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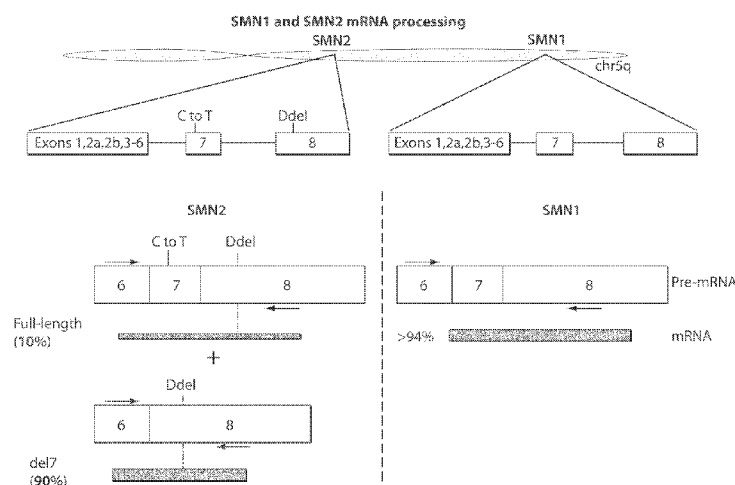


Fig. 1

(57) Abstract: Aspects of the invention provide single stranded oligonucleotides for activating or enhancing expression of SMN1 or SMN2. Further aspects provide compositions and kits comprising single stranded oligonucleotides for activating or enhancing expression of SMN1 or SMN2 that comprises exon 7. Methods for modulating expression of SMN1 or SMN2 using the single stranded oligonucleotides are also provided. Further aspects of the invention provide methods for selecting a candidate oligonucleotide for activating or enhancing expression of SMN1 or SMN2.

COMPOSITIONS AND METHODS FOR MODULATING SMN GENE FAMILY EXPRESSION

CROSS-REFERENCE TO RELATED APPLICATIONS

This application claims the benefit under 35 U.S.C. §119(e) of U.S. Provisional Application No. 61/785,529, entitled “COMPOSITIONS AND METHODS FOR MODULATING SMN GENE FAMILY EXPRESSION”, filed March 14, 2013; U.S. Provisional Application No. 61/719,394, entitled “COMPOSITIONS AND METHODS FOR MODULATING SMN GENE FAMILY EXPRESSION”, filed October 27, 2012; and U.S. Provisional Application No. 61/647,858, entitled “COMPOSITIONS AND METHODS FOR MODULATING SMN GENE FAMILY EXPRESSION”, filed May 16, 2012, the contents of each of which are incorporated herein by reference in their entireties.

FIELD OF THE INVENTION

The invention relates to oligonucleotide based compositions, as well as methods of using oligonucleotide based compositions for treating disease.

BACKGROUND OF THE INVENTION

Spinal muscular atrophy (SMA) is a group of hereditary diseases that causes muscle damage leading to impaired muscle function, difficulty breathing, frequent respiratory infection, and eventually death. There are four types of SMA that are classified based on the onset and severity of the disease. SMA type I is the most severe form and is one of the most common causes of infant mortality, with symptoms of muscle weakness and difficulty breathing occurring at birth. SMA type II occurs later, with muscle weakness and other symptoms developing from ages 6 month to 2 years. Symptoms appear in SMA type III during childhood and in SMA type IV, the mildest form, during adulthood. All four types of SMA have been found to be associated with mutations in the SMN gene family, particularly SMN1.

Survival of motor neuron (SMN) is a protein involved in transcriptional splicing through its involvement in assembly of small nuclear ribonucleoproteins (snRNPs). snRNPs are protein-RNA complexes that bind with pre-mRNA to form a spliceosome, which then splices the pre-mRNA, most often resulting in removal of introns. Three genes encode SMN or a variant of SMN, including SMN1 (survival of motor neuron 1, telomeric), SMN2

(survival of motor neuron 2, centromeric), and SMNP (survival of motor neuron 1, telomeric pseudogene). SMN1 and SMN2 are a result of a gene duplication at 5q13 in humans. A lack of SMN activity results in widespread splicing defects, especially in spinal motor neurons, and degeneration of the spinal cord lower motor neurons.

5

SUMMARY OF THE INVENTION

Aspects of the invention disclosed herein provide methods and compositions that are useful for upregulating the expression of certain genes in cells. In some embodiments, single stranded oligonucleotides are provided that target a PRC2-associated region of a gene and thereby cause upregulation of the gene. Also provided herein are methods and related single stranded oligonucleotides that are useful for selectively inducing expression of particular splice variants of genes. In some embodiments, the methods are useful for controlling the levels in a cell of particular protein isoforms encoded by the splice variants. In some cases, the methods are useful for inducing expression of proteins to levels sufficient to treat disease.

15 In some embodiments, single stranded oligonucleotides are provided that target a PRC2-associated region of a SMN gene (e.g., human SMN1, human SMN2) and thereby cause upregulation of the gene. For example, according to some aspects of the invention methods are provided for increasing expression of full-length SMN protein in a cell for purposes of treating SMA. Accordingly, aspects of the invention disclosed herein provide
20 methods and compositions that are useful for upregulating SMN1 or SMN2 in cells. In some embodiments, single stranded oligonucleotides are provided that target a PRC2-associated region of the gene encoding SMN1 or SMN2. In some embodiments, these single stranded oligonucleotides activate or enhance expression of SMN1 or SMN2 by relieving or preventing PRC2 mediated repression of SMN1 or SMN2.

25 In some embodiments, the methods comprise delivering to the cell a first single stranded oligonucleotide complementary with a PRC2-associated region of an SMN gene, eg., a PRC2-associated region of SMN1 or SMN2, and a second single stranded oligonucleotide complementary with a splice control sequence of a precursor mRNA of an SMN gene, e.g., a precursor mRNA of SMN1 or SMN2, in amounts sufficient to increase
30 expression of a mature mRNA of SMN1 or SMN2 that comprises (or includes) exon 7 in the cell.

According to some aspects of the invention single stranded oligonucleotides are

provided that have a region of complementarity that is complementary with (e.g., at least 8 consecutive nucleotides of) a PRC2-associated region of an SMN gene, e.g., a PRC2-associated region of the nucleotide sequence set forth as SEQ ID NO: 1, 2, 4, or 5. In some embodiments, the oligonucleotide has at least one of the following features: a) a sequence
5 that is 5'-X-Y-Z, in which X is any nucleotide and in which X is at the 5' end of the oligonucleotide, Y is a nucleotide sequence of 6 nucleotides in length that is not a human seed sequence of a microRNA, and Z is a nucleotide sequence of 1 to 23 nucleotides in length; b) a sequence that does not comprise three or more consecutive guanine nucleotides; c) a sequence that has less than a threshold level of sequence identity with every
10 sequence of nucleotides, of equivalent length to the second nucleotide sequence, that are between 50 kilobases upstream of a 5'-end of an off-target gene and 50 kilobases downstream of a 3'-end of the off-target gene; d) a sequence that is complementary to a PRC2-associated region that encodes an RNA that forms a secondary structure comprising at least two single stranded loops; and e) a sequence that has greater than 60% G-C content. In some
15 embodiments, the single stranded oligonucleotide has at least two of features a), b), c), d), and e), each independently selected. In some embodiments, the single stranded oligonucleotide has at least three of features a), b), c), d), and e), each independently selected. In some embodiments, the single stranded oligonucleotide has at least four of features a), b), c), d), and e), each independently selected. In some embodiments, the single stranded
20 oligonucleotide has each of features a), b), c), d), and e). In certain embodiments, the oligonucleotide has the sequence 5'-X-Y-Z, in which the oligonucleotide is 8-50 nucleotides in length.

According to some aspects of the invention, single stranded oligonucleotides are provided that have a sequence X-Y-Z, in which X is any nucleotide, Y is a nucleotide
25 sequence of 6 nucleotides in length that is not a seed sequence of a human microRNA, and Z is a nucleotide sequence of 1 to 23 nucleotides in length, in which the single stranded oligonucleotide is complementary with a PRC2-associated region of an SMN gene, e.g., a PRC2-associated region of the nucleotide sequence set forth as SEQ ID NO: 1, 2, 4, or 5. In some aspects of the invention, single stranded oligonucleotides are provided that have a
30 sequence 5'-X-Y-Z, in which X is any nucleotide, Y is a nucleotide sequence of 6 nucleotides in length that is not a seed sequence of a human microRNA, and Z is a nucleotide sequence of 1 to 23 nucleotides in length, in which the single stranded oligonucleotide is

complementary with at least 8 consecutive nucleotides of a PRC2-associated region of an SMN gene, e.g., a PRC2-associated region of the nucleotide sequence set forth as SEQ ID NO: 1, 2, 4, or 5. In some embodiments, Y is a sequence selected from Table 1. In some embodiments, the PRC2-associated region is a sequence listed in any one of SEQ ID NOS: 9 to 18.

In some embodiments, the single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 30 to 13087, or a fragment thereof that is at least 8 nucleotides. In some embodiments, the single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 30 to 13087, in which the 5' end of the nucleotide sequence provided is the 5' end of the oligonucleotide. In some embodiments, the region of complementarity (e.g., the at least 8 consecutive nucleotides) is also present within the nucleotide sequence set forth as SEQ ID NO: 7 or 8.

In some embodiments, a PRC2-associated region is a sequence listed in any one of SEQ ID NOS: 9 to 14. In some embodiments, the single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 30 to 8329 and 13093 to 13094 or a fragment thereof that is at least 8 nucleotides. In some embodiments, the single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 30 to 8329 and 13093 to 13094, wherein the 5' end of the nucleotide sequence provided is the 5' end of the oligonucleotide. In some embodiments, the at least 8 consecutive nucleotides are also present within the nucleotide sequence set forth as SEQ ID NO: 7.

In some embodiments, a PRC2-associated region is a sequence listed in any one of SEQ ID NOS: 15 to 18. In some embodiments, the single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 1158-1159, 1171, 1482-1483, 1485-1486, 2465-2471, 2488-2490, 2542-2546, 2656-2657, 2833-2835, 3439-3440, 3916-3918, 4469-4472, 4821, 5429, 5537, 6061, 7327, 8330-13061, and 13062-13087 or a fragment thereof that is at least 8 nucleotides. In some embodiments, the at least 8 consecutive nucleotides are present within the nucleotide sequence set forth as SEQ ID NO: 8.

In some embodiments, the single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 30 to 13087. In some embodiments, the

oligonucleotide is up to 50 nucleotides in length. In some embodiments, the single stranded oligonucleotide comprises a fragment of at least 8 nucleotides of a nucleotide sequence as set forth in any one of SEQ ID NOS: 30 to 13087.

In some embodiments, a single stranded oligonucleotide comprises a nucleotide
5 sequence as set forth in Table 4. In some embodiments, the single stranded oligonucleotide comprises a fragment of at least 8 nucleotides of a nucleotide sequence as set forth in Table 4. In some embodiments, a single stranded oligonucleotide consists of a nucleotide sequence as set forth in Table 4.

According to some aspects of the invention, compounds are provided that comprise
10 the general formula A-B-C, wherein A is a single stranded oligonucleotide complementary with at least 8 consecutive nucleotides of a PRC2-associated region of a gene, B is a linker, and C is a single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA of the gene. In some embodiments, B comprises an oligonucleotide, peptide, low pH labile bond, or disulfide bond. In some embodiments, the splice control
15 sequence resides in an exon of the gene. In some embodiments, the splice control sequence traverses an intron-exon junction of the gene. In some embodiments, the splice control sequence resides in an intron of the gene. In some embodiments, the splice control sequence comprises at least one hnRNAP binding sequence. In some embodiments, hybridization of an oligonucleotide having the sequence of C with the splice control sequence of the precursor
20 mRNA in a cell results in inclusion of a particular exon in a mature mRNA that arises from processing of the precursor mRNA in the cell. In some embodiments, hybridization of an oligonucleotide having the sequence of C with the splice control sequence of the precursor mRNA in a cell results in exclusion of a particular intron or exon in a mature mRNA that arises from processing of the precursor mRNA in the cell.

25 In some embodiments, the gene is SMN1 or SMN2. In some embodiments, the splice control sequence resides in intron 6, intron 7, exon 7, exon 8 or at the junction of intron 7 and exon 8 of SMN1 or SMN2. In some embodiments, the splice control sequence comprises the sequence: CAGCAUUAUGAAAG (SEQ ID NO: 13100). In some embodiments, B comprises a sequence selected from: TCACTTTCATAATGCTGG (SEQ ID NO: 13088);
30 TCACTTTCATAATGC (SEQ ID NO: 13089); CACTTTCATAATGCT (SEQ ID NO: 13090); ACTTTCATAATGCTG (SEQ ID NO: 13090); and CTTTCATAATGCTGG (SEQ ID NO: 13092).

In some embodiments, A has a sequence 5'-X-Y-Z, wherein X is any nucleotide, Y is a nucleotide sequence of 6 nucleotides in length that is not a seed sequence of a human microRNA, and Z is a nucleotide sequence of 1-23 nucleotides in length. In some embodiments, the PRC2-associated region of an SMN2 gene is a PRC2-associated region within SEQ ID NO: 1, 2, 4 or 5. In some embodiments, Y is a sequence selected from Table 1. In some embodiments, the PRC2-associated region is a sequence set forth in any one of SEQ ID NOS: 9 to 23. In some embodiments, A comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 30 to 8329 and 13088 to 13094 or a fragment thereof that is at least 8 nucleotides. In some embodiments, A comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 30 to 8329 and 13088 to 13094, wherein the 5' end of the nucleotide sequence provided is the 5' end of A. In some embodiments, the at least 8 consecutive nucleotides are also present within the nucleotide sequence set forth as SEQ ID NO: 7. In some embodiments, the PRC2-associated region is a sequence set forth in any one of SEQ ID NOS: 24 to 29.

In some embodiments, A comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 1158-1159, 1171, 1482-1483, 1485-1486, 2465-2471, 2488-2490, 2542-2546, 2656-2657, 2833-2835, 3439-3440, 3916-3918, 4469-4472, 4821, 5429, 5537, 6061, 7327, 8330-13061, and 13062-13087 or a fragment thereof that is at least 8 nucleotides. In some embodiments, the at least 8 consecutive nucleotides are present within the nucleotide sequence set forth as SEQ ID NO: 8. In some embodiments, A does not comprise three or more consecutive guanosine nucleotides. In some embodiments, A does not comprise four or more consecutive guanosine nucleotides. In some embodiments, A or C is 8 to 30 nucleotides in length. In some embodiments, A is 8 to 10 nucleotides in length and all but 1, 2, or 3 of the nucleotides of the complementary sequence of the PRC2-associated region are cytosine or guanosine nucleotides. In some embodiments, B is an oligonucleotide comprising 1 to 10 thymidines or uridines. In some embodiments, B is more susceptible to cleavage in a mammalian extract than A and C.

In some embodiments, A comprises a nucleotide sequence selected from GCTUTGGGAAGUAUG (SEQ ID NO: 11394), CUTUGGGAAGTATG (SEQ ID NO: 11395) and GGTACATGAGTGGCT (SEQ ID NO: 11419); B comprises the nucleotide sequence TTTT or UUUU; and C comprises the nucleotide sequence TCACTTTCATAATGCTGG (SEQ ID NO: 13088); TCACTTTCATAATGC (SEQ ID NO:

13089); CACTTTCATAATGCT (SEQ ID NO: 13090); ACTTTCATAATGCTG (SEQ ID NO: 13091); or CTTTCATAATGCTGG (SEQ ID NO: 13092), and wherein the 3' end of A is linked to the 5' end of B, and the 3' end of B is linked to 5' end of C.

In some embodiments, the single stranded oligonucleotide does not comprise three or more consecutive guanosine nucleotides. In some embodiments, the single stranded oligonucleotide does not comprise four or more consecutive guanosine nucleotides.

In some embodiments, the single stranded oligonucleotide is 8 to 30 nucleotides in length. In some embodiments, the single stranded oligonucleotide is up to 50 nucleotides in length. In some embodiments, the single stranded oligonucleotide is 8 to 10 nucleotides in length and all but 1, 2, or 3 of the nucleotides of the complementary sequence of the PRC2-associated region are cytosine or guanosine nucleotides.

In some embodiments, the single stranded oligonucleotide is complementary with at least 8 consecutive nucleotides of a PRC2-associated region of an SMN gene, e.g., a PRC2-associated region of a nucleotide sequence set forth as SEQ ID NO: 1, 2, 4, or 5, in which the nucleotide sequence of the single stranded oligonucleotide comprises one or more of a nucleotide sequence selected from the group consisting of

- (a) (X)Xxxxxx, (X)xXxxxx, (X)xxXxxx, (X)xxxXxx, (X)xxxxXx and (X)xxxxxX,
- (b) (X)XXxxxx, (X)XxXxxx, (X)XxxXxx, (X)XxxxXx, (X)XxxxxX, (X)xXXxxx, (X)xXxXxx, (X)xXxxXx, (X)xXxxxX, (X)xxXXxx, (X)xxXxXx, (X)xxXxxX, (X)xxxXXx, (X)xxxXxX and (X)xxxxXX,
- (c) (X)XXXxxx, (X)xXXXxx, (X)xxXXXx, (X)xxxXXX, (X)XXxXxx, (X)XXxxXx, (X)XXxxxX, (X)xXXxXx, (X)xXXxxX, (X)xxXXxX, (X)XxXXxx, (X)XxxXXx, (X)XxxxXX, (X)xXxXXx, (X)xXxxXX, (X)xxXxXX, (X)xXxXxX and (X)XxXxXx,
- (d) (X)xxXXX, (X)xXxXXX, (X)xXXxXX, (X)xXXXxX, (X)xXXXXx, (X)XxxXXXX, (X)XxXxXX, (X)XxXXxX, (X)XxXXx, (X)XXxxXX, (X)XXxXxX, (X)XXxXXx, (X)XXXxxX, (X)XXXxXx, and (X)XXXXxx,
- (e) (X)xXXXXX, (X)XxXXXX, (X)XXxXXX, (X)XXXxXX, (X)XXXXxX and (X)XXXXXx, and
- (f) XXXXXX, XxXXXXX, XXxXXXX, XXXxXXX, XXXXxXX, XXXXXxX and XXXXXXx, wherein "X" denotes a nucleotide analogue, (X) denotes an optional nucleotide analogue, and "x" denotes a DNA or RNA nucleotide unit.

In some embodiments, at least one nucleotide of the oligonucleotide is a nucleotide

analogue. In some embodiments, the at least one nucleotide analogue results in an increase in T_m of the oligonucleotide in a range of 1 to 5 °C compared with an oligonucleotide that does not have the at least one nucleotide analogue.

In some embodiments, at least one nucleotide of the oligonucleotide comprises a 2' O-methyl. In some embodiments, each nucleotide of the oligonucleotide comprises a 2' O-methyl. In some embodiments, the oligonucleotide comprises at least one ribonucleotide, at least one deoxyribonucleotide, or at least one bridged nucleotide. In some embodiments, the bridged nucleotide is a LNA nucleotide, a cEt nucleotide or a ENA modified nucleotide. In some embodiments, each nucleotide of the oligonucleotide is a LNA nucleotide.

In some embodiments, the nucleotides of the oligonucleotide comprise alternating deoxyribonucleotides and 2'-fluoro-deoxyribonucleotides. In some embodiments, the nucleotides of the oligonucleotide comprise alternating deoxyribonucleotides and 2'-O-methyl nucleotides. In some embodiments, the nucleotides of the oligonucleotide comprise alternating deoxyribonucleotides and ENA nucleotide analogues. In some embodiments, the nucleotides of the oligonucleotide comprise alternating deoxyribonucleotides and LNA nucleotides. In some embodiments, the 5' nucleotide of the oligonucleotide is a deoxyribonucleotide. In some embodiments, the nucleotides of the oligonucleotide comprise alternating LNA nucleotides and 2'-O-methyl nucleotides. In some embodiments, the 5' nucleotide of the oligonucleotide is a LNA nucleotide. In some embodiments, the nucleotides of the oligonucleotide comprise deoxyribonucleotides flanked by at least one LNA nucleotide on each of the 5' and 3' ends of the deoxyribonucleotides.

In some embodiments, the single stranded oligonucleotide comprises modified internucleotide linkages (e.g., phosphorothioate internucleotide linkages or other linkages) between at least two, at least three, at least four, at least five or more nucleotides. In some embodiments, the single stranded oligonucleotide comprises modified internucleotide linkages (e.g., phosphorothioate internucleotide linkages or other linkages) between all nucleotides.

In some embodiments, the nucleotide at the 3' position of the oligonucleotide has a 3' hydroxyl group. In some embodiments, the nucleotide at the 3' position of the oligonucleotide has a 3' thiophosphate. In some embodiments, the single stranded oligonucleotide has a biotin moiety or other moiety conjugated to its 5' or 3' nucleotide. In some embodiments, the single stranded oligonucleotide has cholesterol, Vitamin A, folate,

sigma receptor ligands, aptamers, peptides, such as CPP, hydrophobic molecules, such as lipids, ASGPR or dynamic polyconjugates and variants thereof at its 5' or 3' end.

According to some aspects of the invention compositions are provided that comprise any of the oligonucleotides disclosed herein, and a carrier. In some embodiments,

5 compositions are provided that comprise any of the oligonucleotides in a buffered solution. In some embodiments, the oligonucleotide is conjugated to the carrier. In some embodiments, the carrier is a peptide. In some embodiments, the carrier is a steroid. According to some aspects of the invention pharmaceutical compositions are provided that comprise any of the oligonucleotides disclosed herein, and a pharmaceutically acceptable carrier.

10 According to other aspects of the invention, kits are provided that comprise a container housing any of the compositions disclosed herein.

According to some aspects of the invention, methods of increasing expression of SMN1 or SMN2 in a cell are provided. In some embodiments, the methods involve delivering any one or more of the single stranded oligonucleotides disclosed herein into the
15 cell. In some embodiments, delivery of the single stranded oligonucleotide into the cell results in a level of expression of SMN1 or SMN2 that is greater (e.g., at least 50% greater) than a level of expression of SMN1 or SMN2 in a control cell that does not comprise the single stranded oligonucleotide.

According to some aspects of the invention, methods of increasing levels of SMN1 or
20 SMN2 in a subject are provided. According to some aspects of the invention, methods of treating a condition (e.g., Spinal muscular atrophy) associated with decreased levels of SMN1 or SMN2 in a subject are provided. In some embodiments, the methods involve administering any one or more of the single stranded oligonucleotides disclosed herein to the subject.

25 Aspects of the invention relate to methods of increasing expression of SMN protein in a cell. In some embodiments, the method comprise delivering to the cell a first single stranded oligonucleotide complementary with at least 8 consecutive nucleotides of a PRC2-associated region of SMN2 and a second single stranded oligonucleotide complementary with a splice control sequence of a precursor mRNA of SMN2, in amounts sufficient to increase
30 expression of a mature mRNA of SMN2 that comprises exon 7 in the cell. In some embodiments, the region of complementarity with at least 8 consecutive nucleotides of a PRC2-associated region of SMN 2 has at least 1, at least 2, at least 3, at least 4, at least 5, at

least 6, at least 7, at least 8, or more mismatches with a corresponding region of SMN1. As used herein the term, “splice control sequence” refers to a nucleotide sequence that when present in a precursor mRNA influences splicing of that precursor mRNA in a cell. In some embodiments, a splice control sequence includes one or more binding sites for a molecule
5 that regulates mRNA splicing, such as a hnRNAP protein. In some embodiments, a splice control sequence comprises the sequence CAG or AAAG. In some embodiments, a splice control sequence resides in an exon (e.g., an exon of SMN1 or SMN2, such as exon 7 or exon 8). In some embodiments, a splice control sequence traverses an intron-exon junction (e.g., an intron-exon junction of SMN1 or SMN2, such as the intron 6/exon 7 junction or the intron
10 7/exon 8 junction). In some embodiments, a splice control sequence resides in an intron (e.g., an intron of SMN1 or SMN2, such as intron 6 or intron 7). In some embodiments, a splice control sequence comprises the sequence: CAGCAUUAUGAAAG (SEQ ID NO: 13100) or a portion thereof.

In some embodiments, the second single stranded oligonucleotide is splice switching
15 oligonucleotide that comprises a sequence selected from: TCACTTTCATAATGCTGG (SEQ ID NO: 13088); TCACTTTCATAATGC (SEQ ID NO: 13089); CACTTTCATAATGCT (SEQ ID NO: 13090); ACTTTCATAATGCTG (SEQ ID NO: 13091); and CTTTCATAATGCTGG (SEQ ID NO: 13092). In some embodiments, the second single stranded oligonucleotide is 8 to 30 nucleotides in length.

20 In some embodiments, the first single stranded oligonucleotide has a sequence 5'-X-Y-Z, wherein X is any nucleotide, Y is a nucleotide sequence of 6 nucleotides in length that is not a seed sequence of a human microRNA, and Z is a nucleotide sequence of 1-23 nucleotides in length. In some embodiments, the PRC2-associated region of an SMN2 gene is a PRC2-associated region within SEQ ID NO: 1, 2, 4 or 5. In some embodiments, Y is a
25 sequence selected from Table 1. In some embodiments, the PRC2-associated region is a sequence set forth in any one of SEQ ID NOS: 9 to 23. In some embodiments, the first single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 30 to 8329 and 13088 to 13094 or a fragment thereof that is at least 8 nucleotides. In some embodiments, the first single stranded oligonucleotide comprises a nucleotide sequence
30 as set forth in any one of SEQ ID NOS: 30 to 8329 and 13088 to 13094, wherein the 5' end of the nucleotide sequence provided is the 5' end of the first single stranded oligonucleotide.

In some embodiments, the at least 8 consecutive nucleotides are also present within the nucleotide sequence set forth as SEQ ID NO: 7.

In some embodiments, the PRC2-associated region is a sequence set forth in any one of SEQ ID NOS: 24 to 29. In some embodiments, the first single stranded oligonucleotide
5 comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 1158-1159, 1171, 1482-1483, 1485-1486, 2465-2471, 2488-2490, 2542-2546, 2656-2657, 2833-2835, 3439-3440, 3916-3918, 4469-4472, 4821, 5429, 5537, 6061, 7327, 8330-13061, and 13062-13087 or a fragment thereof that is at least 8 nucleotides. In some embodiments, the at least 8 consecutive nucleotides are present within the nucleotide sequence set forth as SEQ ID NO:

10 8. In some embodiments, the first single stranded oligonucleotide does not comprise three or more consecutive guanosine nucleotides. In some embodiments, the first single stranded oligonucleotide does not comprise four or more consecutive guanosine nucleotides. In some embodiments, the first single stranded oligonucleotide is 8 to 30 nucleotides in length. In some embodiments, the first single stranded oligonucleotide is 8 to 10 nucleotides in length
15 and all but 1, 2, or 3 of the nucleotides of the complementary sequence of the PRC2-associated region are cytosine or guanosine nucleotides.

In some embodiments, the first single stranded oligonucleotide and the second single stranded oligonucleotide are delivered to the cell simultaneously. In some embodiments, the cell is in a subject and the step of delivering to the cell comprises administering the first
20 single stranded oligonucleotide and the second single stranded oligonucleotide to the subject as a co-formulation. In some embodiments, the first single stranded oligonucleotide is covalently linked to the second single stranded oligonucleotide through a linker. In some embodiments, the linker comprises an oligonucleotide, a peptide, a low pH-labile bond, or a disulfide bond. In some embodiments, the linker comprises an oligonucleotide, optionally
25 wherein the oligonucleotide comprises 1 to 10 thymidines or uridines. In some embodiments, the linker is more susceptible to cleavage in a mammalian extract than the first and second single stranded oligonucleotides. In some embodiments, the first single stranded oligonucleotide is not covalently linked to the second single stranded oligonucleotide. In some embodiments, the first single stranded oligonucleotide and the second single stranded
30 oligonucleotide are delivered to the cell separately.

According to some aspects of the invention, methods are provided for treating spinal muscular atrophy in a subject. The methods, in some embodiments, comprise administering

to the subject a first single stranded oligonucleotide complementary with at least 8 consecutive nucleotides of a PRC2-associated region of SMN2 and a second single stranded oligonucleotide complementary with a splice control sequence of a precursor mRNA of SMN2, in amounts sufficient to increase expression of SMN protein in the subject.

5 According to some aspects of the invention methods are provided for treating spinal muscular atrophy in a subject that involve administering to the subject a first single stranded oligonucleotide complementary with a PRC2-associated region of SMN2 and a second single stranded oligonucleotide complementary with a splice control sequence of a precursor mRNA of SMN2, in amounts sufficient to increase expression of SMN protein in the subject.

10 Related compositions are also provided. In some embodiments, compositions are provided that comprise a first single stranded oligonucleotide complementary with at least 8 consecutive nucleotides of a PRC2-associated region of SMN2, and a second single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA of SMN2. In some embodiments, compositions are provided that comprise a single stranded
15 oligonucleotide complementary with at least 8 consecutive nucleotides of a PRC2-associated region of a gene, linked via a linker to a single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA of the gene. Related kits comprising single stranded oligonucleotides that regulate SMN1 or SMN2 expression are also provided.

 According to some aspects of the invention compositions are provided that comprise
20 any of the oligonucleotides or compounds disclosed herein, and a carrier. In some embodiments, compositions are provided that comprise any of the oligonucleotides or compounds in a buffered solution. In some embodiments, the oligonucleotide is conjugated to the carrier. In some embodiments, the carrier is a peptide. In some embodiments, the carrier is a steroid. According to some aspects of the invention pharmaceutical compositions are
25 provided that comprise any of the oligonucleotides disclosed herein, and a pharmaceutically acceptable carrier.

 According to some aspects of the invention, compositions are provided that comprise a first single stranded oligonucleotide complementary with at least 8 consecutive nucleotides of a PRC2-associated region of SMN2, and a second single stranded oligonucleotide
30 complementary to a splice control sequence of a precursor mRNA of SMN2. In some embodiments, the splice control sequence resides in an exon of SMN2. In some embodiments, the exon is exon 7 or exon 8. In some embodiments, the splice control

sequence traverses an intron-exon junction of SMN2. In some embodiments, the intron-exon junction is the intron 6/exon 7 junction or the intron 7/exon 8 junction. In some embodiments, the splice control sequence resides in an intron of SMN2. In some embodiments, the intron is intron 6 or intron 7 (SEQ ID NO: 13101). In some embodiments, the splice control sequence comprises the sequence: CAGCAUUAUGAAAG (SEQ ID NO: 13100) or a portion thereof. In some embodiments, the splice control sequence comprises at least one hnRNAP binding sequence. In some embodiments, the second single stranded oligonucleotide comprises a sequence selected from: TCACTTTCATAATGCTGG (SEQ ID NO: 13088); TCACTTTCATAATGC (SEQ ID NO: 13089); CACTTTCATAATGCT (SEQ ID NO: 13090); ACTTTCATAATGCTG (SEQ ID NO: 13091); and CTTTCATAATGCTGG (SEQ ID NO: 13092). In some embodiments, the first single stranded oligonucleotide has a sequence 5'-X-Y-Z, wherein X is any nucleotide, Y is a nucleotide sequence of 6 nucleotides in length that is not a seed sequence of a human microRNA, and Z is a nucleotide sequence of 1-23 nucleotides in length. In some embodiments, the PRC2-associated region of SMN2 is a PRC2-associated region within SEQ ID NO: 1, 2, 4 or 5. In some embodiments, Y is a sequence selected from Table 1. In some embodiments, the PRC2-associated region is a sequence set forth in any one of SEQ ID NOS: 9 to 23. In some embodiments, the first single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 30 to 8329 and 13093 to 13094 or a fragment thereof that is at least 8 nucleotides. In some embodiments, the first single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 30 to 8329 and 13093 to 13094, wherein the 5' end of the nucleotide sequence provided is the 5' end of the first single stranded oligonucleotide. In some embodiments, the at least 8 consecutive nucleotides are also present within the nucleotide sequence set forth as SEQ ID NO: 7. In some embodiments, the PRC2-associated region is a sequence set forth in any one of SEQ ID NOS: 24 to 29. In some embodiments, the first single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 1158-1159, 1171, 1482-1483, 1485-1486, 2465-2471, 2488-2490, 2542-2546, 2656-2657, 2833-2835, 3439-3440, 3916-3918, 4469-4472, 4821, 5429, 5537, 6061, 7327, 8330-13061, and 13062-13087 or a fragment thereof that is at least 8 nucleotides. In some embodiments, the at least 8 consecutive nucleotides are present within the nucleotide sequence set forth as SEQ ID NO: 8. In some embodiments, the first single stranded oligonucleotide does not

comprise three or more consecutive guanosine nucleotides. In some embodiments, the first single stranded oligonucleotide does not comprise four or more consecutive guanosine nucleotides. In some embodiments, the first and/or second single stranded oligonucleotide is 8 to 30 nucleotides in length. In some embodiments, the first single stranded oligonucleotide is 8 to 10 nucleotides in length and all but 1, 2, or 3 of the nucleotides of the complementary sequence of the PRC2-associated region are cytosine or guanosine nucleotides. In some embodiments, the first single stranded oligonucleotide is covalently linked to the second single stranded oligonucleotide through a linker. In some embodiments, the linker comprises an oligonucleotide, a peptide, a low pH-labile bond, or a disulfide bond. In some embodiments, the linker comprises an oligonucleotide, optionally wherein the oligonucleotide comprises 1 to 10 thymidines or uridines. In some embodiments, the linker is more susceptible to cleavage in a mammalian extract than the first and second single stranded oligonucleotides. In some embodiments, the first single stranded oligonucleotide is not covalently linked to the second single stranded oligonucleotide. In some embodiments, the composition further comprises a carrier. In some embodiments, the carrier is a pharmaceutically acceptable carrier.

Further aspects of the invention provide methods for selecting oligonucleotides for activating or enhancing expression of SMN1 or SMN2. In some embodiments, methods are provided for selecting a set of oligonucleotides that is enriched in candidates (e.g., compared with a random selection of oligonucleotides) for activating or enhancing expression of SMN1 or SMN2. Accordingly, the methods may be used to establish sets of clinical candidates that are enriched in oligonucleotides that activate or enhance expression of SMN1 or SMN2. Such libraries may be utilized, for example, to identify lead oligonucleotides for developing therapeutics to treat SMN1 or SMN2. Furthermore, in some embodiments, oligonucleotide chemistries are provided that are useful for controlling the pharmacokinetics, biodistribution, bioavailability and/or efficacy of the single stranded oligonucleotides for activating expression of SMN1 or SMN2.

According to other aspects of the invention, kits are provided that comprise a container housing any of the compositions disclosed herein. According to other aspects of the invention, kits are provided that comprise a first container housing first single stranded oligonucleotide complementary with at least 8 consecutive nucleotides of a PRC2-associated region of a gene; and a second container housing a second single stranded oligonucleotide

complementary to a splice control sequence of a precursor mRNA of the gene. In some embodiments, the splice control sequence resides in an exon of the gene. In some embodiments, the splice control sequence traverses an intron-exon junction of the gene. In some embodiments, the splice control sequence resides in an intron of the gene. In some
 5 embodiments, the splice control sequence comprises at least one hnRNAP binding sequence. In some embodiments, hybridization of an oligonucleotide having the sequence of C with the splice control sequence of the precursor mRNA in a cell results in inclusion of a particular exon in a mature mRNA that arises from processing of the precursor mRNA in the cell. In some embodiments, hybridization of an oligonucleotide having the sequence of C with the
 10 splice control sequence of the precursor mRNA in a cell results in exclusion of a particular intron or exon in a mature mRNA that arises from processing of the precursor mRNA in the cell. In some embodiments, the gene is SMN1 or SMN2. In some embodiments, the splice control sequence resides in intron 6, intron 7, exon 7, exon 8 or at the junction of intron 7 and exon 8. In some embodiments, the splice control sequence comprises the sequence:

15 CAGCAUUAUGAAAG (SEQ ID NO: 13100). In some embodiments, the second single stranded oligonucleotide comprises a sequence selected from: TCACTTTCATAATGCTGG (SEQ ID NO: 13088); TCACTTTCATAATGC (SEQ ID NO: 13089); CACTTTCATAATGCT (SEQ ID NO: 13090); ACTTTCATAATGCTG (SEQ ID NO: XX); and CTTTCATAATGCTGG (SEQ ID NO: 13091). In some embodiments, the first single
 20 stranded oligonucleotide has a sequence 5'-X-Y-Z, wherein X is any nucleotide, Y is a nucleotide sequence of 6 nucleotides in length that is not a seed sequence of a human microRNA, and Z is a nucleotide sequence of 1-23 nucleotides in length. In some embodiments, the PRC2-associated region of an SMN2 gene is a PRC2-associated region within SEQ ID NO: 1, 2, 4 or 5. In some embodiments, Y is a sequence selected from Table
 25 1. In some embodiments, the PRC2-associated region is a sequence set forth in any one of SEQ ID NOS: 9 to 23. In some embodiments, the first single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 30 to 8329 and 13093 to 13094 or a fragment thereof that is at least 8 nucleotides. In some embodiments, the first single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one
 30 of SEQ ID NOS: 30 to 8329 and 13093 to 13094, wherein the 5' end of the nucleotide sequence provided is the 5' end of the first single stranded oligonucleotide. In some embodiments, the at least 8 consecutive nucleotides are also present within the nucleotide

sequence set forth as SEQ ID NO: 7. In some embodiments, the PRC2-associated region is a sequence set forth in any one of SEQ ID NOS: 24 to 29. In some embodiments, the first single stranded oligonucleotide comprises a nucleotide sequence as set forth in any one of SEQ ID NOS: 1158-1159, 1171, 1482-1483, 1485-1486, 2465-2471, 2488-2490, 2542-2546, 2656-2657, 2833-2835, 3439-3440, 3916-3918, 4469-4472, 4821, 5429, 5537, 6061, 7327, 8330-13061, and 13062-13087 or a fragment thereof that is at least 8 nucleotides. In some embodiments, the at least 8 consecutive nucleotides are present within the nucleotide sequence set forth as SEQ ID NO: 8.

BRIEF DESCRIPTION OF DRAWINGS

FIG. 1 provides a schematic of SMN1 and SMN2 mRNA processing

FIG. 2 provides a table outlining genotypes and patent information, including SMA classification, of cell lines tested in Example 2. Baseline SMN protein levels in the cell lines are also depicted.

FIG. 3 depicts results of RT-PCR assays showing effects on SMN mRNA expression of oligonucleotides directed against a PRC2-associated region of SMN2 (oligos 1-52 and 59-101) and splice switching oligonucleotides (oligos 53-58) (PCR primers directed against exon 1 of SMN1/2.) in cell line 3814.

FIG. 4 depicts results of RT-PCR assays showing effects on SMN mRNA expression of oligonucleotides directed against a PRC2-associated region of SMN2 (oligos 1-52 and 59-101) and splice switching oligonucleotides (oligos 53-58) (PCR primers directed against exon 1 of SMN1/2.) in cell line 3813.

FIG. 5 shows that splice switching oligonucleotides (oligos 53-58) increase expression of full length SMN2. Results are based on a gel separation analysis of PCR products obtained following a DdeI restriction digest. Two cell lines were tested, 3813 and 9677. Oligo 84, which targets a PRC2-associated region of SMN2, did not exhibit an increase in full length SMN2 expression when delivered alone to cells.

FIG. 6 provides results of an SMN ELISA (Enzo) showing that certain oligonucleotides directed against a PRC2-associated region of SMN2 alone do not significantly increase SMN2 protein 24h post-transfection in certain SMA patient fibroblasts (compared to Lipofectamine treated cells – dashed line).

FIG. 7 provides results of an SMN ELISA showing that oligonucleotides directed against a PRC2-associated region of SMN2 in combination with a splice switching oligonucleotide (oligo 53) significantly increase SMN2 protein 24h post-transfection in SMA patient fibroblasts (compared to Lipofectamine treated cells – dashed line).

FIG. 8 provides results of an SMN ELISA showing that oligonucleotides directed against a PRC2-associated region of SMN2 in combination with a splice switching oligonucleotide (oligo 54) significantly increase SMN2 protein 24h post-transfection in SMA patient fibroblasts (compared to Lipofectamine treated cells – dashed line).

FIG. 9 provides results of an RT-PCR assay showing that oligonucleotides directed against a PRC2-associated region of SMN2 in combination with a splice switching oligonucleotide (oligo 53 or 54) significantly increase SMN2 protein 24h post-transfection in SMA patient fibroblasts (compared to negative control oligo and Lipofectamine treated cells). LNA/2'OMe alternating oligonucleotide (LM design) and DNA/LNA alternating oligonucleotides (DL design) were tested.

BRIEF DESCRIPTION OF TABLES

Table 1: Hexamers that are not seed sequences of human miRNAs

Table 2: Oligonucleotide sequences made for testing in the lab. RQ (column 2) and RQ SE (column 3) shows the activity of the oligo relative to a control well (usually carrier alone) and the standard error or the triplicate replicates of the experiment. [oligo] is shown in nanomolar for in vitro experiments and in milligrams per kilogram of body weight for in vivo experiments.

Table 3: A listing of oligonucleotide modifications

Table 4: Oligonucleotide sequences made for testing human cells obtained from subjects with Spinal Muscular Atrophy. The table shows the sequence of the modified nucleotides, where lnaX represents an LNA nucleotide with 3' phosphorothioate linkage, omeX is a 2'-O-methyl nucleotide, dX is a deoxy nucleotide. An s at the end of a nucleotide code indicates that the nucleotide had a 3' phosphorothioate linkage. The "-Sup" at the end of the sequence marks the fact that the 3' end lacks either a phosphate or thiophosphate on the 3' linkage. The Formatted Sequence column shows the sequence of the oligonucleotide, including modified nucleotides, for the oligonucleotides tested in Table 2, 5, 6 and 7.

Table 8: Cell lines

BRIEF DESCRIPTION OF THE APPENDICES

- **Appendix A**; Appendix A contains Table 5, which shows RT-PCR data from testing of different oligonucleotides.
- **Appendix B**; Appendix B contains Table 6, which shows RT-PCR data from testing of different combination treatments (*e.g.*, two oligonucleotides, an oligonucleotide and a drug).
- **Appendix C**; Appendix C contains Table 7, which shows ELISA data from testing of different oligonucleotides

Note the following column information for Tables 5-7 in Appendices A-C, respectively. SEQID: sequence identifier of base sequence of oligonucleotide used; Oligo Name: name of oligonucleotide; Avg RQ: average relative quantification of RT-PCR based expression levels of target gene(s); Avg RQ SE: standard error of mean of relative quantification of RT-PCR based expression level; “%SMN over lipo only control” refers to the ratio of SMN protein levels (ng/mg total protein) when compared to Lipofectamine2000 (transfection reagent) treated cells converted into %; “%SMN CVV” refers to coefficient of variation; Exp #: Experiment reference number; Target: target gene; [oligo]: concentration of oligonucleotide used in nM unless otherwise indicated; Cell Line: cell line used; Assay Type: assay used; Time(hr): time of assay following treatment; 2nd Drug: name of second oligonucleotide (identified by Oligo Name) or drug used in combination experiment; [2nd]: concentration of second oligonucleotide or drug; Units: units of concentration; 3rd Drug: name of third oligonucleotide (identified by Oligo Name) or drug used in combination experiment; [3rd]: concentration of third oligonucleotide or drug; Notes: comments regarding experiment. Oligo Names correspond to those in Tables 2 and 4.

DETAILED DESCRIPTION OF CERTAIN EMBODIMENTS OF THE INVENTION

Spinal muscular atrophy (SMA), the most common genetic cause of infant mortality, is an autosomal recessive neuromuscular disease characterized by progressive loss of α -motor neurons in the anterior horns of the spinal cord, leading to limb and trunk paralysis and atrophy of voluntary muscles. Based on the severity and age of onset, SMA is clinically subdivided into types I, II, and III (MIMs 253300, 253550, and 253400), with type I generally understood as being the most severe.

Loss of function of the SMN1 gene is responsible for SMA. Humans have an extra SMN gene copy, called SMN2. Both SMN genes reside within a segmental duplication on Chromosome 5q13 as inverted repeats. SMN1 and SMN2 are almost identical. In some cases, SMN1 and SMN2 differ by 11 nucleotide substitutions, including seven in intron 6, two in intron 7, one in coding exon 7, and one in non-coding exon 8. The substitution in exon 7 involves a translationally silent C to T transition compared with SMN1, that results in alternative splicing because the substitution disrupts recognition of the upstream 3' splice site, in which exon 7 is frequently skipped during precursor mRNA splicing. Consequently, SMN2 encodes primarily the exon 7-skipped protein isoform (SMN Δ 7), which is unstable, mislocalized, and only partially functional.

Methods and related single stranded oligonucleotides that are useful for selectively inducing expression of particular splice variants of SMN1 or SMN2 are provided herein. The methods are useful for controlling the levels in a cell of particular SMN protein isoforms encoded by the splice variants. In some cases, the methods are useful for inducing expression of SMN proteins to levels sufficient to treat SMA. For example, according to some aspects of the invention methods are provided for increasing expression of full-length SMN protein in a cell for purposes of treating SMA. In some embodiments, the methods comprise delivering to the cell a first single stranded oligonucleotide complementary with a PRC2-associated region of SMN1 or SMN2 and a second single stranded oligonucleotide complementary with a splice control sequence of a precursor mRNA of SMN1 or SMN2, in amounts sufficient to increase expression of a mature mRNA of SMN1 or SMN2 that comprises (or includes) exon 7 in the cell. Further aspects of the invention are described in detailed herein.

Polycomb repressive complex 2 (PRC2)-interacting RNAs

Aspects of the invention provided herein relate to the discovery of polycomb repressive complex 2 (PRC2)-interacting RNAs. Polycomb repressive complex 2 (PRC2) is a histone methyltransferase and a known epigenetic regulator involved in silencing of genomic regions through methylation of histone H3. Among other functions, PRC2 interacts with long noncoding RNAs (lncRNAs), such as RepA, Xist, and Tsix, to catalyze trimethylation of histone H3-lysine27. PRC2 contains four subunits, Eed, Suz12, RbAp48, and Ezh2. Aspects of the invention relate to the recognition that single stranded

oligonucleotides that bind to PRC2-associated regions of RNAs (*e.g.*, lncRNAs) that are expressed from within a genomic region that encompasses or that is in functional proximity to the SMN1 or SMN2 gene can induce or enhance expression of SMN1 or SMN2. In some embodiments, this upregulation is believed to result from inhibition of PRC2 mediated repression of SMN1 or SMN2.

As used herein, the term “PRC2-associated region” refers to a region of a nucleic acid that comprises or encodes a sequence of nucleotides that interact directly or indirectly with a component of PRC2. A PRC2-associated region may be present in a RNA (*e.g.*, a long non-coding RNA (lncRNA)) that interacts with a PRC2. A PRC2-associated region may be present in a DNA that encodes an RNA that interacts with PRC2. In some cases, the PRC2-associated region is equivalently referred to as a PRC2-interacting region.

In some embodiments, a PRC2-associated region is a region of an RNA that crosslinks to a component of PRC2 in response to *in situ* ultraviolet irradiation of a cell that expresses the RNA, or a region of genomic DNA that encodes that RNA region. In some embodiments, a PRC2-associated region is a region of an RNA that immunoprecipitates with an antibody that targets a component of PRC2, or a region of genomic DNA that encodes that RNA region. In some embodiments, a PRC2-associated region is a region of an RNA that immunoprecipitates with an antibody that binds specifically to SUZ12, EED, EZH2 or RBBP4 (which as noted above are components of PRC2), or a region of genomic DNA that encodes that RNA region.

In some embodiments, a PRC2-associated region is a region of an RNA that is protected from nucleases (*e.g.*, RNases) in an RNA-immunoprecipitation assay that employs an antibody that targets a component of PRC2, or a region of genomic DNA that encodes that protected RNA region. In some embodiments, a PRC2-associated region is a region of an RNA that is protected from nucleases (*e.g.*, RNases) in an RNA-immunoprecipitation assay that employs an antibody that targets SUZ12, EED, EZH2 or RBBP4, or a region of genomic DNA that encodes that protected RNA region.

In some embodiments, a PRC2-associated region is a region of an RNA within which occur a relatively high frequency of sequence reads in a sequencing reaction of products of an RNA-immunoprecipitation assay that employs an antibody that targets a component of PRC2, or a region of genomic DNA that encodes that RNA region. In some embodiments, a PRC2-associated region is a region of an RNA within which occur a relatively high frequency of

sequence reads in a sequencing reaction of products of an RNA-immunoprecipitation assay that employs an antibody that binds specifically to SUZ12, EED, EZH2 or RBBP4, or a region of genomic DNA that encodes that protected RNA region. In such embodiments, the PRC2-associated region may be referred to as a “peak.”

5 In some embodiments, a PRC2-associated region comprises a sequence of 40 to 60 nucleotides that interact with PRC2 complex. In some embodiments, a PRC2-associated region comprises a sequence of 40 to 60 nucleotides that encode an RNA that interacts with PRC2. In some embodiments, a PRC2-associated region comprises a sequence of up to 5kb in length that comprises a sequence (*e.g.*, of 40 to 60 nucleotides) that interacts with
10 PRC2. In some embodiments, a PRC2-associated region comprises a sequence of up to 5kb in length within which an RNA is encoded that has a sequence (*e.g.*, of 40 to 60 nucleotides) that is known to interact with PRC2. In some embodiments, a PRC2-associated region comprises a sequence of about 4kb in length that comprise a sequence (*e.g.*, of 40 to 60 nucleotides) that interacts with PRC2. In some embodiments, a PRC2-associated region
15 comprises a sequence of about 4kb in length within which an RNA is encoded that includes a sequence (*e.g.*, of 40 to 60 nucleotides) that is known to interact with PRC2. In some embodiments, a PRC2-associated region has a sequence as set forth in any one of SEQ ID NOS: 9 to 29. In some embodiments, a PRC2-associated region has a sequence as set forth in any one of SEQ ID NOS: 24 to 29.

20 In some embodiments, single stranded oligonucleotides are provided that specifically bind to, or are complementary to, a PRC2-associated region in a genomic region that encompasses or that is in proximity to the SMN1 or SMN2 gene. In some embodiments, single stranded oligonucleotides are provided that specifically bind to, or are complementary to, a PRC2-associated region that has a sequence as set forth in any one of SEQ ID NOS: 9 to
25 29. In some embodiments, single stranded oligonucleotides are provided that specifically bind to, or are complementary to, a PRC2-associated region that has a sequence as set forth in any one of SEQ ID NOS: 9 to 29 combined with up to 2kb, up to 5kb, or up to 10kb of flanking sequences from a corresponding genomic region to which these SEQ IDs map (*e.g.*, in a human genome). In some embodiments, single stranded oligonucleotides have a
30 sequence as set forth in any one of SEQ ID NOS: 30 to 13087. In some embodiments, single stranded oligonucleotides have a sequence as set forth in Table 2. In some embodiments, a PRC2 associated region of SMN1 or SMN2 against which a single stranded oligonucleotide

is complementary is selected from SEQ ID NOS: 24-29. In some embodiments, a single stranded oligonucleotide that is complementary with a PRC2 associated region of SMN1 or SMN2 comprises a sequence selected from SEQ ID NOS: 1158-1159, 1171, 1482-1483, 1485-1486, 2465-2471, 2488-2490, 2542-2546, 2656-2657, 2833-2835, 3439-3440, 3916-3918, 4469-4472, 4821, 5429, 5537, 6061, 7327, 8330-13061, and 13062-13087. In some
5 embodiments, a single stranded oligonucleotide that is complementary with a PRC2 associated region of SMN1 or SMN2 comprises a sequence selected from 11395, 11394, 10169, and 10170.

Without being bound by a theory of invention, these oligonucleotides are able to
10 interfere with the binding of and function of PRC2, by preventing recruitment of PRC2 to a specific chromosomal locus. For example, a single administration of single stranded oligonucleotides designed to specifically bind a PRC2-associated region lncRNA can stably displace not only the lncRNA, but also the PRC2 that binds to the lncRNA, from binding chromatin. After displacement, the full complement of PRC2 is not recovered for up to 24
15 hours. Further, lncRNA can recruit PRC2 in a cis fashion, repressing gene expression at or near the specific chromosomal locus from which the lncRNA was transcribed.

Methods of modulating gene expression are provided, in some embodiments, that may be carried out *in vitro*, *ex vivo*, or *in vivo*. It is understood that any reference to uses of compounds throughout the description contemplates use of the compound in preparation of a
20 pharmaceutical composition or medicament for use in the treatment of condition (*e.g.*, Spinal muscular atrophy) associated with decreased levels or activity of SMN1 or SMN2. Thus, as one nonlimiting example, this aspect of the invention includes use of such single stranded oligonucleotides in the preparation of a medicament for use in the treatment of disease, wherein the treatment involves upregulating expression of SMN1 or SMN2.

25 In further aspects of the invention, methods are provided for selecting a candidate oligonucleotide for activating expression of SMN1 or SMN2. The methods generally involve selecting as a candidate oligonucleotide, a single stranded oligonucleotide comprising a nucleotide sequence that is complementary to a PRC2-associated region (*e.g.*, a nucleotide sequence as set forth in any one of SEQ ID NOS: 9 to 29). In some embodiments, sets of
30 oligonucleotides may be selected that are enriched (*e.g.*, compared with a random selection of oligonucleotides) in oligonucleotides that activate expression of SMN1 or SMN2.

Single Stranded Oligonucleotides for Modulating Expression of SMN1 or SMN2

In one aspect of the invention, single stranded oligonucleotides complementary to the PRC2-associated regions are provided for modulating expression of SMN1 or SMN2 in a cell. In some embodiments, expression of SMN1 or SMN2 is upregulated or increased. In some embodiments, single stranded oligonucleotides complementary to these PRC2-associated regions inhibit the interaction of PRC2 with long RNA transcripts such that gene expression is upregulated or increased. In some embodiments, single stranded oligonucleotides complementary to these PRC2-associated regions inhibit the interaction of PRC2 with long RNA transcripts, resulting in reduced methylation of histone H3 and reduced gene inactivation, such that gene expression is upregulated or increased. In some embodiments, this interaction may be disrupted or inhibited due to a change in the structure of the long RNA that prevents or reduces binding to PRC2. The oligonucleotide may be selected using any of the methods disclosed herein for selecting a candidate oligonucleotide for activating expression of SMN1 or SMN2.

The single stranded oligonucleotide may comprise a region of complementarity that is complementary with a PRC2-associated region of a nucleotide sequence set forth in any one of SEQ ID NOS: 1 to 8. The region of complementarity of the single stranded oligonucleotide may be complementary with at least 6, *e.g.*, at least 7, at least 8, at least 9, at least 10, at least 15 or more consecutive nucleotides of the PRC2-associated region.

It should be appreciated that due the high homology between SMN1 and SMN2, single stranded oligonucleotides that are complementary with a PRC2-associated region of SMN1 are often also complementary with a corresponding PRC2-associated region of SMN2.

The PRC2-associated region may map to a position in a chromosome between 50 kilobases upstream of a 5'-end of the SMN1 or SMN2 gene and 50 kilobases downstream of a 3'-end of the SMN1 or SMN2 gene. The PRC2-associated region may map to a position in a chromosome between 25 kilobases upstream of a 5'-end of the SMN1 or SMN2 gene and 25 kilobases downstream of a 3'-end of the SMN1 or SMN2 gene. The PRC2-associated region may map to a position in a chromosome between 12 kilobases upstream of a 5'-end of the SMN1 or SMN2 gene and 12 kilobases downstream of a 3'-end of the SMN1 or SMN2 gene. The PRC2-associated region may map to a position in a chromosome between 5 kilobases upstream of a 5'-end of the SMN1 or SMN2 gene and 5 kilobases downstream of a 3'-end of the SMN1 or SMN2 gene.

The genomic position of the selected PRC2-associated region relative to the SMN1 or SMN2 gene may vary. For example, the PRC2-associated region may be upstream of the 5' end of the SMN1 or SMN2 gene. The PRC2-associated region may be downstream of the 3' end of the SMN1 or SMN2 gene. The PRC2-associated region may be within an intron of the SMN1 or SMN2 gene. The PRC2-associated region may be within an exon of the SMN1 or SMN2 gene. The PRC2-associated region may traverse an intron-exon junction, a 5'-UTR-exon junction or a 3'-UTR-exon junction of the SMN1 or SMN2 gene.

The single stranded oligonucleotide may comprise a sequence having the formula X-Y-Z, in which X is any nucleotide, Y is a nucleotide sequence of 6 nucleotides in length that is not a human seed sequence of a microRNA, and Z is a nucleotide sequence of varying length. In some embodiments X is the 5' nucleotide of the oligonucleotide. In some embodiments, when X is anchored at the 5' end of the oligonucleotide, the oligonucleotide does not have any nucleotides or nucleotide analogs linked 5' to X. In some embodiments, other compounds such as peptides or sterols may be linked at the 5' end in this embodiment as long as they are not nucleotides or nucleotide analogs. In some embodiments, the single stranded oligonucleotide has a sequence 5'-X-Y-Z and is 8-50 nucleotides in length. Oligonucleotides that have these sequence characteristics are predicted to avoid the miRNA pathway. Therefore, in some embodiments, oligonucleotides having these sequence characteristics are unlikely to have an unintended consequence of functioning in a cell as a miRNA molecule. The Y sequence may be a nucleotide sequence of 6 nucleotides in length set forth in Table 1.

The single stranded oligonucleotide may have a sequence that does not contain guanosine nucleotide stretches (*e.g.*, 3 or more, 4 or more, 5 or more, 6 or more consecutive guanosine nucleotides). In some embodiments, oligonucleotides having guanosine nucleotide stretches have increased non-specific binding and/or off-target effects, compared with oligonucleotides that do not have guanosine nucleotide stretches.

The single stranded oligonucleotide may have a sequence that has less than a threshold level of sequence identity with every sequence of nucleotides, of equivalent length, that map to a genomic position encompassing or in proximity to an off-target gene. For example, an oligonucleotide may be designed to ensure that it does not have a sequence that maps to genomic positions encompassing or in proximity with all known genes (*e.g.*, all known protein coding genes) other than SMN1 or SMN2. In a similar embodiment, an

oligonucleotide may be designed to ensure that it does not have a sequence that maps to any other known PRC2-associated region, particularly PRC2-associated regions that are functionally related to any other known gene (*e.g.*, any other known protein coding gene). In either case, the oligonucleotide is expected to have a reduced likelihood of having off-target effects. The threshold level of sequence identity may be 50%, 60%, 70%, 80%, 85%, 90%, 95%, 99% or 100% sequence identity.

The single stranded oligonucleotide may have a sequence that is complementary to a PRC2-associated region that encodes an RNA that forms a secondary structure comprising at least two single stranded loops. It has been discovered that, in some embodiments, oligonucleotides that are complementary to a PRC2-associated region that encodes an RNA that forms a secondary structure comprising one or more single stranded loops (*e.g.*, at least two single stranded loops) have a greater likelihood of being active (*e.g.*, of being capable of activating or enhancing expression of a target gene) than a randomly selected oligonucleotide. In some cases, the secondary structure may comprise a double stranded stem between the at least two single stranded loops. Accordingly, the region of complementarity between the oligonucleotide and the PRC2-associated region may be at a location of the PRC2 associated region that encodes at least a portion of at least one of the loops. In some cases, the region of complementarity between the oligonucleotide and the PRC2-associated region may be at a location of the PRC2-associated region that encodes at least a portion of at least two of the loops. In some cases, the region of complementarity between the oligonucleotide and the PRC2-associated region may be at a location of the PRC2 associated region that encodes at least a portion of the double stranded stem. In some embodiments, a PRC2-associated region (*e.g.*, of an lncRNA) is identified (*e.g.*, using RIP-Seq methodology or information derived therefrom). In some embodiments, the predicted secondary structure RNA (*e.g.*, lncRNA) containing the PRC2-associated region is determined using RNA secondary structure prediction algorithms, *e.g.*, RNAfold, mfold. In some embodiments, oligonucleotides are designed to target a region of the RNA that forms a secondary structure comprising one or more single stranded loop (*e.g.*, at least two single stranded loops) structures which may comprise a double stranded stem between the at least two single stranded loops.

The single stranded oligonucleotide may have a sequence that is has greater than 30% G-C content, greater than 40% G-C content, greater than 50% G-C content, greater than 60%

G-C content, greater than 70% G-C content, or greater than 80% G-C content. The single stranded oligonucleotide may have a sequence that has up to 100% G-C content, up to 95% G-C content, up to 90% G-C content, or up to 80% G-C content. In some embodiments in which the oligonucleotide is 8 to 10 nucleotides in length, all but 1, 2, 3, 4, or 5 of the
5 nucleotides of the complementary sequence of the PRC2-associated region are cytosine or guanosine nucleotides. In some embodiments, the sequence of the PRC2-associated region to which the single stranded oligonucleotide is complementary comprises no more than 3 nucleotides selected from adenine and uracil.

The single stranded oligonucleotide may be complementary to a chromosome of a
10 different species (*e.g.*, a mouse, rat, rabbit, goat, monkey, *etc.*) at a position that encompasses or that is in proximity to that species' homolog of SMN1 or SMN2. The single stranded oligonucleotide may be complementary to a human genomic region encompassing or in proximity to the SMN1 or SMN2 gene and also be complementary to a mouse genomic region encompassing or in proximity to the mouse homolog of SMN1 or SMN2. For
15 example, the single stranded oligonucleotide may be complementary to a sequence as set forth in SEQ ID NO: 1, 2, 4, or 5, which is a human genomic region encompassing or in proximity to the SMN1 or SMN2 gene, and also be complementary to a sequence as set forth in SEQ ID NO:7 or 8, which is a mouse genomic region encompassing or in proximity to the mouse homolog of the SMN1 or SMN2 gene. Oligonucleotides having these characteristics
20 may be tested *in vivo* or *in vitro* for efficacy in multiple species (*e.g.*, human and mouse). This approach also facilitates development of clinical candidates for treating human disease by selecting a species in which an appropriate animal exists for the disease.

In some embodiments, the region of complementarity of the single stranded oligonucleotide is complementary with at least 8 to 15, 8 to 30, 8 to 40, or 10 to 50, or 5 to
25 50, or 5 to 40 bases, *e.g.*, 5, 6, 7, 8, 9, 10, 11, 12, 13, 14, 15, 16, 17, 18, 19, 20, 21, 22, 23, 24, 25, 26, 27, 28, 29, 30, 31, 32, 33, 34, 35, 36, 37, 38, 39, 40, 41, 42, 43, 44, 45, 46, 47, 48, 49, or 50 consecutive nucleotides of a PRC2-associated region. In some embodiments, the region of complementarity is complementary with at least 8 consecutive nucleotides of a PRC2-associated region. In some embodiments the sequence of the single stranded
30 oligonucleotide is based on an RNA sequence that binds to PRC2, or a portion thereof, said portion having a length of from 5 to 40 contiguous base pairs, or about 8 to 40 bases, or about 5 to 15, or about 5 to 30, or about 5 to 40 bases, or about 5 to 50 bases. Complementary, as the

term is used in the art, refers to the capacity for precise pairing between two nucleotides. For example, if a nucleotide at a certain position of an oligonucleotide is capable of hydrogen bonding with a nucleotide at the same position of PRC2-associated region, then the single stranded nucleotide and PRC2-associated region are considered to be complementary to each other at that position. The single stranded nucleotide and PRC2-associated region are complementary to each other when a sufficient number of corresponding positions in each molecule are occupied by nucleotides that can hydrogen bond with each other through their bases. Thus, "complementary" is a term which is used to indicate a sufficient degree of complementarity or precise pairing such that stable and specific binding occurs between the single stranded nucleotide and PRC2-associated region. For example, if a base at one position of a single stranded nucleotide is capable of hydrogen bonding with a base at the corresponding position of a PRC2-associated region, then the bases are considered to be complementary to each other at that position. 100% complementarity is not required.

The single stranded oligonucleotide may be at least 80% complementary to (optionally one of at least 85%, 90%, 91%, 92%, 93%, 94%, 95%, 96%, 97%, 98%, 99% or 100% complementary to) the consecutive nucleotides of a PRC2-associated region. In some embodiments the single stranded oligonucleotide may contain 1, 2 or 3 base mismatches compared to the portion of the consecutive nucleotides of a PRC2-associated region. In some embodiments the single stranded oligonucleotide may have up to 3 mismatches over 15 bases, or up to 2 mismatches over 10 bases.

It is understood in the art that a complementary nucleotide sequence need not be 100% complementary to that of its target to be specifically hybridizable. In some embodiments, a complementary nucleic acid sequence for purposes of the present disclosure is specifically hybridizable when binding of the sequence to the target molecule (*e.g.*, lncRNA) interferes with the normal function of the target (*e.g.*, lncRNA) to cause a loss of activity (*e.g.*, inhibiting PRC2-associated repression with consequent up-regulation of gene expression) and there is a sufficient degree of complementarity to avoid non-specific binding of the sequence to non-target sequences under conditions in which avoidance of non-specific binding is desired, *e.g.*, under physiological conditions in the case of *in vivo* assays or therapeutic treatment, and in the case of *in vitro* assays, under conditions in which the assays are performed under suitable conditions of stringency.

In some embodiments, the single stranded oligonucleotide is 7, 8, 9, 10, 11, 12, 13, 14, 15, 16, 17, 18, 19, 20, 21, 22, 23, 24, 25, 26, 27, 28, 29, 30, 35, 40, 45, 50 or more nucleotides in length. In a preferred embodiment, the oligonucleotide is 8 to 30 nucleotides in length.

5 In some embodiments, the PRC2-associated region occurs on the same DNA strand as a gene sequence (sense). In some embodiments, the PRC2-associated region occurs on the opposite DNA strand as a gene sequence (anti-sense). Oligonucleotides complementary to a PRC2-associated region can bind either sense or anti-sense sequences. Base pairings may include both canonical Watson-Crick base pairing and non-Watson-Crick base pairing (e.g.,
10 Wobble base pairing and Hoogsteen base pairing). It is understood that for complementary base pairings, adenosine-type bases (A) are complementary to thymidine-type bases (T) or uracil-type bases (U), that cytosine-type bases (C) are complementary to guanosine-type bases (G), and that universal bases such as 3-nitropyrrole or 5-nitroindole can hybridize to and are considered complementary to any A, C, U, or T. Inosine (I) has also been considered
15 in the art to be a universal base and is considered complementary to any A, C, U or T.

In some embodiments, any one or more thymidine (T) nucleotides (or modified nucleotide thereof) or uridine (U) nucleotides (or a modified nucleotide thereof) in a sequence provided herein, including a sequence provided in the sequence listing, may be replaced with any other nucleotide suitable for base pairing (e.g., via a Watson-Crick base
20 pair) with an adenosine nucleotide. In some embodiments, any one or more thymidine (T) nucleotides (or modified nucleotide thereof) or uridine (U) nucleotides (or a modified nucleotide thereof) in a sequence provided herein, including a sequence provided in the sequence listing, may be suitably replaced with a different pyrimidine nucleotide or vice versa. In some embodiments, any one or more thymidine (T) nucleotides (or modified
25 nucleotide thereof) in a sequence provided herein, including a sequence provided in the sequence listing, may be suitably replaced with a uridine (U) nucleotide (or a modified nucleotide thereof) or vice versa. In some embodiments, GC content of the single stranded oligonucleotide is preferably between about 30-60 %. Contiguous runs of three or more Gs or Cs may not be preferable in some embodiments. Accordingly, in some embodiments, the
30 oligonucleotide does not comprise a stretch of three or more guanosine nucleotides.

In some embodiments, the single stranded oligonucleotide specifically binds to, or is complementary to an RNA that is encoded in a genome (e.g., a human genome) as a single

contiguous transcript (e.g., a non-spliced RNA). In some embodiments, the single stranded oligonucleotide specifically binds to, or is complementary to an RNA that is encoded in a genome (e.g., a human genome), in which the distance in the genome between the 5' end of the coding region of the RNA and the 3' end of the coding region of the RNA is less than 1 kb, less than 2 kb, less than 3 kb, less than 4 kb, less than 5 kb, less than 7 kb, less than 8 kb, less than 9 kb, less than 10 kb, or less than 20 kb.

It is to be understood that any oligonucleotide provided herein can be excluded.

In some embodiments, single stranded oligonucleotides disclosed herein may increase expression of mRNA corresponding to the gene by at least about 50% (i.e. 150% of normal or 1.5 fold), or by about 2 fold to about 5 fold. In some embodiments, expression may be increased by at least about 15 fold, 20 fold, 30 fold, 40 fold, 50 fold or 100 fold, or any range between any of the foregoing numbers. It has also been found that increased mRNA expression has been shown to correlate to increased protein expression.

In some or any of the embodiments of the oligonucleotides described herein, or processes for designing or synthesizing them, the oligonucleotides will upregulate gene expression and may specifically bind or specifically hybridize or be complementary to the PRC2 binding RNA that is transcribed from the same strand as a protein coding reference gene. The oligonucleotide may bind to a region of the PRC2 binding RNA that originates within or overlaps an intron, exon, intron exon junction, 5' UTR, 3' UTR, a translation initiation region, or a translation termination region of a protein coding sense strand of a reference gene (refGene).

In some or any of the embodiments of oligonucleotides described herein, or processes for designing or synthesizing them, the oligonucleotides will upregulate gene expression and may specifically bind or specifically hybridize or be complementary to a PRC2 binding RNA that transcribed from the opposite strand (the antisense strand) of a protein coding reference gene. The oligonucleotide may bind to a region of the PRC2 binding RNA that originates within or overlaps an intron, exon, intron exon junction, 5' UTR, 3' UTR, a translation initiation region, or a translation termination region of a protein coding antisense strand of a reference gene.

The oligonucleotides described herein may be modified, e.g., comprise a modified sugar moiety, a modified internucleoside linkage, a modified nucleotide and/or combinations thereof. In addition, the oligonucleotides can exhibit one or more of the following properties:

do not induce substantial cleavage or degradation of the target RNA; do not cause substantially complete cleavage or degradation of the target RNA; do not activate the RNase H pathway; do not activate RISC; do not recruit any Argonaute family protein; are not cleaved by Dicer; do not mediate alternative splicing; are not immune stimulatory; are
5 nuclease resistant; have improved cell uptake compared to unmodified oligonucleotides; are not toxic to cells or mammals; may have improved endosomal exit; do interfere with interaction of lncRNA with PRC2, preferably the Ezh2 subunit but optionally the Suz12, Eed, RbAp46/48 subunits or accessory factors such as Jarid2; do decrease histone H3 lysine27 methylation and/or do upregulate gene expression.

10 Oligonucleotides that are designed to interact with RNA to modulate gene expression are a distinct subset of base sequences from those that are designed to bind a DNA target (e.g., are complementary to the underlying genomic DNA sequence from which the RNA is transcribed).

15 *Splice Switching Oligonucleotides*

Aspects of the invention provide strategies for targeting SMN1 or SMN2 precursor mRNA to affect splicing to minimize exon skipping. Accordingly, aspects of the invention provide therapeutic compounds useful for the treatment of SMA. In some embodiments, oligonucleotides, referred to herein as “splice switching oligonucleotides” are provided that
20 modulate SMN2 splicing. Methods and related compositions, compounds, and kits are provided, in some embodiments, that are useful for increasing expression of full-length. SMN protein in a cell. The methods generally involve delivering to a cell a first single stranded oligonucleotide complementary with at least 8 consecutive nucleotides of a PRC2-associated region of SMN2 and a second single stranded oligonucleotide complementary with
25 a splice control sequence of a precursor mRNA of SMN2, in amounts sufficient to increase expression of a mature mRNA of SMN2 that comprises (or includes) exon 7 in the cell. Any of the single stranded oligonucleotides that are complementary with at least 8 consecutive nucleotides of a PRC2-associated region of SMN1 or SMN2 may be used. It should be appreciated that single stranded oligonucleotides that are complementary with a splice control
30 sequence may alternatively be referred herein, as splice switching oligonucleotides.

Splice switching oligonucleotides typically comprise a sequence complementary to a splice control sequence (*e.g.*, a intronic splicing silencer sequence) of a precursor mRNA, and are capable of binding to and affecting processing of the precursor mRNA. Splice switching oligonucleotides may be complementary with a region of an exon, a region of an intron or an intron/exon junction. In some embodiments, the splice control sequence comprises the sequence: CAGCAUUAUGAAAG (SEQ ID NO: 13100) or a portion thereof. In some embodiments, the splice control sequence comprises at least one hnRNAP binding sequence. In some embodiments, splice switching oligonucleotides that target SMN1 or SMN2 function based on the premise that there is a competition between the 3' splice sites of exons 7 and 8 for pairing with the 5' splice site of exon 6, so impairing the recognition of the 3' splice site of exon 8 favors exon 7 inclusion. In some embodiments, splice switching oligonucleotides are provided that promote SMN2 exon 7 inclusion and full-length SMN protein expression, in which the oligonucleotides are complementary to the intron 7/exon 8 junction. In some embodiments, splice switching oligonucleotide are composed of a segment complementary to an exon of SMN1 or SMN2 (*e.g.*, exon 7). In some embodiments, splice switching oligonucleotides comprise a tail (*e.g.*, a non-complementary tail) consisting of RNA sequences with binding motifs recognized by a serine/arginine-rich (SR) protein. In some embodiments, splice switching oligonucleotides are complementary (at least partially) with an intronic splicing silencer (ISS). In some embodiments, the ISS is in intron 6 or intron 7 of SMN1 or SMN2. In some embodiments, splice switching oligonucleotides comprise an antisense moiety complementary to a target exon or intron (*e.g.*, of SMN1 or SMN2) and a minimal RS domain peptide similar to the splicing activation domain of SR proteins. In some embodiments, the splice switching oligonucleotide is 7, 8, 9, 10, 11, 12, 13, 14, 15, 16, 17, 18, 19, 20, 21, 22, 23, 24, 25, 26, 27, 28, 29, 30, 35, 40, 45, 50 or more nucleotides in length. In one embodiment, the oligonucleotide is 8 to 30 nucleotides in length.

Linkers

Any of the oligonucleotides disclosed herein may be linked to one or more other oligonucleotides disclosed herein by a linker, *e.g.*, a cleavable linker. Accordingly, in some embodiments, compounds are provided that comprise a single stranded oligonucleotide complementary with a PRC2-associated region of a gene that is linked via a linker to a single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA

of the gene. In some embodiments, compounds are provided that have the general formula A-B-C, in which A is a single stranded oligonucleotide complementary with a PRC2-associated region of a gene, B is a linker, and C is a single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA of the gene. In some
5 embodiments, linker B comprises an oligonucleotide, peptide, low pH labile bond, or disulfide bond. In some embodiments, the compound is orientated as 5'-A-B-C-3'. In some embodiments, the compound is orientated as 3'-A-B-C-5'. In some embodiments, where B is an oligonucleotide, the 3' end of A is linked to the 5' end of B, and the 3' end of B is linked to 5' end of C. In some embodiments, where B is an oligonucleotide, the 5' end of A is
10 linked to the 3' end of B, and the 5' end of B is linked to 3' end of C. In some embodiments, where B is an oligonucleotide, the 5' end of A is linked to the 5' end of B, and/or the 3' end of B is linked to the 3' end of C. In some embodiments, where B is an oligonucleotide, the 3' end of A is linked to the 3' end of B, and/or the 5' end of B is linked to the 5' end of C.

15 The term "linker" generally refers to a chemical moiety that is capable of covalently linking two or more oligonucleotides. In some embodiments, at least one bond comprised or contained within the linker is capable of being cleaved (e.g., in a biological context, such as in a mammalian extract, such as an endosomal extract), such that at least two oligonucleotides are no longer covalently linked to one another after bond cleavage. It will
20 be appreciated that, in some embodiments, a provided linker may include a region that is non-cleavable, as long as the linker also comprises at least one bond that is cleavable.

In some embodiments, the linker comprises a polypeptide that is more susceptible to cleavage by an endopeptidase in the mammalian extract than the oligonucleotides. The endopeptidase may be a trypsin, chymotrypsin, elastase, thermolysin, pepsin, or
25 endopeptidase V8. The endopeptidase may be a cathepsin B, cathepsin D, cathepsin L, cathepsin C, papain, cathepsin S or endosomal acidic insulinase. For example, the linker comprise a peptide having an amino acid sequence selected from: ALAL, APISFFELG, FL, GFN, R/KXX, GRWHTVGLRWE, YL, GF, and FF, in which X is any amino acid.

In some embodiments, the linker comprises the formula $-(CH_2)_nS-S(CH_2)_m-$, wherein
30 n and m are independently integers from 0 to 10.

In some embodiments, the linker may comprise an oligonucleotide that is more susceptible to cleavage by an endonuclease in the mammalian extract than the

oligonucleotides. The linker may have a nucleotide sequence comprising from 1 to 10 thymidines or uridines. The linker may have a nucleotide sequence comprising deoxyribonucleotides linked through phosphodiester internucleotide linkages. The linker may have a nucleotide sequence comprising from 1 to 10 thymidines or uridines linked
5 through phosphodiester internucleotide linkages. The linker may have a nucleotide sequence comprising from 1 to 10 thymidines or uridines linked through phosphorothioate internucleotide linkages.

In some embodiments, at least one linker is 2-fold, 3-fold, 4-fold, 5-fold, 10-fold or more sensitive to enzymatic cleavage in the presence of a mammalian extract than at least
10 two oligonucleotides. It should be appreciated that different linkers can be designed to be cleaved at different rates and/or by different enzymes in compounds comprising two or more linkers. Similarly different linkers can be designed to be sensitive to cleavage in different tissues, cells or subcellular compartments in compounds comprising two or more linkers. This can advantageously permit compounds to have oligonucleotides that are released from
15 compounds at different rates, by different enzymes, or in different tissues, cells or subcellular compartments thereby controlling release of the monomeric oligonucleotides to a desired *in vivo* location or at a desired time following administration.

In certain embodiments, linkers are stable (e.g., more stable than the oligonucleotides they link together) in plasma, blood or serum which are richer in exonucleases, and less
20 stable in the intracellular environments which are relatively rich in endonucleases. In some embodiments, a linker is considered “non-cleavable” if the linker’s half-life is at least 24, or 28, 32, 36, 48, 72, 96 hours or longer under the conditions described here, such as in liver homogenates. Conversely, in some embodiments, a linker is considered “cleavable” if the half-life of the linker is at most 10, or 8, 6, 5 hours or shorter.

In some embodiments, the linker is a nuclease-cleavable oligonucleotide linker. In some embodiments, the nuclease-cleavable linker contains one or more phosphodiester bonds in the oligonucleotide backbone. For example, the linker may contain a single
25 phosphodiester bridge or 2, 3, 4, 5, 6, 7 or more phosphodiester linkages, for example as a string of 1-10 deoxynucleotides, e.g., dT, or ribonucleotides, e.g., rU, in the case of RNA linkers. In the case of dT or other DNA nucleotides dN in the linker, in certain embodiments
30 the cleavable linker contains one or more phosphodiester linkages. In other embodiments, in the case of rU or other RNA nucleotides rN, the cleavable linker may consist of

phosphorothioate linkages only. In contrast to phosphorothioate-linked deoxynucleotides, which in some embodiments are cleaved relatively slowly by nucleases (thus termed “noncleavable”), phosphorothioate-linked rU undergoes relatively rapid cleavage by ribonucleases and therefore is considered cleavable herein in some embodiments. It is also possible to combine dN and rN into the linker region, which are connected by phosphodiester or phosphorothioate linkages. In other embodiments, the linker can also contain chemically modified nucleotides, which are still cleavable by nucleases, such as, e.g., 2'-O-modified analogs. In particular, 2'-O-methyl or 2'-fluoro nucleotides can be combined with each other or with dN or rN nucleotides. Generally, in the case of nucleotide linkers, the linker is a part of the compound that is usually not complementary to a target, although it could be. This is because the linker is generally cleaved prior to action of the oligonucleotides on the target, and therefore, the linker identity with respect to a target is inconsequential. Accordingly, in some embodiments, a linker is an (oligo)nucleotide linker that is not complementary to any of the targets against which the oligonucleotides are designed.

In some embodiments, the cleavable linker is an oligonucleotide linker that contains a continuous stretch of deliberately introduced Rp phosphorothioate stereoisomers (e.g., 4, 5, 6, 7 or longer stretches). The Rp stereoisomer, unlike Sp isoform, is known to be susceptible to nuclease cleavage (Krieg et al., 2003, Oligonucleotides, 13:491-499). Such a linker would not include a racemic mix of PS linkaged oligonucleotides since the mixed linkages are relatively stable and are not likely to contain long stretches of the Rp stereoisomers, and therefore, considered “non-cleavable” herein. Thus, in some embodiments, a linker comprises a stretch of 4, 5, 6, 7 or more phosphorothioated nucleotides, wherein the stretch does not contain a substantial amount or any of the Sp stereoisomer. The amount could be considered substantial if it exceeds 10% on a per-mole basis.

In some embodiments, the linker is a non-nucleotide linker, for example, a single phosphodiester bridge. Another example of such cleavable linkers is a chemical group comprising a disulfide bond, for example, $-(CH_2)_nS-S(CH_2)_m-$, wherein n and m are integers from 0 to 10. In illustrative embodiments, $n=m=6$. Additional examples of non-nucleotide linkers are described below.

The linker can be designed so as to undergo a chemical or enzymatic cleavage reaction. Chemical reactions involve, for example, cleavage in acidic environments (e.g., endosomes), reductive cleavage (e.g., cytosolic cleavage) or oxidative cleavage (e.g., in liver

microsomes). The cleavage reaction can also be initiated by a rearrangement reaction.

Enzymatic reactions can include reactions mediated by nucleases, peptidases, proteases, phosphatases, oxidases, reductases, etc. For example, a linker can be pH-sensitive, cathepsin-sensitive, or predominantly cleaved in endosomes and/or cytosol.

5 In some embodiments, the linker comprises a peptide. In certain embodiments, the linker comprises a peptide which includes a sequence that is cleavable by an endopeptidase. In addition to the cleavable peptide sequence, the linker may comprise additional amino acid residues and/or non-peptide chemical moieties, such as an alkyl chain. In certain
10 embodiments, the linker comprises Ala-Leu-Ala-Leu, which is a substrate for cathepsin B. See, for example, the maleimidocaproyl-Arg-Arg-Ala-Leu-Ala-Leu linkers described in Schmid et al, *Bioconjugate Chem* 2007, 18, 702-716. In certain embodiments, a cathepsin B-cleavable linker is cleaved in tumor cells. In certain embodiments, the linker comprises Ala-Pro-Ile-Ser-Phe-Phe-Glu-Leu-Gly, which is a substrate for cathepsins D, L, and B (see, for example, Fischer et al, *Chembiochem* 2006, 7, 1428-1434). In certain embodiments, a
15 cathepsin-cleavable linker is cleaved in HeLA cells. In some embodiments, the linker comprises Phe-Lys, which is a substrate for cathepsin B. For example, in certain embodiments, the linker comprises Phe-Lys-p-aminobenzoic acid (PABA). See, e.g., the maleimidocaproyl-Phe-Lys-PABA linker described in Walker et al, *Bioorg. Med. Chem. Lett.* 2002, 12, 217-219. In certain embodiments, the linker comprises Gly-Phe-2-
20 naphthylamide, which is a substrate for cathepsin C (see, for example, Berg et al. *Biochem. J.* 1994, 300, 229-235). In certain embodiments, a cathepsin C-cleavable linker is cleaved in hepatocytes. In some embodiments, the linker comprises a cathepsin S cleavage site. For example, in some embodiments, the linker comprises Gly-Arg-Trp-His-Thr-Val-Gly-Leu-Arg-Trp-Glu, Gly-Arg-Trp-Pro-Pro-Met-Gly-Leu-Pro-Trp-Glu, or Gly-Arg-Trp-His-Pro-
25 Met-Gly-Ala-Pro-Trp-Glu, for example, as described in Lutzner et al, *J. Biol. Chem.* 2008, 283, 36185-36194. In certain embodiments, a cathepsin S-cleavable linker is cleaved in antigen presenting cells. In some embodiments, the linker comprises a papain cleavage site. Papain typically cleaves a peptide having the sequence -R/K-X-X (see Chapman et al, *Annu. Rev. Physiol* 1997, 59, 63-88). In certain embodiments, a papain-cleavable linker is cleaved
30 in endosomes. In some embodiments, the linker comprises an endosomal acidic insulinase cleavage site. For example, in some embodiments, the linker comprises Tyr-Leu, Gly-Phe, or

Phe-Phe (see, e.g., Authier et al, FEBS Lett. 1996, 389, 55-60). In certain embodiments, an endosomal acidic insulinase-cleavable linker is cleaved in hepatic cells.

In some embodiments, the linker is pH sensitive. In certain embodiments, the linker comprises a low pH-labile bond. As used herein, a low-pH labile bond is a bond that is selectively broken under acidic conditions ($\text{pH} < 7$). Such bonds may also be termed endosomally labile bonds, because cell endosomes and lysosomes have a pH less than 7. For example, in certain embodiments, the linker comprises an amine, an imine, an ester, a benzoic imine, an amino ester, a diortho ester, a polyphosphoester, a polyphosphazene, an acetal, a vinyl ether, a hydrazone, an azidomethyl-methylmaleic anhydride, a thiopropionate, a masked endosomolytic agent or a citraconyl group.

In certain embodiments, the linker comprises a low pH-labile bond selected from the following: ketals that are labile in acidic environments (e.g., pH less than 7, greater than about 4) to form a diol and a ketone; acetals that are labile in acidic environments (e.g., pH less than 7, greater than about 4) to form a diol and an aldehyde; imines or iminiums that are labile in acidic environments (e.g., pH less than 7, greater than about 4) to form an amine and an aldehyde or a ketone; silicon-oxygen-carbon linkages that are labile under acidic condition; silicon-nitrogen (silazane) linkages; silicon-carbon linkages (e.g., arylsilanes, vinylsilanes, and allylsilanes); maleamates (amide bonds synthesized from maleic anhydride derivatives and amines); ortho esters; hydrazones; activated carboxylic acid derivatives (e.g., esters, amides) designed to undergo acid catalyzed hydrolysis); or vinyl ethers. Further examples may be found in International Patent Appln. Pub. No. WO 2008/022309, entitled POLYCONJUGATES FOR *IN VIVO* DELIVERY OF POLYNUCLEOTIDES, the contents of which are incorporated herein by reference.

In some embodiments, the linker comprises a masked endosomolytic agent. Endosomolytic polymers are polymers that, in response to a change in pH, are able to cause disruption or lysis of an endosome or provide for escape of a normally membrane-impermeable compound, such as a polynucleotide or protein, from a cellular internal membrane-enclosed vesicle, such as an endosome or lysosome. A subset of endosomolytic compounds is fusogenic compounds, including fusogenic peptides. Fusogenic peptides can facilitate endosomal release of agents such as oligomeric compounds to the cytoplasm. See, for example, US Patent Application Publication Nos. 20040198687, 20080281041, 20080152661, and 20090023890, which are incorporated herein by reference.

The linker can also be designed to undergo an organ/ tissue-specific cleavage. For example, for certain targets, which are expressed in multiple tissues, only the knock-down in liver may be desirable, as knock-down in other organs may lead to undesired side effects. Thus, linkers susceptible to liver-specific enzymes, such as pyrrolase (TPO) and glucose-6-phosphatase (G-6-Pase), can be engineered, so as to limit the antisense effect to the liver mainly. Alternatively, linkers not susceptible to liver enzymes but susceptible to kidney-specific enzymes, such as gamma-glutamyltranspeptidase, can be engineered, so that the antisense effect is limited to the kidneys mainly. Analogously, intestine-specific peptidases cleaving Phe-Ala and Leu-Ala could be considered for orally administered multimeric oligonucleotides. Similarly, by placing an enzyme recognition site into the linker, which is recognized by an enzyme over-expressed in tumors, such as plasmin (e.g., PHEA-D-Val-Leu-Lys recognition site), tumor-specific knock-down should be feasible. By selecting the right enzyme recognition site in the linker, specific cleavage and knock-down should be achievable in many organs. In addition, the linker can also contain a targeting signal, such as *N*-acetyl galactosamine for liver targeting, or folate, vitamin A or RGD-peptide in the case of tumor or activated macrophage targeting. Accordingly, in some embodiments, the cleavable linker is organ- or tissue-specific, for example, liver-specific, kidney-specific, intestine-specific, etc.

The oligonucleotides can be linked through any part of the individual oligonucleotide, e.g., via the phosphate, the sugar (e.g., ribose, deoxyribose), or the nucleobase. In certain embodiments, when linking two oligonucleotides together, the linker can be attached e.g. to the 5'-end of the first oligonucleotide and the 3'-end of the second nucleotide, to the 5'-end of the first oligonucleotide and the 5'-end of the second nucleotide, to the 3'-end of the first oligonucleotide and the 3'-end of the second nucleotide. In other embodiments, when linking two oligonucleotides together, the linker can attach internal residues of each oligonucleotides, e.g., via a modified nucleobase. One of ordinary skill in the art will understand that many such permutations are available for multimers. Further examples of appropriate linkers as well as methods for producing compounds having such linkers are disclosed in International Patent Application Number, PCT/US2012/05535, entitled MULTIMERIC OLIGONUCLEOTIDE COMPOUNDS the contents of which relating to linkers and related chemistries are incorporated herein by referenced in its entirety.

Methods for Selecting Candidate Oligonucleotides for Activating Expression of SMN1 or SMN2

Methods are provided herein for selecting a candidate oligonucleotide for activating or enhancing expression of SMN1 or SMN2. The target selection methods may generally involve steps for selecting single stranded oligonucleotides having any of the structural and functional characteristics disclosed herein. Typically, the methods involve one or more steps aimed at identifying oligonucleotides that target a PRC2-associated region that is functionally related to SMN1 or SMN2, for example a PRC2-associated region of a lncRNA that regulates expression of SMN1 or SMN2 by facilitating (*e.g.*, in a *cis*-regulatory manner) the recruitment of PRC2 to the SMN1 or SMN2 gene. Such oligonucleotides are expected to be candidates for activating expression of SMN1 or SMN2 because of their ability to hybridize with the PRC2-associated region of a nucleic acid (*e.g.*, a lncRNA). In some embodiments, this hybridization event is understood to disrupt interaction of PRC2 with the nucleic acid (*e.g.*, a lncRNA) and as a result disrupt recruitment of PRC2 and its associated co-repressors (*e.g.*, chromatin remodeling factors) to the SMN1 or SMN2 gene locus.

Methods of selecting a candidate oligonucleotide may involve selecting a PRC2-associated region (*e.g.*, a nucleotide sequence as set forth in any one of SEQ ID NOS: 9 to 29) that maps to a chromosomal position encompassing or in proximity to the SMN1 or SMN2 gene (*e.g.*, a chromosomal position having a sequence as set forth in any one of SEQ ID NOS: 1 to 8). The PRC2-associated region may map to the strand of the chromosome comprising the sense strand of the SMN1 or SMN2 gene, in which case the candidate oligonucleotide is complementary to the sense strand of the SMN1 or SMN2 gene (*i.e.*, is antisense to the SMN1 or SMN2 gene). Alternatively, the PRC2-associated region may map to the strand of the first chromosome comprising the antisense strand of the SMN1 or SMN2 gene, in which case the oligonucleotide is complementary to the antisense strand (the template strand) of the SMN1 or SMN2 gene (*i.e.*, is sense to the SMN1 or SMN2 gene).

Methods for selecting a set of candidate oligonucleotides that is enriched in oligonucleotides that activate expression of SMN1 or SMN2 may involve selecting one or more PRC2-associated regions that map to a chromosomal position that encompasses or that is in proximity to the SMN1 or SMN2 gene and selecting a set of oligonucleotides, in which each oligonucleotide in the set comprises a nucleotide sequence that is complementary with the one or more PRC2-associated regions. As used herein, the phrase, “a set of

oligonucleotides that is enriched in oligonucleotides that activate expression of" refers to a set of oligonucleotides that has a greater number of oligonucleotides that activate expression of a target gene (*e.g.*, SMN1 or SMN2) compared with a random selection of oligonucleotides of the same physicochemical properties (*e.g.*, the same GC content, T_m , length *etc.*) as the enriched set.

Where the design and/or synthesis of a single stranded oligonucleotide involves design and/or synthesis of a sequence that is complementary to a nucleic acid or PRC2-associated region described by such sequence information, the skilled person is readily able to determine the complementary sequence, *e.g.*, through understanding of Watson Crick base pairing rules which form part of the common general knowledge in the field.

In some embodiments design and/or synthesis of a single stranded oligonucleotide involves manufacture of an oligonucleotide from starting materials by techniques known to those of skill in the art, where the synthesis may be based on a sequence of a PRC2-associated region, or portion thereof.

Methods of design and/or synthesis of a single stranded oligonucleotide may involve one or more of the steps of:

Identifying and/or selecting PRC2-associated region;

Designing a nucleic acid sequence having a desired degree of sequence identity or complementarity to a PRC2-associated region or a portion thereof;

Synthesizing a single stranded oligonucleotide to the designed sequence;

Purifying the synthesized single stranded oligonucleotide; and

Optionally mixing the synthesized single stranded oligonucleotide with at least one pharmaceutically acceptable diluent, carrier or excipient to form a pharmaceutical composition or medicament.

Single stranded oligonucleotides so designed and/or synthesized may be useful in method of modulating gene expression as described herein.

Preferably, single stranded oligonucleotides of the invention are synthesized chemically. Oligonucleotides used to practice this invention can be synthesized *in vitro* by well-known chemical synthesis techniques.

Oligonucleotides of the invention can be stabilized against nucleolytic degradation such as by the incorporation of a modification, *e.g.*, a nucleotide modification. For example, nucleic acid sequences of the invention include a phosphorothioate at least the first, second,

or third internucleotide linkage at the 5' or 3' end of the nucleotide sequence. As another example, the nucleic acid sequence can include a 2'-modified nucleotide, *e.g.*, a 2'-deoxy, 2'-deoxy-2'-fluoro, 2'-O-methyl, 2'-O-methoxyethyl (2'-O-MOE), 2'-O-aminopropyl (2'-O-AP), 2'-O-dimethylaminoethyl (2'-O-DMAOE), 2'-O-dimethylaminopropyl (2'-O-DMAP), 2'-O-dimethylaminoethyloxyethyl (2'-O-DMAEOE), or 2'-O--N-methylacetamido (2'-O--NMA). As another example, the nucleic acid sequence can include at least one 2'-O-methyl-modified nucleotide, and in some embodiments, all of the nucleotides include a 2'-O-methyl modification. In some embodiments, the nucleic acids are "locked," *i.e.*, comprise nucleic acid analogues in which the ribose ring is "locked" by a methylene bridge connecting the 2'-O atom and the 4'-C atom.

It is understood that any of the modified chemistries or formats of single stranded oligonucleotides described herein can be combined with each other, and that one, two, three, four, five, or more different types of modifications can be included within the same molecule.

In some embodiments, the method may further comprise the steps of amplifying the synthesized single stranded oligonucleotide, and/or purifying the single stranded oligonucleotide (or amplified single stranded oligonucleotide), and/or sequencing the single stranded oligonucleotide so obtained.

As such, the process of preparing a single stranded oligonucleotide may be a process that is for use in the manufacture of a pharmaceutical composition or medicament for use in the treatment of disease, optionally wherein the treatment involves modulating expression of a gene associated with a PRC2-associated region.

In the methods described above a PRC2-associated region may be, or have been, identified, or obtained, by a method that involves identifying RNA that binds to PRC2.

Such methods may involve the following steps: providing a sample containing nuclear ribonucleic acids, contacting the sample with an agent that binds specifically to PRC2 or a subunit thereof, allowing complexes to form between the agent and protein in the sample, partitioning the complexes, synthesizing nucleic acid that is complementary to nucleic acid present in the complexes.

Where the single stranded oligonucleotide is based on a PRC2-associated region, or a portion of such a sequence, it may be based on information about that sequence, *e.g.*, sequence information available in written or electronic form, which may include sequence information contained in publicly available scientific publications or sequence databases.

Nucleotide Analogues

In some embodiments, the oligonucleotide may comprise at least one ribonucleotide, at least one deoxyribonucleotide, and/or at least one bridged nucleotide. In some
5 embodiments, the oligonucleotide may comprise a bridged nucleotide, such as a locked nucleic acid (LNA) nucleotide, a constrained ethyl (cEt) nucleotide, or an ethylene bridged nucleic acid (ENA) nucleotide. Examples of such nucleotides are disclosed herein and known in the art. In some embodiments, the oligonucleotide comprises a nucleotide analog disclosed in one of the following United States Patent or Patent Application Publications: US
10 7,399,845, US 7,741,457, US 8,022,193, US 7,569,686, US 7,335,765, US 7,314,923, US 7,335,765, and US 7,816,333, US 20110009471, the entire contents of each of which are incorporated herein by reference for all purposes. The oligonucleotide may have one or more 2' O-methyl nucleotides. The oligonucleotide may consist entirely of 2' O-methyl nucleotides.

15 Often the single stranded oligonucleotide has one or more nucleotide analogues. For example, the single stranded oligonucleotide may have at least one nucleotide analogue that results in an increase in T_m of the oligonucleotide in a range of 1 °C, 2 °C, 3 °C, 4 °C, or 5 °C compared with an oligonucleotide that does not have the at least one nucleotide analogue. The single stranded oligonucleotide may have a plurality of nucleotide analogues that results
20 in a total increase in T_m of the oligonucleotide in a range of 2 °C, 3 °C, 4 °C, 5 °C, 6 °C, 7 °C, 8 °C, 9 °C, 10 °C, 15 °C, 20 °C, 25 °C, 30 °C, 35 °C, 40 °C, 45 °C or more compared with an oligonucleotide that does not have the nucleotide analogue.

The oligonucleotide may be of up to 50 nucleotides in length in which 2 to 10, 2 to 15, 2 to 16, 2 to 17, 2 to 18, 2 to 19, 2 to 20, 2 to 25, 2 to 30, 2 to 40, 2 to 45, or more
25 nucleotides of the oligonucleotide are nucleotide analogues. The oligonucleotide may be of 8 to 30 nucleotides in length in which 2 to 10, 2 to 15, 2 to 16, 2 to 17, 2 to 18, 2 to 19, 2 to 20, 2 to 25, 2 to 30 nucleotides of the oligonucleotide are nucleotide analogues.

The oligonucleotide may be of 8 to 15 nucleotides in length in which 2 to 4, 2 to 5, 2 to 6, 2 to 7, 2 to 8, 2 to 9, 2 to 10, 2 to 11, 2 to 12, 2 to 13, 2 to 14 nucleotides of the
30 oligonucleotide are nucleotide analogues. Optionally, the oligonucleotides may have every nucleotide except 1, 2, 3, 4, 5, 6, 7, 8, 9, or 10 nucleotides modified.

The oligonucleotide may consist entirely of bridged nucleotides (*e.g.*, LNA nucleotides, cEt nucleotides, ENA nucleotides). The oligonucleotide may comprise alternating deoxyribonucleotides and 2'-fluoro-deoxyribonucleotides. The oligonucleotide may comprise alternating deoxyribonucleotides and 2'-O-methyl nucleotides. The oligonucleotide may comprise alternating deoxyribonucleotides and ENA nucleotide analogues. The oligonucleotide may comprise alternating deoxyribonucleotides and LNA nucleotides. The oligonucleotide may comprise alternating LNA nucleotides and 2'-O-methyl nucleotides. The oligonucleotide may have a 5' nucleotide that is a bridged nucleotide (*e.g.*, a LNA nucleotide, cEt nucleotide, ENA nucleotide). The oligonucleotide may have a 5' nucleotide that is a deoxyribonucleotide.

The oligonucleotide may comprise deoxyribonucleotides flanked by at least one bridged nucleotide (*e.g.*, a LNA nucleotide, cEt nucleotide, ENA nucleotide) on each of the 5' and 3' ends of the deoxyribonucleotides. The oligonucleotide may comprise deoxyribonucleotides flanked by 1, 2, 3, 4, 5, 6, 7, 8 or more bridged nucleotides (*e.g.*, LNA nucleotides, cEt nucleotides, ENA nucleotides) on each of the 5' and 3' ends of the deoxyribonucleotides. The 3' position of the oligonucleotide may have a 3' hydroxyl group. The 3' position of the oligonucleotide may have a 3' thiophosphate.

The oligonucleotide may be conjugated with a label. For example, the oligonucleotide may be conjugated with a biotin moiety, cholesterol, Vitamin A, folate, sigma receptor ligands, aptamers, peptides, such as CPP, hydrophobic molecules, such as lipids, ASGPR or dynamic polyconjugates and variants thereof at its 5' or 3' end.

Preferably the single stranded oligonucleotide comprises one or more modifications comprising: a modified sugar moiety, and/or a modified internucleoside linkage, and/or a modified nucleotide and/or combinations thereof. It is not necessary for all positions in a given oligonucleotide to be uniformly modified, and in fact more than one of the modifications described herein may be incorporated in a single oligonucleotide or even at within a single nucleoside within an oligonucleotide.

In some embodiments, the single stranded oligonucleotides are chimeric oligonucleotides that contain two or more chemically distinct regions, each made up of at least one nucleotide. These oligonucleotides typically contain at least one region of modified nucleotides that confers one or more beneficial properties (such as, for example, increased nuclease resistance, increased uptake into cells, increased binding affinity for the target) and

a region that is a substrate for enzymes capable of cleaving RNA:DNA or RNA:RNA hybrids. Chimeric single stranded oligonucleotides of the invention may be formed as composite structures of two or more oligonucleotides, modified oligonucleotides, oligonucleosides and/or oligonucleotide mimetics as described above. Such compounds have
5 also been referred to in the art as hybrids or gapmers. Representative United States patents that teach the preparation of such hybrid structures comprise, but are not limited to, US patent nos. 5,013,830; 5,149,797; 5,220,007; 5,256,775; 5,366,878; 5,403,711; 5,491,133; 5,565,350; 5,623,065; 5,652,355; 5,652,356; and 5,700,922, each of which is herein incorporated by reference.

10 In some embodiments, the single stranded oligonucleotide comprises at least one nucleotide modified at the 2' position of the sugar, most preferably a 2'-O-alkyl, 2'-O-alkyl-O-alkyl or 2'-fluoro-modified nucleotide. In other preferred embodiments, RNA modifications include 2'-fluoro, 2'-amino and 2' O-methyl modifications on the ribose of pyrimidines, abasic residues or an inverted base at the 3' end of the RNA. Such modifications are
15 routinely incorporated into oligonucleotides and these oligonucleotides have been shown to have a higher T_m (i.e., higher target binding affinity) than 2'-deoxyoligonucleotides against a given target.

A number of nucleotide and nucleoside modifications have been shown to make the oligonucleotide into which they are incorporated more resistant to nuclease digestion than the
20 native oligodeoxynucleotide; these modified oligos survive intact for a longer time than unmodified oligonucleotides. Specific examples of modified oligonucleotides include those comprising modified backbones, for example, phosphorothioates, phosphotriesters, methyl phosphonates, short chain alkyl or cycloalkyl intersugar linkages or short chain heteroatomic or heterocyclic intersugar linkages. Most preferred are oligonucleotides with
25 phosphorothioate backbones and those with heteroatom backbones, particularly $\text{CH}_2\text{-NH-O-CH}_2$, $\text{CH}_2\text{-N(CH}_3\text{)-O-CH}_2$ (known as a methylene(methylimino) or MMI backbone, $\text{CH}_2\text{-O-N(CH}_3\text{)-CH}_2$, $\text{CH}_2\text{-N(CH}_3\text{)-N(CH}_3\text{)-CH}_2$ and $\text{O-N(CH}_3\text{)-CH}_2\text{-CH}_2$ backbones, wherein the native phosphodiester backbone is represented as O-P-O-CH_2); amide backbones (see De Mesmaeker et al. *Acc. Chem. Res.* 1995, 28:366-374); morpholino
30 backbone structures (see Summerton and Weller, U.S. Pat. No. 5,034,506); peptide nucleic acid (PNA) backbone (wherein the phosphodiester backbone of the oligonucleotide is replaced with a polyamide backbone, the nucleotides being bound directly or indirectly to the

aza nitrogen atoms of the polyamide backbone, see Nielsen et al., Science 1991, 254, 1497). Phosphorus-containing linkages include, but are not limited to, phosphorothioates, chiral phosphorothioates, phosphorodithioates, phosphotriesters, aminoalkylphosphotriesters, methyl and other alkyl phosphonates comprising 3'-alkylene phosphonates and chiral
5 phosphonates, phosphinates, phosphoramidates comprising 3'-amino phosphoramidate and aminoalkylphosphoramidates, thionophosphoramidates, thionoalkylphosphonates, thionoalkylphosphotriesters, and boranophosphates having normal 3'-5' linkages, 2'-5' linked analogs of these, and those having inverted polarity wherein the adjacent pairs of nucleoside units are linked 3'-5' to 5'-3' or 2'-5' to 5'-2'; see US patent nos. 3,687,808; 4,469,863;
10 4,476,301; 5,023,243; 5,177,196; 5,188,897; 5,264,423; 5,276,019; 5,278,302; 5,286,717; 5,321,131; 5,399,676; 5,405,939; 5,453,496; 5,455,233; 5,466,677; 5,476,925; 5,519,126; 5,536,821; 5,541,306; 5,550,111; 5,563,253; 5,571,799; 5,587,361; and 5,625,050.

Morpholino-based oligomeric compounds are described in Dwaine A. Braasch and David R. Corey, Biochemistry, 2002, 41(14), 4503-4510; Genesis, volume 30, issue 3, 2001;
15 Heasman, J., Dev. Biol., 2002, 243, 209-214; Nasevicius et al., Nat. Genet., 2000, 26, 216-220; Lacerra et al., Proc. Natl. Acad. Sci., 2000, 97, 9591-9596; and U.S. Pat. No. 5,034,506, issued Jul. 23, 1991. In some embodiments, the morpholino-based oligomeric compound is a phosphorodiamidate morpholino oligomer (PMO) (e.g., as described in Iverson, Curr. Opin. Mol. Ther., 3:235-238, 2001; and Wang et al., J. Gene Med., 12:354-364, 2010; the
20 disclosures of which are incorporated herein by reference in their entireties).

Cyclohexenyl nucleic acid oligonucleotide mimetics are described in Wang et al., J. Am. Chem. Soc., 2000, 122, 8595-8602.

Modified oligonucleotide backbones that do not include a phosphorus atom therein have backbones that are formed by short chain alkyl or cycloalkyl internucleoside linkages, mixed heteroatom and alkyl or cycloalkyl internucleoside linkages, or one or more short chain heteroatomic or heterocyclic internucleoside linkages. These comprise those having morpholino linkages (formed in part from the sugar portion of a nucleoside); siloxane backbones; sulfide, sulfoxide and sulfone backbones; formacetyl and thioformacetyl backbones; methylene formacetyl and thioformacetyl backbones; alkene containing
30 backbones; sulfamate backbones; methyleneimino and methylenehydrazino backbones; sulfonate and sulfonamide backbones; amide backbones; and others having mixed N, O, S and CH₂ component parts; see US patent nos. 5,034,506; 5,166,315; 5,185,444; 5,214,134;

5,216,141; 5,235,033; 5,264, 562; 5, 264,564; 5,405,938; 5,434,257; 5,466,677; 5,470,967; 5,489,677; 5,541,307; 5,561,225; 5,596, 086; 5,602,240; 5,610,289; 5,602,240; 5,608,046; 5,610,289; 5,618,704; 5,623, 070; 5,663,312; 5,633,360; 5,677,437; and 5,677,439, each of which is herein incorporated by reference.

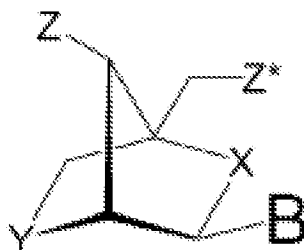
5 Modified oligonucleotides are also known that include oligonucleotides that are based on or constructed from arabinonucleotide or modified arabinonucleotide residues.

Arabinonucleosides are stereoisomers of ribonucleosides, differing only in the configuration at the 2'-position of the sugar ring. In some embodiments, a 2'-arabino modification is 2'-F arabino. In some embodiments, the modified oligonucleotide is 2'-fluoro-D-arabinonucleic acid (FANA) (as described in, for example, Lon et al., *Biochem.*, 41:3457-3467, 2002 and
10 Min et al., *Bioorg. Med. Chem. Lett.*, 12:2651-2654, 2002; the disclosures of which are incorporated herein by reference in their entireties). Similar modifications can also be made at other positions on the sugar, particularly the 3' position of the sugar on a 3' terminal nucleoside or in 2'-5' linked oligonucleotides and the 5' position of 5' terminal nucleotide.

15 PCT Publication No. WO 99/67378 discloses arabinonucleic acids (ANA) oligomers and their analogues for improved sequence specific inhibition of gene expression via association to complementary messenger RNA.

Other preferred modifications include ethylene-bridged nucleic acids (ENAs) (*e.g.*, International Patent Publication No. WO 2005/042777, Morita et al., *Nucleic Acid Res.*,
20 Suppl 1:241-242, 2001; Suroso et al., *Hum. Gene Ther.*, 15:749-757, 2004; Koizumi, *Curr. Opin. Mol. Ther.*, 8:144-149, 2006 and Horie et al., *Nucleic Acids Symp. Ser (Oxf)*, 49:171-172, 2005; the disclosures of which are incorporated herein by reference in their entireties). Preferred ENAs include, but are not limited to, 2'-O,4'-C-ethylene-bridged nucleic acids.

Examples of LNAs are described in WO/2008/043753 and include compounds of the following general formula.



5

where X and Y are independently selected among the groups -O-,

-S-, -N(H)-, N(R)-, -CH₂- or -CH- (if part of a double bond),

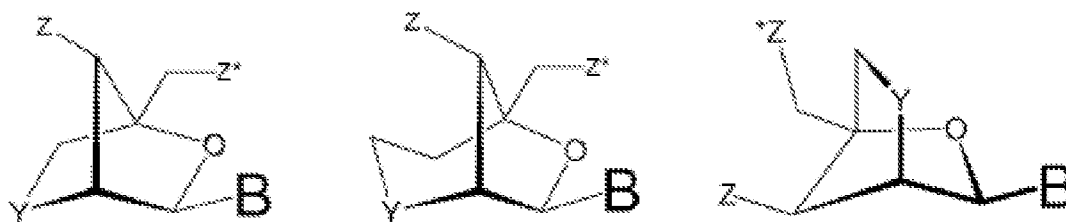
-CH₂-O-, -CH₂-S-, -CH₂-N(H)-, -CH₂-N(R)-, -CH₂-CH₂- or -CH₂-CH- (if part of a double bond),

10

-CH=CH-, where R is selected from hydrogen and C₁₋₄-alkyl; Z and Z* are independently selected among an internucleoside linkage, a terminal group or a protecting group; B constitutes a natural or non-natural nucleotide base moiety; and the asymmetric groups may be found in either orientation.

Preferably, the LNA used in the oligonucleotides described herein comprises at least one LNA unit according any of the formulas

15



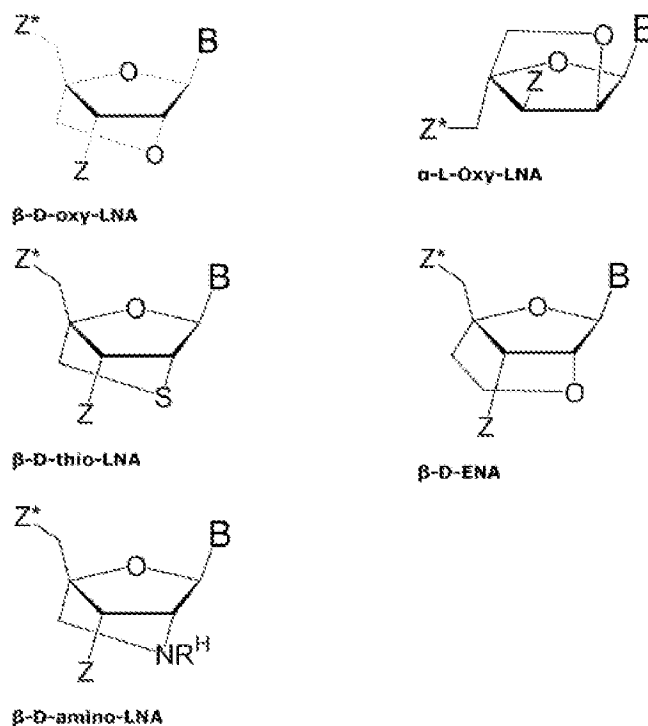
wherein Y is -O-, -S-, -NH-, or N(R^H); Z and Z* are independently selected among an internucleoside linkage, a terminal group or a protecting group; B constitutes a natural or non-natural nucleotide base moiety, and RH is selected from hydrogen and C₁₋₄-alkyl.

20

In some embodiments, the Locked Nucleic Acid (LNA) used in the oligonucleotides described herein comprises at least one Locked Nucleic Acid (LNA) unit according any of the formulas shown in Scheme 2 of PCT/DK2006/000512.

In some embodiments, the LNA used in the oligomer of the invention comprises internucleoside linkages selected from -O-P(O)₂-O-, -O-P(O,S)-O-, -O-P(S)₂-O-, -S-P(O)₂-O-, -S-P(O,S)-O-, -S-P(S)₂-O-, -O-P(O)₂-S-, -O-P(O,S)-S-, -S-P(O)₂-S-, -O-PO(R^H)-O-, O-PO(OCH₃)-O-, -O-PO(NR^H)-O-, -O-PO(OCH₂CH₂S-R)-O-, -O-PO(BH₃)-O-, -O-PO(NHR^H)-O-, -O-P(O)₂-NR^H-, -NR^H-P(O)₂-O-, -NR^H-CO-O-, where R^H is selected from hydrogen and C₁₋₄-alkyl.

Specifically preferred LNA units are shown in scheme 2:



Scheme 2

The term "thio-LNA" comprises a locked nucleotide in which at least one of X or Y in the general formula above is selected from S or -CH₂-S-. Thio-LNA can be in both beta-D and alpha-L-configuration.

The term "amino-LNA" comprises a locked nucleotide in which at least one of X or Y in the general formula above is selected from -N(H)-, N(R)-, CH₂-N(H)-, and -CH₂-N(R)-

where R is selected from hydrogen and C₁₋₄-alkyl. Amino-LNA can be in both beta-D and alpha-L-configuration.

The term "oxy-LNA" comprises a locked nucleotide in which at least one of X or Y in the general formula above represents -O- or -CH₂-O-. Oxy-LNA can be in both beta-D and
5 alpha-L-configuration.

The term "ena-LNA" comprises a locked nucleotide in which Y in the general formula above is -CH₂-O- (where the oxygen atom of -CH₂-O- is attached to the 2'-position relative to the base B).

LNAs are described in additional detail herein.

10 One or more substituted sugar moieties can also be included, *e.g.*, one of the following at the 2' position: OH, SH, SCH₃, F, OCN, OCH₃, OCH₃, OCH₃ O(CH₂)_n CH₃, O(CH₂)_n NH₂ or O(CH₂)_n CH₃ where n is from 1 to about 10; C₁ to C₁₀ lower alkyl, alkoxyalkoxy, substituted lower alkyl, alkaryl or aralkyl; Cl; Br; CN; CF₃; OCF₃; O-, S-, or N-alkyl; O-, S-, or N-alkenyl; SOCH₃; SO₂ CH₃; ONO₂; NO₂; N₃; NH₂; heterocycloalkyl; heterocycloalkaryl; aminoalkylamino; polyalkylamino; substituted silyl; an RNA cleaving
15 group; a reporter group; an intercalator; a group for improving the pharmacokinetic properties of an oligonucleotide; or a group for improving the pharmacodynamic properties of an oligonucleotide and other substituents having similar properties. A preferred modification includes 2'-methoxyethoxy [2'-O-CH₂CH₂OCH₃, also known as 2'-O-(2-methoxyethyl)]
20 (Martin et al, *Helv. Chim. Acta*, 1995, 78, 486). Other preferred modifications include 2'-methoxy (2'-O-CH₃), 2'-propoxy (2'-OCH₂ CH₂CH₃) and 2'-fluoro (2'-F). Similar modifications may also be made at other positions on the oligonucleotide, particularly the 3' position of the sugar on the 3' terminal nucleotide and the 5' position of 5' terminal nucleotide. Oligonucleotides may also have sugar mimetics such as cyclobutyls in place of
25 the pentofuranosyl group.

Single stranded oligonucleotides can also include, additionally or alternatively, nucleobase (often referred to in the art simply as "base") modifications or substitutions. As used herein, "unmodified" or "natural" nucleobases include adenine (A), guanine (G), thymine (T), cytosine (C) and uracil (U). Modified nucleobases include nucleobases found
30 only infrequently or transiently in natural nucleic acids, *e.g.*, hypoxanthine, 6-methyladenine, 5-Me pyrimidines, particularly 5-methylcytosine (also referred to as 5-methyl-2'

deoxycytosine and often referred to in the art as 5-Me-C), 5-hydroxymethylcytosine (HMC), glycosyl HMC and gentobiosyl HMC, isocytosine, pseudoisocytosine, as well as synthetic nucleobases, *e.g.*, 2-aminoadenine, 2- (methylamino)adenine, 2-(imidazolylalkyl)adenine, 2- (aminoalkylamino)adenine or other heterosubstituted alkyladenines, 2-thiouracil, 2-
5 thiothymine, 5-bromouracil, 5-hydroxymethyluracil, 5-propynyluracil, 8-azaguanine, 7-deazaguanine, N6 (6-aminohexyl)adenine, 6-aminopurine, 2-aminopurine, 2-chloro-6-aminopurine and 2,6-diaminopurine or other diaminopurines. See, *e.g.*, Kornberg, "DNA Replication," W. H. Freeman & Co., San Francisco, 1980, pp75-77; and Gebeyehu, G., et al. Nucl. Acids Res., 15:4513 (1987)). A "universal" base known in the art, *e.g.*, inosine, can
10 also be included. 5-Me-C substitutions have been shown to increase nucleic acid duplex stability by 0.6-1.2°C. (Sanghvi, in Crooke, and Lebleu, eds., Antisense Research and Applications, CRC Press, Boca Raton, 1993, pp. 276-278) and may be used as base substitutions.

It is not necessary for all positions in a given oligonucleotide to be uniformly
15 modified, and in fact more than one of the modifications described herein may be incorporated in a single oligonucleotide or even at within a single nucleoside within an oligonucleotide.

In some embodiments, both a sugar and an internucleoside linkage, *i.e.*, the backbone, of the nucleotide units are replaced with novel groups. The base units are maintained for
20 hybridization with an appropriate nucleic acid target compound. One such oligomeric compound, an oligonucleotide mimetic that has been shown to have excellent hybridization properties, is referred to as a peptide nucleic acid (PNA). In PNA compounds, the sugar-backbone of an oligonucleotide is replaced with an amide containing backbone, for example, an aminoethylglycine backbone. The nucleobases are retained and are bound directly or
25 indirectly to aza nitrogen atoms of the amide portion of the backbone. Representative United States patents that teach the preparation of PNA compounds include, but are not limited to, US patent nos. 5,539,082; 5,714,331; and 5,719,262, each of which is herein incorporated by reference. Further teaching of PNA compounds can be found in Nielsen et al, Science, 1991, 254, 1497-1500.

30 Single stranded oligonucleotides can also include one or more nucleobase (often referred to in the art simply as "base") modifications or substitutions. As used herein, "unmodified" or "natural" nucleobases comprise the purine bases adenine (A) and guanine

(G), and the pyrimidine bases thymine (T), cytosine (C) and uracil (U). Modified nucleobases comprise other synthetic and natural nucleobases such as 5-methylcytosine (5-me-C), 5-hydroxymethyl cytosine, xanthine, hypoxanthine, 2-aminoadenine, 6-methyl and other alkyl derivatives of adenine and guanine, 2-propyl and other alkyl derivatives of adenine and guanine, 2-thiouracil, 2-thiothymine and 2-thiocytosine, 5-halouracil and cytosine, 5-propynyl uracil and cytosine, 6-azo uracil, cytosine and thymine, 5-uracil (pseudo-uracil), 4-thiouracil, 8-halo, 8-amino, 8-thiol, 8- thioalkyl, 8-hydroxyl and other 8-substituted adenines and guanines, 5-halo particularly 5- bromo, 5-trifluoromethyl and other 5-substituted uracils and cytosines, 7-methylguanine and 7-methyladenine, 8-azaguanine and 8-azaadenine, 7-deazaguanine and 7-deazaadenine and 3- deazaguanine and 3-deazaadenine.

Further, nucleobases comprise those disclosed in United States Patent No. 3,687,808, those disclosed in "The Concise Encyclopedia of Polymer Science And Engineering", pages 858-859, Kroschwitz, ed. John Wiley & Sons, 1990;, those disclosed by Englisch et al., Angewandte Chemie, International Edition, 1991, 30, page 613, and those disclosed by Sanghvi, Chapter 15, Antisense Research and Applications," pages 289- 302, Crooke, and Lebleu, eds., CRC Press, 1993. Certain of these nucleobases are particularly useful for increasing the binding affinity of the oligomeric compounds of the invention. These include 5-substituted pyrimidines, 6-azapyrimidines and N-2, N-6 and O-6 substituted purines, comprising 2-aminopropyladenine, 5-propynyluracil and 5- propynylcytosine. 5-methylcytosine substitutions have been shown to increase nucleic acid duplex stability by 0.6-1.2°C (Sanghvi, et al., eds, "Antisense Research and Applications," CRC Press, Boca Raton, 1993, pp. 276-278) and are presently preferred base substitutions, even more particularly when combined with 2'-O-methoxyethyl sugar modifications. Modified nucleobases are described in US patent nos. 3,687,808, as well as 4,845,205; 5,130,302; 5,134,066; 5,175, 273; 5, 367,066; 5,432,272; 5,457,187; 5,459,255; 5,484,908; 5,502,177; 5,525,711; 5,552,540; 5,587,469; 5,596,091; 5,614,617; 5,750,692, and 5,681,941, each of which is herein incorporated by reference.

In some embodiments, the single stranded oligonucleotides are chemically linked to one or more moieties or conjugates that enhance the activity, cellular distribution, or cellular uptake of the oligonucleotide. For example, one or more single stranded oligonucleotides, of the same or different types, can be conjugated to each other; or single stranded oligonucleotides can be conjugated to targeting moieties with enhanced specificity for a cell

type or tissue type. Such moieties include, but are not limited to, lipid moieties such as a cholesterol moiety (Letsinger et al., Proc. Natl. Acad. Sci. USA, 1989, 86, 6553-6556), cholic acid (Manoharan et al., Bioorg. Med. Chem. Lett., 1994, 4, 1053-1060), a thioether, *e.g.*, hexyl-S- tritylthiol (Manoharan et al, Ann. N. Y. Acad. Sci., 1992, 660, 306-309; Manoharan et al., Bioorg. Med. Chem. Lett., 1993, 3, 2765-2770), a thiocholesterol (Oberhauser et al., Nucl. Acids Res., 1992, 20, 533-538), an aliphatic chain, *e.g.*, dodecandiol or undecyl residues (Kabanov et al., FEBS Lett., 1990, 259, 327-330; Svinarchuk et al., Biochimie, 1993, 75, 49- 54), a phospholipid, *e.g.*, di-hexadecyl-rac-glycerol or triethylammonium 1,2-di-O-hexadecyl- rac-glycero-3-H-phosphonate (Manoharan et al., Tetrahedron Lett., 1995, 36, 3651-3654; Shea et al., Nucl. Acids Res., 1990, 18, 3777-3783), a polyamine or a polyethylene glycol chain (Mancharan et al., Nucleosides & Nucleotides, 1995, 14, 969-973), or adamantane acetic acid (Manoharan et al., Tetrahedron Lett., 1995, 36, 3651-3654), a palmityl moiety (Mishra et al., Biochim. Biophys. Acta, 1995, 1264, 229-237), or an octadecylamine or hexylamino-carbonyl-t oxycholesterol moiety (Crooke et al., J. Pharmacol. Exp. Ther., 1996, 277, 923-937). See also US patent nos. 4,828,979; 4,948,882; 5,218,105; 5,525,465; 5,541,313; 5,545,730; 5,552, 538; 5,578,717; 5,580,731; 5,580,731; 5,591,584; 5,109,124; 5,118,802; 5,138,045; 5,414,077; 5,486, 603; 5,512,439; 5,578,718; 5,608,046; 4,587,044; 4,605,735; 4,667,025; 4,762, 779; 4,789,737; 4,824,941; 4,835,263; 4,876,335; 4,904,582; 4,958,013; 5,082, 830; 5,112,963; 5,214,136; 5,082,830; 5,112,963; 5,214,136; 5, 245,022; 5,254,469; 5,258,506; 5,262,536; 5,272,250; 5,292,873; 5,317,098; 5,371,241, 5,391, 723; 5,416,203, 5,451,463; 5,510,475; 5,512,667; 5,514,785; 5, 565,552; 5,567,810; 5,574,142; 5,585,481; 5,587,371; 5,595,726; 5,597,696; 5,599,923; 5,599, 928 and 5,688,941, each of which is herein incorporated by reference.

These moieties or conjugates can include conjugate groups covalently bound to functional groups such as primary or secondary hydroxyl groups. Conjugate groups of the invention include intercalators, reporter molecules, polyamines, polyamides, polyethylene glycols, polyethers, groups that enhance the pharmacodynamic properties of oligomers, and groups that enhance the pharmacokinetic properties of oligomers. Typical conjugate groups include cholesterol, lipids, phospholipids, biotin, phenazine, folate, phenanthridine, anthraquinone, acridine, fluoresceins, rhodamines, coumarins, and dyes. Groups that enhance the pharmacodynamic properties, in the context of this invention, include groups that improve uptake, enhance resistance to degradation, and/or strengthen sequence-specific hybridization

with the target nucleic acid. Groups that enhance the pharmacokinetic properties, in the context of this invention, include groups that improve uptake, distribution, metabolism or excretion of the compounds of the present invention. Representative conjugate groups are disclosed in International Patent Application No. PCT/US92/09196, filed Oct. 23, 1992, and U.S. Pat. No. 6,287,860, which are incorporated herein by reference. Conjugate moieties include, but are not limited to, lipid moieties such as a cholesterol moiety, cholic acid, a thioether, *e.g.*, hexyl-5-tritylthiol, a thiocholesterol, an aliphatic chain, *e.g.*, dodecandiol or undecyl residues, a phospholipid, *e.g.*, di-hexadecyl-rac- glycerol or triethylammonium 1,2-di-O-hexadecyl-rac-glycero-3-H-phosphonate, a polyamine or a polyethylene glycol chain, or adamantane acetic acid, a palmityl moiety, or an octadecylamine or hexylamino-carbonyl-oxy cholesterol moiety. See, *e.g.*, U.S. Pat. Nos. 4,828,979; 4,948,882; 5,218,105; 5,525,465; 5,541,313; 5,545,730; 5,552,538; 5,578,717; 5,580,731; 5,580,731; 5,591,584; 5,109,124; 5,118,802; 5,138,045; 5,414,077; 5,486,603; 5,512,439; 5,578,718; 5,608,046; 4,587,044; 4,605,735; 4,667,025; 4,762,779; 4,789,737; 4,824,941; 4,835,263; 4,876,335; 4,904,582; 4,958,013; 5,082,830; 5,112,963; 5,214,136; 5,082,830; 5,112,963; 5,214,136; 5,245,022; 5,254,469; 5,258,506; 5,262,536; 5,272,250; 5,292,873; 5,317,098; 5,371,241; 5,391,723; 5,416,203; 5,451,463; 5,510,475; 5,512,667; 5,514,785; 5,565,552; 5,567,810; 5,574,142; 5,585,481; 5,587,371; 5,595,726; 5,597,696; 5,599,923; 5,599,928 and 5,688,941.

In some embodiments, single stranded oligonucleotide modification include modification of the 5' or 3' end of the oligonucleotide. In some embodiments, the 3' end of the oligonucleotide comprises a hydroxyl group or a thiophosphate. It should be appreciated that additional molecules (*e.g.* a biotin moiety or a fluorophor) can be conjugated to the 5' or 3' end of the single stranded oligonucleotide. In some embodiments, the single stranded oligonucleotide comprises a biotin moiety conjugated to the 5' nucleotide.

In some embodiments, the single stranded oligonucleotide comprises locked nucleic acids (LNA), ENA modified nucleotides, 2'-O-methyl nucleotides, or 2'-fluoro-deoxyribonucleotides. In some embodiments, the single stranded oligonucleotide comprises alternating deoxyribonucleotides and 2'-fluoro-deoxyribonucleotides. In some embodiments, the single stranded oligonucleotide comprises alternating deoxyribonucleotides and 2'-O-methyl nucleotides. In some embodiments, the single stranded oligonucleotide comprises alternating deoxyribonucleotides and ENA modified nucleotides. In some embodiments, the single stranded oligonucleotide comprises alternating deoxyribonucleotides and locked

nucleic acid nucleotides. In some embodiments, the single stranded oligonucleotide comprises alternating locked nucleic acid nucleotides and 2'-O-methyl nucleotides.

In some embodiments, the 5' nucleotide of the oligonucleotide is a deoxyribonucleotide. In some embodiments, the 5' nucleotide of the oligonucleotide is a locked nucleic acid nucleotide. In some embodiments, the nucleotides of the oligonucleotide comprise deoxyribonucleotides flanked by at least one locked nucleic acid nucleotide on each of the 5' and 3' ends of the deoxyribonucleotides. In some embodiments, the nucleotide at the 3' position of the oligonucleotide has a 3' hydroxyl group or a 3' thiophosphate.

In some embodiments, the single stranded oligonucleotide comprises phosphorothioate internucleotide linkages. In some embodiments, the single stranded oligonucleotide comprises phosphorothioate internucleotide linkages between at least two nucleotides. In some embodiments, the single stranded oligonucleotide comprises phosphorothioate internucleotide linkages between all nucleotides.

It should be appreciated that the single stranded oligonucleotide can have any combination of modifications as described herein.

The oligonucleotide may comprise a nucleotide sequence having one or more of the following modification patterns.

(a) (X)XXXXXX, (X)xXXXX, (X)xxXXX, (X)xxxXX, (X)xxxxX and (X)xxxxX,

(b) (X)XXXXXX, (X)XxXXXX, (X)XxxXX, (X)XxxxX, (X)XxxxxX, (X)xXXxxx, (X)xXxXxx, (X)xXxxX, (X)xxXXX, (X)xxXXxx, (X)xxXxX, (X)xxXxxX, (X)xxxXXx, (X)xxxXxX and (X)xxxxXX,

(c) (X)XXXxxx, (X)xXXXxx, (X)xxXXXx, (X)xxxXXX, (X)XXxXxx, (X)XXxxXx, (X)XXxxxX, (X)xXXxXx, (X)xXXxxX, (X)xxXXxX, (X)XxXXxx, (X)XxxXXx, (X)XxxxXX, (X)xXxXXx, (X)xXxxXX, (X)xxXxXX, (X)xXxXxX and (X)XxXxXx,

(d) (X)xxXXX, (X)xXxXXX, (X)xXXxXX, (X)xXXXxX, (X)xXXXXx, (X)XxxXXXX, (X)XxXxXX, (X)XxXXxX, (X)XxXXx, (X)XXxxXX, (X)XXxXxX, (X)XXxXXx, (X)XXXxxX, (X)XXXxXx, and (X)XXXXxx,

(e) (X)xxxxxx, (X)Xxxxxx, (X)XXxxxX, (X)XXXxXX, (X)XXXXxX and (X)XXXXxx, and

(f) XXXXXX, Xxxxxx, XXxxxx, XXXxxx, XXXxXX, XXXXXx, XXXXXxX and XXXXXXx, in which "X" denotes a nucleotide analogue, (X) denotes an optional nucleotide analogue, and "x" denotes a DNA or RNA nucleotide unit. Each of the above listed patterns

may appear one or more times within an oligonucleotide, alone or in combination with any of the other disclosed modification patterns.

Methods for Modulating Gene Expression

5 In some embodiments, methods are provided for increasing expression of SMN protein in a cell. The methods, in some embodiments, involve delivering to the cell a first single stranded oligonucleotide complementary with a PRC2-associated region of SMN1 or SMN2 and a second single stranded oligonucleotide complementary with a splice control sequence of a precursor mRNA of SMN1 or SMN2, in amounts sufficient to increase
10 expression of a mature mRNA of SMN1 or SMN2 that comprises (or includes) exon 7 in the cell. The first and second single stranded oligonucleotides may be delivered together or separately. The first and second single stranded oligonucleotides may be linked together, or unlinked, i.e., separate.

15 In some embodiments, methods are provided for treating spinal muscular atrophy in a subject. The methods, in some embodiments, involve administering to a subject a first single stranded oligonucleotide complementary with a PRC2-associated region of SMN1 or SMN2 and a second single stranded oligonucleotide complementary with a splice control sequence of a precursor mRNA of SMN1 or SMN2, in amounts sufficient to increase expression of full length SMN protein in the subject to levels sufficient to improve one or more conditions
20 associated with SMA. The first and second single stranded oligonucleotides may be administered together or separately. The first and second single stranded oligonucleotides may be linked together, or unlinked, i.e., separate. The first single stranded oligonucleotide may be administered within 1 hour, 2 hours, 3 hours, 4 hours, 8 hours, 12 hours, 24 hours, 48 hours or more of administration of the second single stranded oligonucleotide. The first
25 single stranded oligonucleotide may be administered before or after the second single stranded oligonucleotide. The oligonucleotides may be administered once or on multiple occasions depending on the needs of the subject and/or judgment of the treating physician. In some cases, the oligonucleotides may be administered in cycles. The administration cycles may vary; for example, the administration cycle may be 2nd oligo - 1st oligo - 2nd oligo - 1st
30 oligo and so on; or 1st oligo-2nd oligo-1st oligo-2nd oligo, and so on; or 1st oligo - 2nd oligo - 2nd oligo -1st oligo-1st oligo - 2nd oligo - 2nd oligo -1st oligo, and so on. The skilled artisan will

be capable of selecting administration cycles and intervals between each administration that are appropriate for treating a particular subject.

In one aspect, the invention relates to methods for modulating gene expression in a cell (*e.g.*, a cell for which SMN1 or SMN2 levels are reduced) for research purposes (*e.g.*, to study the function of the gene in the cell). In another aspect, the invention relates to methods for modulating gene expression in a cell (*e.g.*, a cell for which SMN1 or SMN2 levels are reduced) for gene or epigenetic therapy. The cells can be *in vitro*, *ex vivo*, or *in vivo* (*e.g.*, in a subject who has a disease resulting from reduced expression or activity of SMN1 or SMN2). In some embodiments, methods for modulating gene expression in a cell comprise delivering a single stranded oligonucleotide as described herein. In some embodiments, delivery of the single stranded oligonucleotide to the cell results in a level of expression of gene that is at least 5%, 10%, 20%, 30%, 40%, 50%, 60%, 70%, 80%, 90%, 100%, 200% or more greater than a level of expression of gene in a control cell to which the single stranded oligonucleotide has not been delivered. In certain embodiments, delivery of the single stranded oligonucleotide to the cell results in a level of expression of gene that is at least 50% greater than a level of expression of gene in a control cell to which the single stranded oligonucleotide has not been delivered.

In another aspect of the invention, methods comprise administering to a subject (*e.g.* a human) a composition comprising a single stranded oligonucleotide as described herein to increase protein levels in the subject. In some embodiments, the increase in protein levels is at least 5%, 10%, 20%, 30%, 40%, 50%, 60%, 70%, 80%, 90%, 100%, 200%, or more, higher than the amount of a protein in the subject before administering.

As another example, to increase expression of SMN1 or SMN2 in a cell, the methods include introducing into the cell a single stranded oligonucleotide that is sufficiently complementary to a PRC2-associated region (*e.g.*, of a long non-coding RNA) that maps to a genomic position encompassing or in proximity to the SMN1 or SMN2 gene.

In another aspect of the invention provides methods of treating a condition (*e.g.*, Spinal muscular atrophy) associated with decreased levels of expression of SMN1 or SMN2 in a subject, the method comprising administering a single stranded oligonucleotide as described herein.

A subject can include a non-human mammal, *e.g.* mouse, rat, guinea pig, rabbit, cat, dog, goat, cow, or horse. In preferred embodiments, a subject is a human. Single stranded

oligonucleotides have been employed as therapeutic moieties in the treatment of disease states in animals, including humans. Single stranded oligonucleotides can be useful therapeutic modalities that can be configured to be useful in treatment regimes for the treatment of cells, tissues and animals, especially humans.

5 For therapeutics, an animal, preferably a human, suspected of having Spinal muscular atrophy is treated by administering single stranded oligonucleotide in accordance with this invention. For example, in one non-limiting embodiment, the methods comprise the step of administering to the animal in need of treatment, a therapeutically effective amount of a single stranded oligonucleotide as described herein.

10 *Formulation, Delivery, And Dosing*

The oligonucleotides described herein can be formulated for administration to a subject for treating a condition (*e.g.*, Spinal muscular atrophy) associated with decreased levels of SMN protein. It should be understood that the formulations, compositions and methods can be practiced with any of the oligonucleotides disclosed herein. In some 15 embodiments, formulations are provided that comprise a first single stranded oligonucleotide complementary with a PRC2-associated region of a gene and a second single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA of the gene. In some embodiments, formulations are provided that comprise a first single stranded 20 oligonucleotide complementary with a PRC2-associated region of a gene that is linked via a linker with a second single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA of the gene. Thus, it should be appreciated that in some embodiments, a first single stranded oligonucleotide complementary with a PRC2-associated region of a gene is linked with a second single stranded oligonucleotide complementary to a 25 splice control sequence of a precursor mRNA of the gene, and in other embodiments, the single stranded oligonucleotides are not linked. Single stranded oligonucleotides that are not linked may be administered to a subject or delivered to a cell simultaneously (*e.g.*, within the same composition) or separately.

The formulations may conveniently be presented in unit dosage form and may be 30 prepared by any methods well known in the art of pharmacy. The amount of active ingredient (*e.g.*, an oligonucleotide or compound of the invention) which can be combined with a carrier material to produce a single dosage form will vary depending upon the host

being treated, the particular mode of administration, *e.g.*, intradermal or inhalation. The amount of active ingredient which can be combined with a carrier material to produce a single dosage form will generally be that amount of the compound which produces a therapeutic effect, *e.g.* tumor regression.

5 Pharmaceutical formulations of this invention can be prepared according to any method known to the art for the manufacture of pharmaceuticals. Such formulations can contain sweetening agents, flavoring agents, coloring agents and preserving agents. A formulation can be admixed with nontoxic pharmaceutically acceptable excipients which are suitable for manufacture. Formulations may comprise one or more diluents, emulsifiers,
10 preservatives, buffers, excipients, etc. and may be provided in such forms as liquids, powders, emulsions, lyophilized powders, sprays, creams, lotions, controlled release formulations, tablets, pills, gels, on patches, in implants, etc.

 A formulated single stranded oligonucleotide composition can assume a variety of states. In some examples, the composition is at least partially crystalline, uniformly
15 crystalline, and/or anhydrous (*e.g.*, less than 80, 50, 30, 20, or 10% water). In another example, the single stranded oligonucleotide is in an aqueous phase, *e.g.*, in a solution that includes water. The aqueous phase or the crystalline compositions can, *e.g.*, be incorporated into a delivery vehicle, *e.g.*, a liposome (particularly for the aqueous phase) or a particle (*e.g.*, a microparticle as can be appropriate for a crystalline composition). Generally, the single
20 stranded oligonucleotide composition is formulated in a manner that is compatible with the intended method of administration.

 In some embodiments, the composition is prepared by at least one of the following methods: spray drying, lyophilization, vacuum drying, evaporation, fluid bed drying, or a combination of these techniques; or sonication with a lipid, freeze-drying, condensation and
25 other self-assembly.

 A single stranded oligonucleotide preparation can be formulated or administered (together or separately) in combination with another agent, *e.g.*, another therapeutic agent or an agent that stabilizes a single stranded oligonucleotide, *e.g.*, a protein that complexes with single stranded oligonucleotide. Still other agents include chelators, *e.g.*, EDTA (*e.g.*, to
30 remove divalent cations such as Mg^{2+}), salts, RNase inhibitors (*e.g.*, a broad specificity RNase inhibitor such as RNasin) and so forth. In some embodiments, the other agent used in combination with the single stranded oligonucleotide is an agent that also regulates SMN

expression. In some embodiments, the other agent is a growth hormone, a histone deacetylase inhibitor, a hydroxycarbamide (hydroxyurea), a natural polyphenol compound (e.g., resveratrol, curcumin), prolactin, or salbutamol. Examples of histone deacetylase inhibitors that may be used include aliphatic compounds (e.g., butyrates (e.g., sodium butyrate and sodium phenylbutyrate) and valproic acid), benzamides (e.g., M344), and hydroxamic acids (e.g., CBHA, SBHA, Entinostat (MS-275)) Panobinostat (LBH-589), Trichostatin A, Vorinostat (SAHA)),

In one embodiment, the single stranded oligonucleotide preparation includes another single stranded oligonucleotide, e.g., a second single stranded oligonucleotide that modulates expression and/or mRNA processing of a second gene or a second single stranded oligonucleotide that modulates expression of the first gene. Still other preparation can include at least 3, 5, ten, twenty, fifty, or a hundred or more different single stranded oligonucleotide species. Such single stranded oligonucleotides can mediated gene expression with respect to a similar number of different genes. In one embodiment, the single stranded oligonucleotide preparation includes at least a second therapeutic agent (e.g., an agent other than an oligonucleotide).

Route of Delivery

A composition that includes a single stranded oligonucleotide can be delivered to a subject by a variety of routes. Exemplary routes include: intravenous, intradermal, topical, rectal, parenteral, anal, intravaginal, intranasal, pulmonary, ocular. The term "therapeutically effective amount" is the amount of oligonucleotide present in the composition that is needed to provide the desired level of SMN1 or SMN2 expression in the subject to be treated to give the anticipated physiological response. The term "physiologically effective amount" is that amount delivered to a subject to give the desired palliative or curative effect. The term "pharmaceutically acceptable carrier" means that the carrier can be administered to a subject with no significant adverse toxicological effects to the subject.

The single stranded oligonucleotide molecules of the invention can be incorporated into pharmaceutical compositions suitable for administration. Such compositions typically include one or more species of single stranded oligonucleotide and a pharmaceutically acceptable carrier. As used herein the language "pharmaceutically acceptable carrier" is intended to include any and all solvents, dispersion media, coatings, antibacterial and

antifungal agents, isotonic and absorption delaying agents, and the like, compatible with pharmaceutical administration. The use of such media and agents for pharmaceutically active substances is well known in the art. Except insofar as any conventional media or agent is incompatible with the active compound, use thereof in the compositions is contemplated.

5 Supplementary active compounds can also be incorporated into the compositions.

The pharmaceutical compositions of the present invention may be administered in a number of ways depending upon whether local or systemic treatment is desired and upon the area to be treated. Administration may be topical (including ophthalmic, vaginal, rectal, intranasal, transdermal), oral or parenteral. Parenteral administration includes intravenous
10 drip, subcutaneous, intraperitoneal or intramuscular injection, or intrathecal or intraventricular administration.

The route and site of administration may be chosen to enhance targeting. For example, to target muscle cells, intramuscular injection into the muscles of interest would be a logical choice. Lung cells might be targeted by administering the single stranded
15 oligonucleotide in aerosol form. The vascular endothelial cells could be targeted by coating a balloon catheter with the single stranded oligonucleotide and mechanically introducing the oligonucleotide.

Topical administration refers to the delivery to a subject by contacting the formulation directly to a surface of the subject. The most common form of topical delivery is to the skin,
20 but a composition disclosed herein can also be directly applied to other surfaces of the body, *e.g.*, to the eye, a mucous membrane, to surfaces of a body cavity or to an internal surface. As mentioned above, the most common topical delivery is to the skin. The term encompasses several routes of administration including, but not limited to, topical and transdermal. These modes of administration typically include penetration of the skin's permeability barrier and
25 efficient delivery to the target tissue or stratum. Topical administration can be used as a means to penetrate the epidermis and dermis and ultimately achieve systemic delivery of the composition. Topical administration can also be used as a means to selectively deliver oligonucleotides to the epidermis or dermis of a subject, or to specific strata thereof, or to an underlying tissue.

30 Formulations for topical administration may include transdermal patches, ointments, lotions, creams, gels, drops, suppositories, sprays, liquids and powders. Conventional

pharmaceutical carriers, aqueous, powder or oily bases, thickeners and the like may be necessary or desirable. Coated condoms, gloves and the like may also be useful.

Transdermal delivery is a valuable route for the administration of lipid soluble therapeutics. The dermis is more permeable than the epidermis and therefore absorption is much more rapid through abraded, burned or denuded skin. Inflammation and other physiologic conditions that increase blood flow to the skin also enhance transdermal adsorption. Absorption via this route may be enhanced by the use of an oily vehicle (inunction) or through the use of one or more penetration enhancers. Other effective ways to deliver a composition disclosed herein via the transdermal route include hydration of the skin and the use of controlled release topical patches. The transdermal route provides a potentially effective means to deliver a composition disclosed herein for systemic and/or local therapy. In addition, iontophoresis (transfer of ionic solutes through biological membranes under the influence of an electric field), phonophoresis or sonophoresis (use of ultrasound to enhance the absorption of various therapeutic agents across biological membranes, notably the skin and the cornea), and optimization of vehicle characteristics relative to dose position and retention at the site of administration may be useful methods for enhancing the transport of topically applied compositions across skin and mucosal sites.

Both the oral and nasal membranes offer advantages over other routes of administration. For example, oligonucleotides administered through these membranes may have a rapid onset of action, provide therapeutic plasma levels, avoid first pass effect of hepatic metabolism, and avoid exposure of the oligonucleotides to the hostile gastrointestinal (GI) environment. Additional advantages include easy access to the membrane sites so that the oligonucleotide can be applied, localized and removed easily.

In oral delivery, compositions can be targeted to a surface of the oral cavity, *e.g.*, to sublingual mucosa which includes the membrane of ventral surface of the tongue and the floor of the mouth or the buccal mucosa which constitutes the lining of the cheek. The sublingual mucosa is relatively permeable thus giving rapid absorption and acceptable bioavailability of many agents. Further, the sublingual mucosa is convenient, acceptable and easily accessible.

A pharmaceutical composition of single stranded oligonucleotide may also be administered to the buccal cavity of a human being by spraying into the cavity, without inhalation, from a metered dose spray dispenser, a mixed micellar pharmaceutical

formulation as described above and a propellant. In one embodiment, the dispenser is first shaken prior to spraying the pharmaceutical formulation and propellant into the buccal cavity.

Compositions for oral administration include powders or granules, suspensions or solutions in water, syrups, slurries, emulsions, elixirs or non-aqueous media, tablets, capsules, lozenges, or troches. In the case of tablets, carriers that can be used include lactose, sodium citrate and salts of phosphoric acid. Various disintegrants such as starch, and lubricating agents such as magnesium stearate, sodium lauryl sulfate and talc, are commonly used in tablets. For oral administration in capsule form, useful diluents are lactose and high molecular weight polyethylene glycols. When aqueous suspensions are required for oral use, the nucleic acid compositions can be combined with emulsifying and suspending agents. If desired, certain sweetening and/or flavoring agents can be added.

Parenteral administration includes intravenous drip, subcutaneous, intraperitoneal or intramuscular injection, intrathecal or intraventricular administration. In some embodiments, parental administration involves administration directly to the site of disease (*e.g.* injection into a tumor).

Formulations for parenteral administration may include sterile aqueous solutions which may also contain buffers, diluents and other suitable additives. Intraventricular injection may be facilitated by an intraventricular catheter, for example, attached to a reservoir. For intravenous use, the total concentration of solutes should be controlled to render the preparation isotonic.

Any of the single stranded oligonucleotides described herein can be administered to ocular tissue. For example, the compositions can be applied to the surface of the eye or nearby tissue, *e.g.*, the inside of the eyelid. For ocular administration, ointments or droppable liquids may be delivered by ocular delivery systems known to the art such as applicators or eye droppers. Such compositions can include mucomimetics such as hyaluronic acid, chondroitin sulfate, hydroxypropyl methylcellulose or poly(vinyl alcohol), preservatives such as sorbic acid, EDTA or benzylchromium chloride, and the usual quantities of diluents and/or carriers. The single stranded oligonucleotide can also be administered to the interior of the eye, and can be introduced by a needle or other delivery device which can introduce it to a selected area or structure.

Pulmonary delivery compositions can be delivered by inhalation by the patient of a dispersion so that the composition, preferably single stranded oligonucleotides, within the

dispersion can reach the lung where it can be readily absorbed through the alveolar region directly into blood circulation. Pulmonary delivery can be effective both for systemic delivery and for localized delivery to treat diseases of the lungs.

Pulmonary delivery can be achieved by different approaches, including the use of nebulized, aerosolized, micellular and dry powder-based formulations. Delivery can be achieved with liquid nebulizers, aerosol-based inhalers, and dry powder dispersion devices. Metered-dose devices are preferred. One of the benefits of using an atomizer or inhaler is that the potential for contamination is minimized because the devices are self-contained. Dry powder dispersion devices, for example, deliver agents that may be readily formulated as dry powders. A single stranded oligonucleotide composition may be stably stored as lyophilized or spray-dried powders by itself or in combination with suitable powder carriers. The delivery of a composition for inhalation can be mediated by a dosing timing element which can include a timer, a dose counter, time measuring device, or a time indicator which when incorporated into the device enables dose tracking, compliance monitoring, and/or dose triggering to a patient during administration of the aerosol medicament.

The term "powder" means a composition that consists of finely dispersed solid particles that are free flowing and capable of being readily dispersed in an inhalation device and subsequently inhaled by a subject so that the particles reach the lungs to permit penetration into the alveoli. Thus, the powder is said to be "respirable." Preferably the average particle size is less than about 10 μm in diameter preferably with a relatively uniform spheroidal shape distribution. More preferably the diameter is less than about 7.5 μm and most preferably less than about 5.0 μm . Usually the particle size distribution is between about 0.1 μm and about 5 μm in diameter, particularly about 0.3 μm to about 5 μm .

The term "dry" means that the composition has a moisture content below about 10% by weight (% w) water, usually below about 5% w and preferably less than about 3% w. A dry composition can be such that the particles are readily dispersible in an inhalation device to form an aerosol.

The types of pharmaceutical excipients that are useful as carrier include stabilizers such as human serum albumin (HSA), bulking agents such as carbohydrates, amino acids and polypeptides; pH adjusters or buffers; salts such as sodium chloride; and the like. These carriers may be in a crystalline or amorphous form or may be a mixture of the two.

Suitable pH adjusters or buffers include organic salts prepared from organic acids and bases, such as sodium citrate, sodium ascorbate, and the like; sodium citrate is preferred. Pulmonary administration of a micellar single stranded oligonucleotide formulation may be achieved through metered dose spray devices with propellants such as tetrafluoroethane, heptafluoroethane, dimethylfluoropropane, tetrafluoropropane, butane, isobutane, dimethyl ether and other non-CFC and CFC propellants.

Exemplary devices include devices which are introduced into the vasculature, *e.g.*, devices inserted into the lumen of a vascular tissue, or which devices themselves form a part of the vasculature, including stents, catheters, heart valves, and other vascular devices. These devices, *e.g.*, catheters or stents, can be placed in the vasculature of the lung, heart, or leg.

Other devices include non-vascular devices, *e.g.*, devices implanted in the peritoneum, or in organ or glandular tissue, *e.g.*, artificial organs. The device can release a therapeutic substance in addition to a single stranded oligonucleotide, *e.g.*, a device can release insulin.

In one embodiment, unit doses or measured doses of a composition that includes single stranded oligonucleotide are dispensed by an implanted device. The device can include a sensor that monitors a parameter within a subject. For example, the device can include pump, *e.g.*, and, optionally, associated electronics.

Tissue, *e.g.*, cells or organs can be treated with a single stranded oligonucleotide, *ex vivo* and then administered or implanted in a subject. The tissue can be autologous, allogeneic, or xenogeneic tissue. *E.g.*, tissue can be treated to reduce graft v. host disease. In other embodiments, the tissue is allogeneic and the tissue is treated to treat a disorder characterized by unwanted gene expression in that tissue. *E.g.*, tissue, *e.g.*, hematopoietic cells, *e.g.*, bone marrow hematopoietic cells, can be treated to inhibit unwanted cell proliferation. Introduction of treated tissue, whether autologous or transplant, can be combined with other therapies. In some implementations, the single stranded oligonucleotide treated cells are insulated from other cells, *e.g.*, by a semi-permeable porous barrier that prevents the cells from leaving the implant, but enables molecules from the body to reach the cells and molecules produced by the cells to enter the body. In one embodiment, the porous barrier is formed from alginate.

In one embodiment, a contraceptive device is coated with or contains a single stranded oligonucleotide. Exemplary devices include condoms, diaphragms, IUD (implantable uterine devices, sponges, vaginal sheaths, and birth control devices.

Dosage

In one aspect, the invention features a method of administering a single stranded oligonucleotide (*e.g.*, as a compound or as a component of a composition) to a subject (*e.g.*, a human subject). In some embodiments, the methods involve administering a compound (*e.g.*, a compound of the general formula A-B-C, as disclosed herein, or a single stranded oligonucleotide,) in a unit dose to a subject. In one embodiment, the unit dose is between about 10 mg and 25 mg per kg of bodyweight. In one embodiment, the unit dose is between about 1 mg and 100 mg per kg of bodyweight. In one embodiment, the unit dose is between about 0.1 mg and 500 mg per kg of bodyweight. In some embodiments, the unit dose is more than 0.001, 0.005, 0.01, 0.05, 0.1, 0.5, 1, 2, 5, 10, 25, 50 or 100 mg per kg of bodyweight.

The defined amount can be an amount effective to treat or prevent a disease or disorder, *e.g.*, a disease or disorder associated with the SMN1 or SMN2. The unit dose, for example, can be administered by injection (*e.g.*, intravenous or intramuscular), an inhaled dose, or a topical application.

In some embodiments, the unit dose is administered daily. In some embodiments, less frequently than once a day, *e.g.*, less than every 2, 4, 8 or 30 days. In another embodiment, the unit dose is not administered with a frequency (*e.g.*, not a regular frequency). For example, the unit dose may be administered a single time. In some embodiments, the unit dose is administered more than once a day, *e.g.*, once an hour, two hours, four hours, eight hours, twelve hours, etc.

In one embodiment, a subject is administered an initial dose and one or more maintenance doses of a single stranded oligonucleotide. The maintenance dose or doses are generally lower than the initial dose, *e.g.*, one-half less of the initial dose. A maintenance regimen can include treating the subject with a dose or doses ranging from 0.0001 to 100 mg/kg of body weight per day, *e.g.*, 100, 10, 1, 0.1, 0.01, 0.001, or 0.0001 mg per kg of bodyweight per day. The maintenance doses may be administered no more than once every 1, 5, 10, or 30 days. Further, the treatment regimen may last for a period of time which will vary depending upon the nature of the particular disease, its severity and the overall condition

of the patient. In some embodiments the dosage may be delivered no more than once per day, *e.g.*, no more than once per 24, 36, 48, or more hours, *e.g.*, no more than once for every 5 or 8 days. Following treatment, the patient can be monitored for changes in his condition and for alleviation of the symptoms of the disease state. The dosage of the oligonucleotide
5 may either be increased in the event the patient does not respond significantly to current dosage levels, or the dose may be decreased if an alleviation of the symptoms of the disease state is observed, if the disease state has been ablated, or if undesired side-effects are observed.

The effective dose can be administered in a single dose or in two or more doses, as
10 desired or considered appropriate under the specific circumstances. If desired to facilitate repeated or frequent infusions, implantation of a delivery device, *e.g.*, a pump, semi-permanent stent (*e.g.*, intravenous, intraperitoneal, intracisternal or intracapsular), or reservoir may be advisable.

In some embodiments, the pharmaceutical composition includes a plurality of single
15 stranded oligonucleotide species. In some embodiments, the pharmaceutical composition comprises a first single stranded oligonucleotide complementary with a PRC2-associated region of a gene (*e.g.*, SMN1 or SMN2), and a second single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA of a gene (*e.g.*, SMN1 or SMN2). In some embodiments, the pharmaceutical composition includes a compound
20 comprising the general formula A-B-C, in which A is a single stranded oligonucleotide complementary with a PRC2-associated region of a gene, B is a linker, and C is a single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA of the gene.

In another embodiment, the single stranded oligonucleotide species has sequences that
25 are non-overlapping and non-adjacent to another species with respect to a naturally occurring target sequence (*e.g.*, a PRC2-associated region). In another embodiment, the plurality of single stranded oligonucleotide species is specific for different PRC2-associated regions. In another embodiment, the single stranded oligonucleotide is allele specific. In some cases, a patient is treated with a single stranded oligonucleotide in conjunction with other therapeutic
30 modalities.

Following successful treatment, it may be desirable to have the patient undergo maintenance therapy to prevent the recurrence of the disease state, wherein the compound of

the invention is administered in maintenance doses, ranging from 0.0001 mg to 100 mg per kg of body weight.

The concentration of the single stranded oligonucleotide composition is an amount sufficient to be effective in treating or preventing a disorder or to regulate a physiological condition in humans. The concentration or amount of single stranded oligonucleotide administered will depend on the parameters determined for the agent and the method of administration, *e.g.* nasal, buccal, pulmonary. For example, nasal formulations may tend to require much lower concentrations of some ingredients in order to avoid irritation or burning of the nasal passages. It is sometimes desirable to dilute an oral formulation up to 10-100 times in order to provide a suitable nasal formulation.

Certain factors may influence the dosage required to effectively treat a subject, including but not limited to the severity of the disease or disorder, previous treatments, the general health and/or age of the subject, and other diseases present. Moreover, treatment of a subject with a therapeutically effective amount of a single stranded oligonucleotide can include a single treatment or, preferably, can include a series of treatments. It will also be appreciated that the effective dosage of a single stranded oligonucleotide used for treatment may increase or decrease over the course of a particular treatment. For example, the subject can be monitored after administering a single stranded oligonucleotide composition. Based on information from the monitoring, an additional amount of the single stranded oligonucleotide composition can be administered.

Dosing is dependent on severity and responsiveness of the disease condition to be treated, with the course of treatment lasting from several days to several months, or until a cure is effected or a diminution of disease state is achieved. Optimal dosing schedules can be calculated from measurements of SMN1 or SMN2 expression levels in the body of the patient. Persons of ordinary skill can easily determine optimum dosages, dosing methodologies and repetition rates. Optimum dosages may vary depending on the relative potency of individual compounds, and can generally be estimated based on EC50s found to be effective in *in vitro* and *in vivo* animal models. In some embodiments, the animal models include transgenic animals that express a human SMN1 or SMN2. In another embodiment, the composition for testing includes a single stranded oligonucleotide that is complementary, at least in an internal region, to a sequence that is conserved between SMN1 or SMN2 in the animal model and the SMN1 or SMN2 in a human.

In one embodiment, the administration of the single stranded oligonucleotide composition is parenteral, *e.g.* intravenous (*e.g.*, as a bolus or as a diffusible infusion), intradermal, intraperitoneal, intramuscular, intrathecal, intraventricular, intracranial, subcutaneous, transmucosal, buccal, sublingual, endoscopic, rectal, oral, vaginal, topical, pulmonary, intranasal, urethral or ocular. Administration can be provided by the subject or by another person, *e.g.*, a health care provider. The composition can be provided in measured doses or in a dispenser which delivers a metered dose. Selected modes of delivery are discussed in more detail below.

Kits

In certain aspects of the invention, kits are provided, comprising a container housing a composition comprising a single stranded oligonucleotide. In some embodiments, the kits comprise a container housing a single stranded oligonucleotide complementary with of a PRC2-associated region of a gene; and a second container housing a single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA of the gene. In some embodiments, the kits comprise a container housing a single stranded oligonucleotide complementary with of a PRC2-associated region of a gene and a single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA of the gene. In some embodiments, the composition is a pharmaceutical composition comprising a single stranded oligonucleotide and a pharmaceutically acceptable carrier. In some embodiments, the individual components of the pharmaceutical composition may be provided in one container. Alternatively, it may be desirable to provide the components of the pharmaceutical composition separately in two or more containers, *e.g.*, one container for single stranded oligonucleotides, and at least another for a carrier compound. The kit may be packaged in a number of different configurations such as one or more containers in a single box. The different components can be combined, *e.g.*, according to instructions provided with the kit. The components can be combined according to a method described herein, *e.g.*, to prepare and administer a pharmaceutical composition. The kit can also include a delivery device.

The present invention is further illustrated by the following Examples, which in no way should be construed as further limiting. The entire contents of all of the references

(including literature references, issued patents, published patent applications, and co-pending patent applications) cited throughout this application are hereby expressly incorporated by reference.

EXAMPLES

The invention is further described in the following examples, which do not limit the scope of the invention described in the claims.

Example 1: Oligonucleotides targeting PRC2-associated regions that upregulate SMN1.

MATERIALS AND METHODS:

Real Time PCR

RNA was harvested from the cells using Promega SV 96 Total RNA Isolation system or Trizol omitting the DNase step. In separate pilot experiments, 50 ng of RNA was determined to be sufficient template for the reverse transcriptase reaction. RNA harvested from cells was normalized so that 50ng of RNA was input to each reverse transcription reaction. For the few samples that were too dilute to reach this limit, the maximum input volume was added. Reverse transcriptase reaction was performed using the Superscript II kit and real time PCR performed on cDNA samples using icycler SYBR green chemistry (Biorad). A baseline level of mRNA expression for each target gene was determined through quantitative PCR as outlined above. Baseline levels were also determined for mRNA of various housekeeping genes which are constitutively expressed. A “control” housekeeping gene with approximately the same level of baseline expression as the target gene was chosen for comparison purposes.

Protein expression (ELISA)

ELISA to determine SMN protein was carried out per manufacturer’s instructions (SMN ELISA kit #ADI-900-209, Enzo Life Sciences).

Cell Culture

Human hepatocyte Hep3B, human hepatocyte HepG2 cells, mouse hepatoma Hepa1-6 cells, and human renal proximal tubule epithelial cells (RPTEC) were cultured using conditions known in the art (see, *e.g.* Current Protocols in Cell Biology). Other cell lines tested were neuronal cell lines (SK-N-AS, U-87) and SMN patient fibroblasts. Details of the cell lines used in the experiments described herein are provided in Table 8.

Table 8. Cell lines

Cell line	Source	Species	Gender	Cell Type	Tissue	Status	Culture Conditions
RPTEC	Lonza	human	N/A	proximal tubule epithelial cells	kidney	primary	Clonetics™ REGM™ BulletKit™ (CC-3190)
Hep3B	ATCC	human	M	hepatocytes	liver	immortalized	Eagle's MEM + 10% FBS
SK-N-AS	ATCC	human	F	neuroblast	brain	immortalized	DMEM + 10% FBS
U-87	ATCC	human	M	glioblastoma	brain	immortalized	Eagle's MEM + 10% FBS
GM03813	Coriell Institute	human	F	fibroblast	skin	immortalized	MEM + 10% FBS
GM03814	Coriell Institute	human	M	fibroblast	skin	immortalized	MEM + 10% FBS
GM09677	Coriell Institute	human	M	fibroblast	skin	immortalized	MEM + 10% FBS
GM00232	Coriell Institute	human	M	fibroblast	skin	immortalized	MEM + 10% FBS
GM03815	Coriell Institute	human	M	fibroblast	skin	immortalized	MEM + 10% FBS
GM22592	Coriell Institute	human	M	fibroblast	skin	immortalized	MEM + 10% FBS
GM10684	Coriell Institute	human	F	B-lymphocyte	blood	immortalized	MEM + 10% FBS
GM00321 (normal)	Coriell Institute	human	F	fibroblast	skin	immortalized	MEM + 10% FBS

Oligonucleotide design

Oligonucleotides were designed within PRC2-interacting regions in order to upregulate SMN1. The sequence and structure of each oligonucleotide is shown in Table 2. The following table provides a description of the nucleotide analogs, modifications and intranucleotide linkages used for certain oligonucleotides tested and described in Table 2.

***In vitro* transfection of cells with oligonucleotides**

Cells were seeded into each well of 24-well plates at a density of 25,000 cells per 500uL and transfections were performed with Lipofectamine and the single stranded oligonucleotides. Control wells contained Lipofectamine alone. At 48 hours post-transfection, approximately 200 uL of cell culture supernatants were stored at -80 C for ELISA. At 48 hours post-transfection, RNA was harvested from the cells and quantitative PCR was carried out as outlined above. The percent induction of target mRNA expression by each oligonucleotide was determined by normalizing mRNA levels in the presence of the oligonucleotide to the mRNA levels in the presence of control (Lipofectamine alone). This was compared side-by-side with the increase in mRNA expression of the “control” housekeeping gene.

RESULTS:***In vitro* delivery of single stranded oligonucleotides upregulated SMN1 expression**

Oligonucleotides were designed as candidates for upregulating SMN1 expression. A total of 52 single stranded oligonucleotides were designed to be complementary to a PRC2-interacting region within a sequence as set forth in SEQ ID NO: 1, 2, 4, or 5. Oligonucleotides were tested in at least duplicate. The sequence and structural features of the oligonucleotides are set forth in Table 2. Briefly, cells were transfected in vitro with the oligonucleotides as described above. SMN1 expression in cells following treatment was evaluated by qRT-PCR. Oligonucleotides that upregulated SMN1 expression were identified. Further details are outlined in Table 2.

Table 5 shows further results from experiments in which oligonucleotides were transfected into cells at a particular concentration [oligo] and 48 or 72h later RNA was prepared and qRT-PCR assays carried out to determine mRNA levels of full length (FL) or

delta7 SMN. In other cases, oligos were administered gymnotically to cells at 10 μ M and RNA harvested 9 days post treatment. The cell lines tested were neuronal cell lines (SK-N-AS, U-87) and SMN patient fibroblasts.

Table 6 shows results from experiments in which oligonucleotides were transfected into cells in combination with either one or two more oligos or small molecule compounds at a particular concentration ([oligo], [2nd], [3rd]) and 48 or 72h later RNA was prepared and qRTPCR assays carried out to determine mRNA levels of full length (FL) or delta7 SMN. The cell lines tested were SMN patient fibroblasts.

Table 7 shows results from experiments in which oligonucleotides were transfected into cells in combination with either one or two more oligos or as dimers or by gymnotic treatment at a particular concentration ([oligo], [2nd], [3rd]) and 24, 48, 72 or 216h later cell lysates were prepared and ELISA assays carried out to determine SMN protein levels. The cell lines tested were SMN patient fibroblasts.

Tables

Table 1: Non-Seed hexamer sequences.

AAAAAA, AAAAAG, AAAACA, AAAAGA, AAAAGC, AAAAGG, AAAAUA, AAACAA, AAACAC, AAACAG,
 AAACAU, AAACCC, AAACCU, AAACGA, AAACGC, AAACGU, AAACUA, AAACUC, AAACUU, AAAGAU,
 5 AAAGCC, AAAGGA, AAAGGG, AAAGUC, AAAUAC, AAAUAU, AAAUCG, AAAUCU, AAAUGC, AAAUGU,
 AAAUUA, AAAUUG, AACAAC, AACAAG, AACAAU, AACACA, AACACG, AACAGA, AACAGC, AACAGG,
 AACAUC, AACAUG, AACCAA, AACCAC, AACCAG, AACCAU, AACCCC, AACCCG, AACCGA, AACCGC,
 AACCGG, AACCUA, AACCUU, AACGAA, AACGAC, AACGAG, AACGAU, AACGCU, AACGGG, AACGGU,
 AACGUA, AACGUC, AACGUG, AACGUU, AACUAU, AACUCA, AACUCC, AACUCG, AACUGA, AACUGC,
 10 AACUGU, AACUUA, AACUUC, AACUUG, AACUUU, AAGAAA, AAGAAG, AAGAAU, AAGACG, AAGAGA,
 AAGAGC, AAGAGG, AAGAGU, AAGAUU, AAGCAA, AAGCAC, AAGCAG, AAGCAU, AAGCCA, AAGCCC,
 AAGCCG, AAGCCU, AAGCGA, AAGCGG, AAGCGU, AAGCUA, AAGGAA, AAGGAC, AAGGCU, AAGGGC,
 AAGGGU, AAGGUU, AAGUAA, AAGUAC, AAGUAU, AAGUCC, AAGUCG, AAGUGA, AAGUGG, AAGUUA,
 AAGUUU, AAUAAA, AAUAAC, AAUAAG, AAUAU, AAUACA, AAUACC, AAUACG, AAUAGA, AAUAGC,
 15 AAUAGG, AAUAGU, AAUAUC, AAUAUU, AAUCAA, AAUCAU, AAUCCA, AAUCCC, AAUCCG, AAUCGA,
 AAUCGC, AAUCGU, AAUCUA, AAUCUG, AAUCUU, AAUGAA, AAUGAC, AAUGAG, AAUGAU, AAUGCG,
 AAUGCU, AAUGGA, AAUGGU, AAUGUA, AAUGUC, AAUGUG, AAUUAA, AAUUAC, AAUUAG, AAUUCC,
 AAUUCG, AAUUGA, AAUUGG, AAUUGU, AAUUUC, AAUUUG, ACAAAA, ACAAAC, ACAAAG, ACAAUU,
 ACAACC, ACAACG, ACAACU, ACAAGA, ACAAGC, ACAAGU, ACAAUC, ACAAUG, ACAAUU, ACACAG,
 20 ACACCA, ACACCC, ACACCG, ACACCU, ACACGA, ACACGC, ACACGU, ACACUC, ACACUG, ACACUU,
 ACAGAA, ACAGAC, ACAGCC, ACAGCG, ACAGCU, ACAGGG, ACAGUC, ACAGUG, ACAGUU, ACAUAA,
 ACAUAC, ACAUCC, ACAUCG, ACAUCU, ACAUGA, ACAUGC, ACAUGU, ACAUUG, ACAUUU, ACCAAA,
 ACCAAC, ACCAAG, ACCAAU, ACCACC, ACCACG, ACCAGA, ACCAGU, ACCAUA, ACCAUG, ACCAUU,
 ACCCAA, ACCCAC, ACCCCA, ACCCCG, ACCCGA, ACCCGC, ACCCUA, ACCCUC, ACCCUU, ACCGAA,
 25 ACCGAC, ACCGAU, ACCGCA, ACCGCC, ACCGCG, ACCGCU, ACCGGA, ACCGGC, ACCGGU, ACCGUA,
 ACCGUC, ACCGUG, ACCGUU, ACCUAA, ACCUAC, ACCUAG, ACCUAU, ACCUCA, ACCUCC, ACCUCG,
 ACCUCU, ACCUGA, ACCUGC, ACCUGU, ACCUUA, ACCUUC, ACCUUU, ACGAAA, ACGAAC, ACGAAG,
 ACGAAU, ACGACA, ACGACC, ACGACG, ACGACU, ACGAGA, ACGAGC, ACGAGG, ACGAGU, ACGAUA,
 ACGAUC, ACGAUG, ACGAUU, ACGCAA, ACGCAG, ACGCAU, ACGCCC, ACGCCG, ACGCCU, ACGCGA,
 30 ACGCGG, ACGCGU, ACGCUA, ACGCUG, ACGCUU, ACGGAA, ACGGAC, ACGGAG, ACGGAU, ACGGCC,
 ACGGCG, ACGGCU, ACGGGC, ACGGGG, ACGGGU, ACGGUA, ACGGUC, ACGGUG, ACGGUU, ACGUAA,
 ACGUAC, ACGUAU, ACGUCC, ACGUCG, ACGUCU, ACGUGA, ACGUGC, ACGUGG, ACGUGU, ACGUUA,
 ACGUUC, ACGUUG, ACGUUU, ACUAAA, ACUAAG, ACUAAU, ACUACA, ACUACC, ACUACG, ACUACU,
 ACUAGG, ACUAUC, ACUAUG, ACUAUU, ACUCAU, ACUCCC, ACUCCG, ACUCCU, ACUCGA, ACUCGC,
 35 ACUCGG, ACUCUC, ACUCUU, ACUGAG, ACUGAU, ACUGCC, ACUGCG, ACUGCU, ACUGGG, ACUGGU,
 ACUGUC, ACUUAA, ACUUAC, ACUUAU, ACUUCA, ACUUCC, ACUUCG, ACUUCU, ACUUGA, ACUUGC,
 ACUUGU, ACUUUA, ACUUUC, ACUUUG, AGAAAA, AGAAAC, AGAAAG, AGAACC, AGAACG, AGAACU,
 AGAAGC, AGAAGU, AGAAUA, AGAAUC, AGAAUG, AGAAUU, AGACAA, AGACAC, AGACAU, AGACCA,
 AGACCC, AGACCG, AGACCU, AGACGA, AGACGC, AGACGU, AGACUA, AGACUC, AGACUU, AGAGAC,
 40 AGAGAG, AGAGAU, AGAGCC, AGAGCG, AGAGCU, AGAGGC, AGAGGG, AGAGGU, AGAGUA, AGAGUU,

AGAUAC, AGAUAG, AGAUAU, AGAUCC, AGAUCG, AGAUCU, AGAUGA, AGAUGC, AGAUGG, AGAUUA,
 AGAUUC, AGAUUG, AGAUUU, AGCAAC, AGCACA, AGCACG, AGCACU, AGCAGA, AGCAUA, AGCAUC,
 AGCAUG, AGCCAA, AGCCAU, AGCCCA, AGCCGA, AGCCGC, AGCCGG, AGCCGU, AGCCUA, AGCCUC,
 AGCGAA, AGCGAG, AGCGAU, AGCGCA, AGCGCC, AGCGCG, AGCGCU, AGCGGA, AGCGGC, AGCGGU,
 5 AGCGUA, AGCGUC, AGCGUG, AGCGUU, AGCUAA, AGCUAC, AGCUAG, AGCUAU, AGCUCA, AGCUCC,
 AGCUCG, AGCUCU, AGCUGA, AGCUGG, AGCUGU, AGCUUC, AGCUUU, AGGAAU, AGGACC, AGGACG,
 AGGAGA, AGGAGU, AGGAUA, AGGCAA, AGGCAU, AGGCCG, AGGCGA, AGGCGC, AGGCGG, AGGCUA,
 AGGCUC, AGGCUU, AGGGAC, AGGGAU, AGGGGA, AGGGGU, AGGGUA, AGGGUG, AGGUAA,
 AGGUAC, AGGUCA, AGGUCC, AGGUCU, AGGUGA, AGGUGC, AGGUGG, AGGUGU, AGGUUC,
 10 AGGUUG, AGUAAA, AGUAAG, AGUAAU, AGUACA, AGUACG, AGUAGC, AGUAGG, AGUAUA, AGUAUC,
 AGUAUG, AGUAUU, AGUCAA, AGUCAC, AGUCAG, AGUCAU, AGUCCA, AGUCCG, AGUCCU, AGUCGA,
 AGUCGC, AGUCGG, AGUCGU, AGUCUA, AGUCUC, AGUCUG, AGUCUU, AGUGAA, AGUGAC, AGUGCG,
 AGUGGG, AGUGUC, AGUUAA, AGUUAC, AGUUAG, AGUUC, AGUUCG, AGUUGA, AGUUGC,
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 15 AUA ACU, AUA AGA, AUA AGC, AUA AGG, AUA AGU, AUA AUC, AUA AUG, AUA AUU, AUACAC, AUACAG,
 AUACAU, AUACCA, AUACCC, AUACCG, AUACGA, AUACGC, AUACGG, AUACGU, AUACUA, AUACUC,
 AUACUG, AUACUU, AUAGAA, AUAGAC, AUAGAU, AUAGCA, AUAGCG, AUAGCU, AUAGGA, AUAGGU,
 AUAGUA, AUAGUC, AUAGUG, AUAGUU, AUAUAC, AUAUAG, AUAUCC, AUAUCG, AUAUCU, AUAUGA,
 AUAUGC, AUAUGG, AUAUGU, AUAUUC, AUAUUG, AUAUUU, AUCAAA, AUCAAC, AUCAAG, AUCAAU,
 20 AUCACA, AUCACC, AUCACG, AUCAGC, AUCAGG, AUCCAA, AUCCAU, AUCCCC, AUCCCG, AUCCGA,
 AUCCGC, AUCCGG, AUCCUA, AUCCUC, AUCCUG, AUCGAA, AUCGAC, AUCGAG, AUCGAU, AUCGCA,
 AUCGCC, AUCGCG, AUCGCU, AUCGGC, AUCGGG, AUCGGU, AUCGUC, AUCGUG, AUCGUU, AUCUAA,
 AUCUAC, AUCUAG, AUCUAU, AUCUCC, AUCUCG, AUCUGU, AUCUUG, AUCUUU, AUGAAA, AUGAAC,
 AUGAAG, AUGAAU, AUGACC, AUGACU, AUGAGG, AUGAGU, AUGAUA, AUGAUC, AUGAUU, AUGCAA,
 25 AUGCAG, AUGCCA, AUGCCC, AUGCCG, AUGCGA, AUGCGG, AUGCGU, AUGCUC, AUGCUU, AUGGAC,
 AUGGCC, AUGGGA, AUGGGC, AUGGGU, AUGGUC, AUGGUG, AUGUAC, AUGUAU, AUGUCA,
 AUGUCC, AUGUCG, AUGUGU, AUGUUA, AUGUUC, AUUAAA, AUUAAC, AUUAAG, AUUAAU, AUUACA,
 AUUACC, AUUACG, AUUACU, AUUAGA, AUUAGC, AUUAGG, AUUAGU, AUUAUA, AUUAUC, AUUAUG,
 AUUCAC, AUUCCA, AUUCCG, AUUCCU, AUUCGA, AUUCGC, AUUCGG, AUUCGU, AUUCUA, AUUCUC,
 30 AUUCUU, AUUGAA, AUUGAC, AUUGAU, AUUGCC, AUUGCG, AUUGCU, AUUGGA, AUUGGC,
 AUUGGG, AUUGGU, AUUGUA, AUUGUC, AUUGUG, AUUGUU, AUUUAA, AUUUAG, AUUUAU,
 AUUUCC, AUUUUCG, AUUUUCU, AUUUUGA, AUUUUGC, AUUUUGU, AUUUUA, AUUUUC, AUUUUG,
 AUUUUU, CAAAAG, CAAACA, CAAACC, CAAACG, CAAACU, CAAAGA, CAAAGG, CAAUA, CAAUU,
 CAACAC, CAACAU, CAACCA, CAACCC, CAACCG, CAACGA, CAACGC, CAACGG, CAACGU, CAACUA,
 35 CAACUC, CAACUG, CAACUU, CAAGAA, CAAGAC, CAAGAU, CAAGCA, CAAGCC, CAAGCG, CAAGCU,
 CAAGGA, CAAGGG, CAAGUC, CAAGUG, CAAGUU, CAAUAA, CAAUAC, CAAUAG, CAAUCC, CAAUCG,
 CAAUCU, CAAUGA, CAAUGC, CAAUGG, CAAUGU, CAAUUC, CAAUUG, CAAUUU, CACAAU, CACACA,
 CACACG, CACACU, CACAGA, CACAGC, CACAGG, CACAU, CACAUC, CACAUU, CACCAA, CACCAC,
 CACCAU, CACCCA, CACCCC, CACCCG, CACCGA, CACCGC, CACCGG, CACCGU, CACCUA, CACCUU,
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UCUCGU, UCUCUC, UCUGAA, UCUGAU, UCUGCA, UCUGCG, UCUGCU, UCUGGC, UCUGGU, UCUGUC,
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UGAGCA, UGAGCC, UGAGCU, UGAGGC, UGAGGU, UGAGUA, UGAGUU, UGAUAC, UGAUAG,
10 UGAUUA, UGAUCA, UGAUCG, UGAUCU, UGAUGA, UGAUGC, UGAUGG, UGAUGU, UGAUUA,
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UGGUCG, UGGUCU, UGGUGA, UGGUGC, UGGUGG, UGGUGU, UGGUUA, UGGUUG, UGUAAA,
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UGUUGU, UGUUUA, UGUUUC, UGUUUG, UGUUUU, UUA AAA, UUA AAC, UUA AAG, UUA AAU,
UUA ACC, UUA ACG, UUA ACU, UUA AGU, UUA AUA, UUA AUC, UUA AUG, UUA AUU, UUA CAA, UUA CAC,
25 UUA CAG, UUA CAU, UUA CCA, UUA CCC, UUA CCG, UUA CCU, UUA CGA, UUA CGC, UUA CGG, UUA CGU,
UUA CUA, UUA CUC, UUA CUG, UUA CUU, UUA GAA, UUA GAC, UUA GCC, UUA GCG, UUA GCU, UUA GGC,
UUA GGU, UUA GUA, UUA GUC, UUA GUU, UUA UAA, UUA UAC, UUA UAG, UUA UAU, UUA UCC,
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30 UUA AUC, UUA AUG, UUA AUU, UUA CAA, UUA CCA, UUA CCG, UUA CCGA, UUA CCGU, UUA CCU, UUA CGAA,
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40 UUA AUG, UUA AUU, UUA CAU, UUA CCA, UUA CCG, UUA CCU, UUA CGA, UUA CGC, UUA CGG,
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UUUUGU, UUUUUA, UUUUUC, UUUUUU

Table 2: Oligonucleotide sequences made for testing

Oligo Name	RQ	RQ SE	Gene Name	Expt Type	Cell Line/Tissue	[Oligo]	Assay Type	Coordinates_g
SMN1-01	0.8126719 52	0.1352513 51	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21157U20
SMN1-01	0.8570321 01	0.0273187 37	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21157U20
SMN1-01	0.1679989 15	0.1679986 72	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21157U20
SMN1-01	1.0481253 02	0.0393027 84	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21157U20
SMN1-01	1.3817042 07	0.0532905 65	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21157U20
SMN1-01	0.9798692 47	0.0205152 27	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21157U20
SMN1-02	0.7600003 18	0.0429932 12	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21158U20
SMN1-02	0.9871384 47	0.0681879 98	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21158U20
SMN1-02	2.2524945 26	1.8031906 69	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21158U20
SMN1-02	1.1143879 73	0.0267332 51	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21158U20
SMN1-02	1.3464192 9	0.0276412 81	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21158U20
SMN1-02	1.1536970 83	0.0249999 91	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21158U20
SMN1-03	1.9072297 5	0.5259392 96	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21159U20
SMN1-03	1.1327582 64	0.0946401 77	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21159U20
SMN1-03	0.2961917 4	0.1732823 09	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21159U20
SMN1-03	1.4881793 5	0.1727195 07	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21159U20
SMN1-03	1.2993282 6	0.0598252 28	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21159U20
SMN1-03	1.5115678 14	0.0541781 75	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21159U20
SMN1-04	1.0483065 17	0.2439345 43	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21160U20
SMN1-04	1.3224072 67	0.1000223 92	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21160U20
SMN1-04	0.1331700 13	0.0328243 91	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21160U20
SMN1-04	1.2895501 63	0.3301959 87	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21160U20

Oligo Name	RQ	RQ SE	Gene Name	Expt Type	Cell Line/Tissue	[Oligo]	Assay Type	Coordinates_g
SMN1-04	1.280225492	0.062577972	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21160U20
SMN1-04	1.488482795	0.044641287	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21160U20
SMN1-05	0.876747527	0.087392504	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21161U20
SMN1-05	1.167120345	0.069814091	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21161U20
SMN1-05	0.088317863	0.039887014	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21161U20
SMN1-05	1.310053256	0.234231348	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21161U20
SMN1-05	1.038699643	0.056421362	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21161U20
SMN1-05	0.859144751	0.039970015	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21161U20
SMN1-06	0.704659891	0.087244119	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21162U20
SMN1-06	1.11194006	0.088571377	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21162U20
SMN1-06	0.57685962	0.246186541	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21162U20
SMN1-06	1.419418884	0.432447122	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21162U20
SMN1-06	1.146251704	0.051891541	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21162U20
SMN1-06	1.030682317	0.013070835	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21162U20
SMN1-07	0.682085732	0.084885351	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21163U20
SMN1-07	0.975853552	0.034178542	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21163U20
SMN1-07	1.013252314	0.118540759	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21163U20
SMN1-07	1.039381902	0.059815387	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21163U20
SMN1-07	1.156949605	0.107385405	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21163U20
SMN1-07	1.239503954	0.134603844	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21163U20
SMN1-08	0.948714888	0.142708231	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21164U20
SMN1-08	1.312080445	0.058464993	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21164U20
SMN1-08	0.216530007	0.177400555	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21164U20
SMN1-08	2.082151781	0.815184252	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21164U20
SMN1-08	1.010090604	0.200588791	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21164U20

Oligo Name	RQ	RQ SE	Gene Name	Expt Type	Cell Line/Tissue	[Oligo]	Assay Type	Coordinates_g
SMN1-08	1.223947667	0.295307243	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21164U20
SMN1-09	0.77519063	0.098695118	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21165U20
SMN1-09	1.685731616	0.014884028	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21165U20
SMN1-09	0.621406781	0.227211261	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21165U20
SMN1-09	0.85593922	0.256108337	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21165U20
SMN1-09	0.940186097	0.197008464	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21165U20
SMN1-09	0.864481145	0.162739271	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21165U20
SMN1-10	0.945730986	0.08072952	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21166U20
SMN1-10	1.574526902	0.123062684	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21166U20
SMN1-10	0.482822242	0.131557474	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21166U20
SMN1-10	1.280629128	0.17088425	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21166U20
SMN1-10	1.127254654	0.152486374	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21166U20
SMN1-10	1.069571458	0.122106758	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21166U20
SMN1-11	0.774436979	0.038076182	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21167U20
SMN1-11	1.562714254	0.158043098	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21167U20
SMN1-11	0.463655938	0.295513886	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21167U20
SMN1-11	0.957611652	0.334137541	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21167U20
SMN1-11	1.225973818	0.223472758	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21167U20
SMN1-11	1.089302259	0.126414268	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21167U20
SMN1-12	0.981429476	0.07937384	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21168U20
SMN1-12	1.585088128	0.05291912	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21168U20
SMN1-12	0.208586047	0.187017655	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21168U20
SMN1-12	3.266965896	2.002074369	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21168U20
SMN1-12	1.03381379	0.204376291	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21168U20
SMN1-12	1.137471671	0.246791954	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21168U20

Oligo Name	RQ	RQ SE	Gene Name	Expt Type	Cell Line/Tissue	[Oligo]	Assay Type	Coordinates_g
SMN1-13	0.749636437	0.103277003	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21169U20
SMN1-13	1.175989263	0.122355585	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21169U20
SMN1-13	0.161499159	0.079356287	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21169U20
SMN1-13	1.287763591	0.090306717	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21169U20
SMN1-13	1.336851675	0.177814975	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21169U20
SMN1-13	1.037772291	0.039404507	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21169U20
SMN1-14	0.771635177	0.086041959	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21170U20
SMN1-14	1.467048548	0.073113884	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21170U20
SMN1-14	1.978254154	1.352951156	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21170U20
SMN1-14	1.311990937	0.073121634	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21170U20
SMN1-14	1.12892777	0.147162701	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21170U20
SMN1-14	0.855795121	0.017797181	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21170U20
SMN1-15	0.891491964	0.039822032	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21171U20
SMN1-15	1.573440342	0.117453017	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21171U20
SMN1-15	0.366043104	0.117162019	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21171U20
SMN1-15	1.738217394	0.520148155	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21171U20
SMN1-15	1.383201337	0.101830776	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21171U20
SMN1-15	1.619495052	0.038364989	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21171U20
SMN1-16	0.73721881	0.038067583	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21172U20
SMN1-16	1.441616196	0.059823944	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21172U20
SMN1-16	0.510056605	0.286522659	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21172U20
SMN1-16	1.381914214	0.247880229	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21172U20
SMN1-16	1.310073573	0.026347093	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21172U20
SMN1-16	1.418132646	0.082371708	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21172U20
SMN1-17	1.219065651	0.281987674	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21173U20

Oligo Name	RQ	RQ SE	Gene Name	Expt Type	Cell Line/Tissue	[Oligo]	Assay Type	Coordinates_g
SMN1-17	1.274819195	0.179527293	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21173U20
SMN1-17	0.416739222	0.066066242	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21173U20
SMN1-17	3.331843017	0.970174873	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21173U20
SMN1-17	1.260856522	0.038565799	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21173U20
SMN1-17	1.609045311	0.10487434	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21173U20
SMN1-18	0.868441941	0.088184698	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21174U20
SMN1-18	1.221663574	0.064445539	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21174U20
SMN1-18	10.28455167	3.929310832	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21174U20
SMN1-18	1.800920764	0.42559045	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21174U20
SMN1-18	1.261752602	0.069817143	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21174U20
SMN1-18	1.592700796	0.199280916	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21174U20
SMN1-19	0.705512452	0.06496675	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21175U20
SMN1-19	1.43433309	0.075936965	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21175U20
SMN1-19	0.538932156	0.309273594	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21175U20
SMN1-19	1.17374637	0.179415746	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21175U20
SMN1-19	1.186141471	0.036729063	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21175U20
SMN1-19	1.834775368	0.155761723	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21175U20
SMN1-20	0.826303453	0.062998254	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21176U20
SMN1-20	1.505786689	0.170697984	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21176U20
SMN1-20	0.06244992	0.049069571	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21176U20
SMN1-20	1.541480855	0.461158669	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21176U20
SMN1-20	1.089985692	0.043750568	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21176U20
SMN1-20	1.41531375	0.146502726	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21176U20
SMN1-21	0.865566453	0.209455026	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21177U20
SMN1-21	1.466688787	0.116267764	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21177U20

Oligo Name	RQ	RQ SE	Gene Name	Expt Type	Cell Line/Tissue	[Oligo]	Assay Type	Coordinates_g
SMN1-21	0.388233514	0.139680869	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21177U20
SMN1-21	1.366269447	0.239420557	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21177U20
SMN1-21	1.354554841	0.013175463	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21177U20
SMN1-21	2.026968382	0.27827902	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21177U20
SMN1-22	0.639934851	0.011679891	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21178U20
SMN1-22	1.242593923	0.02840519	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21178U20
SMN1-22	0.229857922	0.128101282	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21178U20
SMN1-22	1.499722255	0.568788539	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21178U20
SMN1-22	1.234783764	0.017119432	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21178U20
SMN1-22	1.509695591	0.175764156	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21178U20
SMN1-23	0.748031845	0.083732479	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21179U20
SMN1-23	1.33910973	0.070877143	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21179U20
SMN1-23	0.384143384	0.14723735	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21179U20
SMN1-23	2.620195611	0.342101826	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21179U20
SMN1-23	1.473663866	0.053762605	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21179U20
SMN1-23	1.920800418	0.127336842	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21179U20
SMN1-24	0.907436601	0.24201681	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21180U20
SMN1-24	1.28379369	0.158661709	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21180U20
SMN1-24	0.963100208	0.117802422	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21180U20
SMN1-24	0.994753299	0.268415648	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21180U20
SMN1-24	0.965440348	0.032646295	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21180U20
SMN1-24	1.140566171	0.10163121	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21180U20
SMN1-25	0.908854808	0.076026035	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21181U20
SMN1-25	1.226185041	0.044422705	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21181U20
SMN1-25	1.055082301	0.326768036	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21181U20

Oligo Name	RQ	RQ SE	Gene Name	Expt Type	Cell Line/Tissue	[Oligo]	Assay Type	Coordinates_g
SMN1-25	0.969185038	0.226484866	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21181U20
SMN1-25	1.00974636	0.122120737	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21181U20
SMN1-25	1.081303639	0.101827303	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21181U20
SMN1-26	0.876444072	0.070457909	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21182U20
SMN1-26	1.632434888	0.061512357	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21182U20
SMN1-26	0.071593319	0.071592884	SMN1	in vitro	Hep3B	50	qRTPCR	SMN1:21182U20
SMN1-26	1.999202516	0.420387669	SMN1	in vitro	Hep3B	100	qRTPCR	SMN1:21182U20
SMN1-26	0.974107584	0.066863661	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21182U20
SMN1-26	1.030227891	0.096105098	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21182U20
SMN1-27	0.834365703	0.108102871	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21157U15
SMN1-27	1.589954219	0.093101653	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21157U15
SMN1-27	0.747186714	0.007807701	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21157U15
SMN1-27	1.049068744	0.092645193	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21157U15
SMN1-28	1.058343694	0.208931576	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21158U15
SMN1-28	1.402348414	0.101950771	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21158U15
SMN1-28	1.150224316	0.080077707	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21158U15
SMN1-28	1.219828396	0.031782762	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21158U15
SMN1-29	0.712268587	0.077572838	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21159U15
SMN1-29	1.145305552	0.044575389	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21159U15
SMN1-29	0.937393865	0.015700783	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21159U15
SMN1-29	1.208521962	0.084021899	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21159U15
SMN1-30	0.869504109	0.147682779	SMN1	in vitro	RPTEC	100	qRTPCR	SMN1:21160U15
SMN1-30	1.166995709	0.128900531	SMN1	in vitro	RPTEC	50	qRTPCR	SMN1:21160U15
SMN1-30	1.069533423	0.042258392	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21160U15
SMN1-30	1.004618999	0.068245537	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21160U15

Oligo Name	RQ	RQ SE	Gene Name	Expt Type	Cell Line/Tissue	[Oligo]	Assay Type	Coordinates_g
SMN1-31	1.223685297	0.155258366	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21161U15
SMN1-31	0.936569575	0.083367899	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21161U15
SMN1-32	1.032978469	0.02312057	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21162U15
SMN1-32	1.053045821	0.030158389	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21162U15
SMN1-33	1.046361407	0.038971809	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21163U15
SMN1-33	1.233302232	0.063255341	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21163U15
SMN1-34	1.079876751	0.09859402	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21164U15
SMN1-34	1.271026183	0.067019476	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21164U15
SMN1-35	0.861464008	0.024095912	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21165U15
SMN1-35	0.836966392	0.054159619	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21165U15
SMN1-36	1.26636324	0.046963681	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21166U15
SMN1-36	1.326257117	0.039674649	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21166U15
SMN1-37	1.232690086	0.043476252	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21167U15
SMN1-37	1.144632987	0.058433353	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21167U15
SMN1-38	0.843241863	0.033808043	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21168U15
SMN1-38	0.93818033	0.011376217	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21168U15
SMN1-39	0.663746249	0.045527014	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21169U15
SMN1-39	0.891764551	0.019395327	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21169U15
SMN1-40	0.888138653	0.081401804	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21170U15
SMN1-40	0.871602899	0.065372936	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21170U15
SMN1-41	0.882466148	0.031016749	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21171U15
SMN1-41	1.093694765	0.025996502	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21171U15
SMN1-42	0.956860836	0.043558382	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21172U15
SMN1-42	1.151755999	0.067662107	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21172U15
SMN1-43	1.341919782	0.08080776	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21173U15

Oligo Name	RQ	RQ SE	Gene Name	Expt Type	Cell Line/Tissue	[Oligo]	Assay Type	Coordinates_g
SMN1-43	1.692919815	0.084669198	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21173U15
SMN1-44	0	0	SMN1	NA	NA	0	NA	SMN1:21174U15
SMN1-45	1.807236897	0.11410948	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21175U15
SMN1-45	1.377773703	0.108540058	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21175U15
SMN1-46	1.545649538	0.064006814	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21176U15
SMN1-46	1.354291504	0.038498944	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21176U15
SMN1-47	2.711598361	0.260043446	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21177U15
SMN1-47	1.986674786	0.119436675	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21177U15
SMN1-48	1.482342195	0.063036343	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21178U15
SMN1-48	2.597350628	0.145439801	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21178U15
SMN1-49	1.534493905	0.110688365	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21179U15
SMN1-49	2.223340784	0.148702992	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21179U15
SMN1-50	0.897421396	0.034254931	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21180U15
SMN1-50	1.132362781	0.078523003	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21180U15
SMN1-51	1.157921368	0.044256319	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21181U15
SMN1-51	1.177604665	0.038060353	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21181U15
SMN1-52	0.973548353	0.051461583	SMN1	in vitro	Hep3B	30	qRTPCR	SMN1:21182U15
SMN1-52	1.068355642	0.060851146	SMN1	in vitro	Hep3B	10	qRTPCR	SMN1:21182U15

Table 3: Oligonucleotide Modifications

Symbol	Feature Description
bio	5' biotin
dAs	DNA w/3' thiophosphate
dCs	DNA w/3' thiophosphate
dGs	DNA w/3' thiophosphate

Symbol	Feature Description
dTs	DNA w/3' thiophosphate
dG	DNA
enaAs	ENA w/3' thiophosphate
enaCs	ENA w/3' thiophosphate
enaGs	ENA w/3' thiophosphate
enaTs	ENA w/3' thiophosphate
fluAs	2'-fluoro w/3' thiophosphate
fluCs	2'-fluoro w/3' thiophosphate
fluGs	2'-fluoro w/3' thiophosphate
fluUs	2'-fluoro w/3' thiophosphate
lnaAs	LNA w/3' thiophosphate
lnaCs	LNA w/3' thiophosphate
lnaGs	LNA w/3' thiophosphate
lnaTs	LNA w/3' thiophosphate
omeAs	2'-OMe w/3' thiophosphate
omeCs	2'-OMe w/3' thiophosphate
omeGs	2'-OMe w/3' thiophosphate
omeTs	2'-OMe w/3' thiophosphate
lnaAs-Sup	LNA w/3' thiophosphate at 3' terminus
lnaCs-Sup	LNA w/3' thiophosphate at 3' terminus
lnaGs-Sup	LNA w/3' thiophosphate at 3' terminus
lnaTs-Sup	LNA w/3' thiophosphate at 3' terminus
lnaA-Sup	LNA w/3' OH at 3' terminus
lnaC-Sup	LNA w/3' OH at 3' terminus
lnaG-Sup	LNA w/3' OH at 3' terminus
lnaT-Sup	LNA w/3' OH at 3' terminus
omeA-Sup	2'-OMe w/3' OH at 3' terminus
omeC-Sup	2'-OMe w/3' OH at 3' terminus
omeG-Sup	2'-OMe w/3' OH at 3' terminus
omeU-Sup	2'-OMe w/3' OH at 3' terminus
dAs-Sup	DNA w/3' thiophosphate at 3' terminus
dCs-Sup	DNA w/3' thiophosphate at 3' terminus
dGs-Sup	DNA w/3' thiophosphate at 3' terminus
dTs-Sup	DNA w/3' thiophosphate at 3' terminus
dA-Sup	DNA w/3' OH at 3' terminus
dC-Sup	DNA w/3' OH at 3' terminus
dG-Sup	DNA w/3' OH at 3' terminus
dT-Sup	DNA w/3' OH at 3' terminus

BRIEF DESCRIPTION OF SEQUENCE LISTING

SeqID	Chrom	Gene	Chrom Start	Chrom End	Strand	Name
1	chr5	SMN1	70208768	70260838	+	human SMN1
2	chr5	SMN2	69333350	69385422	+	human SMN2
3	chr9	SMNP	20319406	20344375	+	human SMNP
4	chr5	SMN1	70208768	70260838	-	human SMN1_revComp
5	chr5	SMN2	69333350	69385422	-	human SMN2_revComp
6	chr9	SMNP	20319406	20344375	-	human SMNP_revComp
7	chr13	Smn1	100881160	100919653	+	mouse Smn1
8	chr13	Smn1	100881160	100919653	-	mouse Smn1_revComp
9	chr5	SMN1	70240095	70240127	+	S48-193240
9	chr5	SMN2	69364672	69364704	+	S48-193240
10	chr5	SMN1	70214393	70214822	+	S48-441814
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25	chr5	SMN2	69366499	69366543	-	S48-193242
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27	chr5	SMN2	69363087	69367128	-	S48-193241 +2K
28	chr5	SMN1	70239924	70243968	-	S48-193242 +2K
29	chr5	SMN2	69364499	69368543	-	S48-193242 +2K
13100	chr5	SMN1	70247831	70247845	+	Splice control sequence
13100	chr5	SMN2	69372411	69372425	+	Splice control sequence

- 90 -

13101 chr5 SMN2 69372402 69372845 + Intron 7

Single Strand Oligonucleotides (Antisense Strand of Target Gene)

SeqID range: 30 to 8329, 13088-13094

SeqIDs w/o G Runs:

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Single Strand Oligonucleotides (Sense Strand of Target Gene)

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Example 2: Selective upregulation of exon 7 containing SMN2 transcripts using oligonucleotides targeting PRC2-interacting regions that upregulate SMN2 and splice-switching oligonucleotides.

20 ***Oligo design:***

Oligonucleotides targeting PRC2-interacting regions (lncRNA peaks) in the SMN1/2 gene loci were designed. These oligos were synthesized with various DNA base modifications, modification placements, inter-nucleoside bonds and inter-oligo linkers (oligos 1-52 and 59-101) as outlined in Table 4.

25 Splice switching oligos (SSO) were designed based on sequences of SMN2. Various modifications of such SSOs in length and chemistry were prepared (oligos 53-58).

Universal negative control oligos (oligo 232 and 293) were also designed using on bioinformatic analysis.

Methods:

30 ***Cell Culture:***

Six SMA fibroblast cell lines and one lymphoblast cell line were obtained from the Coriell Institute (FIG. 2). The cells were either transfected with the oligos using

Lipofectamine 2000 (Fibroblasts) or by electroporation or unassisted delivery (lymphoblast) to ascertain effects of the oligonucleotides on SMN1/2 mRNA and protein expression. All experiments were carried out as biological triplicates.

mRNA and protein expression:

5 *mRNA expression -*

1. On day 1, SMA fibroblasts were seeded into each well of 96-well plates at a density of 5,000 cells per 100uL.

2. On day 2, transfections were performed using Lipofectamine2000 per manufacturer's instructions with oligos at either 10nM or 30nM.

10 3. 48 hours post-transfection, Ambion Cells-to-CT kit was used to directly obtain qRT-PCR results from the cells per manufacturer's instructions.

4. Quantitative PCR evaluation was completed using Taqman FAST qPCR on StepOne Plus, and change in mRNA expression was quantified using the delta delta Ct method by normalizing SMN expression to a housekeeper gene (B2M).

15 *Protein expression (ELISA) -*

ELISA to determine SMN protein was carried out per manufacturer's instructions (SMN ELISA kit #ADI-900-209, Enzo Life Sciences). Briefly, SMN fibroblasts were cultures at 40,000 cells/well of a 24-well tissue culture coated plate on day 1. Cells were transfected with the oligos using Lipofectamine2000 on day 2 and cell lysates prepared at 24
20 and 48 hours post-treatment. ELISA was carried out per manufacturer's instructions. Subsequently fold induction of SMN protein was determined by normalizing SMN protein levels induced by oligonucleotides to the SMN protein levels induced by Lipofectamine treatment alone.

Splice switching assay (DdeI assay) -

25 SMN2-derived transcripts contain a unique DdeI restriction element introduced because of a nucleotide polymorphism not present in SMN1 and are differentiated from SMN1-derived transcripts because of the faster migration of the SMN2 products. Briefly, SMA fibroblasts were treated with oligonucleotides targeting PRC2-interacting regions with or without SSO at 30nM each as described before. RT-PCR was carried out with an SMN
30 exon 5 forward primer and an exon 8 reverse primer to generate cDNAs that were then digested with DdeI. The SMN1 transcript, if present, migrates at a slower rate than the DdeI-digested SMN2 transcript and is seen as the first band from the top of the gel. The second

band from the top indicates full length SMN2 (accurately spliced form) and the third band indicates the incorrectly spliced SMN2delta7. (FIG. 5)

Results

In Spinal Muscular Atrophy patients, the SMN1 gene is often mutated in such a way that it is unable to correctly code the SMN protein - due to either a deletion encompassing at least a portion of exon 7 or to other mutations. SMA patients, however, generally retain at least one copy of the SMN2 gene (with many having 2 or more copies) that still expresses small amounts of SMN protein. The SMN2 gene has a C to T mutation (compared with SMN1) in exon 7 that alters splicing of its precursor mRNA such that exon 7 is spliced out at a high frequency. Consequently, only about 10% of the normal levels of full length SMN protein are produced from SMN2. (See FIG. 1)

Six SMA fibroblast cell lines and one lymphoblast cell line were obtained from the Coriell Institute (FIG. 2). Cells were transfected with oligonucleotides (oligos 1-52 and 59-101) directed against a PRC2-associated region of SMN2 and RT-PCR assays were conducted to evaluate effects on SMN mRNA expression. (See FIGS. 3 and 4 for results in cell lines 3814 and 3813). In separate experiments, cells were transfected with oligonucleotides (oligos 53-58) directed at a splice control sequence in intron 7 of SMN2 and RT-PCR assays were conducted to evaluate effects on SMN mRNA expression (See FIGS. 3 and 4 for results in cell lines 3814 and 3813). Splice switching oligonucleotides (oligos 53-58) were found to increase expression of full length SMN2 based on a gel separation analysis of PCR products obtained following a DdeI restriction digest; whereas certain oligonucleotides directed against a PRC2-associated region of SMN2 did not. (FIG. 5)

SMN ELISA (Enzo) assays were conducted and revealed that certain oligonucleotides directed against a PRC2-associated region of SMN2 alone did not significantly increase full length SMN protein 24h post-transfection in certain SMA patient fibroblasts. (FIG. 6) However, the same SMN ELISA assays showed that oligonucleotides directed against a PRC2-associated region of SMN2 in combination with a splice switching oligonucleotide (oligo 53 or 54) significantly increase full length SMN protein 24h post-transfection in SMA patient fibroblasts above that observed with splice switching oligonucleotides alone. (FIGS. 7 and 8). RT-PCR assays were conducted and showed that oligonucleotides directed against a PRC2-associated region of SMN2 in combination with a splice switching oligonucleotide (oligo 53 or 54) significantly increased SMN2 protein 24h post-transfection in SMA patient

fibroblasts. (FIG 9.) These experiments were conducted modified oligonucleotides with either alternating LNA and 2'OMe nucleotides or alternating DNA and LNA nucleotides.

In summary, the results of Example 2 show that certain oligos targeting PRC2 associated regions of SMN2 induce SMN RNA expression (e.g., of the SMN Δ 7 transcript) SMA patient-derived fibroblasts. The results also show that, in some embodiments, splice-switching oligos may not induce SMN RNA expression, but rather shift SMN RNA splicing to the full-length transcript. Finally, the results show that combination of splice switching oligos with PRC2-associated region targeting oligos substantially increases full length SMN protein in cells from SMA patients.

Table 4: Oligonucleotide sequences made for testing human cells obtained from subjects with Spinal Muscular Atrophy (See Table 3 for structural features of formatted sequence).

Oligo Name	Base Sequence	Formatted Sequence	SeqID
SMN1-01 m03	ATTCTCTTGA TGATGCTGAT	omeAs;omeUs;omeUs;omeCs;omeUs;omeCs;omeUs;omeUs;omeGs;omeAs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeU-Sup	13062
SMN1-02 m03	TTCTCTTGAT GATGCTGAT G	omeUs;omeUs;omeCs;omeUs;omeCs;omeUs;omeUs;omeGs;omeAs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeG-Sup	13063
SMN1-03 m03	TCTCTTGATG ATGCTGATGC	omeUs;omeCs;omeUs;omeCs;omeUs;omeUs;omeGs;omeAs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeC-Sup	13064
SMN1-04 m03	CTCTTGATGA TGCTGATGCT	omeCs;omeUs;omeCs;omeUs;omeUs;omeGs;omeAs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeU-Sup	13065
SMN1-05 m03	TCTTGATGAT GCTGATGCTT	omeUs;omeCs;omeUs;omeUs;omeGs;omeAs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeU-Sup	13066
SMN1-06 m03	CTTGATGATG CTGATGCTTT	omeCs;omeUs;omeUs;omeGs;omeAs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeU-Sup	13067
SMN1-07 m03	TTGATGATGC TGATGCTTTG	omeUs;omeUs;omeGs;omeAs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeU-Sup	13068
SMN1-08 m03	TGATGATGCT GATGCTTTGG	omeUs;omeGs;omeAs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeG-Sup	13069
SMN1-09 m03	GATGATGCT GATGCTTTGG G	omeGs;omeAs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeG-Sup	13070
SMN1-10 m03	ATGATGCTGA TGCTTTGGGA	omeAs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeG-Sup	13071

		;omeA-Sup	
SMN1-11 m03	TGATGCTGAT GCTTTGGGA A	omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeA-Sup	13072
SMN1-12 m03	GATGCTGAT GCTTTGGGA AG	omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeG-Sup	13073
SMN1-13 m03	ATGCTGATGC TTTGGGAAGT	omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeU-Sup	13074
SMN1-14 m03	TGCTGATGCT TTGGGAAGT A	omeUs;omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeUs;omeA-Sup	13075
SMN1-15 m03	GCTGATGCTT TGGGAAGTA T	omeGs;omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeUs;omeAs;omeU-Sup	13076
SMN1-16 m03	CTGATGCTTT GGGAAGTAT G	omeCs;omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeUs;omeAs;omeUs;omeG-Sup	13077
SMN1-17 m03	TGATGCTTTG GGAAGTATG T	omeUs;omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeUs;omeAs;omeUs;omeGs;omeU-Sup	13078
SMN1-18 m03	GATGCTTTGG GAAGTATGTT	omeGs;omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeUs;omeU-Sup	13079
SMN1-19 m03	ATGCTTTGGG AAGTATGTTA	omeAs;omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeUs;omeUs;omeA-Sup	13080
SMN1-20 m03	TGCTTTGGGA AGTATGTAA	omeUs;omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeUs;omeAs;omeUs;omeGs;omeUs;omeUs;omeAs;omeA-Sup	13081
SMN1-21 m03	GCTTTGGGA AGTATGTAA T	omeGs;omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeUs;omeAs;omeUs;omeGs;omeUs;omeUs;omeAs;omeAs;omeU-Sup	13082
SMN1-22 m03	CTTTGGGAA GTATGTAAAT T	omeCs;omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeUs;omeAs;omeUs;omeGs;omeUs;omeUs;omeAs;omeAs;omeUs;omeU-Sup	13083
SMN1-23 m03	TTTGGGAAGT ATGTAAATTT	omeUs;omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeCs;omeUs;omeAs;omeUs;omeGs;omeUs;omeUs;omeAs;omeAs;omeUs;omeUs;omeU-Sup	13084
SMN1-24 m03	TTGGGAAGT ATGTAAATTT C	omeUs;omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeUs;omeAs;omeUs;omeGs;omeUs;omeUs;omeUs;omeAs;omeAs;omeUs;omeUs;omeUs;omeC-Sup	13085
SMN1-25 m03	TGGGAAGTA TGTTAATTT A	omeUs;omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeUs;omeAs;omeCs;omeUs;omeGs;omeUs;omeUs;omeAs;omeAs;omeUs;omeUs;omeUs;omeCs;omeA-Sup	13086
SMN1-26 m03	GGAAGTAT GTAAATTTCA T	omeGs;omeGs;omeGs;omeAs;omeAs;omeGs;omeUs;omeAs;omeUs;omeGs;omeUs;omeUs;omeAs;omeAs;omeUs;omeCs;omeAs;omeU-Sup	13087
SMN1-27 m01	ATTCTTTGA TGATG	lNaAs;omeUs;lNaTs;omeCs;lNaTs;omeCs;lNaTs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaAs;omeUs;lNaG-Sup	11374
SMN1-	TTCTCTTGAT	lNaTs;omeUs;lNaCs;omeUs;lNaCs;omeUs;lNaTs;omeGs;lNaAs;omeUs;lNa	11375

28 m01	GATGC	Gs;omeAs;lNaTs;omeGs;lNaC-Sup	
SMN1-29 m01	TCTCTTGATG ATGCT	lNaTs;omeCs;lNaTs;omeCs;lNaTs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeCs;lNaT-Sup	11376
SMN1-30 m01	CTCTTGATGA TGCTG	lNaCs;omeUs;lNaCs;omeUs;lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaCs;omeUs;lNaG-Sup	11377
SMN1-31 m01	TCTTGATGAT GCTGA	lNaTs;omeCs;lNaTs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeCs;lNaTs;omeGs;lNaA-Sup	11378
SMN1-32 m01	CTTGATGATG CTGAT	lNaCs;omeUs;lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaCs;omeUs;lNaGs;omeAs;lNaT-Sup	11379
SMN1-33 m01	TTGATGATGC TGATG	lNaTs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeCs;lNaTs;omeGs;lNaAs;omeUs;lNaG-Sup	11380
SMN1-34 m01	TGATGATGCT GATGC	lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaCs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaC-Sup	11381
SMN1-35 m01	GATGATGCT GATGCT	lNaGs;omeAs;lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeCs;lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeCs;lNaT-Sup	11382
SMN1-36 m01	ATGATGCTGA TGCTT	lNaAs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaCs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaCs;omeUs;lNaT-Sup	11383
SMN1-37 m01	TGATGCTGAT GCTTT	lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeCs;lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeCs;lNaTs;omeUs;lNaT-Sup	11384
SMN1-38 m01	GATGCTGAT GCTTTG	lNaGs;omeAs;lNaTs;omeGs;lNaCs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaCs;omeUs;lNaTs;omeUs;lNaG-Sup	11385
SMN1-39 m01	ATGCTGATGC TTTGG	lNaAs;omeUs;lNaGs;omeCs;lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeCs;lNaTs;omeUs;lNaTs;omeGs;lNaG-Sup	11386
SMN1-40 m01	TGCTGATGCT TTGGG	lNaTs;omeGs;lNaCs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaCs;omeUs;lNaTs;omeUs;lNaGs;omeGs;lNaG-Sup	11387
SMN1-41 m01	GCTGATGCTT TGGGA	lNaGs;omeCs;lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeCs;lNaTs;omeUs;lNaTs;omeGs;lNaGs;omeGs;lNaA-Sup	11388
SMN1-42 m01	CTGATGCTTT GGGAA	lNaCs;omeUs;lNaGs;omeAs;lNaTs;omeGs;lNaCs;omeUs;lNaTs;omeUs;lNaGs;omeGs;lNaGs;omeAs;lNaA-Sup	11389
SMN1-43 m01	TGATGCTTTG GGAAG	lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeCs;lNaTs;omeUs;lNaTs;omeGs;lNaGs;omeCs;lNaAs;omeAs;lNaG-Sup	11390
SMN1-44 m01	GATGCTTTGG GAAGT	lNaGs;omeAs;lNaTs;omeGs;lNaCs;omeUs;lNaTs;omeUs;lNaGs;omeGs;lNaGs;omeAs;lNaAs;omeGs;lNaT-Sup	11391
SMN1-45 m01	ATGCTTTGGG AAGTA	lNaAs;omeUs;lNaGs;omeCs;lNaTs;omeUs;lNaTs;omeGs;lNaGs;omeGs;lNaAs;omeAs;lNaGs;omeUs;lNaA-Sup	11392
SMN1-46 m01	TGCTTTGGGA AGTAT	lNaTs;omeGs;lNaCs;omeUs;lNaTs;omeUs;lNaGs;omeGs;lNaGs;omeAs;lNaAs;omeGs;lNaTs;omeAs;lNaT-Sup	11393
SMN1-47 m02	GCTTTGGGA AGTATG	dGs;lNaCs;dTs;lNaTs;dTs;lNaGs;dGs;lNaGs;dAs;lNaAs;dGs;lNaTs;dAs;lNaTs;dG-Sup	11394
SMN1-47 m01	GCTTTGGGA AGTATG	lNaGs;omeCs;lNaTs;omeUs;lNaTs;omeGs;lNaGs;omeGs;lNaAs;omeAs;lNaGs;omeUs;lNaAs;omeUs;lNaG-Sup	11394
SMN1-48 m05	CTTTGGGAA GTATGT	dCs;lNaTs;dTs;lNaTs;dGs;lNaGs;dGs;lNaAs;dAs;lNaGs;dTs;lNaAs;dTs;lNaGs;dT-Sup	11395
SMN1-48 m01	CTTTGGGAA GTATGT	lNaCs;omeUs;lNaTs;omeUs;lNaGs;omeGs;lNaGs;omeAs;lNaAs;omeGs;lNaTs;omeAs;lNaTs;omeGs;lNaT-Sup	11395
SMN1-49 m01	TTTGGGAAGT ATGTT	lNaTs;omeUs;lNaTs;omeGs;lNaGs;omeGs;lNaAs;omeAs;lNaGs;omeUs;lNaAs;omeUs;lNaGs;omeUs;lNaT-Sup	11396
SMN1-50 m01	TTGGGAAGT ATGTTA	lNaTs;omeUs;lNaGs;omeGs;lNaGs;omeAs;lNaAs;omeGs;lNaTs;omeAs;lNaTs;omeGs;lNaTs;omeUs;lNaA-Sup	11397
SMN1-51 m01	TGGGAAGTA TGTTAA	lNaTs;omeGs;lNaGs;omeGs;lNaAs;omeAs;lNaGs;omeUs;lNaAs;omeUs;lNaGs;omeUs;lNaTs;omeAs;lNaA-Sup	11398
SMN1-	GGGGAAGTAT	lNaGs;omeGs;lNaGs;omeAs;lNaAs;omeGs;lNaTs;omeAs;lNaTs;omeGs;lNaTs	11399

52 m01	GTTAAT	s;omeUs;lnaAs;omeAs;lnaT-Sup	
SMN1-53 m02	TCAC TTCAT AATGCTGG	dTs;lnaCs;dAs;lnaCs;dTs;lnaTs;dTs;lnaCs;dAs;lnaTs;dAs;lnaAs;dTs;lnaGs; dCs;lnaTs;dGs;lnaG-Sup	13088
SMN1-53 m12	TCAC TTCAT AATGCTGG	lnaTs;dCs;lnaAs;dCs;lnaTs;dTs;lnaTs;dCs;lnaAs;dTs;lnaAs;dAs;lnaTs;dGs;l naCs;dTs;lnaGs;dG-Sup	13088
SMN1-54 m01	TCAC TTCAT AATGCTGG	lnaTs;omeCs;lnaAs;omeCs;lnaTs;omeUs;lnaTs;omeCs;lnaAs;omeUs;lnaA s;omeAs;lnaTs;omeGs;lnaCs;omeUs;lnaGs;omeG-Sup	13088
SMN1-53 m03	TCAC TTCAT AATGCTGG	omeUs;omeCs;omeAs;omeCs;omeUs;omeUs;omeUs;omeCs;omeAs;ome Us;omeAs;omeAs;omeUs;omeGs;omeCs;omeUs;omeGs;omeG-Sup	13088
SMN1-55 m01	TCAC TTCAT AATGC	lnaTs;omeCs;lnaAs;omeCs;lnaTs;omeUs;lnaTs;omeCs;lnaAs;omeUs;lnaA s;omeAs;lnaTs;omeGs;lnaC-Sup	13089
SMN1-56 m01	CACTTTCATA ATGCT	lnaCs;omeAs;lnaCs;omeUs;lnaTs;omeUs;lnaCs;omeAs;lnaTs;omeAs;lnaA s;omeUs;lnaGs;omeCs;lnaT-Sup	13090
SMN1-57 m02	ACTTTCATAA TGCTG	dAs;lnaCs;dTs;lnaTs;dTs;lnaCs;dAs;lnaTs;dAs;lnaAs;dTs;lnaGs;dCs;lnaTs; dG-Sup	13091
SMN1-57 m01	ACTTTCATAA TGCTG	lnaAs;omeCs;lnaTs;omeUs;lnaTs;omeCs;lnaAs;omeUs;lnaAs;omeAs;lnaT s;omeGs;lnaCs;omeUs;lnaG-Sup	13091
SMN1-58 m01	CTTTCATAAT GCTGG	lnaCs;omeUs;lnaTs;omeUs;lnaCs;omeAs;lnaTs;omeAs;lnaAs;omeUs;lnaG s;omeCs;lnaTs;omeGs;lnaG-Sup	13092
SMN1-59 m01	AGACCAGTTT TACCT	lnaAs;omeGs;lnaAs;omeCs;lnaCs;omeAs;lnaGs;omeUs;lnaTs;omeUs;lnaT s;omeAs;lnaCs;omeCs;lnaT-Sup	3650
SMN1-60 m01	CCTAGCTACT TTGAA	lnaCs;omeCs;lnaTs;omeAs;lnaGs;omeCs;lnaTs;omeAs;lnaCs;omeUs;lnaT s;omeUs;lnaGs;omeAs;lnaA-Sup	13093
SMN1-61 m01	TCCTAGCTAC TTTGA	lnaTs;omeCs;lnaCs;omeUs;lnaAs;omeGs;lnaCs;omeUs;lnaAs;omeCs;lnaT s;omeUs;lnaTs;omeGs;lnaA-Sup	13094
SMN1-62 m01	GAAATATTCC TTATA	lnaGs;omeAs;lnaAs;omeAs;lnaTs;omeAs;lnaTs;omeUs;lnaCs;omeCs;lnaT s;omeUs;lnaAs;omeUs;lnaA-Sup	10065
SMN1-63 m01	AAATATTCCT TATAG	lnaAs;omeAs;lnaAs;omeUs;lnaAs;omeUs;lnaTs;omeCs;lnaCs;omeUs;lnaT s;omeAs;lnaTs;omeAs;lnaG-Sup	10066
SMN1-64 m01	AATATTCCTT ATAGC	lnaAs;omeAs;lnaTs;omeAs;lnaTs;omeUs;lnaCs;omeCs;lnaTs;omeUs;lnaA s;omeUs;lnaAs;omeGs;lnaC-Sup	10067
SMN1-65 m01	ATATTCCTTA TAGCC	lnaAs;omeUs;lnaAs;omeUs;lnaTs;omeCs;lnaCs;omeUs;lnaTs;omeAs;lnaT s;omeAs;lnaGs;omeCs;lnaC-Sup	10068
SMN1-66 m01	TATTCCTTAT AGCCA	lnaTs;omeAs;lnaTs;omeUs;lnaCs;omeCs;lnaTs;omeUs;lnaAs;omeUs;lnaA s;omeGs;lnaCs;omeCs;lnaA-Sup	10069
SMN1-67 m01	ATTCTTATA GCCAG	lnaAs;omeUs;lnaTs;omeCs;lnaCs;omeUs;lnaTs;omeAs;lnaTs;omeAs;lnaG s;omeCs;lnaCs;omeAs;lnaG-Sup	10070
SMN1-68 m01	TTCCTTATAG CCAGG	lnaTs;omeUs;lnaCs;omeCs;lnaTs;omeUs;lnaAs;omeUs;lnaAs;omeGs;lnaC s;omeCs;lnaAs;omeGs;lnaG-Sup	10071
SMN1-69 m01	TCCTTATAGC CAGGT	lnaTs;omeCs;lnaCs;omeUs;lnaTs;omeAs;lnaTs;omeAs;lnaGs;omeCs;lnaC s;omeAs;lnaGs;omeGs;lnaT-Sup	10072
SMN1-70 m01	CCTTATAGCC AGGTC	lnaCs;omeCs;lnaTs;omeUs;lnaAs;omeUs;lnaAs;omeGs;lnaCs;omeCs;lnaA s;omeGs;lnaGs;omeUs;lnaC-Sup	10073
SMN1-71 m01	CTTATAGCCA GGTCT	lnaCs;omeUs;lnaTs;omeAs;lnaTs;omeAs;lnaGs;omeCs;lnaCs;omeAs;lnaG s;omeGs;lnaTs;omeCs;lnaT-Sup	10074
SMN1-72 m01	TTATAGCCAG GTCTA	lnaTs;omeUs;lnaAs;omeUs;lnaAs;omeGs;lnaCs;omeCs;lnaAs;omeGs;lna Gs;omeUs;lnaCs;omeUs;lnaA-Sup	10075
SMN1-73 m01	GCCAGGTCTA AAATT	lnaGs;omeCs;lnaCs;omeAs;lnaGs;omeGs;lnaTs;omeCs;lnaTs;omeAs;lnaA s;omeAs;lnaAs;omeUs;lnaT-Sup	10080
SMN1-74 m01	CCAGGTCTAA AATTC	lnaCs;omeCs;lnaAs;omeGs;lnaGs;omeUs;lnaCs;omeUs;lnaAs;omeAs;lna As;omeAs;lnaTs;omeUs;lnaC-Sup	10081
SMN1-	CAGGTCTAAA	lnaCs;omeAs;lnaGs;omeGs;lnaTs;omeCs;lnaTs;omeAs;lnaAs;omeAs;lnaA	10082

75 m01	ATTCA	s;omeUs;lNaTs;omeCs;lNaA-Sup	
SMN1-76 m01	GGTCTAAAAT TCAAT	lNaGs;omeGs;lNaTs;omeCs;lNaTs;omeAs;lNaAs;omeAs;lNaAs;omeUs;lNaTs;omeCs;lNaAs;omeAs;lNaT-Sup	10084
SMN1-77 m01	CTAAAATTCA ATGGC	lNaCs;omeUs;lNaAs;omeAs;lNaAs;omeAs;lNaTs;omeUs;lNaCs;omeAs;lNaAs;omeUs;lNaGs;omeGs;lNaC-Sup	10087
SMN1-77 m03	CTAAAATTCA ATGGC	omeCs;omeUs;omeAs;omeAs;omeAs;omeAs;omeUs;omeUs;omeCs;omeAs;omeAs;omeUs;omeGs;omeGs;omeC-Sup	10087
SMN1-78 m01	GGACCACCA GTAAGT	lNaGs;omeGs;lNaAs;omeCs;lNaCs;omeAs;lNaCs;omeCs;lNaAs;omeGs;lNaTs;omeAs;lNaAs;omeGs;lNaT-Sup	10168
SMN1-79 m02	GACCACCAGT AAGTA	dGs;lNaAs;dCs;lNaCs;dAs;lNaCs;dCs;lNaAs;dGs;lNaTs;dAs;lNaAs;dGs;lNaTs;dA-Sup	10169
SMN1-79 m01	GACCACCAGT AAGTA	lNaGs;omeAs;lNaCs;omeCs;lNaAs;omeCs;lNaCs;omeAs;lNaGs;omeUs;lNaAs;omeAs;lNaGs;omeUs;lNaA-Sup	10169
SMN1-80 m02	ACCACCAGTA AGTAA	dAs;lNaCs;dCs;lNaAs;dCs;lNaCs;dAs;lNaGs;dTs;lNaAs;dAs;lNaGs;dTs;lNaAs;dA-Sup	10170
SMN1-80 m01	ACCACCAGTA AGTAA	lNaAs;omeCs;lNaCs;omeAs;lNaCs;omeCs;lNaAs;omeGs;lNaTs;omeAs;lNaAs;omeGs;lNaTs;omeAs;lNaA-Sup	10170
SMN1-81 m01	TTCTGTTACC CAGAT	lNaTs;omeUs;lNaCs;omeUs;lNaGs;omeUs;lNaTs;omeAs;lNaCs;omeCs;lNaCs;omeAs;lNaGs;omeAs;lNaT-Sup	10337
SMN1-82 m01	TCTGTTACCC AGATG	lNaTs;omeCs;lNaTs;omeGs;lNaTs;omeUs;lNaAs;omeCs;lNaCs;omeCs;lNaAs;omeGs;lNaAs;omeUs;lNaG-Sup	10338
SMN1-83 m01	CTGTTACCCA GATGC	lNaCs;omeUs;lNaGs;omeUs;lNaTs;omeAs;lNaCs;omeCs;lNaCs;omeAs;lNaGs;omeAs;lNaTs;omeGs;lNaC-Sup	10339
SMN1-84 m02	TTTTTAGGTA TTAAC	dTs;lNaTs;dTs;lNaTs;dTs;lNaAs;dGs;lNaGs;dTs;lNaAs;dTs;lNaTs;dAs;lNaAs;dC-Sup	10763
SMN1-84 m01	TTTTTAGGTA TTAAC	lNaTs;omeUs;lNaTs;omeUs;lNaTs;omeAs;lNaGs;omeGs;lNaTs;omeAs;lNaTs;omeUs;lNaAs;omeAs;lNaC-Sup	10763
SMN1-85 m01	TTTTAGGTAT TAACA	lNaTs;omeUs;lNaTs;omeUs;lNaAs;omeGs;lNaGs;omeUs;lNaAs;omeUs;lNaTs;omeAs;lNaAs;omeCs;lNaA-Sup	10764
SMN1-86 m01	CATAGCTTCA TAGTG	lNaCs;omeAs;lNaTs;omeAs;lNaGs;omeCs;lNaTs;omeUs;lNaCs;omeAs;lNaTs;omeAs;lNaGs;omeUs;lNaG-Sup	10949
SMN1-87 m01	TAGCTTCATA GTGGA	lNaTs;omeAs;lNaGs;omeCs;lNaTs;omeUs;lNaCs;omeAs;lNaTs;omeAs;lNaGs;omeUs;lNaGs;omeGs;lNaA-Sup	10951
SMN1-88 m01	AGCTTCATAG TGGA	lNaAs;omeGs;lNaCs;omeUs;lNaTs;omeCs;lNaAs;omeUs;lNaAs;omeGs;lNaTs;omeGs;lNaGs;omeAs;lNaA-Sup	10952
SMN1-89 m01	GCTTCATAGT GGAAC	lNaGs;omeCs;lNaTs;omeUs;lNaCs;omeAs;lNaTs;omeAs;lNaGs;omeUs;lNaGs;omeGs;lNaAs;omeAs;lNaC-Sup	10953
SMN1-90 m01	CTTCATAGTG GAACA	lNaCs;omeUs;lNaTs;omeCs;lNaAs;omeUs;lNaAs;omeGs;lNaTs;omeGs;lNaGs;omeAs;lNaAs;omeCs;lNaA-Sup	10954
SMN1-91 m01	TCATGGTACA TGAGT	lNaTs;omeCs;lNaAs;omeUs;lNaGs;omeGs;lNaTs;omeAs;lNaCs;omeAs;lNaTs;omeGs;lNaAs;omeGs;lNaT-Sup	11415
SMN1-92 m01	TGGTACATGA GTGGC	lNaTs;omeGs;lNaGs;omeUs;lNaAs;omeCs;lNaAs;omeUs;lNaGs;omeAs;lNaGs;omeUs;lNaGs;omeGs;lNaC-Sup	11418
SMN1-93 m02	GGTACATGA GTGGCT	dGs;lNaGs;dTs;lNaAs;dCs;lNaAs;dTs;lNaGs;dAs;lNaGs;dTs;lNaGs;dGs;lNaCs;dT-Sup	11419
SMN1-93 m01	GGTACATGA GTGGCT	lNaGs;omeGs;lNaTs;omeAs;lNaCs;omeAs;lNaTs;omeGs;lNaAs;omeGs;lNaTs;omeGs;lNaGs;omeCs;lNaT-Sup	11419
SMN1-94 m01	TACATGAGTG GCTAT	lNaTs;omeAs;lNaCs;omeAs;lNaTs;omeGs;lNaAs;omeGs;lNaTs;omeGs;lNaGs;omeCs;lNaTs;omeAs;lNaT-Sup	11421
SMN1-95 m01	ACATGAGTG GCTATC	lNaAs;omeCs;lNaAs;omeUs;lNaGs;omeAs;lNaGs;omeUs;lNaGs;omeGs;lNaCs;omeUs;lNaAs;omeUs;lNaC-Sup	11422
SMN1-	CATGAGTGG	lNaCs;omeAs;lNaTs;omeGs;lNaAs;omeGs;lNaTs;omeGs;lNaGs;omeCs;lNaTs;omeGs;lNaAs;omeCs;lNaT-Sup	11423

96 m01	CTATCA	s;omeAs;lNaTs;omeCs;lNaA-Sup	
SMN1-97 m01	CTGGCTATTA TATGG	lNaCs;omeUs;lNaGs;omeGs;lNaCs;omeUs;lNaAs;omeUs;lNaTs;omeAs;lNaTs;omeAs;lNaTs;omeGs;lNaG-Sup	11440
SMN1-98 m01	TGGCTATTAT ATGGT	lNaTs;omeGs;lNaGs;omeCs;lNaTs;omeAs;lNaTs;omeUs;lNaAs;omeUs;lNaA s;omeUs;lNaGs;omeGs;lNaT-Sup	11441
SMN1-99 m01	GGCTATTATA TGGTA	lNaGs;omeGs;lNaCs;omeUs;lNaAs;omeUs;lNaTs;omeAs;lNaTs;omeAs;lNaT s;omeGs;lNaGs;omeUs;lNaA-Sup	11442
SMN1-100 m01	GCTATTATAT GGTAA	lNaGs;omeCs;lNaTs;omeAs;lNaTs;omeUs;lNaAs;omeUs;lNaAs;omeUs;lNa Gs;omeGs;lNaTs;omeAs;lNaA-Sup	11443
SMN1-101 m01	GTATCATCTG TGTGT	lNaGs;omeUs;lNaAs;omeUs;lNaCs;omeAs;lNaTs;omeCs;lNaTs;omeGs;lNaT s;omeGs;lNaTs;omeGs;lNaT-Sup	12369
SMN1-102 m01	GCTTTGGGA AGTATGTTTT TCACTTTCAT AATGCTGG	lNaGs;omeCs;lNaTs;omeUs;lNaTs;omeGs;lNaGs;omeGs;lNaAs;omeAs;lNa Gs;omeUs;lNaAs;omeUs;lNaG;dT;dT;dT;lNaTs;omeCs;lNaAs;omeCs;lNa Ts;omeUs;lNaTs;omeCs;lNaAs;omeUs;lNaAs;omeAs;lNaTs;omeGs;lNaCs;o meUs;lNaGs;omeG-Sup	13097
SMN1-103 m01	CTTTGGGAA GTATGTTTT TCACTTTCAT AATGCTGG	lNaCs;omeUs;lNaTs;omeUs;lNaGs;omeGs;lNaGs;omeAs;lNaAs;omeGs;lNa Ts;omeAs;lNaTs;omeGs;lNaT;dT;dT;dT;lNaTs;omeCs;lNaAs;omeCs;lNaT s;omeUs;lNaTs;omeCs;lNaAs;omeUs;lNaAs;omeAs;lNaTs;omeGs;lNaCs;o meUs;lNaGs;omeG-Sup	13102
SMN1-104 m01	GGTACATGA GTGGCTTTTT TCACTTTCAT AATGCTGG	lNaGs;omeGs;lNaTs;omeAs;lNaCs;omeAs;lNaTs;omeGs;lNaAs;omeGs;lNaT s;omeGs;lNaGs;omeCs;lNaT;dT;dT;dT;lNaTs;omeCs;lNaAs;omeCs;lNaTs ;omeUs;lNaTs;omeCs;lNaAs;omeUs;lNaAs;omeAs;lNaTs;omeGs;lNaCs;om eUs;lNaGs;omeG-Sup	13099
SMN1-105 m01	TGATGCTGAT GCTTTTTTCT AAAATTCAAT GGC	lNaTs;omeGs;lNaAs;omeUs;lNaGs;omeCs;lNaTs;omeGs;lNaAs;omeUs;lNa Gs;omeCs;lNaTs;omeUs;lNaT;dT;dT;dT;lNaCs;omeUs;lNaAs;omeAs;lNa As;omeAs;lNaTs;omeUs;lNaCs;omeAs;lNaAs;omeUs;lNaGs;omeGs;lNaC- Sup	13103
SMN1-106 m01	CTAAAATTCA ATGGCTTTTC TAAAATTCAA TGGC	lNaCs;omeUs;lNaAs;omeAs;lNaAs;omeAs;lNaTs;omeUs;lNaCs;omeAs;lNaA s;omeUs;lNaGs;omeGs;lNaC;dT;dT;dT;lNaCs;omeUs;lNaAs;omeAs;lNaA s;omeAs;lNaTs;omeUs;lNaCs;omeAs;lNaAs;omeUs;lNaGs;omeGs;lNaC- Sup	13104
SMN1-107 m01	CTGTTACCCA GATGCTTTTC TAAAATTCAA TGGC	lNaCs;omeUs;lNaGs;omeUs;lNaTs;omeAs;lNaCs;omeCs;lNaCs;omeAs;lNaG s;omeAs;lNaTs;omeGs;lNaC;dT;dT;dT;lNaCs;omeUs;lNaAs;omeAs;lNaA s;omeAs;lNaTs;omeUs;lNaCs;omeAs;lNaAs;omeUs;lNaGs;omeGs;lNaC- Sup	13105
SMN1-108 m01	CTTCATAGTG GAACATTTTC TAAAATTCAA TGGC	lNaCs;omeUs;lNaTs;omeCs;lNaAs;omeUs;lNaAs;omeGs;lNaTs;omeGs;lNaG s;omeAs;lNaAs;omeCs;lNaA;dT;dT;dT;lNaCs;omeUs;lNaAs;omeAs;lNaA s;omeAs;lNaTs;omeUs;lNaCs;omeAs;lNaAs;omeUs;lNaGs;omeGs;lNaC- Sup	13106
SMN1-109 m01	TCACTTTCAT AATGCTGGTT TTTCACTTTC ATAATGCTGG	lNaTs;omeCs;lNaAs;omeCs;lNaTs;omeUs;lNaTs;omeCs;lNaAs;omeUs;lNaA s;omeAs;lNaTs;omeGs;lNaCs;omeUs;lNaGs;omeG;dT;dT;dT;lNaTs;ome Cs;lNaAs;omeCs;lNaTs;omeUs;lNaTs;omeCs;lNaAs;omeUs;lNaAs;omeAs;lNa aTs;omeGs;lNaCs;omeUs;lNaGs;omeG-Sup	13107
SMN1-110 m01	AAATTCAATG GCCCCA	lNaAs;omeAs;lNaAs;omeUs;lNaTs;omeCs;lNaAs;omeAs;lNaTs;omeGs;lNaG s;omeCs;lNaCs;omeCs;lNaA-Sup	10090
SMN1-111 m01	AATTCAATGG CCCAC	lNaAs;omeAs;lNaTs;omeUs;lNaCs;omeAs;lNaAs;omeUs;lNaGs;omeGs;lNa Cs;omeCs;lNaCs;omeAs;lNaC-Sup	10091
SMN1-112	ATTCAATGGC CCACC	lNaAs;omeUs;lNaTs;omeCs;lNaAs;omeAs;lNaTs;omeGs;lNaGs;omeCs;lNaC s;omeCs;lNaAs;omeCs;lNaC-Sup	10092

m01			
SMN1-113 m01	TTCAATGGCC CACCA	lnaTs;omeUs;lnaCs;omeAs;lnaAs;omeUs;lnaGs;omeGs;lnaCs;omeCs;lnaCs;omeAs;lnaCs;omeCs;lnaA-Sup	10093
SMN1-114 m01	TCAATGGCCC ACCAC	lnaTs;omeCs;lnaAs;omeAs;lnaTs;omeGs;lnaGs;omeCs;lnaCs;omeCs;lnaA s;omeCs;lnaCs;omeAs;lnaC-Sup	10094
SMN1-115 m01	CAATGGCCCA CCACC	lnaCs;omeAs;lnaAs;omeUs;lnaGs;omeGs;lnaCs;omeCs;lnaCs;omeAs;lnaC s;omeCs;lnaAs;omeCs;lnaC-Sup	10095
SMN1-116 m01	AATGGCCCA CACCG	lnaAs;omeAs;lnaTs;omeGs;lnaGs;omeCs;lnaCs;omeCs;lnaAs;omeCs;lnaC s;omeAs;lnaCs;omeCs;lnaG-Sup	10096
SMN1-117 m01	ATGGCCCA ACCGC	lnaAs;omeUs;lnaGs;omeGs;lnaCs;omeCs;lnaCs;omeAs;lnaCs;omeCs;lnaA s;omeCs;lnaCs;omeGs;lnaC-Sup	10097
SMN1-118 m01	AATGCCTTTC TGTTA	lnaAs;omeAs;lnaTs;omeGs;lnaCs;omeCs;lnaTs;omeUs;lnaTs;omeCs;lnaTs ;omeGs;lnaTs;omeUs;lnaA-Sup	10330
SMN1-119 m01	ATGCCTTCT GTTAC	lnaAs;omeUs;lnaGs;omeCs;lnaCs;omeUs;lnaTs;omeUs;lnaCs;omeUs;lna Gs;omeUs;lnaTs;omeAs;lnaC-Sup	10331
SMN1-120 m01	TGCCTTCTG TTACC	lnaTs;omeGs;lnaCs;omeCs;lnaTs;omeUs;lnaTs;omeCs;lnaTs;omeGs;lnaTs ;omeUs;lnaAs;omeCs;lnaC-Sup	10332
SMN1-121 m01	GCCTTCTGT TACCC	lnaGs;omeCs;lnaCs;omeUs;lnaTs;omeUs;lnaCs;omeUs;lnaGs;omeUs;lnaT s;omeAs;lnaCs;omeCs;lnaC-Sup	10333
SMN1-122 m01	CCTTCTGTT ACCCA	lnaCs;omeCs;lnaTs;omeUs;lnaTs;omeCs;lnaTs;omeGs;lnaTs;omeUs;lnaA s;omeCs;lnaCs;omeCs;lnaA-Sup	10334
SMN1-123 m01	CTTCTGTTA CCCAG	lnaCs;omeUs;lnaTs;omeUs;lnaCs;omeUs;lnaGs;omeUs;lnaTs;omeAs;lnaC s;omeCs;lnaCs;omeAs;lnaG-Sup	10335
SMN1-124 m01	TTTCTGTAC CCAGA	lnaTs;omeUs;lnaTs;omeCs;lnaTs;omeGs;lnaTs;omeUs;lnaAs;omeCs;lnaC s;omeCs;lnaAs;omeGs;lnaA-Sup	10336
SMN1-125 m01	TGTTACCCAG ATGCA	lnaTs;omeGs;lnaTs;omeUs;lnaAs;omeCs;lnaCs;omeCs;lnaAs;omeGs;lnaA s;omeUs;lnaGs;omeCs;lnaA-Sup	10340
SMN1-126 m01	GTTACCCAGA TGCAG	lnaGs;omeUs;lnaTs;omeAs;lnaCs;omeCs;lnaCs;omeAs;lnaGs;omeAs;lnaT s;omeGs;lnaCs;omeAs;lnaG-Sup	10341
SMN1-127 m01	TTACCCAGAT GCAGT	lnaTs;omeUs;lnaAs;omeCs;lnaCs;omeCs;lnaAs;omeGs;lnaAs;omeUs;lnaG s;omeCs;lnaAs;omeGs;lnaT-Sup	10342
SMN1-128 m01	ACCCAGATGC AGTGC	lnaAs;omeCs;lnaCs;omeCs;lnaAs;omeGs;lnaAs;omeUs;lnaGs;omeCs;lnaA s;omeGs;lnaTs;omeGs;lnaC-Sup	10344
SMN1-129 m01	CCCAGATGCA GTGCT	lnaCs;omeCs;lnaCs;omeAs;lnaGs;omeAs;lnaTs;omeGs;lnaCs;omeAs;lnaG s;omeUs;lnaGs;omeCs;lnaT-Sup	10345

SMN1-130 m01	CCAGATGCA GTGCTC	lnaCs;omeCs;lnaAs;omeGs;lnaAs;omeUs;lnaGs;omeCs;lnaAs;omeGs;lnaTs;omeCs;lnaCs;omeUs;lnaC-Sup	10346
SMN1-131 m01	CAGATGCAGT GCTCT	lnaCs;omeAs;lnaGs;omeAs;lnaTs;omeGs;lnaCs;omeAs;lnaGs;omeUs;lnaGs;omeCs;lnaTs;omeCs;lnaT-Sup	10347
SMN1-132 m01	AGATGCAGT GCTCTT	lnaAs;omeGs;lnaAs;omeUs;lnaGs;omeCs;lnaAs;omeGs;lnaTs;omeGs;lnaCs;omeUs;lnaCs;omeUs;lnaT-Sup	10348
SMN1-133 m01	TTTTACTCAT AGCTT	lnaTs;omeUs;lnaTs;omeUs;lnaAs;omeCs;lnaTs;omeCs;lnaAs;omeUs;lnaAs;omeGs;lnaCs;omeUs;lnaT-Sup	10942
SMN1-134 m01	TTTACTCATA GCTTC	lnaTs;omeUs;lnaTs;omeAs;lnaCs;omeUs;lnaCs;omeAs;lnaTs;omeAs;lnaGs;omeCs;lnaTs;omeUs;lnaC-Sup	10943
SMN1-135 m01	TTACTCATAG CTTCA	lnaTs;omeUs;lnaAs;omeCs;lnaTs;omeCs;lnaAs;omeUs;lnaAs;omeGs;lnaCs;omeUs;lnaTs;omeCs;lnaA-Sup	10944
SMN1-136 m01	TACTCATAGC TTCAT	lnaTs;omeAs;lnaCs;omeUs;lnaCs;omeAs;lnaTs;omeAs;lnaGs;omeCs;lnaTs;omeUs;lnaCs;omeAs;lnaT-Sup	10945
SMN1-137 m01	ACTCATAGCT TCATA	lnaAs;omeCs;lnaTs;omeCs;lnaAs;omeUs;lnaAs;omeGs;lnaCs;omeUs;lnaTs;omeCs;lnaAs;omeUs;lnaA-Sup	10946
SMN1-138 m01	CTCATAGCTT CATAG	lnaCs;omeUs;lnaCs;omeAs;lnaTs;omeAs;lnaGs;omeCs;lnaTs;omeUs;lnaCs;omeAs;lnaTs;omeAs;lnaG-Sup	10947
SMN1-139 m01	TCATAGCTTC ATAGT	lnaTs;omeCs;lnaAs;omeUs;lnaAs;omeGs;lnaCs;omeUs;lnaTs;omeCs;lnaAs;omeUs;lnaAs;omeGs;lnaT-Sup	10948
SMN1-140 m01	ATAGCTTCAT AGTGG	lnaAs;omeUs;lnaAs;omeGs;lnaCs;omeUs;lnaTs;omeCs;lnaAs;omeUs;lnaAs;omeGs;lnaTs;omeGs;lnaG-Sup	10950
SMN1-141 m01	TTCATAGTGG AACAG	lnaTs;omeUs;lnaCs;omeAs;lnaTs;omeAs;lnaGs;omeUs;lnaGs;omeGs;lnaAs;omeAs;lnaCs;omeAs;lnaG-Sup	10955
SMN1-142 m01	TCATAGTGGA ACAGA	lnaTs;omeCs;lnaAs;omeUs;lnaAs;omeGs;lnaTs;omeGs;lnaGs;omeAs;lnaAs;omeCs;lnaAs;omeGs;lnaA-Sup	10956
SMN1-143 m01	CATAGTGGA ACAGAT	lnaCs;omeAs;lnaTs;omeAs;lnaGs;omeUs;lnaGs;omeGs;lnaAs;omeAs;lnaCs;omeAs;lnaGs;omeAs;lnaT-Sup	10957
SMN1-144 m01	ATAGTGGA CAGATA	lnaAs;omeUs;lnaAs;omeGs;lnaTs;omeGs;lnaGs;omeAs;lnaAs;omeCs;lnaAs;omeGs;lnaAs;omeUs;lnaA-Sup	10958
SMN1-145 m01	TAGTGGAAC AGATAC	lnaTs;omeAs;lnaGs;omeUs;lnaGs;omeGs;lnaAs;omeAs;lnaCs;omeAs;lnaGs;omeAs;lnaTs;omeAs;lnaC-Sup	10959
SMN1-146 m01	AGTGGAACA GATACA	lnaAs;omeGs;lnaTs;omeGs;lnaGs;omeAs;lnaAs;omeCs;lnaAs;omeGs;lnaAs;omeUs;lnaAs;omeCs;lnaA-Sup	10960
SMN1-147	GTGGAACAG ATACAT	lnaGs;omeUs;lnaGs;omeGs;lnaAs;omeAs;lnaCs;omeAs;lnaGs;omeAs;lnaTs;omeAs;lnaCs;omeAs;lnaT-Sup	10961

m01			
SMN1-148 m01	TGGAACAGATACATA	InaTs;omeGs;InaGs;omeAs;InaAs;omeCs;InaAs;omeGs;InaAs;omeUs;InaAs;omeCs;InaAs;omeUs;InaA-Sup	10962
SMN1-149 m01	TGTCCAGATTCTCTT	InaTs;omeGs;InaTs;omeCs;InaCs;omeAs;InaGs;omeAs;InaTs;omeUs;InaCs;omeUs;InaCs;omeUs;InaT-Sup	11367
SMN1-150 m01	GTCCAGATTCCTTG	InaGs;omeUs;InaCs;omeCs;InaAs;omeGs;InaAs;omeUs;InaTs;omeCs;InaTs;omeCs;InaTs;omeUs;InaG-Sup	11368
SMN1-151 m01	TCCAGATTCTCTTGA	InaTs;omeCs;InaCs;omeAs;InaGs;omeAs;InaTs;omeUs;InaCs;omeUs;InaCs;omeUs;InaCs;omeUs;InaA-Sup	11369
SMN1-152 m01	CCAGATTCTCTTGAT	InaCs;omeCs;InaAs;omeGs;InaAs;omeUs;InaTs;omeCs;InaTs;omeCs;InaTs;omeCs;InaTs;omeUs;InaGs;omeAs;InaT-Sup	11370
SMN1-153 m01	CAGATTCTCTTGATG	InaCs;omeAs;InaGs;omeAs;InaTs;omeUs;InaCs;omeUs;InaCs;omeUs;InaTs;omeGs;InaAs;omeUs;InaG-Sup	11371
SMN1-154 m01	AGATTCTCTTGATGA	InaAs;omeGs;InaAs;omeUs;InaTs;omeCs;InaTs;omeCs;InaTs;omeUs;InaGs;omeAs;InaTs;omeGs;InaA-Sup	11372
SMN1-155 m01	GATTCTCTTGATGAT	InaGs;omeAs;InaTs;omeUs;InaCs;omeUs;InaCs;omeUs;InaTs;omeGs;InaA;s;omeUs;InaGs;omeAs;InaT-Sup	11373
SMN1-156 m01	GGAAGTATGTTAATT	InaGs;omeGs;InaAs;omeAs;InaGs;omeUs;InaAs;omeUs;InaGs;omeUs;InaTs;omeAs;InaAs;omeUs;InaT-Sup	11400
SMN1-157 m01	GAAGTATGTTAATTT	InaGs;omeAs;InaAs;omeGs;InaTs;omeAs;InaTs;omeGs;InaTs;omeUs;InaAs;s;omeAs;InaTs;omeUs;InaT-Sup	11401
SMN1-158 m01	AAGTATGTTAATTTTC	InaAs;omeAs;InaGs;omeUs;InaAs;omeUs;InaGs;omeUs;InaTs;omeAs;InaAs;omeUs;InaTs;omeUs;InaC-Sup	11402
SMN1-159 m01	AGTATGTTAATTTTCA	InaAs;omeGs;InaTs;omeAs;InaTs;omeGs;InaTs;omeUs;InaAs;omeAs;InaTs;s;omeUs;InaTs;omeCs;InaA-Sup	11403
SMN1-160 m01	GTATGTTAATTTCAT	InaGs;omeUs;InaAs;omeUs;InaGs;omeUs;InaTs;omeAs;InaAs;omeUs;InaTs;omeUs;InaCs;omeAs;InaT-Sup	11404
SMN1-161 m01	TATGTTAATTTCATG	InaTs;omeAs;InaTs;omeGs;InaTs;omeUs;InaAs;omeAs;InaTs;omeUs;InaTs;s;omeCs;InaAs;omeUs;InaG-Sup	11405
SMN1-162 m01	ATGTTAATTTTCATGG	InaAs;omeUs;InaGs;omeUs;InaTs;omeAs;InaAs;omeUs;InaTs;omeUs;InaCs;omeAs;InaTs;omeGs;InaG-Sup	11406
SMN1-163 m01	TGAAATATTCCTTAT	InaTs;omeGs;InaAs;omeAs;InaAs;omeUs;InaAs;omeUs;InaTs;omeCs;InaCs;s;omeUs;InaTs;omeAs;InaT-Sup	10064
SMN1-164 m01	TATAGCCAGGTCTAA	InaTs;omeAs;InaTs;omeAs;InaGs;omeCs;InaCs;omeAs;InaGs;omeGs;InaTs;s;omeCs;InaTs;omeAs;InaA-Sup	10076

SMN1-165 m01	ATAGCCAGGT CTAAA	InaAs;omeUs;InaAs;omeGs;InaCs;omeCs;InaAs;omeGs;InaGs;omeUs;InaCs;omeUs;InaAs;omeAs;InaA-Sup	10077
SMN1-166 m01	AGGTCTAAAA TTCAA	InaAs;omeGs;InaGs;omeUs;InaCs;omeUs;InaAs;omeAs;InaAs;omeAs;InaTs;omeUs;InaCs;omeAs;InaA-Sup	10083
SMN1-167 m01	GTCTAAAATT CAATG	InaGs;omeUs;InaCs;omeUs;InaAs;omeAs;InaAs;omeAs;InaTs;omeUs;InaCs;omeAs;InaAs;omeUs;InaG-Sup	10085
SMN1-168 m01	TCTAAAATTC AATGG	InaTs;omeCs;InaTs;omeAs;InaAs;omeAs;InaAs;omeUs;InaTs;omeCs;InaAs;omeAs;InaTs;omeGs;InaG-Sup	10086
SMN1-169 m01	TAAAATTCAA TGGCC	InaTs;omeAs;InaAs;omeAs;InaAs;omeUs;InaTs;omeCs;InaAs;omeAs;InaTs;omeGs;InaGs;omeCs;InaC-Sup	10088
SMN1-170 m01	AAAATTCAAT GGCCC	InaAs;omeAs;InaAs;omeAs;InaTs;omeUs;InaCs;omeAs;InaAs;omeUs;InaGs;omeGs;InaCs;omeCs;InaC-Sup	10089
unc-232 m12	CTACGCGTCG ACGGT	InaCs;dTs;InaAs;dCs;InaGs;dCs;InaGs;dTs;InaCs;dGs;InaAs;dCs;InaGs;dGs;InaT-Sup	13095
unc-232 m01	CTACGCGTCG ACGGT	InaCs;omeUs;InaAs;omeCs;InaGs;omeCs;InaGs;omeUs;InaCs;omeGs;InaAs;omeCs;InaGs;omeGs;InaT-Sup	13095
unc-293 m12	CCGATTCGCG CGTAA	InaCs;dCs;InaGs;dAs;InaTs;dTs;InaCs;dGs;InaCs;dGs;InaCs;dGs;InaTs;dAs;InaA-Sup	13096
unc-293 m01	CCGATTCGCG CGTAA	InaCs;omeCs;InaGs;omeAs;InaTs;omeUs;InaCs;omeGs;InaCs;omeGs;InaCs;omeGs;InaTs;omeAs;InaA-Sup	13096

The foregoing written specification is considered to be sufficient to enable one skilled in the art to practice the invention. The present invention is not to be limited in scope by examples provided, since the examples are intended as a single illustration of one aspect of the invention and other functionally equivalent embodiments are within the scope of the invention. Various modifications of the invention in addition to those shown and described herein will become apparent to those skilled in the art from the foregoing description and fall within the scope of the appended claims. The advantages and objects of the invention are not necessarily encompassed by each embodiment of the invention.

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13062	SMN1-01 m3	0.81267195	0.135251351	SMN1	100	RPTEC	48	qRTPCR	
13063	SMN1-02 m3	0.76000032	0.042993212	SMN1	100	RPTEC	48	qRTPCR	
13064	SMN1-03 m3	1.90722975	0.525939296	SMN1	100	RPTEC	48	qRTPCR	
13065	SMN1-04 m3	1.04830652	0.243934543	SMN1	100	RPTEC	48	qRTPCR	
13066	SMN1-05 m3	0.87674753	0.087392504	SMN1	100	RPTEC	48	qRTPCR	
13067	SMN1-06 m3	0.70465989	0.087244119	SMN1	100	RPTEC	48	qRTPCR	
13068	SMN1-07 m3	0.68208573	0.084885351	SMN1	100	RPTEC	48	qRTPCR	
13069	SMN1-08 m3	0.94871489	0.142708231	SMN1	100	RPTEC	48	qRTPCR	
13070	SMN1-09 m3	0.77519063	0.098695118	SMN1	100	RPTEC	48	qRTPCR	
13071	SMN1-10 m3	0.94573099	0.08072952	SMN1	100	RPTEC	48	qRTPCR	
13072	SMN1-11 m3	0.77443698	0.038076182	SMN1	100	RPTEC	48	qRTPCR	
13073	SMN1-12 m3	0.98142948	0.07937384	SMN1	100	RPTEC	48	qRTPCR	
13074	SMN1-13 m3	0.74963644	0.103277003	SMN1	100	RPTEC	48	qRTPCR	
13075	SMN1-14	0.77163518	0.086041959	SMN1	100	RPTEC	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m03								
13076	SMN1-15 m03	0.89149196	0.039822032	SMN1	100	RPTEC	48	qRTPCR	
13077	SMN1-16 m03	0.73721881	0.038067583	SMN1	100	RPTEC	48	qRTPCR	
13078	SMN1-17 m03	1.21906565	0.281987674	SMN1	100	RPTEC	48	qRTPCR	
13079	SMN1-18 m03	0.86844194	0.088184698	SMN1	100	RPTEC	48	qRTPCR	
13080	SMN1-19 m03	0.70551245	0.064966675	SMN1	100	RPTEC	48	qRTPCR	
13081	SMN1-20 m03	0.82630345	0.062998254	SMN1	100	RPTEC	48	qRTPCR	
13082	SMN1-21 m03	0.86556645	0.209455026	SMN1	100	RPTEC	48	qRTPCR	
13083	SMN1-22 m03	0.63993485	0.011679891	SMN1	100	RPTEC	48	qRTPCR	
13084	SMN1-23 m03	0.74803184	0.083732479	SMN1	100	RPTEC	48	qRTPCR	
13085	SMN1-24 m03	0.9074366	0.24201681	SMN1	100	RPTEC	48	qRTPCR	
13086	SMN1-25 m03	0.90885481	0.076026035	SMN1	100	RPTEC	48	qRTPCR	
13087	SMN1-26 m03	0.87644407	0.070457909	SMN1	100	RPTEC	48	qRTPCR	
11374	SMN1-27 m01	0.8343657	0.108102871	SMN1	100	RPTEC	48	qRTPCR	
11375	SMN1-28	1.05834369	0.208931576	SMN1	100	RPTEC	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
11376	SMN1-29 m01	0.712226859	0.077572838	SMN1	100	RPTEC	48	qRTPCR	
11377	SMN1-30 m01	0.86950411	0.147682779	SMN1	100	RPTEC	48	qRTPCR	
	Ctrl Un	0.77123156	0.109637491	SMN1	100	RPTEC	48	qRTPCR	
	Ctrl Lipo	1	0.0256921	SMN1	100	RPTEC	48	qRTPCR	
13062	SMN1-01 m03	0.8570321	0.027318737	SMN1	50	RPTEC	48	qRTPCR	
13063	SMN1-02 m03	0.98713845	0.068187998	SMN1	50	RPTEC	48	qRTPCR	
13064	SMN1-03 m03	1.13275826	0.094640177	SMN1	50	RPTEC	48	qRTPCR	
13065	SMN1-04 m03	1.32240727	0.100022392	SMN1	50	RPTEC	48	qRTPCR	
13066	SMN1-05 m03	1.16712034	0.069814091	SMN1	50	RPTEC	48	qRTPCR	
13067	SMN1-06 m03	1.11194006	0.088571377	SMN1	50	RPTEC	48	qRTPCR	
13068	SMN1-07 m03	0.97585355	0.034178542	SMN1	50	RPTEC	48	qRTPCR	
13069	SMN1-08 m03	1.31208044	0.058464993	SMN1	50	RPTEC	48	qRTPCR	
13070	SMN1-09 m03	1.68573162	0.014884028	SMN1	50	RPTEC	48	qRTPCR	
13071	SMN1-10 m03	1.5745269	0.123062684	SMN1	50	RPTEC	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13072	SMN1-11 m03	1.56271425	0.158043098	SMN1	50	RPTEC	48	qRTPCR	
13073	SMN1-12 m03	1.58508813	0.05291912	SMN1	50	RPTEC	48	qRTPCR	
13074	SMN1-13 m03	1.17598926	0.122355585	SMN1	50	RPTEC	48	qRTPCR	
13075	SMN1-14 m03	1.46704855	0.073113884	SMN1	50	RPTEC	48	qRTPCR	
13076	SMN1-15 m03	1.57344034	0.117453017	SMN1	50	RPTEC	48	qRTPCR	
13077	SMN1-16 m03	1.4416162	0.059823944	SMN1	50	RPTEC	48	qRTPCR	
13078	SMN1-17 m03	1.27481919	0.179527293	SMN1	50	RPTEC	48	qRTPCR	
13079	SMN1-18 m03	1.22166357	0.064445539	SMN1	50	RPTEC	48	qRTPCR	
13080	SMN1-19 m03	1.43433309	0.075936965	SMN1	50	RPTEC	48	qRTPCR	
13081	SMN1-20 m03	1.50578669	0.170697984	SMN1	50	RPTEC	48	qRTPCR	
13082	SMN1-21 m03	1.46668879	0.116267764	SMN1	50	RPTEC	48	qRTPCR	
13083	SMN1-22 m03	1.24259392	0.02840519	SMN1	50	RPTEC	48	qRTPCR	
13084	SMN1-23 m03	1.33910973	0.070877143	SMN1	50	RPTEC	48	qRTPCR	
13085	SMN1-24 m03	1.28379369	0.158661709	SMN1	50	RPTEC	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13086	SMN1-25 m03	1.22618504	0.044422705	SMN1	50	RPTEC	48	qRTPCR	
13087	SMN1-26 m03	1.63243489	0.061512357	SMN1	50	RPTEC	48	qRTPCR	
11374	SMN1-27 m01	1.58995422	0.093101653	SMN1	50	RPTEC	48	qRTPCR	
11375	SMN1-28 m01	1.40234841	0.101950771	SMN1	50	RPTEC	48	qRTPCR	
11376	SMN1-29 m01	1.14530555	0.044575389	SMN1	50	RPTEC	48	qRTPCR	
11377	SMN1-30 m01	1.16699571	0.128900531	SMN1	50	RPTEC	48	qRTPCR	
	Ctrl Un	0.91941798	0.06956953	SMN1	50	RPTEC	48	qRTPCR	
	Ctrl Lipo	1	0.04685214	SMN1	50	RPTEC	48	qRTPCR	
11374	SMN1-27 m01	0.74718671	0.007807701	SMN1	30	Hep3B	48	qRTPCR	
11375	SMN1-28 m01	1.15022432	0.080077707	SMN1	30	Hep3B	48	qRTPCR	
11376	SMN1-29 m01	0.93739387	0.015700783	SMN1	30	Hep3B	48	qRTPCR	
11377	SMN1-30 m01	1.06953342	0.042258392	SMN1	30	Hep3B	48	qRTPCR	
11378	SMN1-31 m01	1.2236853	0.155258366	SMN1	30	Hep3B	48	qRTPCR	
11379	SMN1-32 m01	1.03297847	0.02312057	SMN1	30	Hep3B	48	qRTPCR	
11380	SMN1-33 m01	1.04636141	0.038971809	SMN1	30	Hep3B	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11381	SMN1-34 m01	1.07987675	0.09859402	SMN1	30	Hep3B	48	qRTPCR	
11382	SMN1-35 m01	0.86146401	0.024095912	SMN1	30	Hep3B	48	qRTPCR	
11383	SMN1-36 m01	1.26636324	0.046963681	SMN1	30	Hep3B	48	qRTPCR	
11384	SMN1-37 m01	1.23269009	0.043476252	SMN1	30	Hep3B	48	qRTPCR	
11385	SMN1-38 m01	0.84324186	0.033808043	SMN1	30	Hep3B	48	qRTPCR	
11386	SMN1-39 m01	0.66374625	0.045527014	SMN1	30	Hep3B	48	qRTPCR	
11387	SMN1-40 m01	0.88813865	0.081401804	SMN1	30	Hep3B	48	qRTPCR	
11388	SMN1-41 m01	0.88246615	0.031016749	SMN1	30	Hep3B	48	qRTPCR	
11389	SMN1-42 m01	0.95686084	0.043558382	SMN1	30	Hep3B	48	qRTPCR	
11390	SMN1-43 m01	1.34191978	0.08080776	SMN1	30	Hep3B	48	qRTPCR	
	Ctrl Un	0.63609664	0.027473819	SMN1	30	Hep3B	48	qRTPCR	
11392	SMN1-45 m01	1.8072369	0.11410948	SMN1	30	Hep3B	48	qRTPCR	
11393	SMN1-46 m01	1.54564954	0.064006814	SMN1	30	Hep3B	48	qRTPCR	
11394	SMN1-47 m01	2.71159836	0.260043446	SMN1	30	Hep3B	48	qRTPCR	
11395	SMN1-48	1.4823422	0.063036343	SMN1	30	Hep3B	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
11396	SMN1-49 m01	1.53449391	0.110688365	SMN1	30	Hep3B	48	qRTPCR	
11397	SMN1-50 m01	0.8974214	0.034254931	SMN1	30	Hep3B	48	qRTPCR	
11398	SMN1-51 m01	1.15792137	0.044256319	SMN1	30	Hep3B	48	qRTPCR	
11399	SMN1-52 m01	0.97354835	0.051461583	SMN1	30	Hep3B	48	qRTPCR	
	Ctrl Un	0.79784081	0.014934034	SMN1	30	Hep3B	48	qRTPCR	
	Ctrl Lipo	1	0.143365418	SMN1	30	Hep3B	48	qRTPCR	
11374	SMN1-27 m01	1.04906874	0.092645193	SMN1	10	Hep3B	48	qRTPCR	
11375	SMN1-28 m01	1.2198284	0.031782762	SMN1	10	Hep3B	48	qRTPCR	
11376	SMN1-29 m01	1.20852196	0.084021899	SMN1	10	Hep3B	48	qRTPCR	
11377	SMN1-30 m01	1.004619	0.068245537	SMN1	10	Hep3B	48	qRTPCR	
11378	SMN1-31 m01	0.93656958	0.083367899	SMN1	10	Hep3B	48	qRTPCR	
11379	SMN1-32 m01	1.05304582	0.030158389	SMN1	10	Hep3B	48	qRTPCR	
11380	SMN1-33 m01	1.23330223	0.063255341	SMN1	10	Hep3B	48	qRTPCR	
11381	SMN1-34 m01	1.27102618	0.067019476	SMN1	10	Hep3B	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11382	SMN1-35 m01	0.83696639	0.054159619	SMN1	10	Hep3B	48	qRTPCR	
11383	SMN1-36 m01	1.32625712	0.039674649	SMN1	10	Hep3B	48	qRTPCR	
11384	SMN1-37 m01	1.14463299	0.058433353	SMN1	10	Hep3B	48	qRTPCR	
11385	SMN1-38 m01	0.93818033	0.011376217	SMN1	10	Hep3B	48	qRTPCR	
11386	SMN1-39 m01	0.89176455	0.019395327	SMN1	10	Hep3B	48	qRTPCR	
11387	SMN1-40 m01	0.8716029	0.065372936	SMN1	10	Hep3B	48	qRTPCR	
11388	SMN1-41 m01	1.09369477	0.025996502	SMN1	10	Hep3B	48	qRTPCR	
11389	SMN1-42 m01	1.151756	0.067662107	SMN1	10	Hep3B	48	qRTPCR	
11390	SMN1-43 m01	1.69291981	0.084669198	SMN1	10	Hep3B	48	qRTPCR	
	Ctrl Un	0.79359897	0.051012309	SMN1	10	Hep3B	48	qRTPCR	
11392	SMN1-45 m01	1.37777737	0.108540058	SMN1	10	Hep3B	48	qRTPCR	
11393	SMN1-46 m01	1.3542915	0.038498944	SMN1	10	Hep3B	48	qRTPCR	
11394	SMN1-47 m01	1.98667479	0.119436675	SMN1	10	Hep3B	48	qRTPCR	
11395	SMN1-48 m01	2.59735063	0.145439801	SMN1	10	Hep3B	48	qRTPCR	
11396	SMN1-49	2.22334078	0.148702992	SMN1	10	Hep3B	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
11397	SMN1-50 m01	1.13236278	0.078523003	SMN1	10	Hep3B	48	qRTPCR	
11398	SMN1-51 m01	1.17760467	0.038060353	SMN1	10	Hep3B	48	qRTPCR	
11399	SMN1-52 m01	1.06835564	0.060851146	SMN1	10	Hep3B	48	qRTPCR	
	Ctrl Un	0.94100856	0.011653526	SMN1	10	Hep3B	48	qRTPCR	
	Ctrl Lipo	1	0.087075677	SMN1	10	Hep3B	48	qRTPCR	
13062	SMN1-01 m03	0.16799891	0.167998672	SMN1	50	Hep3B	48	qRTPCR	
13063	SMN1-02 m03	2.25249453	1.803190669	SMN1	50	Hep3B	48	qRTPCR	
13064	SMN1-03 m03	0.29619174	0.173282309	SMN1	50	Hep3B	48	qRTPCR	
13065	SMN1-04 m03	0.13317001	0.032824391	SMN1	50	Hep3B	48	qRTPCR	
13066	SMN1-05 m03	0.08831786	0.039887014	SMN1	50	Hep3B	48	qRTPCR	
13067	SMN1-06 m03	0.57685962	0.246186541	SMN1	50	Hep3B	48	qRTPCR	
13068	SMN1-07 m03	1.01325231	0.118540759	SMN1	50	Hep3B	48	qRTPCR	
13069	SMN1-08 m03	0.21653001	0.177400555	SMN1	50	Hep3B	48	qRTPCR	
13070	SMN1-09 m03	0.62140678	0.227211261	SMN1	50	Hep3B	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13071	SMN1-10 m03	0.48282224	0.131557474	SMN1	50	Hep3B	48	qRTPCR	
13072	SMN1-11 m03	0.46365594	0.295513886	SMN1	50	Hep3B	48	qRTPCR	
13073	SMN1-12 m03	0.20858605	0.187017655	SMN1	50	Hep3B	48	qRTPCR	
13074	SMN1-13 m03	0.16149916	0.079356287	SMN1	50	Hep3B	48	qRTPCR	
13075	SMN1-14 m03	1.97825415	1.352951156	SMN1	50	Hep3B	48	qRTPCR	
13076	SMN1-15 m03	0.3660431	0.117162019	SMN1	50	Hep3B	48	qRTPCR	
13077	SMN1-16 m03	0.5100566	0.286522659	SMN1	50	Hep3B	48	qRTPCR	
13078	SMN1-17 m03	0.41673922	0.066066242	SMN1	50	Hep3B	48	qRTPCR	
13079	SMN1-18 m03	10.2845517	3.929310832	SMN1	50	Hep3B	48	qRTPCR	
13080	SMN1-19 m03	0.53893216	0.309273594	SMN1	50	Hep3B	48	qRTPCR	
13081	SMN1-20 m03	0.06244992	0.049069571	SMN1	50	Hep3B	48	qRTPCR	
13082	SMN1-21 m03	0.38823351	0.139680869	SMN1	50	Hep3B	48	qRTPCR	
13083	SMN1-22 m03	0.22985792	0.128101282	SMN1	50	Hep3B	48	qRTPCR	
13084	SMN1-23 m03	0.38414338	0.14723735	SMN1	50	Hep3B	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13085	SMN1-24 m03	0.96310021	0.117802422	SMN1	50	Hep3B	48	qRTPCR	
13086	SMN1-25 m03	1.0550823	0.326768036	SMN1	50	Hep3B	48	qRTPCR	
13087	SMN1-26 m03	0.07159332	0.071592884	SMN1	50	Hep3B	48	qRTPCR	
	Ctrl Un	0.80420836	0.023544282	SMN1	50	Hep3B	48	qRTPCR	
	Ctrl Un	0.51071098	0.050976007	SMN1	50	Hep3B	48	qRTPCR	
	Ctrl Un	1.03079733	0.623706913	SMN1	50	Hep3B	48	qRTPCR	
	Ctrl Un	0.52223297	0.314895313	SMN1	50	Hep3B	48	qRTPCR	
	Ctrl Un	1.37924539	0.838262759	SMN1	50	Hep3B	48	qRTPCR	
	Ctrl Lipo	1	0.366142387	SMN1	50	Hep3B	48	qRTPCR	
13062	SMN1-01 m03	1.0481253	0.039302784	SMN1	100	Hep3B	48	qRTPCR	
13063	SMN1-02 m03	1.11438797	0.026733251	SMN1	100	Hep3B	48	qRTPCR	
13064	SMN1-03 m03	1.48817935	0.172719507	SMN1	100	Hep3B	48	qRTPCR	
13065	SMN1-04 m03	1.28955016	0.330195987	SMN1	100	Hep3B	48	qRTPCR	
13066	SMN1-05 m03	1.31005326	0.234231348	SMN1	100	Hep3B	48	qRTPCR	
13067	SMN1-06 m03	1.41941888	0.432447122	SMN1	100	Hep3B	48	qRTPCR	
13068	SMN1-07 m03	1.0393819	0.059815387	SMN1	100	Hep3B	48	qRTPCR	
13069	SMN1-08 m03	2.08215178	0.815184252	SMN1	100	Hep3B	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m03								
13070	SMN1-09 m03	0.85593922	0.256108337	SMN1	100	Hep3B	48	qRTPCR	
13071	SMN1-10 m03	1.28062913	0.17088425	SMN1	100	Hep3B	48	qRTPCR	
13072	SMN1-11 m03	0.95761165	0.334137541	SMN1	100	Hep3B	48	qRTPCR	
13073	SMN1-12 m03	3.2669659	2.002074369	SMN1	100	Hep3B	48	qRTPCR	
13074	SMN1-13 m03	1.28776359	0.090306717	SMN1	100	Hep3B	48	qRTPCR	
13075	SMN1-14 m03	1.31199094	0.073121634	SMN1	100	Hep3B	48	qRTPCR	
13076	SMN1-15 m03	1.73821739	0.520148155	SMN1	100	Hep3B	48	qRTPCR	
13077	SMN1-16 m03	1.38191421	0.247880229	SMN1	100	Hep3B	48	qRTPCR	
13078	SMN1-17 m03	3.33184302	0.970174873	SMN1	100	Hep3B	48	qRTPCR	
13079	SMN1-18 m03	1.80092076	0.42559045	SMN1	100	Hep3B	48	qRTPCR	
13080	SMN1-19 m03	1.17374637	0.179415746	SMN1	100	Hep3B	48	qRTPCR	
13081	SMN1-20 m03	1.54148085	0.461158669	SMN1	100	Hep3B	48	qRTPCR	
13082	SMN1-21 m03	1.36626945	0.239420557	SMN1	100	Hep3B	48	qRTPCR	
13083	SMN1-22	1.49972226	0.568788539	SMN1	100	Hep3B	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m03								
13084	SMN1-23 m03	2.62019561	0.342101826	SMN1	100	Hep3B	48	qRTPCR	
13085	SMN1-24 m03	0.9947533	0.268415648	SMN1	100	Hep3B	48	qRTPCR	
13086	SMN1-25 m03	0.96918504	0.226484866	SMN1	100	Hep3B	48	qRTPCR	
13087	SMN1-26 m03	1.99920252	0.420387669	SMN1	100	Hep3B	48	qRTPCR	
	Ctrl Un	2.27647789	1.013039331	SMN1	100	Hep3B	48	qRTPCR	
	Ctrl Un	1.49042646	0.075799845	SMN1	100	Hep3B	48	qRTPCR	
	Ctrl Un	2.12590142	0.755142009	SMN1	100	Hep3B	48	qRTPCR	
	Ctrl Un	2.22467362	0.327856883	SMN1	100	Hep3B	48	qRTPCR	
	Ctrl Un	2.35024717	1.025162247	SMN1	100	Hep3B	48	qRTPCR	
	Ctrl Lipo	1	0.094046289	SMN1	100	Hep3B	48	qRTPCR	
13062	SMN1-01 m03	1.38170421	0.053290565	SMN1	10	Hep3B	48	qRTPCR	
13063	SMN1-02 m03	1.34641929	0.027641281	SMN1	10	Hep3B	48	qRTPCR	
13064	SMN1-03 m03	1.29932826	0.059825228	SMN1	10	Hep3B	48	qRTPCR	
13065	SMN1-04 m03	1.28022549	0.062577972	SMN1	10	Hep3B	48	qRTPCR	
13066	SMN1-05 m03	1.03869964	0.056421362	SMN1	10	Hep3B	48	qRTPCR	
13067	SMN1-06 m03	1.1462517	0.051891541	SMN1	10	Hep3B	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13068	SMN1-07 m03	1.15694961	0.107385405	SMN1	10	Hep3B	48	qRTPCR	
13069	SMN1-08 m03	1.0100906	0.200588791	SMN1	10	Hep3B	48	qRTPCR	
13070	SMN1-09 m03	0.9401861	0.197008464	SMN1	10	Hep3B	48	qRTPCR	
13071	SMN1-10 m03	1.12725465	0.152486374	SMN1	10	Hep3B	48	qRTPCR	
13072	SMN1-11 m03	1.22597382	0.223472758	SMN1	10	Hep3B	48	qRTPCR	
13073	SMN1-12 m03	1.03381379	0.204376291	SMN1	10	Hep3B	48	qRTPCR	
13074	SMN1-13 m03	1.3368516	0.177814975	SMN1	10	Hep3B	48	qRTPCR	
13075	SMN1-14 m03	1.12892777	0.147162701	SMN1	10	Hep3B	48	qRTPCR	
13076	SMN1-15 m03	1.38320134	0.101830776	SMN1	10	Hep3B	48	qRTPCR	
13077	SMN1-16 m03	1.31007357	0.026347093	SMN1	10	Hep3B	48	qRTPCR	
13078	SMN1-17 m03	1.26085652	0.038565799	SMN1	10	Hep3B	48	qRTPCR	
13079	SMN1-18 m03	1.2617526	0.069817143	SMN1	10	Hep3B	48	qRTPCR	
13080	SMN1-19 m03	1.18614147	0.036729063	SMN1	10	Hep3B	48	qRTPCR	
13081	SMN1-20 m03	1.08998569	0.043750568	SMN1	10	Hep3B	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13082	SMN1-21 m03	1.35455484	0.013175463	SMN1	10	Hep3B	48	qRTPCR	
13083	SMN1-22 m03	1.23478376	0.017119432	SMN1	10	Hep3B	48	qRTPCR	
13084	SMN1-23 m03	1.47366387	0.053762605	SMN1	10	Hep3B	48	qRTPCR	
13085	SMN1-24 m03	0.96544035	0.032646295	SMN1	10	Hep3B	48	qRTPCR	
13086	SMN1-25 m03	1.00974636	0.122120737	SMN1	10	Hep3B	48	qRTPCR	
13087	SMN1-26 m03	0.97410758	0.066863661	SMN1	10	Hep3B	48	qRTPCR	
	Ctrl Un	1.04177401	0.051819332	SMN1	10	Hep3B	48	qRTPCR	
	Ctrl Lipo	1	0.031344	SMN1	10	Hep3B	48	qRTPCR	
13062	SMN1-01 m03	0.97986925	0.020515227	SMN1	30	Hep3B	48	qRTPCR	
13063	SMN1-02 m03	1.15369708	0.024999991	SMN1	30	Hep3B	48	qRTPCR	
13064	SMN1-03 m03	1.51156781	0.054178175	SMN1	30	Hep3B	48	qRTPCR	
13065	SMN1-04 m03	1.48848279	0.044641287	SMN1	30	Hep3B	48	qRTPCR	
13066	SMN1-05 m03	0.85914475	0.039970015	SMN1	30	Hep3B	48	qRTPCR	
13067	SMN1-06 m03	1.03068232	0.013070835	SMN1	30	Hep3B	48	qRTPCR	
13068	SMN1-07 m03	1.23950395	0.134603844	SMN1	30	Hep3B	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13069	SMN1-08 m03	1.22394767	0.295307243	SMN1	30	Hep3B	48	qRTPCR	
13070	SMN1-09 m03	0.86448114	0.162739271	SMN1	30	Hep3B	48	qRTPCR	
13071	SMN1-10 m03	1.06957146	0.122106758	SMN1	30	Hep3B	48	qRTPCR	
13072	SMN1-11 m03	1.08930226	0.126414268	SMN1	30	Hep3B	48	qRTPCR	
13073	SMN1-12 m03	1.13747167	0.246791954	SMN1	30	Hep3B	48	qRTPCR	
13074	SMN1-13 m03	1.03777229	0.039404507	SMN1	30	Hep3B	48	qRTPCR	
13075	SMN1-14 m03	0.85579512	0.017797181	SMN1	30	Hep3B	48	qRTPCR	
13076	SMN1-15 m03	1.61949505	0.038364989	SMN1	30	Hep3B	48	qRTPCR	
13077	SMN1-16 m03	1.41813265	0.082371708	SMN1	30	Hep3B	48	qRTPCR	
13078	SMN1-17 m03	1.60904531	0.10487434	SMN1	30	Hep3B	48	qRTPCR	
13079	SMN1-18 m03	1.5927008	0.199280916	SMN1	30	Hep3B	48	qRTPCR	
13080	SMN1-19 m03	1.83477537	0.155761723	SMN1	30	Hep3B	48	qRTPCR	
13081	SMN1-20 m03	1.41531375	0.146502726	SMN1	30	Hep3B	48	qRTPCR	
13082	SMN1-21 m03	2.02696838	0.27827902	SMN1	30	Hep3B	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13083	SMN1-22 m03	1.50969559	0.175764156	SMN1	30	Hep3B	48	qRTPCR	
13084	SMN1-23 m03	1.92080042	0.127336842	SMN1	30	Hep3B	48	qRTPCR	
13085	SMN1-24 m03	1.14056617	0.10163121	SMN1	30	Hep3B	48	qRTPCR	
13086	SMN1-25 m03	1.08130364	0.101827303	SMN1	30	Hep3B	48	qRTPCR	
13087	SMN1-26 m03	1.03022789	0.096105098	SMN1	30	Hep3B	48	qRTPCR	
	Ctrl Un	0.93755934	0.066825398	SMN1	30	Hep3B	48	qRTPCR	
	Ctrl Lipo	1	0.11263538	SMN1	30	Hep3B	48	qRTPCR	
11374	SMN1-27 m01	0.91480247	0.06029412	SMN	30	SK-N-AS	48	qRTPCR	
11375	SMN1-28 m01	1.14513577	0.073064244	SMN	30	SK-N-AS	48	qRTPCR	
11376	SMN1-29 m01	1.09193595	0.046058037	SMN	30	SK-N-AS	48	qRTPCR	
11377	SMN1-30 m01	1.00777268	0.042900759	SMN	30	SK-N-AS	48	qRTPCR	
11378	SMN1-31 m01	0.84025249	0.028179921	SMN	30	SK-N-AS	48	qRTPCR	
11379	SMN1-32 m01	0.79364182	0.031255602	SMN	30	SK-N-AS	48	qRTPCR	
11380	SMN1-33 m01	1.05952837	0.026275242	SMN	30	SK-N-AS	48	qRTPCR	
11381	SMN1-34 m01	0.90733827	0.135540433	SMN	30	SK-N-AS	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11382	SMN1-35 m01	0.71280704	0.084307759	SMN	30	SK-N-AS	48	qRT-PCR	
11383	SMN1-36 m01	0.9563418	0.059940328	SMN	30	SK-N-AS	48	qRT-PCR	
11384	SMN1-37 m01	0.88794313	0.046479463	SMN	30	SK-N-AS	48	qRT-PCR	
11385	SMN1-38 m01	0.85381763	0.098224845	SMN	30	SK-N-AS	48	qRT-PCR	
11386	SMN1-39 m01	0.84169353	0.030220248	SMN	30	SK-N-AS	48	qRT-PCR	
11387	SMN1-40 m01	1.00989002	0.049436731	SMN	30	SK-N-AS	48	qRT-PCR	
11388	SMN1-41 m01	1.24346831	0.083429431	SMN	30	SK-N-AS	48	qRT-PCR	
11389	SMN1-42 m01	1.26993842	0.117969563	SMN	30	SK-N-AS	48	qRT-PCR	
11390	SMN1-43 m01	1.14083308	0.043391451	SMN	30	SK-N-AS	48	qRT-PCR	
11391	SMN1-44 m01	1.40837345	0.09773958	SMN	30	SK-N-AS	48	qRT-PCR	
11392	SMN1-45 m01	1.08919975	0.074440731	SMN	30	SK-N-AS	48	qRT-PCR	
11393	SMN1-46 m01	1.0563416	0.043185977	SMN	30	SK-N-AS	48	qRT-PCR	
11394	SMN1-47 m01	1.49210181	0.034626588	SMN	30	SK-N-AS	48	qRT-PCR	
11395	SMN1-48 m01	1.34231867	0.041173721	SMN	30	SK-N-AS	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11396	SMN1-49 m01	1.37311316	0.042503656	SMN	30	SK-N-AS	48	qRTPCR	
11397	SMN1-50 m01	0.89505319	0.025534007	SMN	30	SK-N-AS	48	qRTPCR	
11398	SMN1-51 m01	0.95901306	0.024799397	SMN	30	SK-N-AS	48	qRTPCR	
11399	SMN1-52 m01	0.90222059	0.022401106	SMN	30	SK-N-AS	48	qRTPCR	
	Ctrl Lipo	1	0.050167818	SMN	30	SK-N-AS	48	qRTPCR	
	Ctrl Un	0.90829997	0.028519073	SMN	30	SK-N-AS	48	qRTPCR	
11374	SMN1-27 m01	0.92625828	0.026226701	SMN	50	SK-N-AS	48	qRTPCR	
11375	SMN1-28 m01	1.17877148	0.062941901	SMN	50	SK-N-AS	48	qRTPCR	
11376	SMN1-29 m01	1.32061386	0.038630476	SMN	50	SK-N-AS	48	qRTPCR	
11377	SMN1-30 m01	1.20908487	0.089525425	SMN	50	SK-N-AS	48	qRTPCR	
11378	SMN1-31 m01	0.79421497	0.031491594	SMN	50	SK-N-AS	48	qRTPCR	
11379	SMN1-32 m01	0.7882657	0.041343893	SMN	50	SK-N-AS	48	qRTPCR	
11380	SMN1-33 m01	0.93474946	0.039788349	SMN	50	SK-N-AS	48	qRTPCR	
11381	SMN1-34 m01	0.81568625	0.086370017	SMN	50	SK-N-AS	48	qRTPCR	
11382	SMN1-35 m01	0.83126356	0.144440932	SMN	50	SK-N-AS	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11383	SMN1-36 m01	1.22419056	0.194554189	SMN	50	SK-N-AS	48	qRT-PCR	
11384	SMN1-37 m01	1.07598009	0.11828228	SMN	50	SK-N-AS	48	qRT-PCR	
11385	SMN1-38 m01	0.89248269	0.117182711	SMN	50	SK-N-AS	48	qRT-PCR	
11386	SMN1-39 m01	0.94335653	0.024717496	SMN	50	SK-N-AS	48	qRT-PCR	
11387	SMN1-40 m01	1.12005295	0.057300095	SMN	50	SK-N-AS	48	qRT-PCR	
11388	SMN1-41 m01	1.41931772	0.06614803	SMN	50	SK-N-AS	48	qRT-PCR	
11389	SMN1-42 m01	1.28656423	0.1568116	SMN	50	SK-N-AS	48	qRT-PCR	
11390	SMN1-43 m01	1.21225527	0.155449912	SMN	50	SK-N-AS	48	qRT-PCR	
11391	SMN1-44 m01	1.44675629	0.099931629	SMN	50	SK-N-AS	48	qRT-PCR	
11392	SMN1-45 m01	1.24699418	0.072776827	SMN	50	SK-N-AS	48	qRT-PCR	
11393	SMN1-46 m01	1.0815571	0.162576736	SMN	50	SK-N-AS	48	qRT-PCR	
11394	SMN1-47 m01	2.35225429	0.154049964	SMN	50	SK-N-AS	48	qRT-PCR	
11395	SMN1-48 m01	1.44737202	0.092512204	SMN	50	SK-N-AS	48	qRT-PCR	
11396	SMN1-49 m01	1.47140858	0.114116276	SMN	50	SK-N-AS	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11397	SMN1-50 m01	1.05016678	0.056199143	SMN	50	SK-N-AS	48	qRTPCR	
11398	SMN1-51 m01	1.26062559	0.02900481	SMN	50	SK-N-AS	48	qRTPCR	
11399	SMN1-52 m01	1.15959826	0.067122609	SMN	50	SK-N-AS	48	qRTPCR	
	Ctrl Lipo	1	0.019877689	SMN	50	SK-N-AS	48	qRTPCR	
	Ctrl Un	1.13429966	0.062815882	SMN	50	SK-N-AS	48	qRTPCR	
11374	SMN1-27 m01	0.90380922	0.028775573	SMN	30	U-87	48	qRTPCR	
11375	SMN1-28 m01	1.36281182	0.091249714	SMN	30	U-87	48	qRTPCR	
11376	SMN1-29 m01	0.92753342	0.043982925	SMN	30	U-87	48	qRTPCR	
11377	SMN1-30 m01	1.27040273	0.083227292	SMN	30	U-87	48	qRTPCR	
11378	SMN1-31 m01	0.60349487	0.021601592	SMN	30	U-87	48	qRTPCR	
11379	SMN1-32 m01	0.87172918	0.027385229	SMN	30	U-87	48	qRTPCR	
11380	SMN1-33 m01	1.0506283	0.050883615	SMN	30	U-87	48	qRTPCR	
11381	SMN1-34 m01	0.87044581	0.113868287	SMN	30	U-87	48	qRTPCR	
11382	SMN1-35 m01	0.77286743	0.113245918	SMN	30	U-87	48	qRTPCR	
11383	SMN1-36 m01	1.01814352	0.092114524	SMN	30	U-87	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11384	SMN1-37 m01	1.02442784	0.082814512	SMN	30	U-87	48	qRT-PCR	
11385	SMN1-38 m01	0.88824852	0.116558429	SMN	30	U-87	48	qRT-PCR	
11386	SMN1-39 m01	1.1535991	0.064147943	SMN	30	U-87	48	qRT-PCR	
11387	SMN1-40 m01	1.07971998	0.03425257	SMN	30	U-87	48	qRT-PCR	
11388	SMN1-41 m01	1.08239092	0.033138384	SMN	30	U-87	48	qRT-PCR	
11389	SMN1-42 m01	0.93090579	0.033554463	SMN	30	U-87	48	qRT-PCR	
11390	SMN1-43 m01	1.33766819	0.071324972	SMN	30	U-87	48	qRT-PCR	
11391	SMN1-44 m01	1.61330555	0.03255805	SMN	30	U-87	48	qRT-PCR	
11392	SMN1-45 m01	1.13867715	0.03593228	SMN	30	U-87	48	qRT-PCR	
11393	SMN1-46 m01	1.19251782	0.029622835	SMN	30	U-87	48	qRT-PCR	
11394	SMN1-47 m01	1.95722311	0.072700493	SMN	30	U-87	48	qRT-PCR	
11395	SMN1-48 m01	1.31709902	0.064666933	SMN	30	U-87	48	qRT-PCR	
11396	SMN1-49 m01	1.65838881	0.084762831	SMN	30	U-87	48	qRT-PCR	
11397	SMN1-50 m01	1.00549885	0.013808888	SMN	30	U-87	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11398	SMN1-51 m01	1.0102985	0.046175017	SMN	30	U-87	48	qRT-PCR	
11399	SMN1-52 m01	1.02353389	0.043958517	SMN	30	U-87	48	qRT-PCR	
	Ctrl Lipo	1	0.036230807	SMN	30	U-87	48	qRT-PCR	
	Ctrl Un	1.07036321	0.020341592	SMN	30	U-87	48	qRT-PCR	
11374	SMN1-27 m01	0.88787687	0.020515076	SMN	50	U-87	48	qRT-PCR	
11375	SMN1-28 m01	1.283286	0.007583976	SMN	50	U-87	48	qRT-PCR	
11376	SMN1-29 m01	0.9912161	0.009556459	SMN	50	U-87	48	qRT-PCR	
11377	SMN1-30 m01	1.47477303	0.118905158	SMN	50	U-87	48	qRT-PCR	
11378	SMN1-31 m01	0.79045095	0.022369992	SMN	50	U-87	48	qRT-PCR	
11379	SMN1-32 m01	1.33952911	0.032601975	SMN	50	U-87	48	qRT-PCR	
11380	SMN1-33 m01	1.22920827	0.109540088	SMN	50	U-87	48	qRT-PCR	
11381	SMN1-34 m01	0.91666557	0.148062154	SMN	50	U-87	48	qRT-PCR	
11382	SMN1-35 m01	0.88255794	0.176458849	SMN	50	U-87	48	qRT-PCR	
11383	SMN1-36 m01	1.06022141	0.132504995	SMN	50	U-87	48	qRT-PCR	
11384	SMN1-37 m01	1.29315186	0.218150138	SMN	50	U-87	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11385	SMN1-38 m01	0.8674373	0.106103303	SMN	50	U-87	48	qRT-PCR	
11386	SMN1-39 m01	1.35916125	0.054080137	SMN	50	U-87	48	qRT-PCR	
11387	SMN1-40 m01	1.02428776	0.017685951	SMN	50	U-87	48	qRT-PCR	
11388	SMN1-41 m01	1.27130372	0.116137705	SMN	50	U-87	48	qRT-PCR	
11389	SMN1-42 m01	0.93818952	0.075457949	SMN	50	U-87	48	qRT-PCR	
11390	SMN1-43 m01	1.19178569	0.01422073	SMN	50	U-87	48	qRT-PCR	
11391	SMN1-44 m01	1.75004832	0.04201986	SMN	50	U-87	48	qRT-PCR	
11392	SMN1-45 m01	1.27464677	0.03794454	SMN	50	U-87	48	qRT-PCR	
11393	SMN1-46 m01	1.43440122	0.005611125	SMN	50	U-87	48	qRT-PCR	
11394	SMN1-47 m01	2.50979319	0.008427116	SMN	50	U-87	48	qRT-PCR	
11395	SMN1-48 m01	0.83955595	0.048056453	SMN	50	U-87	48	qRT-PCR	
11396	SMN1-49 m01	1.24250902	0.012870656	SMN	50	U-87	48	qRT-PCR	
11397	SMN1-50 m01	1.09574695	0.029715018	SMN	50	U-87	48	qRT-PCR	
11398	SMN1-51 m01	1.0260093	0.019239702	SMN	50	U-87	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11399	SMN1-52 m01	0.94399356	0.034899449	SMN	50	U-87	48	qRTPCR	
	Ctrl Lipo	1	0.058551292	SMN	50	U-87	48	qRTPCR	
	Ctrl Un	0.91334263	0.041751467	SMN	50	U-87	48	qRTPCR	
3650	SMN1-59 m01	1.91022705	0.042063629	SMN	50	SK-N-AS	48	qRTPCR	
13093	SMN1-60 m01	1.28634888	0.081658616	SMN	50	SK-N-AS	48	qRTPCR	
13094	SMN1-61 m01	1.37828275	0.146823059	SMN	50	SK-N-AS	48	qRTPCR	
10065	SMN1-62 m01	1.52512518	0.351450295	SMN	50	SK-N-AS	48	qRTPCR	
10066	SMN1-63 m01	1.27737921	0.068864088	SMN	50	SK-N-AS	48	qRTPCR	
10067	SMN1-64 m01	1.17032308	0.041286164	SMN	50	SK-N-AS	48	qRTPCR	
10068	SMN1-65 m01	1.15071588	0.008208311	SMN	50	SK-N-AS	48	qRTPCR	
10069	SMN1-66 m01	1.21121453	0.034534827	SMN	50	SK-N-AS	48	qRTPCR	
10070	SMN1-67 m01	0.98963431	0.020730408	SMN	50	SK-N-AS	48	qRTPCR	
10071	SMN1-68 m01	1.09073306	0.074092701	SMN	50	SK-N-AS	48	qRTPCR	
10072	SMN1-69 m01	1.03471171	0.088857018	SMN	50	SK-N-AS	48	qRTPCR	
10073	SMN1-70 m01	1.39281773	0.000607478	SMN	50	SK-N-AS	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10074	SMN1-71 m01	1.7630648	0.280179736	SMN	50	SK-N-AS	48	qRT-PCR	
10075	SMN1-72 m01	1.23296496	0.061360559	SMN	50	SK-N-AS	48	qRT-PCR	
10080	SMN1-73 m01	1.46168104	0.061621464	SMN	50	SK-N-AS	48	qRT-PCR	
10081	SMN1-74 m01	1.41790556	0.013868966	SMN	50	SK-N-AS	48	qRT-PCR	
10082	SMN1-75 m01	1.33354972	0.033881591	SMN	50	SK-N-AS	48	qRT-PCR	
10084	SMN1-76 m01	1.01678386	0.082597989	SMN	50	SK-N-AS	48	qRT-PCR	
10087	SMN1-77 m01	1.21299034	0.04194719	SMN	50	SK-N-AS	48	qRT-PCR	
10168	SMN1-78 m01	1.35729348	0.091749492	SMN	50	SK-N-AS	48	qRT-PCR	
10169	SMN1-79 m01	2.08007913	0.032381338	SMN	50	SK-N-AS	48	qRT-PCR	
10170	SMN1-80 m01	1.56178714	0.044728311	SMN	50	SK-N-AS	48	qRT-PCR	
10337	SMN1-81 m01	1.11364919	0.089749199	SMN	50	SK-N-AS	48	qRT-PCR	
10338	SMN1-82 m01	1.04563958	0.008966312	SMN	50	SK-N-AS	48	qRT-PCR	
10339	SMN1-83 m01	0.96092903	0.012050036	SMN	50	SK-N-AS	48	qRT-PCR	
10763	SMN1-84 m01	1.64866424	0.116099279	SMN	50	SK-N-AS	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10764	SMN1-85 m01	0.991164	0.044203573	SMN	50	SK-N-AS	48	qRT-PCR	
10949	SMN1-86 m01	1.08498873	0.109848717	SMN	50	SK-N-AS	48	qRT-PCR	
10951	SMN1-87 m01	1.20420537	0.029743659	SMN	50	SK-N-AS	48	qRT-PCR	
10952	SMN1-88 m01	1.33826283	0.074971011	SMN	50	SK-N-AS	48	qRT-PCR	
	Ctrl Un	1.13057953	0.116388295	SMN	50	SK-N-AS	48	qRT-PCR	
	Ctrl Lipo	1	0.101348242	SMN	50	SK-N-AS	48	qRT-PCR	
10953	SMN1-89 m01	0.80414703	0.035911687	SMN	50	SK-N-AS	48	qRT-PCR	
10954	SMN1-90 m01	1.1232011	0.076107434	SMN	50	SK-N-AS	48	qRT-PCR	
11415	SMN1-91 m01	1.18175055	0.091606813	SMN	50	SK-N-AS	48	qRT-PCR	
11418	SMN1-92 m01	1.18058087	0.167599705	SMN	50	SK-N-AS	48	qRT-PCR	
11419	SMN1-93 m01	1.38757673	0.113324416	SMN	50	SK-N-AS	48	qRT-PCR	
11421	SMN1-94 m01	1.02107651	0.082537842	SMN	50	SK-N-AS	48	qRT-PCR	
11422	SMN1-95 m01	1.02858872	0.043883154	SMN	50	SK-N-AS	48	qRT-PCR	
11423	SMN1-96 m01	0.88930577	0.123744889	SMN	50	SK-N-AS	48	qRT-PCR	
11440	SMN1-97 m01	0.69386206	0.075034478	SMN	50	SK-N-AS	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11441	SMN1-98 m01	1.05550748	0.1111104446	SMN	50	SK-N-AS	48	qRTPCR	
11443	SMN1-100 m01	1.03253876	0.096969602	SMN	50	SK-N-AS	48	qRTPCR	
12369	SMN1-101 m01	0.53047313	0.06385017	SMN	50	SK-N-AS	48	qRTPCR	
13095	unc-232 m12	1.33098861	0.113806694	SMN	50	SK-N-AS	48	qRTPCR	
13095	unc-232 m01	1.21833282	0.094695532	SMN	50	SK-N-AS	48	qRTPCR	
13096	unc-293 m12	1.05934522	0.008702948	SMN	50	SK-N-AS	48	qRTPCR	
13096	unc-293 m01	0.961045	0.038674252	SMN	50	SK-N-AS	48	qRTPCR	
	Ctrl Un	1.12455044	0.022110785	SMN	50	SK-N-AS	48	qRTPCR	
	Ctrl Lipo	1	0.039724098	SMN	50	SK-N-AS	48	qRTPCR	
3650	SMN1-59 m01	1.08317288	0.011158109	SMN	30	SK-N-AS	48	qRTPCR	
13093	SMN1-60 m01	0.97826255	0.074282578	SMN	30	SK-N-AS	48	qRTPCR	
13094	SMN1-61 m01	1.07999379	0.092227537	SMN	30	SK-N-AS	48	qRTPCR	
10065	SMN1-62 m01	0.94288012	0.029846055	SMN	30	SK-N-AS	48	qRTPCR	
10066	SMN1-63 m01	0.98172459	0.069780403	SMN	30	SK-N-AS	48	qRTPCR	
10067	SMN1-64 m01	0.90013768	0.013828094	SMN	30	SK-N-AS	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10068	SMN1-65 m01	0.91845158	0.022428024	SMN	30	SK-N-AS	48	qRT-PCR	
10069	SMN1-66 m01	0.99043689	0.074395897	SMN	30	SK-N-AS	48	qRT-PCR	
10070	SMN1-67 m01	0.71402846	0.006715541	SMN	30	SK-N-AS	48	qRT-PCR	
10071	SMN1-68 m01	0.83496069	0.018724163	SMN	30	SK-N-AS	48	qRT-PCR	
10072	SMN1-69 m01	0.94583703	0.021867654	SMN	30	SK-N-AS	48	qRT-PCR	
10073	SMN1-70 m01	0.98611871	0.015730276	SMN	30	SK-N-AS	48	qRT-PCR	
10074	SMN1-71 m01	1.43908652	0.194274257	SMN	30	SK-N-AS	48	qRT-PCR	
10075	SMN1-72 m01	0.87991096	0.015146769	SMN	30	SK-N-AS	48	qRT-PCR	
10080	SMN1-73 m01	1.05570734	0.014543206	SMN	30	SK-N-AS	48	qRT-PCR	
10081	SMN1-74 m01	1.11478354	0.004149062	SMN	30	SK-N-AS	48	qRT-PCR	
10082	SMN1-75 m01	0.87297646	0.011971055	SMN	30	SK-N-AS	48	qRT-PCR	
10084	SMN1-76 m01	1.05743132	0.092272493	SMN	30	SK-N-AS	48	qRT-PCR	
10087	SMN1-77 m01	0.94300376	0.031147303	SMN	30	SK-N-AS	48	qRT-PCR	
10168	SMN1-78 m01	0.90942262	0.009025999	SMN	30	SK-N-AS	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10169	SMN1-79 m01	1.47532749	0.213339495	SMN	30	SK-N-AS	48	qRT-PCR	
10170	SMN1-80 m01	1.188111149	0.020144997	SMN	30	SK-N-AS	48	qRT-PCR	
10337	SMN1-81 m01	0.89671746	0.055297665	SMN	30	SK-N-AS	48	qRT-PCR	
10338	SMN1-82 m01	0.91879872	0.016685594	SMN	30	SK-N-AS	48	qRT-PCR	
10339	SMN1-83 m01	0.80024698	0.002046436	SMN	30	SK-N-AS	48	qRT-PCR	
10763	SMN1-84 m01	1.15174908	0.018551461	SMN	30	SK-N-AS	48	qRT-PCR	
10764	SMN1-85 m01	0.84982301	0.057948114	SMN	30	SK-N-AS	48	qRT-PCR	
10949	SMN1-86 m01	0.94258868	0.008953789	SMN	30	SK-N-AS	48	qRT-PCR	
10951	SMN1-87 m01	1.03552616	0.084824321	SMN	30	SK-N-AS	48	qRT-PCR	
10952	SMN1-88 m01	0.92731952	0.030459643	SMN	30	SK-N-AS	48	qRT-PCR	
	Ctrl Un	1.06494774	0.080035171	SMN	30	SK-N-AS	48	qRT-PCR	
	Ctrl Lipo	1	0.021518469	SMN	30	SK-N-AS	48	qRT-PCR	
10953	SMN1-89 m01	1.01027962	0.014282098	SMN	30	SK-N-AS	48	qRT-PCR	
10954	SMN1-90 m01	1.09402735	0.005307231	SMN	30	SK-N-AS	48	qRT-PCR	
11415	SMN1-91 m01	1.15907359	0.026911128	SMN	30	SK-N-AS	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11418	SMN1-92 m01	1.27812931	0.010005755	SMN	30	SK-N-AS	48	qRT-PCR	
11419	SMN1-93 m01	1.19694532	0.012393906	SMN	30	SK-N-AS	48	qRT-PCR	
11421	SMN1-94 m01	1.14955752	0.010423907	SMN	30	SK-N-AS	48	qRT-PCR	
11422	SMN1-95 m01	1.09239249	0.044970572	SMN	30	SK-N-AS	48	qRT-PCR	
11423	SMN1-96 m01	0.93556863	0.14718547	SMN	30	SK-N-AS	48	qRT-PCR	
11440	SMN1-97 m01	0.82626603	0.139660313	SMN	30	SK-N-AS	48	qRT-PCR	
11441	SMN1-98 m01	0.93173548	0.049283401	SMN	30	SK-N-AS	48	qRT-PCR	
11443	SMN1-100 m01	1.05795264	0.100144072	SMN	30	SK-N-AS	48	qRT-PCR	
12369	SMN1-101 m01	0.67166165	0.006396268	SMN	30	SK-N-AS	48	qRT-PCR	
13095	unc-232 m12	1.14345152	0.016004894	SMN	30	SK-N-AS	48	qRT-PCR	
13095	unc-232 m01	1.21984387	0.028949128	SMN	30	SK-N-AS	48	qRT-PCR	
13096	unc-293 m12	1.12121877	0.005296535	SMN	30	SK-N-AS	48	qRT-PCR	
13096	unc-293 m01	1.17467747	0.052822903	SMN	30	SK-N-AS	48	qRT-PCR	
	Ctrl Un	1.03905006	0.008112288	SMN	30	SK-N-AS	48	qRT-PCR	
	Ctrl Lipo	1	0.01281112	SMN	30	SK-N-AS	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
3650	SMN1-59 m01	1.08426171	0.046738519	SMN	30	U-87	48	qRTPCR	
13093	SMN1-60 m01	1.12445602	0.038654453	SMN	30	U-87	48	qRTPCR	
13094	SMN1-61 m01	0.93124943	0.015032975	SMN	30	U-87	48	qRTPCR	
10065	SMN1-62 m01	1.0848363	0.029983562	SMN	30	U-87	48	qRTPCR	
10066	SMN1-63 m01	0.90402718	0.0289096	SMN	30	U-87	48	qRTPCR	
10067	SMN1-64 m01	1.30530539	0.036758053	SMN	30	U-87	48	qRTPCR	
10068	SMN1-65 m01	0.81156684	0.025909488	SMN	30	U-87	48	qRTPCR	
10069	SMN1-66 m01	0.80616217	0.043169667	SMN	30	U-87	48	qRTPCR	
10070	SMN1-67 m01	0.60837699	0.014197025	SMN	30	U-87	48	qRTPCR	
10071	SMN1-68 m01	0.74776509	0.013667179	SMN	30	U-87	48	qRTPCR	
10072	SMN1-69 m01	0.74284473	0.110489693	SMN	30	U-87	48	qRTPCR	
10073	SMN1-70 m01	0.84891931	0.012893017	SMN	30	U-87	48	qRTPCR	
10074	SMN1-71 m01	1.0204774	0.012582652	SMN	30	U-87	48	qRTPCR	
10075	SMN1-72 m01	0.84791731	0.00878091	SMN	30	U-87	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10080	SMN1-73 m01	0.91580931	0.002527437	SMN	30	U-87	48	qRT-PCR	
10081	SMN1-74 m01	1.03262566	0.026430898	SMN	30	U-87	48	qRT-PCR	
10082	SMN1-75 m01	0.89053406	0.022754693	SMN	30	U-87	48	qRT-PCR	
10084	SMN1-76 m01	1.18921909	0.034386739	SMN	30	U-87	48	qRT-PCR	
10087	SMN1-77 m01	0.89237301	0.024648193	SMN	30	U-87	48	qRT-PCR	
10168	SMN1-78 m01	0.99897906	0.015452652	SMN	30	U-87	48	qRT-PCR	
10169	SMN1-79 m01	1.32001585	0.003777496	SMN	30	U-87	48	qRT-PCR	
10170	SMN1-80 m01	1.20391965	0.004142413	SMN	30	U-87	48	qRT-PCR	
10337	SMN1-81 m01	1.05067948	0.029104426	SMN	30	U-87	48	qRT-PCR	
10338	SMN1-82 m01	0.93729507	0.002334402	SMN	30	U-87	48	qRT-PCR	
10339	SMN1-83 m01	0.96636421	0.025988679	SMN	30	U-87	48	qRT-PCR	
10763	SMN1-84 m01	1.689737	0.080899567	SMN	30	U-87	48	qRT-PCR	
10764	SMN1-85 m01	1.03238722	0.034356811	SMN	30	U-87	48	qRT-PCR	
10949	SMN1-86 m01	1.50178262	0.073097778	SMN	30	U-87	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10951	SMN1-87 m01	1.16597185	0.059924081	SMN	30	U-87	48	qRTPCR	
10952	SMN1-88 m01	1.10045596	0.030408847	SMN	30	U-87	48	qRTPCR	
	Ctrl Un	0.99874347	0.025342299	SMN	30	U-87	48	qRTPCR	
	Ctrl Lipo	1	0.013594788	SMN	30	U-87	48	qRTPCR	
10953	SMN1-89 m01	0.95148686	0.089550784	SMN	30	U-87	48	qRTPCR	
10954	SMN1-90 m01	1.01592532	0.073162228	SMN	30	U-87	48	qRTPCR	
11415	SMN1-91 m01	0.80879545	0.014675915	SMN	30	U-87	48	qRTPCR	
11418	SMN1-92 m01	1.17434779	0.013839558	SMN	30	U-87	48	qRTPCR	
11419	SMN1-93 m01	1.34876403	0.034459933	SMN	30	U-87	48	qRTPCR	
11421	SMN1-94 m01	1.21180378	0.019064762	SMN	30	U-87	48	qRTPCR	
11422	SMN1-95 m01	1.06371222	0.026967546	SMN	30	U-87	48	qRTPCR	
11423	SMN1-96 m01	0.78544197	0.07131768	SMN	30	U-87	48	qRTPCR	
11440	SMN1-97 m01	0.93531963	0.194700087	SMN	30	U-87	48	qRTPCR	
11441	SMN1-98 m01	0.92718595	0.053882452	SMN	30	U-87	48	qRTPCR	
11443	SMN1-100 m01	0.88026806	0.014546132	SMN	30	U-87	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
12369	SMN1-101 m01	0.45783871	0.101463017	SMN	30	U-87	48	qRT-PCR	
13095	unc-232 m12	0.9234009	0.056513794	SMN	30	U-87	48	qRT-PCR	
13095	unc-232 m01	0.88363709	0.004083924	SMN	30	U-87	48	qRT-PCR	
13096	unc-293 m12	0.94579115	0.053800845	SMN	30	U-87	48	qRT-PCR	
13096	unc-293 m01	0.77694212	0.035840086	SMN	30	U-87	48	qRT-PCR	
	Ctrl Un	1.14267957	0.057694977	SMN	30	U-87	48	qRT-PCR	
	Ctrl Lipo	1	0.033353342	SMN	30	U-87	48	qRT-PCR	
3650	SMN1-59 m01	1.44287443	0.077473953	SMN	50	U-87	48	qRT-PCR	
13093	SMN1-60 m01	1.64763497	0.046110534	SMN	50	U-87	48	qRT-PCR	
13094	SMN1-61 m01	1.098821	0.045042178	SMN	50	U-87	48	qRT-PCR	
10065	SMN1-62 m01	1.32052944	0.082464676	SMN	50	U-87	48	qRT-PCR	
10066	SMN1-63 m01	1.11800123	0.020881949	SMN	50	U-87	48	qRT-PCR	
10067	SMN1-64 m01	1.66912017	0.043823535	SMN	50	U-87	48	qRT-PCR	
10068	SMN1-65 m01	1.0344282	0.034133106	SMN	50	U-87	48	qRT-PCR	
10069	SMN1-66 m01	1.06684244	0.032021956	SMN	50	U-87	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10070	SMN1-67 m01	0.49764932	0.05256605	SMN	50	U-87	48	qRTPCR	
10071	SMN1-68 m01	1.03267478	0.067708876	SMN	50	U-87	48	qRTPCR	
10072	SMN1-69 m01	1.08875847	0.062383562	SMN	50	U-87	48	qRTPCR	
10073	SMN1-70 m01	1.05492664	0.112756013	SMN	50	U-87	48	qRTPCR	
10074	SMN1-71 m01	1.45324963	0.053956025	SMN	50	U-87	48	qRTPCR	
10075	SMN1-72 m01	0.93290782	0.015629669	SMN	50	U-87	48	qRTPCR	
10080	SMN1-73 m01	1.58825563	0.048751072	SMN	50	U-87	48	qRTPCR	
10081	SMN1-74 m01	1.56437988	0.0448992	SMN	50	U-87	48	qRTPCR	
10082	SMN1-75 m01	1.27955748	0.098488946	SMN	50	U-87	48	qRTPCR	
10084	SMN1-76 m01	1.83186084	0.118979665	SMN	50	U-87	48	qRTPCR	
10087	SMN1-77 m01	1.10086	0.028448478	SMN	50	U-87	48	qRTPCR	
10168	SMN1-78 m01	1.352235	0.029767942	SMN	50	U-87	48	qRTPCR	
10169	SMN1-79 m01	1.88486966	0.05823675	SMN	50	U-87	48	qRTPCR	
10170	SMN1-80 m01	1.49747412	0.02452877	SMN	50	U-87	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10337	SMN1-81 m01	1.1460465	0.042571287	SMN	50	U-87	48	qRT-PCR	
10338	SMN1-82 m01	0.92468298	0.022687836	SMN	50	U-87	48	qRT-PCR	
10339	SMN1-83 m01	1.01496185	0.043599152	SMN	50	U-87	48	qRT-PCR	
10763	SMN1-84 m01	2.63329694	0.104402138	SMN	50	U-87	48	qRT-PCR	
10764	SMN1-85 m01	1.25681207	0.078658093	SMN	50	U-87	48	qRT-PCR	
10949	SMN1-86 m01	1.87525327	0.06397273	SMN	50	U-87	48	qRT-PCR	
10951	SMN1-87 m01	1.53023568	0.184873718	SMN	50	U-87	48	qRT-PCR	
10952	SMN1-88 m01	1.43809711	0.091457291	SMN	50	U-87	48	qRT-PCR	
	Ctrl Un	1.02836902	0.055896517	SMN	50	U-87	48	qRT-PCR	
	Ctrl Lipo	1	0.019372556	SMN	50	U-87	48	qRT-PCR	
10953	SMN1-89 m01	1.44971543	0.013205867	SMN	50	U-87	48	qRT-PCR	
10954	SMN1-90 m01	1.67164794	0.008955104	SMN	50	U-87	48	qRT-PCR	
11415	SMN1-91 m01	1.09728182	0.068154011	SMN	50	U-87	48	qRT-PCR	
11418	SMN1-92 m01	1.7841026	0.013132939	SMN	50	U-87	48	qRT-PCR	
11419	SMN1-93 m01	1.98926161	0.108543671	SMN	50	U-87	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11421	SMN1-94 m01	1.52185782	0.028184934	SMN	50	U-87	48	qRT-PCR	
11422	SMN1-95 m01	1.47230805	0.236677508	SMN	50	U-87	48	qRT-PCR	
11423	SMN1-96 m01	0.87520617	0.116238582	SMN	50	U-87	48	qRT-PCR	
11440	SMN1-97 m01	0.96748663	0.210353872	SMN	50	U-87	48	qRT-PCR	
11441	SMN1-98 m01	1.05702982	0.116506452	SMN	50	U-87	48	qRT-PCR	
11443	SMN1-100 m01	1.04695021	0.094728755	SMN	50	U-87	48	qRT-PCR	
12369	SMN1-101 m01	0.31493498	0.17199775	SMN	50	U-87	48	qRT-PCR	
13095	unc-232 m12	1.19076857	0.020533275	SMN	50	U-87	48	qRT-PCR	
13095	unc-232 m01	1.16684135	0.010425283	SMN	50	U-87	48	qRT-PCR	
13096	unc-293 m12	1.11033179	0.027322669	SMN	50	U-87	48	qRT-PCR	
13096	unc-293 m01	0.96484072	0.025822663	SMN	50	U-87	48	qRT-PCR	
	Ctrl Un	1.09507257	0.028915982	SMN	50	U-87	48	qRT-PCR	
	Ctrl Lipo	1	0.010714199	SMN	50	U-87	48	qRT-PCR	
13062	SMN1-01 m03	1.76153061	0.109030274	SMN	30	SK-N-AS	48	qRT-PCR	
13063	SMN1-02 m03	1.49232302	0.124325034	SMN	30	SK-N-AS	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13064	SMN1-03 m03	1.76670197	0.080363586	SMN	30	SK-N-AS	48	qRT-PCR	
13065	SMN1-04 m03	2.06061697	0.079036218	SMN	30	SK-N-AS	48	qRT-PCR	
13066	SMN1-05 m03	2.22979138	0.1344102	SMN	30	SK-N-AS	48	qRT-PCR	
13067	SMN1-06 m03	1.75172919	0.148594324	SMN	30	SK-N-AS	48	qRT-PCR	
13068	SMN1-07 m03	1.48557932	0.068173305	SMN	30	SK-N-AS	48	qRT-PCR	
13069	SMN1-08 m03	1.41275143	0.106861362	SMN	30	SK-N-AS	48	qRT-PCR	
13070	SMN1-09 m03	1.96798314	0.083656818	SMN	30	SK-N-AS	48	qRT-PCR	
13071	SMN1-10 m03	2.8905735	0.1207669	SMN	30	SK-N-AS	48	qRT-PCR	
13072	SMN1-11 m03	2.61387493	0.06296649	SMN	30	SK-N-AS	48	qRT-PCR	
13073	SMN1-12 m03	1.95553907	0.059091514	SMN	30	SK-N-AS	48	qRT-PCR	
13074	SMN1-13 m03	2.26900148	0.069909307	SMN	30	SK-N-AS	48	qRT-PCR	
13075	SMN1-14 m03	2.23992544	0.060869881	SMN	30	SK-N-AS	48	qRT-PCR	
13076	SMN1-15 m03	2.75289981	0.080057758	SMN	30	SK-N-AS	48	qRT-PCR	
13077	SMN1-16 m03	1.4317496	0.025979456	SMN	30	SK-N-AS	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13078	SMN1-17 m03	2.0831693	0.20023673	SMN	30	SK-N-AS	48	qRT-PCR	
13079	SMN1-18 m03	2.56956712	0.111553947	SMN	30	SK-N-AS	48	qRT-PCR	
13080	SMN1-19 m03	2.38121702	0.303778011	SMN	30	SK-N-AS	48	qRT-PCR	
13081	SMN1-20 m03	2.17052026	0.212229254	SMN	30	SK-N-AS	48	qRT-PCR	
13082	SMN1-21 m03	2.48953449	0.130548044	SMN	30	SK-N-AS	48	qRT-PCR	
13083	SMN1-22 m03	1.8049885	0.224362408	SMN	30	SK-N-AS	48	qRT-PCR	
13084	SMN1-23 m03	1.92937893	0.029756205	SMN	30	SK-N-AS	48	qRT-PCR	
13085	SMN1-24 m03	1.65812053	0.06217982	SMN	30	SK-N-AS	48	qRT-PCR	
13086	SMN1-25 m03	1.79285893	0.133462253	SMN	30	SK-N-AS	48	qRT-PCR	
13087	SMN1-26 m03	1.98777316	0.195688275	SMN	30	SK-N-AS	48	qRT-PCR	
13095	unc-232 m12	1.37715601	0.083636562	SMN	30	SK-N-AS	48	qRT-PCR	
13095	unc-232 m01	1.24491795	0.105832582	SMN	30	SK-N-AS	48	qRT-PCR	
13096	unc-293 m12	1.01661197	0.016370439	SMN	30	SK-N-AS	48	qRT-PCR	
13096	unc-293 m01	0.99243308	0.045865487	SMN	30	SK-N-AS	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	Ctrl Un	1.01523451	0.017448707	SMN	30	SK-N-AS	48	qRT-PCR	
	Ctrl Lipo	1	0.029126196	SMN	30	SK-N-AS	48	qRT-PCR	
13062	SMN1-01 m03	1.73166677	0.035564224	SMN	50	SK-N-AS	48	qRT-PCR	
13063	SMN1-02 m03	1.84791335	0.150609652	SMN	50	SK-N-AS	48	qRT-PCR	
13064	SMN1-03 m03	1.99269439	0.071623773	SMN	50	SK-N-AS	48	qRT-PCR	
13065	SMN1-04 m03	2.36449028	0.100570552	SMN	50	SK-N-AS	48	qRT-PCR	
13066	SMN1-05 m03	2.43108466	0.180817974	SMN	50	SK-N-AS	48	qRT-PCR	
13067	SMN1-06 m03	2.09697005	0.06366998	SMN	50	SK-N-AS	48	qRT-PCR	
13068	SMN1-07 m03	2.42413558	0.336942066	SMN	50	SK-N-AS	48	qRT-PCR	
13069	SMN1-08 m03	1.58657165	0.001511565	SMN	50	SK-N-AS	48	qRT-PCR	
13070	SMN1-09 m03	1.74073474	0.135189212	SMN	50	SK-N-AS	48	qRT-PCR	
13071	SMN1-10 m03	2.49528401	0.117753824	SMN	50	SK-N-AS	48	qRT-PCR	
13072	SMN1-11 m03	3.13352758	0.266686515	SMN	50	SK-N-AS	48	qRT-PCR	
13073	SMN1-12 m03	2.18775373	0.081352782	SMN	50	SK-N-AS	48	qRT-PCR	
13074	SMN1-13 m03	2.57805049	0.144974019	SMN	50	SK-N-AS	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13075	SMN1-14 m03	2.68067094	0.283045478	SMN	50	SK-N-AS	48	qRT-PCR	
13076	SMN1-15 m03	6.42561349	0.037787912	SMN	50	SK-N-AS	48	qRT-PCR	
13077	SMN1-16 m03	1.7495812	0.090713246	SMN	50	SK-N-AS	48	qRT-PCR	
13078	SMN1-17 m03	1.95152129	0.039070933	SMN	50	SK-N-AS	48	qRT-PCR	
13079	SMN1-18 m03	2.27080411	0.050183452	SMN	50	SK-N-AS	48	qRT-PCR	
13080	SMN1-19 m03	2.20208412	0.053090251	SMN	50	SK-N-AS	48	qRT-PCR	
13081	SMN1-20 m03	2.5441445	0.209268801	SMN	50	SK-N-AS	48	qRT-PCR	
13082	SMN1-21 m03	2.94442081	0.127216439	SMN	50	SK-N-AS	48	qRT-PCR	
13083	SMN1-22 m03	2.28106599	0.097806544	SMN	50	SK-N-AS	48	qRT-PCR	
13084	SMN1-23 m03	3.44139585	0.236096969	SMN	50	SK-N-AS	48	qRT-PCR	
13085	SMN1-24 m03	1.73785778	0.123432168	SMN	50	SK-N-AS	48	qRT-PCR	
13086	SMN1-25 m03	1.73418306	0.050400387	SMN	50	SK-N-AS	48	qRT-PCR	
13087	SMN1-26 m03	1.98672839	0.137818331	SMN	50	SK-N-AS	48	qRT-PCR	
13095	unc-232 m12	1.01952687	0.02152483	SMN	50	SK-N-AS	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13095	unc-232 m01	1.06265647	0.19162971	SMN	50	SK-N-AS	48	qRTPCR	
13096	unc-293 m12	0.80285492	0.030791547	SMN	50	SK-N-AS	48	qRTPCR	
13096	unc-293 m01	1.45463741	0.088247895	SMN	50	SK-N-AS	48	qRTPCR	
	Ctrl Un	0.87472655	0.043428629	SMN	50	SK-N-AS	48	qRTPCR	
	Ctrl Lipo	1	0.071495321	SMN	50	SK-N-AS	48	qRTPCR	
13088	SMN1-53 m03	1.17693989	0.052165333	SMN	30	GM03813	48	qRTPCR	
13088	SMN1-54 m01	1.53058418	0.065864609	SMN	30	GM03813	48	qRTPCR	
13089	SMN1-55 m01	1.36692604	0.067567719	SMN	30	GM03813	48	qRTPCR	
13090	SMN1-56 m01	1.71988116	0.09933625	SMN	30	GM03813	48	qRTPCR	
13091	SMN1-57 m01	2.527361	0.129310458	SMN	30	GM03813	48	qRTPCR	
13092	SMN1-58 m01	1.59001293	0.078796571	SMN	30	GM03813	48	qRTPCR	
3650	SMN1-59 m01	1.09298064	0.082641104	SMN	30	GM03813	48	qRTPCR	
13093	SMN1-60 m01	1.11236932	0.119612749	SMN	30	GM03813	48	qRTPCR	
13094	SMN1-61 m01	0.98594884	0.003552981	SMN	30	GM03813	48	qRTPCR	
10065	SMN1-62 m01	1.14415618	0.041210967	SMN	30	GM03813	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10066	SMN1-63 m01	1.18667957	0.106666464	SMN	30	GM03813	48	qRT-PCR	
10067	SMN1-64 m01	1.19150677	0.028365555	SMN	30	GM03813	48	qRT-PCR	
10068	SMN1-65 m01	1.07990272	0.063896003	SMN	30	GM03813	48	qRT-PCR	
10069	SMN1-66 m01	1.15746946	0.075254672	SMN	30	GM03813	48	qRT-PCR	
10070	SMN1-67 m01	0.7481427	0.044554047	SMN	30	GM03813	48	qRT-PCR	
10071	SMN1-68 m01	1.08428883	0.04713109	SMN	30	GM03813	48	qRT-PCR	
10072	SMN1-69 m01	1.0075831	0.037381747	SMN	30	GM03813	48	qRT-PCR	
10073	SMN1-70 m01	1.14407698	0.001346995	SMN	30	GM03813	48	qRT-PCR	
10074	SMN1-71 m01	1.20571252	0.064704423	SMN	30	GM03813	48	qRT-PCR	
10075	SMN1-72 m01	1.12664463	0.027384396	SMN	30	GM03813	48	qRT-PCR	
10080	SMN1-73 m01	1.28674219	0.047303459	SMN	30	GM03813	48	qRT-PCR	
10081	SMN1-74 m01	1.39558956	0.036199994	SMN	30	GM03813	48	qRT-PCR	
10082	SMN1-75 m01	1.19280082	0.009453346	SMN	30	GM03813	48	qRT-PCR	
10084	SMN1-76 m01	1.19677155	0.006065234	SMN	30	GM03813	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10087	SMN1-77 m01	1.23494565	0.053055687	SMN	30	GM03813	48	qRT-PCR	
10168	SMN1-78 m01	1.67124565	0.080238561	SMN	30	GM03813	48	qRT-PCR	
10169	SMN1-79 m01	2.73367887	0.120677266	SMN	30	GM03813	48	qRT-PCR	
10170	SMN1-80 m01	2.99729088	0.104995909	SMN	30	GM03813	48	qRT-PCR	
10337	SMN1-81 m01	0.99433432	0.020537838	SMN	30	GM03813	48	qRT-PCR	
10338	SMN1-82 m01	1.0033708	0.021945544	SMN	30	GM03813	48	qRT-PCR	
	Ctrl Un	1.05761842	0.049479996	SMN	30	GM03813	48	qRT-PCR	
	Ctrl Lipo	1	0.007605271	SMN	30	GM03813	48	qRT-PCR	
13088	SMN1-53 m03	0.99042677	0.040377162	SMN	30	GM03814	48	qRT-PCR	
13088	SMN1-54 m01	1.3291862	0.032398287	SMN	30	GM03814	48	qRT-PCR	
13089	SMN1-55 m01	1.07105069	0.009045823	SMN	30	GM03814	48	qRT-PCR	
13090	SMN1-56 m01	1.42636828	0.061352347	SMN	30	GM03814	48	qRT-PCR	
13091	SMN1-57 m01	1.7605377	0.149363212	SMN	30	GM03814	48	qRT-PCR	
13092	SMN1-58 m01	1.40579692	0.050310304	SMN	30	GM03814	48	qRT-PCR	
3650	SMN1-59 m01	1.03169699	0.016777595	SMN	30	GM03814	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13093	SMN1-60 m01	1.05988411	0.052261632	SMN	30	GM03814	48	qRT-PCR	
13094	SMN1-61 m01	0.98026735	0.029457728	SMN	30	GM03814	48	qRT-PCR	
10065	SMN1-62 m01	1.12206312	0.014711891	SMN	30	GM03814	48	qRT-PCR	
10066	SMN1-63 m01	1.14191247	0.092906134	SMN	30	GM03814	48	qRT-PCR	
10067	SMN1-64 m01	1.30014476	0.066542453	SMN	30	GM03814	48	qRT-PCR	
10068	SMN1-65 m01	1.0480548	0.042952858	SMN	30	GM03814	48	qRT-PCR	
10069	SMN1-66 m01	1.01528769	0.037913482	SMN	30	GM03814	48	qRT-PCR	
10070	SMN1-67 m01	0.74104214	0.016222632	SMN	30	GM03814	48	qRT-PCR	
10071	SMN1-68 m01	0.90213667	0.030780176	SMN	30	GM03814	48	qRT-PCR	
10072	SMN1-69 m01	0.96181337	0.037600898	SMN	30	GM03814	48	qRT-PCR	
10073	SMN1-70 m01	1.10014548	0.107168789	SMN	30	GM03814	48	qRT-PCR	
10074	SMN1-71 m01	1.26769519	0.103872704	SMN	30	GM03814	48	qRT-PCR	
10075	SMN1-72 m01	1.30871356	0.062477564	SMN	30	GM03814	48	qRT-PCR	
10080	SMN1-73 m01	1.41163361	0.115569496	SMN	30	GM03814	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10081	SMN1-74 m01	1.22148473	0.023871922	SMN	30	GM03814	48	qRTPCR	
10082	SMN1-75 m01	1.06498572	0.050263043	SMN	30	GM03814	48	qRTPCR	
10084	SMN1-76 m01	1.04325464	0.049661107	SMN	30	GM03814	48	qRTPCR	
10087	SMN1-77 m01	0.99806627	0.111329169	SMN	30	GM03814	48	qRTPCR	
10168	SMN1-78 m01	1.65699963	0.159089837	SMN	30	GM03814	48	qRTPCR	
10169	SMN1-79 m01	2.42069547	0.098699339	SMN	30	GM03814	48	qRTPCR	
10170	SMN1-80 m01	2.62947027	0.186673542	SMN	30	GM03814	48	qRTPCR	
10337	SMN1-81 m01	1.03577703	0.049488616	SMN	30	GM03814	48	qRTPCR	
10338	SMN1-82 m01	0.92632641	0.056255593	SMN	30	GM03814	48	qRTPCR	
	Ctrl Un	0.95678418	0.0253863	SMN	30	GM03814	48	qRTPCR	
	Ctrl Lipo	1	0.138818304	SMN	30	GM03814	48	qRTPCR	
13088	SMN1-53 m03	1.5854441	0.031772568	SMN	30	GM09677	48	qRTPCR	
13088	SMN1-54 m01	1.77358461	0.105394613	SMN	30	GM09677	48	qRTPCR	
13089	SMN1-55 m01	1.42808651	0.043494314	SMN	30	GM09677	48	qRTPCR	
13090	SMN1-56 m01	1.97556225	0.068512755	SMN	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13091	SMN1-57 m01	2.72749147	0.033832205	SMN	30	GM09677	48	qRT-PCR	
13092	SMN1-58 m01	1.64943697	0.062441499	SMN	30	GM09677	48	qRT-PCR	
3650	SMN1-59 m01	1.11693488	0.05926132	SMN	30	GM09677	48	qRT-PCR	
13093	SMN1-60 m01	1.19303961	0.116912241	SMN	30	GM09677	48	qRT-PCR	
13094	SMN1-61 m01	1.04925956	0.081573525	SMN	30	GM09677	48	qRT-PCR	
10065	SMN1-62 m01	1.04453802	0.056925315	SMN	30	GM09677	48	qRT-PCR	
10066	SMN1-63 m01	0.98064072	0.037511153	SMN	30	GM09677	48	qRT-PCR	
10067	SMN1-64 m01	1.09955446	0.034589264	SMN	30	GM09677	48	qRT-PCR	
10068	SMN1-65 m01	1.0836007	0.053876114	SMN	30	GM09677	48	qRT-PCR	
10069	SMN1-66 m01	1.12557096	0.027060841	SMN	30	GM09677	48	qRT-PCR	
10070	SMN1-67 m01	0.68823636	0.010001186	SMN	30	GM09677	48	qRT-PCR	
10071	SMN1-68 m01	0.95611326	0.044013301	SMN	30	GM09677	48	qRT-PCR	
10072	SMN1-69 m01	1.09651623	0.095578189	SMN	30	GM09677	48	qRT-PCR	
10073	SMN1-70 m01	1.12870988	0.028966044	SMN	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10074	SMN1-71 m01	1.01814554	0.031735396	SMN	30	GM09677	48	qRT-PCR	
10075	SMN1-72 m01	1.0453018	0.015341437	SMN	30	GM09677	48	qRT-PCR	
10080	SMN1-73 m01	1.34688281	0.051633688	SMN	30	GM09677	48	qRT-PCR	
10081	SMN1-74 m01	1.21823137	0.043636251	SMN	30	GM09677	48	qRT-PCR	
10082	SMN1-75 m01	1.10129662	0.006915756	SMN	30	GM09677	48	qRT-PCR	
10084	SMN1-76 m01	1.07870912	0.030179237	SMN	30	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	1.41492654	0.081927966	SMN	30	GM09677	48	qRT-PCR	
10168	SMN1-78 m01	2.05474631	0.14774417	SMN	30	GM09677	48	qRT-PCR	
10169	SMN1-79 m01	3.46370384	0.186085222	SMN	30	GM09677	48	qRT-PCR	
10170	SMN1-80 m01	3.30141002	0.153502987	SMN	30	GM09677	48	qRT-PCR	
10337	SMN1-81 m01	0.87600637	0.020502578	SMN	30	GM09677	48	qRT-PCR	
10338	SMN1-82 m01	0.9599919	0.005403034	SMN	30	GM09677	48	qRT-PCR	
	Ctrl Un	1.02123777	0.027305142	SMN	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.076483437	SMN	30	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	1.52037281	0.194916734	SMN	30	GM03813	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10763	SMN1-84 m01	4.92516169	1.194593043	SMN	30	GM03813	48	qRT-PCR	
10764	SMN1-85 m01	1.41371744	0.051139047	SMN	30	GM03813	48	qRT-PCR	
10949	SMN1-86 m01	1.736111155	0.069885536	SMN	30	GM03813	48	qRT-PCR	
10951	SMN1-87 m01	1.8519481	0.05671647	SMN	30	GM03813	48	qRT-PCR	
10952	SMN1-88 m01	1.58365571	0.040175359	SMN	30	GM03813	48	qRT-PCR	
10953	SMN1-89 m01	2.36334667	0.134545493	SMN	30	GM03813	48	qRT-PCR	
10954	SMN1-90 m01	2.00182341	0.153071824	SMN	30	GM03813	48	qRT-PCR	
11415	SMN1-91 m01	1.7314611	0.32623523	SMN	30	GM03813	48	qRT-PCR	
11418	SMN1-92 m01	1.53552076	0.07710905	SMN	30	GM03813	48	qRT-PCR	
11419	SMN1-93 m01	1.85411354	0.096040871	SMN	30	GM03813	48	qRT-PCR	
11421	SMN1-94 m01	1.66244036	0.335378923	SMN	30	GM03813	48	qRT-PCR	
11422	SMN1-95 m01	1.80720913	0.443396594	SMN	30	GM03813	48	qRT-PCR	
11423	SMN1-96 m01	1.37297276	0.092534284	SMN	30	GM03813	48	qRT-PCR	
11440	SMN1-97 m01	1.63963788	0.346835143	SMN	30	GM03813	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11441	SMN1-98 m01	1.37918711	0.134861934	SMN	30	GM03813	48	qRT-PCR	
11443	SMN1-100 m01	1.86055726	0.282144295	SMN	30	GM03813	48	qRT-PCR	
12369	SMN1-101 m01	0.47718837	0.10737891	SMN	30	GM03813	48	qRT-PCR	
13095	unc-232 m12	1.69572477	0.225244558	SMN	30	GM03813	48	qRT-PCR	
13095	unc-232 m01	1.62388063	0.251429623	SMN	30	GM03813	48	qRT-PCR	
13096	unc-293 m12	1.18719437	0.112073109	SMN	30	GM03813	48	qRT-PCR	
13096	unc-293 m01	1.76293672	0.258864352	SMN	30	GM03813	48	qRT-PCR	
	Ctrl Un	1.32788679	0.152969134	SMN	30	GM03813	48	qRT-PCR	
	Ctrl Lipo	1	0.009087149	SMN	30	GM03813	48	qRT-PCR	
10339	SMN1-83 m01	1.01119376	0.045323195	SMN	30	GM03814	48	qRT-PCR	
10763	SMN1-84 m01	2.61064901	0.129208709	SMN	30	GM03814	48	qRT-PCR	
10764	SMN1-85 m01	1.03248361	0.026196629	SMN	30	GM03814	48	qRT-PCR	
10949	SMN1-86 m01	1.13996393	0.040526949	SMN	30	GM03814	48	qRT-PCR	
10951	SMN1-87 m01	1.1863933	0.044201452	SMN	30	GM03814	48	qRT-PCR	
10952	SMN1-88 m01	1.24513971	0.060269432	SMN	30	GM03814	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10953	SMN1-89 m01	1.21898689	0.024279343	SMN	30	GM03814	48	qRT-PCR	
10954	SMN1-90 m01	1.21796187	0.034916973	SMN	30	GM03814	48	qRT-PCR	
11415	SMN1-91 m01	1.00244882	0.077089551	SMN	30	GM03814	48	qRT-PCR	
11418	SMN1-92 m01	1.41552769	0.163341811	SMN	30	GM03814	48	qRT-PCR	
11419	SMN1-93 m01	1.68351777	0.239972216	SMN	30	GM03814	48	qRT-PCR	
11421	SMN1-94 m01	1.08989286	0.074179484	SMN	30	GM03814	48	qRT-PCR	
11422	SMN1-95 m01	1.20372366	0.136986753	SMN	30	GM03814	48	qRT-PCR	
11423	SMN1-96 m01	1.08559928	0.026388389	SMN	30	GM03814	48	qRT-PCR	
11440	SMN1-97 m01	1.03335292	0.022961034	SMN	30	GM03814	48	qRT-PCR	
11441	SMN1-98 m01	0.84056059	0.031260632	SMN	30	GM03814	48	qRT-PCR	
11443	SMN1-100 m01	1.13356194	0.122238842	SMN	30	GM03814	48	qRT-PCR	
12369	SMN1-101 m01	0.41693663	0.020121695	SMN	30	GM03814	48	qRT-PCR	
13095	unc-232 m12	1.38094018	0.024900762	SMN	30	GM03814	48	qRT-PCR	
13095	unc-232 m01	1.24987705	0.072347147	SMN	30	GM03814	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13096	unc-293 m12	1.19914322	0.05320868	SMN	30	GM03814	48	qRTPCR	
13096	unc-293 m01	1.13872311	0.047975132	SMN	30	GM03814	48	qRTPCR	
	Ctrl Un	1.03393862	0.069004637	SMN	30	GM03814	48	qRTPCR	
	Ctrl Lipo	1	0.043466951	SMN	30	GM03814	48	qRTPCR	
10339	SMN1-83 m01	1.21684322	0.01521815	SMN	30	GM09677	48	qRTPCR	
10763	SMN1-84 m01	3.87682799	0.148980303	SMN	30	GM09677	48	qRTPCR	
10764	SMN1-85 m01	1.24686601	0.065959113	SMN	30	GM09677	48	qRTPCR	
10949	SMN1-86 m01	1.53232002	0.070075314	SMN	30	GM09677	48	qRTPCR	
10951	SMN1-87 m01	1.59058353	0.104008047	SMN	30	GM09677	48	qRTPCR	
10952	SMN1-88 m01	1.51302989	0.107170168	SMN	30	GM09677	48	qRTPCR	
10953	SMN1-89 m01	1.4267206	0.038181887	SMN	30	GM09677	48	qRTPCR	
10954	SMN1-90 m01	1.42084039	0.0412365	SMN	30	GM09677	48	qRTPCR	
11415	SMN1-91 m01	1.23090402	0.068969535	SMN	30	GM09677	48	qRTPCR	
11418	SMN1-92 m01	1.77894136	0.071155599	SMN	30	GM09677	48	qRTPCR	
11419	SMN1-93 m01	2.51073515	0.095629595	SMN	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11421	SMN1-94 m01	1.28909	0.035164269	SMN	30	GM09677	48	qRT-PCR	
11422	SMN1-95 m01	1.49608719	0.092832576	SMN	30	GM09677	48	qRT-PCR	
11423	SMN1-96 m01	1.2055077	0.046642931	SMN	30	GM09677	48	qRT-PCR	
11440	SMN1-97 m01	1.18160532	0.035005962	SMN	30	GM09677	48	qRT-PCR	
11441	SMN1-98 m01	1.08732822	0.022657224	SMN	30	GM09677	48	qRT-PCR	
11443	SMN1-100 m01	1.14747354	0.022656066	SMN	30	GM09677	48	qRT-PCR	
12369	SMN1-101 m01	0.288081	0.030736597	SMN	30	GM09677	48	qRT-PCR	
13095	unc-232 m12	1.4711582	0.054811271	SMN	30	GM09677	48	qRT-PCR	
13095	unc-232 m01	1.3240595	0.021340772	SMN	30	GM09677	48	qRT-PCR	
13096	unc-293 m12	1.2827479	0.011628927	SMN	30	GM09677	48	qRT-PCR	
13096	unc-293 m01	1.49956278	0.070843668	SMN	30	GM09677	48	qRT-PCR	
	Ctrl Un	1.15604088	0.048096904	SMN	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.035444734	SMN	30	GM09677	48	qRT-PCR	
13088	SMN1-53 m03	1.08604974	0.044801785	SMN	30	GM00232	48	qRT-PCR	
13088	SMN1-54 m01	1.05266927	0.087997876	SMN	30	GM00232	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13089	SMN1-55 m01	1.21348485	0.10797169	SMN	30	GM00232	48	qRT-PCR	
13090	SMN1-56 m01	1.62533649	0.112388889	SMN	30	GM00232	48	qRT-PCR	
13091	SMN1-57 m01	1.72747938	0.054162932	SMN	30	GM00232	48	qRT-PCR	
13092	SMN1-58 m01	1.33002076	0.236261556	SMN	30	GM00232	48	qRT-PCR	
3650	SMN1-59 m01	1.05342037	0.122301998	SMN	30	GM00232	48	qRT-PCR	
13093	SMN1-60 m01	1.14938369	0.091511069	SMN	30	GM00232	48	qRT-PCR	
13094	SMN1-61 m01	1.03657213	0.077644326	SMN	30	GM00232	48	qRT-PCR	
10065	SMN1-62 m01	1.22300019	0.092886522	SMN	30	GM00232	48	qRT-PCR	
10066	SMN1-63 m01	1.15565627	0.072356952	SMN	30	GM00232	48	qRT-PCR	
10067	SMN1-64 m01	1.07617762	0.039543636	SMN	30	GM00232	48	qRT-PCR	
10068	SMN1-65 m01	1.04874221	0.05549858	SMN	30	GM00232	48	qRT-PCR	
10069	SMN1-66 m01	0.98644251	0.015373474	SMN	30	GM00232	48	qRT-PCR	
10070	SMN1-67 m01	0.6775155	0.024783371	SMN	30	GM00232	48	qRT-PCR	
10071	SMN1-68 m01	0.99744854	0.017661751	SMN	30	GM00232	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10072	SMN1-69 m01	1.03021795	0.058925554	SMN	30	GM00232	48	qRT-PCR	
10073	SMN1-70 m01	1.04249192	0.046218324	SMN	30	GM00232	48	qRT-PCR	
10074	SMN1-71 m01	1.08883082	0.037443412	SMN	30	GM00232	48	qRT-PCR	
10075	SMN1-72 m01	1.10035634	0.046779617	SMN	30	GM00232	48	qRT-PCR	
10080	SMN1-73 m01	1.09314536	0.006129568	SMN	30	GM00232	48	qRT-PCR	
10081	SMN1-74 m01	1.11475475	0.023882237	SMN	30	GM00232	48	qRT-PCR	
10082	SMN1-75 m01	1.10197636	0.015843373	SMN	30	GM00232	48	qRT-PCR	
10084	SMN1-76 m01	1.14570674	0.090880502	SMN	30	GM00232	48	qRT-PCR	
10087	SMN1-77 m01	1.06970023	0.05198802	SMN	30	GM00232	48	qRT-PCR	
10168	SMN1-78 m01	1.11647216	0.059645118	SMN	30	GM00232	48	qRT-PCR	
10169	SMN1-79 m01	1.64496777	0.196747066	SMN	30	GM00232	48	qRT-PCR	
10170	SMN1-80 m01	1.79902695	0.194205069	SMN	30	GM00232	48	qRT-PCR	
10337	SMN1-81 m01	0.98609605	0.059039561	SMN	30	GM00232	48	qRT-PCR	
10338	SMN1-82 m01	1.04134979	0.023971736	SMN	30	GM00232	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	Ctrl Un	1.14411424	0.100050542	SMN	30	GM00232	48	qRT-PCR	
	Ctrl Lipo	1	0.07970209	SMN	30	GM00232	48	qRT-PCR	
13088	SMN1-53 m03	1.52068215	0.144338494	SMN	30	GM03815	48	qRT-PCR	
13088	SMN1-54 m01	1.18327318	0.040613612	SMN	30	GM03815	48	qRT-PCR	
13089	SMN1-55 m01	1.23942939	0.037477765	SMN	30	GM03815	48	qRT-PCR	
13090	SMN1-56 m01	1.71273722	0.268341524	SMN	30	GM03815	48	qRT-PCR	
13091	SMN1-57 m01	1.80090893	0.15887446	SMN	30	GM03815	48	qRT-PCR	
13092	SMN1-58 m01	1.23492808	0.042813295	SMN	30	GM03815	48	qRT-PCR	
3650	SMN1-59 m01	1.04263467	0.070506434	SMN	30	GM03815	48	qRT-PCR	
13093	SMN1-60 m01	1.09528465	0.047057943	SMN	30	GM03815	48	qRT-PCR	
13094	SMN1-61 m01	1.73804062	0.189926172	SMN	30	GM03815	48	qRT-PCR	
10065	SMN1-62 m01	1.16429452	0.054618176	SMN	30	GM03815	48	qRT-PCR	
10066	SMN1-63 m01	1.38313249	0.224089478	SMN	30	GM03815	48	qRT-PCR	
10067	SMN1-64 m01	1.6809956	0.073702107	SMN	30	GM03815	48	qRT-PCR	
10068	SMN1-65 m01	1.17806284	0.032625899	SMN	30	GM03815	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10069	SMN1-66 m01	1.16023332	0.033138619	SMN	30	GM03815	48	qRT-PCR	
10070	SMN1-67 m01	0.78236474	0.085666716	SMN	30	GM03815	48	qRT-PCR	
10071	SMN1-68 m01	1.04864537	0.025950423	SMN	30	GM03815	48	qRT-PCR	
10072	SMN1-69 m01	1.24683638	0.113429653	SMN	30	GM03815	48	qRT-PCR	
10073	SMN1-70 m01	1.17315928	0.069791373	SMN	30	GM03815	48	qRT-PCR	
10074	SMN1-71 m01	1.31853371	0.048885968	SMN	30	GM03815	48	qRT-PCR	
10075	SMN1-72 m01	1.5222552	0.109184626	SMN	30	GM03815	48	qRT-PCR	
10080	SMN1-73 m01	1.61723289	0.130134486	SMN	30	GM03815	48	qRT-PCR	
10081	SMN1-74 m01	1.3550506	0.098787876	SMN	30	GM03815	48	qRT-PCR	
10082	SMN1-75 m01	1.11930164	0.074015162	SMN	30	GM03815	48	qRT-PCR	
10084	SMN1-76 m01	1.09864632	0.019061995	SMN	30	GM03815	48	qRT-PCR	
10087	SMN1-77 m01	1.55822024	0.052007964	SMN	30	GM03815	48	qRT-PCR	
10168	SMN1-78 m01	1.74326871	0.154153286	SMN	30	GM03815	48	qRT-PCR	
10169	SMN1-79 m01	2.22158214	0.109943722	SMN	30	GM03815	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10170	SMN1-80 m01	3.27182486	0.185738825	SMN	30	GM03815	48	qRTPCR	
10337	SMN1-81 m01	1.32881709	0.092470664	SMN	30	GM03815	48	qRTPCR	
10338	SMN1-82 m01	1.05797861	0.02617898	SMN	30	GM03815	48	qRTPCR	
	Ctrl Un	1.06639452	0.070275912	SMN	30	GM03815	48	qRTPCR	
	Ctrl Lipo	1	0.066757547	SMN	30	GM03815	48	qRTPCR	
13088	SMN1-53 m03	1.14636231	0.055933965	SMN	30	GM22592	48	qRTPCR	
13088	SMN1-54 m01	0.96784555	0.035714254	SMN	30	GM22592	48	qRTPCR	
13089	SMN1-55 m01	1.06424159	0.015782764	SMN	30	GM22592	48	qRTPCR	
13090	SMN1-56 m01	1.34188275	0.055318681	SMN	30	GM22592	48	qRTPCR	
13091	SMN1-57 m01	1.59390924	0.018177266	SMN	30	GM22592	48	qRTPCR	
13092	SMN1-58 m01	1.11544326	0.031493292	SMN	30	GM22592	48	qRTPCR	
3650	SMN1-59 m01	1.07036319	0.020138881	SMN	30	GM22592	48	qRTPCR	
13093	SMN1-60 m01	1.28630481	0.093794834	SMN	30	GM22592	48	qRTPCR	
13094	SMN1-61 m01	1.17898071	0.042702668	SMN	30	GM22592	48	qRTPCR	
10065	SMN1-62 m01	1.19028785	0.019560255	SMN	30	GM22592	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10066	SMN1-63 m01	1.13536151	0.047030129	SMN	30	GM22592	48	qRT-PCR	
10067	SMN1-64 m01	1.06356685	0.031189402	SMN	30	GM22592	48	qRT-PCR	
10068	SMN1-65 m01	1.13906042	0.055852567	SMN	30	GM22592	48	qRT-PCR	
10069	SMN1-66 m01	1.00851377	0.042958179	SMN	30	GM22592	48	qRT-PCR	
10070	SMN1-67 m01	0.75570038	0.041773931	SMN	30	GM22592	48	qRT-PCR	
10071	SMN1-68 m01	1.31865368	0.100668141	SMN	30	GM22592	48	qRT-PCR	
10072	SMN1-69 m01	1.08934578	0.010014409	SMN	30	GM22592	48	qRT-PCR	
10073	SMN1-70 m01	1.00967299	0.025819726	SMN	30	GM22592	48	qRT-PCR	
10074	SMN1-71 m01	1.04026164	0.048730555	SMN	30	GM22592	48	qRT-PCR	
10075	SMN1-72 m01	0.94866004	0.046886869	SMN	30	GM22592	48	qRT-PCR	
10080	SMN1-73 m01	1.1226587	0.072560256	SMN	30	GM22592	48	qRT-PCR	
10081	SMN1-74 m01	1.09506726	0.070354868	SMN	30	GM22592	48	qRT-PCR	
10082	SMN1-75 m01	1.0863241	0.037711526	SMN	30	GM22592	48	qRT-PCR	
10084	SMN1-76 m01	1.27567006	0.125653848	SMN	30	GM22592	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10087	SMN1-77 m01	1.0190716	0.010503333	SMN	30	GM22592	48	qRTPCR	
10168	SMN1-78 m01	1.15709306	0.03959328	SMN	30	GM22592	48	qRTPCR	
10169	SMN1-79 m01	1.73804588	0.032706562	SMN	30	GM22592	48	qRTPCR	
10170	SMN1-80 m01	1.92972222	0.033387117	SMN	30	GM22592	48	qRTPCR	
10337	SMN1-81 m01	1.02035355	0.06295254	SMN	30	GM22592	48	qRTPCR	
10338	SMN1-82 m01	0.88831056	0.010722375	SMN	30	GM22592	48	qRTPCR	
	Ctrl Un	1.08878253	0.0281464	SMN	30	GM22592	48	qRTPCR	
	Ctrl Lipo	1	0.033122379	SMN	30	GM22592	48	qRTPCR	
10339	SMN1-83 m01	0.92690312	0.040173213	SMN	30	GM00232	48	qRTPCR	
10763	SMN1-84 m01	1.74567159	0.137322108	SMN	30	GM00232	48	qRTPCR	
10764	SMN1-85 m01	1.13205865	0.075376875	SMN	30	GM00232	48	qRTPCR	
10949	SMN1-86 m01	1.0702942	0.018996158	SMN	30	GM00232	48	qRTPCR	
10951	SMN1-87 m01	1.1867941	0.04720604	SMN	30	GM00232	48	qRTPCR	
10952	SMN1-88 m01	1.17636071	0.069177517	SMN	30	GM00232	48	qRTPCR	
10953	SMN1-89 m01	1.17289802	0.044681682	SMN	30	GM00232	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10954	SMN1-90 m01	1.1840071	0.145210715	SMN	30	GM00232	48	qRT-PCR	
11415	SMN1-91 m01	1.17818752	0.098879035	SMN	30	GM00232	48	qRT-PCR	
11418	SMN1-92 m01	1.18956481	0.122045104	SMN	30	GM00232	48	qRT-PCR	
11419	SMN1-93 m01	1.32373285	0.06539985	SMN	30	GM00232	48	qRT-PCR	
11421	SMN1-94 m01	0.97555596	0.069660084	SMN	30	GM00232	48	qRT-PCR	
11422	SMN1-95 m01	0.99217823	0.037988843	SMN	30	GM00232	48	qRT-PCR	
11423	SMN1-96 m01	0.99922452	0.055920586	SMN	30	GM00232	48	qRT-PCR	
11440	SMN1-97 m01	0.97839737	0.070198663	SMN	30	GM00232	48	qRT-PCR	
11441	SMN1-98 m01	1.25027315	0.099466303	SMN	30	GM00232	48	qRT-PCR	
11443	SMN1-100 m01	1.39494836	0.090235303	SMN	30	GM00232	48	qRT-PCR	
12369	SMN1-101 m01	0.50628311	0.040266894	SMN	30	GM00232	48	qRT-PCR	
13095	unc-232 m12	1.32484367	0.028469083	SMN	30	GM00232	48	qRT-PCR	
13095	unc-232 m01	1.26707302	0.129523425	SMN	30	GM00232	48	qRT-PCR	
13096	unc-293 m12	0.9941798	0.038648744	SMN	30	GM00232	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13096	unc-293 m01	1.09440559	0.105971879	SMN	30	GM00232	48	qRTPCR	
	Ctrl Un	1.32672875	0.160308854	SMN	30	GM00232	48	qRTPCR	
	Ctrl Lipo	1	0.159820797	SMN	30	GM00232	48	qRTPCR	
10339	SMN1-83 m01	0.51779551	0.016708132	SMN	30	GM03815	48	qRTPCR	
10763	SMN1-84 m01	1.0566458	0.184358867	SMN	30	GM03815	48	qRTPCR	
10764	SMN1-85 m01	0.48992996	0.012929421	SMN	30	GM03815	48	qRTPCR	
10949	SMN1-86 m01	0.5449103	0.023095063	SMN	30	GM03815	48	qRTPCR	
10951	SMN1-87 m01	0.51135121	0.036554098	SMN	30	GM03815	48	qRTPCR	
10952	SMN1-88 m01	0.50587993	0.018161073	SMN	30	GM03815	48	qRTPCR	
10953	SMN1-89 m01	0.54552877	0.016044171	SMN	30	GM03815	48	qRTPCR	
10954	SMN1-90 m01	0.67877892	0.088741737	SMN	30	GM03815	48	qRTPCR	
11415	SMN1-91 m01	0.55959319	0.022738763	SMN	30	GM03815	48	qRTPCR	
11418	SMN1-92 m01	0.84682074	0.05583765	SMN	30	GM03815	48	qRTPCR	
11419	SMN1-93 m01	0.71225822	0.032715689	SMN	30	GM03815	48	qRTPCR	
11421	SMN1-94 m01	0.59505425	0.036003454	SMN	30	GM03815	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11422	SMN1-95 m01	0.68669184	0.020059615	SMN	30	GM03815	48	qRT-PCR	
11423	SMN1-96 m01	0.72720958	0.129025367	SMN	30	GM03815	48	qRT-PCR	
11440	SMN1-97 m01	0.4252869	0.021582722	SMN	30	GM03815	48	qRT-PCR	
11441	SMN1-98 m01	0.58050176	0.064707664	SMN	30	GM03815	48	qRT-PCR	
11443	SMN1-100 m01	0.54525598	0.005011356	SMN	30	GM03815	48	qRT-PCR	
12369	SMN1-101 m01	0.18912343	0.024222321	SMN	30	GM03815	48	qRT-PCR	
13095	unc-232 m12	0.58423649	0.039863175	SMN	30	GM03815	48	qRT-PCR	
13095	unc-232 m01	0.98101472	0.18621629	SMN	30	GM03815	48	qRT-PCR	
13096	unc-293 m12	0.65385691	0.073701747	SMN	30	GM03815	48	qRT-PCR	
13096	unc-293 m01	0.72781826	0.085937045	SMN	30	GM03815	48	qRT-PCR	
	Ctrl Un	0.54485293	0.03829476	SMN	30	GM03815	48	qRT-PCR	
	Ctrl Lipo	1	0.111439011	SMN	30	GM03815	48	qRT-PCR	
10339	SMN1-83 m01	1.00695812	0.086137524	SMN	30	GM22592	48	qRT-PCR	
10763	SMN1-84 m01	2.09763841	0.18323225	SMN	30	GM22592	48	qRT-PCR	
10764	SMN1-85 m01	0.97690192	0.032451283	SMN	30	GM22592	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10949	SMN1-86 m01	1.06858446	0.052275381	SMN	30	GM22592	48	qRT-PCR	
10951	SMN1-87 m01	0.88354279	0.021492758	SMN	30	GM22592	48	qRT-PCR	
10952	SMN1-88 m01	1.20182221	0.074552712	SMN	30	GM22592	48	qRT-PCR	
10953	SMN1-89 m01	1.18522046	0.136487889	SMN	30	GM22592	48	qRT-PCR	
10954	SMN1-90 m01	1.5156599	0.15489579	SMN	30	GM22592	48	qRT-PCR	
11415	SMN1-91 m01	1.40991272	0.158816648	SMN	30	GM22592	48	qRT-PCR	
11418	SMN1-92 m01	1.21940342	0.093675536	SMN	30	GM22592	48	qRT-PCR	
11419	SMN1-93 m01	1.15996783	0.054170813	SMN	30	GM22592	48	qRT-PCR	
11421	SMN1-94 m01	1.13663306	0.086434434	SMN	30	GM22592	48	qRT-PCR	
11422	SMN1-95 m01	1.10773856	0.10669286	SMN	30	GM22592	48	qRT-PCR	
11423	SMN1-96 m01	0.9961877	0.106343078	SMN	30	GM22592	48	qRT-PCR	
11440	SMN1-97 m01	1.02484659	0.0958652	SMN	30	GM22592	48	qRT-PCR	
11441	SMN1-98 m01	1.17128556	0.033761231	SMN	30	GM22592	48	qRT-PCR	
11443	SMN1-100 m01	1.44312565	0.094146363	SMN	30	GM22592	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
12369	SMN1-101 m01	0.21276778	0.0117523	SMN	30	GM22592	48	qRTPCR	
13095	unc-232 m12	1.18188663	0.044920061	SMN	30	GM22592	48	qRTPCR	
13095	unc-232 m01	1.29329972	0.061249452	SMN	30	GM22592	48	qRTPCR	
13096	unc-293 m12	1.05631259	0.024663045	SMN	30	GM22592	48	qRTPCR	
13096	unc-293 m01	1.11729872	0.102126603	SMN	30	GM22592	48	qRTPCR	
	Ctrl Un	1.26943109	0.040083922	SMN	30	GM22592	48	qRTPCR	
	Ctrl Lipo	1	0.034336458	SMN Full	30	GM22592	48	qRTPCR	
11384	SMN1-37 m01	5.9542612	0.290640254	SMN Full	100	GM09677	48	qRTPCR	
11384	SMN1-37 m01	5.39087769	0.241884316	SMN Full	50	GM09677	48	qRTPCR	
11384	SMN1-37 m01	2.79888032	0.017318969	SMN Full	25	GM09677	48	qRTPCR	
11384	SMN1-37 m01	1.82559566	0.039136131	SMN Full	12.5	GM09677	48	qRTPCR	
11384	SMN1-37 m01	1.33628617	0.096519403	SMN Full	6.25	GM09677	48	qRTPCR	
11384	SMN1-37 m01	1.06712865	0.066053843	SMN Full	3.125	GM09677	48	qRTPCR	
11384	SMN1-37 m01	1.10936601	0.014082497	SMN Full	1.25	GM09677	48	qRTPCR	
10087	SMN1-77 m01	3.05323733	0.080096111	SMN Full	100	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10087	SMN1-77 m01	3.9171689	0.203914666	SMN Full	50	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	2.57971982	0.116653144	SMN Full	25	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	1.9222121	0.113382861	SMN Full	12.5	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	1.7467303	0.077816887	SMN Full	6.25	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	1.42937738	0.038046739	SMN Full	3.125	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	1.22473848	0.015879533	SMN Full	1.25	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	3.94736096	0.129336341	SMN Full	100	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	3.96658819	0.099613774	SMN Full	50	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	1.96505234	0.144350711	SMN Full	25	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	1.40459561	0.066254462	SMN Full	12.5	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	1.21022789	0.021693529	SMN Full	6.25	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	1.02775504	0.034847841	SMN Full	3.125	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	0.97115843	0.007975519	SMN Full	1.25	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	3.35218523	0.088164478	SMN Full	100	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10954	SMN1-90 m01	3.16330595	0.028689068	SMN Full	50	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	1.5589865	0.041002104	SMN Full	25	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	1.2184128	0.014395758	SMN Full	12.5	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	1.17741173	0.039945158	SMN Full	6.25	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	1.06885864	0.052240464	SMN Full	3.125	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	1.09681422	0.020245023	SMN Full	1.25	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.016095308	SMN Full	0	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.10766465	0.013675601	SMN del7	100	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.07677743	0.007617795	SMN del7	50	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.2405718	0.018419112	SMN del7	25	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.56386612	0.047377291	SMN del7	12.5	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.7988113	0.056316613	SMN del7	6.25	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.92651529	0.017608283	SMN del7	3.125	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	1.13816432	0.006057032	SMN del7	1.25	GM09677	48	qRT-PCR	
10087	SMN1-77	0.16763182	0.004593329	SMN	100	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
10087	SMN1-77 m01	0.0987514	0.016242325	SMN del7	50	GM09677	48	qRTPCR	
10087	SMN1-77 m01	0.11685404	0.005950861	SMN del7	25	GM09677	48	qRTPCR	
10087	SMN1-77 m01	0.17077336	0.008968329	SMN del7	12.5	GM09677	48	qRTPCR	
10087	SMN1-77 m01	0.29594078	0.034339904	SMN del7	6.25	GM09677	48	qRTPCR	
10087	SMN1-77 m01	0.49603609	0.019354816	SMN del7	3.125	GM09677	48	qRTPCR	
10087	SMN1-77 m01	0.71056618	0.049590838	SMN del7	1.25	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.27540994	0.013082079	SMN del7	100	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.21591218	0.024780671	SMN del7	50	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.32948585	0.03767129	SMN del7	25	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.49464481	0.018127851	SMN del7	12.5	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.75743969	0.022196849	SMN del7	6.25	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.86983308	0.044240282	SMN del7	3.125	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.94361766	0.02892766	SMN del7	1.25	GM09677	48	qRTPCR	
10954	SMN1-90	0.93107336	0.019724548	SMN	100	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
10954	SMN1-90 m01	0.95391457	0.041511303	SMN del7	50	GM09677	48	qRTPCR	
10954	SMN1-90 m01	0.72340414	0.054975642	SMN del7	25	GM09677	48	qRTPCR	
10954	SMN1-90 m01	0.73825121	0.062877224	SMN del7	12.5	GM09677	48	qRTPCR	
10954	SMN1-90 m01	0.93965875	0.035183679	SMN del7	6.25	GM09677	48	qRTPCR	
10954	SMN1-90 m01	1.03317202	0.06366904	SMN del7	3.125	GM09677	48	qRTPCR	
10954	SMN1-90 m01	1.07044084	0.057947407	SMN del7	1.25	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.050445935	SMN del7	0	GM09677	48	qRTPCR	
13088	SMN1-53 m03	6.8463352	0.856443314	SMN Full	100	GM09677	48	qRTPCR	
13088	SMN1-53 m03	9.62228167	0.234752009	SMN Full	50	GM09677	48	qRTPCR	
13088	SMN1-53 m03	4.6378624	0.126987377	SMN Full	25	GM09677	48	qRTPCR	
13088	SMN1-53 m03	3.18308378	0.233853217	SMN Full	12.5	GM09677	48	qRTPCR	
13088	SMN1-53 m03	3.08052546	0.199971524	SMN Full	6.25	GM09677	48	qRTPCR	
13088	SMN1-53 m03	2.90931736	0.106957536	SMN Full	3.125	GM09677	48	qRTPCR	
13088	SMN1-53 m03	2.62194563	0.111464606	SMN Full	1.25	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m03								
13088	SMN1-54 m01	3.8336987	0.270658933	SMN Full	100	GM09677	48	qRTPCR	
13088	SMN1-54 m01	5.26802487	0.095288013	SMN Full	50	GM09677	48	qRTPCR	
13088	SMN1-54 m01	4.73743077	0.476930024	SMN Full	25	GM09677	48	qRTPCR	
13088	SMN1-54 m01	2.93080957	0.175656776	SMN Full	12.5	GM09677	48	qRTPCR	
13088	SMN1-54 m01	2.51816601	0.24634728	SMN Full	6.25	GM09677	48	qRTPCR	
13088	SMN1-54 m01	2.70486897	0.195936533	SMN Full	3.125	GM09677	48	qRTPCR	
13088	SMN1-54 m01	2.0202206	0.092539952	SMN Full	1.25	GM09677	48	qRTPCR	
13095	unc-232 m01	1.24791763	0.054920504	SMN Full	100	GM09677	48	qRTPCR	
	Ctrl Un	1.45789687	0.054723979	SMN Full	0	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.042415029	SMN Full	0	GM09677	48	qRTPCR	
13088	SMN1-53 m03	0.06074849	0.002933624	SMN del7	100	GM09677	48	qRTPCR	
13088	SMN1-53 m03	0.04619314	0.01517452	SMN del7	50	GM09677	48	qRTPCR	
13088	SMN1-53 m03	0.04719771	0.002006351	SMN del7	25	GM09677	48	qRTPCR	
13088	SMN1-53 m03	0.05847386	0.007589362	SMN del7	12.5	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13088	SMN1-53 m03	0.07408243	0.009511611	SMN del7	6.25	GM09677	48	qRTPCR	
13088	SMN1-53 m03	0.10840254	0.007380012	SMN del7	3.125	GM09677	48	qRTPCR	
13088	SMN1-53 m03	0.12403134	0.016857354	SMN del7	1.25	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.12438851	0.012295936	SMN del7	100	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.04933631	0.003621923	SMN del7	50	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.02915638	0.005894236	SMN del7	25	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.04690527	0.009339682	SMN del7	12.5	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.05669103	0.010312509	SMN del7	6.25	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.12713748	0.0096972	SMN del7	3.125	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.24966606	0.014247099	SMN del7	1.25	GM09677	48	qRTPCR	
13095	unc-232 m01	1.60419159	0.109607645	SMN del7	100	GM09677	48	qRTPCR	
	Ctrl Un	1.50899881	0.082961814	SMN del7	0	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.079894541	SMN del7	0	GM09677	48	qRTPCR	
11374	SMN1-27 m01	1.06289151	0.025841933	SMN Full	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11375	SMN1-28 m01	1.04865509	0.016855456	SMN Full	30	GM09677	48	qRT-PCR	
11376	SMN1-29 m01	1.01070925	0.013243258	SMN Full	30	GM09677	48	qRT-PCR	
11377	SMN1-30 m01	1.67527223	0.011430256	SMN Full	30	GM09677	48	qRT-PCR	
11378	SMN1-31 m01	1.84472058	0.021575776	SMN Full	30	GM09677	48	qRT-PCR	
11379	SMN1-32 m01	1.16562841	0.03357926	SMN Full	30	GM09677	48	qRT-PCR	
11380	SMN1-33 m01	1.61132054	0.014172718	SMN Full	30	GM09677	48	qRT-PCR	
11381	SMN1-34 m01	1.83630558	0.065951741	SMN Full	30	GM09677	48	qRT-PCR	
11382	SMN1-35 m01	2.14294563	0.0670525	SMN Full	30	GM09677	48	qRT-PCR	
11383	SMN1-36 m01	1.35517564	0.002069697	SMN Full	30	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	1.98891781	0.070088796	SMN Full	30	GM09677	48	qRT-PCR	
11385	SMN1-38 m01	1.60182608	0.003732147	SMN Full	30	GM09677	48	qRT-PCR	
11386	SMN1-39 m01	1.30439676	0.021907618	SMN Full	30	GM09677	48	qRT-PCR	
11387	SMN1-40 m01	0.98382696	0.026234551	SMN Full	30	GM09677	48	qRT-PCR	
11388	SMN1-41 m01	0.96131056	0.017976722	SMN Full	30	GM09677	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11389	SMN1-42 m01	0.96544777	0.039675519	SMN Full	30	GM09677	48	qRT-PCR	
11390	SMN1-43 m01	1.14786301	0.029602393	SMN Full	30	GM09677	48	qRT-PCR	
11391	SMN1-44 m01	0.58910025	0.019381422	SMN Full	30	GM09677	48	qRT-PCR	
11392	SMN1-45 m01	0.89050638	0.030829917	SMN Full	30	GM09677	48	qRT-PCR	
11393	SMN1-46 m01	0.7659958	0.01318192	SMN Full	30	GM09677	48	qRT-PCR	
11394	SMN1-47 m01	0.95365719	0.024094251	SMN Full	30	GM09677	48	qRT-PCR	
11395	SMN1-48 m01	1.02427285	0.036889509	SMN Full	30	GM09677	48	qRT-PCR	
11396	SMN1-49 m01	0.82692071	0.023805443	SMN Full	30	GM09677	48	qRT-PCR	
11397	SMN1-50 m01	1.13263218	0.068915365	SMN Full	30	GM09677	48	qRT-PCR	
11398	SMN1-51 m01	1.19062518	0.069378809	SMN Full	30	GM09677	48	qRT-PCR	
11399	SMN1-52 m01	0.85430672	0.034519845	SMN Full	30	GM09677	48	qRT-PCR	
13088	SMN1-53 m03	2.75059401	0.133550763	SMN Full	30	GM09677	48	qRT-PCR	
13088	SMN1-54 m01	2.40598139	0.08218513	SMN Full	30	GM09677	48	qRT-PCR	
13089	SMN1-55 m01	1.24697911	0.06516549	SMN Full	30	GM09677	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13090	SMN1-56 m01	0.6013976	0.022355997	SMN Full	30	GM09677	48	qRTPCR	
13095	unc-232 m12	1.05797158	0.03096159	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.047369909	SMN Full	30	GM09677	48	qRTPCR	
11374	SMN1-27 m01	0.97917098	0.038180283	SMN del7	30	GM09677	48	qRTPCR	
11375	SMN1-28 m01	0.97976897	0.056019638	SMN del7	30	GM09677	48	qRTPCR	
11376	SMN1-29 m01	1.50572431	0.109802089	SMN del7	30	GM09677	48	qRTPCR	
11377	SMN1-30 m01	0.63237745	0.040101131	SMN del7	30	GM09677	48	qRTPCR	
11378	SMN1-31 m01	0.16643691	0.007954712	SMN del7	30	GM09677	48	qRTPCR	
11379	SMN1-32 m01	0.28375673	0.00468045	SMN del7	30	GM09677	48	qRTPCR	
11380	SMN1-33 m01	0.28415807	0.014291283	SMN del7	30	GM09677	48	qRTPCR	
11381	SMN1-34 m01	0.2523501	0.024700188	SMN del7	30	GM09677	48	qRTPCR	
11382	SMN1-35 m01	0.43627009	0.024860482	SMN del7	30	GM09677	48	qRTPCR	
11383	SMN1-36 m01	0.60271164	0.018112438	SMN del7	30	GM09677	48	qRTPCR	
11384	SMN1-37 m01	0.40219705	0.047669033	SMN del7	30	GM09677	48	qRTPCR	
11385	SMN1-38	0.69242194	0.026898249	SMN	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
11386	SMN1-39 m01	0.81545607	0.009428319	SMN del7	30	GM09677	48	qRTPCR	
11387	SMN1-40 m01	0.95652664	0.036759246	SMN del7	30	GM09677	48	qRTPCR	
11388	SMN1-41 m01	1.092434	0.084831976	SMN del7	30	GM09677	48	qRTPCR	
11389	SMN1-42 m01	0.8202633	0.025709254	SMN del7	30	GM09677	48	qRTPCR	
11390	SMN1-43 m01	0.80734385	0.036848505	SMN del7	30	GM09677	48	qRTPCR	
11391	SMN1-44 m01	1.4106905	0.075965682	SMN del7	30	GM09677	48	qRTPCR	
11392	SMN1-45 m01	1.6206207	0.061607483	SMN del7	30	GM09677	48	qRTPCR	
11393	SMN1-46 m01	1.87305616	0.024525081	SMN del7	30	GM09677	48	qRTPCR	
11394	SMN1-47 m01	2.56683928	0.157809159	SMN del7	30	GM09677	48	qRTPCR	
11395	SMN1-48 m01	0.87628496	0.050643652	SMN del7	30	GM09677	48	qRTPCR	
11396	SMN1-49 m01	1.39489097	0.054331531	SMN del7	30	GM09677	48	qRTPCR	
11397	SMN1-50 m01	0.40660313	0.026604446	SMN del7	30	GM09677	48	qRTPCR	
11398	SMN1-51 m01	0.95306066	0.006201775	SMN del7	30	GM09677	48	qRTPCR	
11399	SMN1-52	0.50250253	0.022757992	SMN	30	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
13088	SMN1-53 m03	0.03672797	0.00648923	SMN del7	30	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.06854374	0.007294301	SMN del7	30	GM09677	48	qRTPCR	
13089	SMN1-55 m01	0.53493451	0.00994796	SMN del7	30	GM09677	48	qRTPCR	
13090	SMN1-56 m01	1.83519037	0.01668585	SMN del7	30	GM09677	48	qRTPCR	
13095	unc-232 m12	1.01967406	0.035007783	SMN del7	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.045704143	SMN del7	30	GM09677	48	qRTPCR	
13091	SMN1-57 m01	1.14138977	0.159603457	SMN Full	30	GM09677	48	qRTPCR	
13092	SMN1-58 m01	2.48463888	0.048867858	SMN Full	30	GM09677	48	qRTPCR	
3650	SMN1-59 m01	0.44643559	0.009187815	SMN Full	30	GM09677	48	qRTPCR	
13093	SMN1-60 m01	1.20287876	0.006618955	SMN Full	30	GM09677	48	qRTPCR	
13094	SMN1-61 m01	1.10479129	0.028468367	SMN Full	30	GM09677	48	qRTPCR	
10065	SMN1-62 m01	0.67155026	0.011630159	SMN Full	30	GM09677	48	qRTPCR	
10066	SMN1-63 m01	0.58859993	0.025523209	SMN Full	30	GM09677	48	qRTPCR	
10067	SMN1-64	0.27895293	0.01381098	SMN Full	30	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
10068	SMN1-65 m01	0.50823198	0.011441603	SMN Full	30	GM09677	48	qRTPCR	
10069	SMN1-66 m01	0.58509547	0.025437693	SMN Full	30	GM09677	48	qRTPCR	
10070	SMN1-67 m01	0.81029559	0.0379367	SMN Full	30	GM09677	48	qRTPCR	
10071	SMN1-68 m01	0.95208277	0.002756169	SMN Full	30	GM09677	48	qRTPCR	
10072	SMN1-69 m01	1.03376563	0.036658892	SMN Full	30	GM09677	48	qRTPCR	
10073	SMN1-70 m01	1.41865584	0.03040738	SMN Full	30	GM09677	48	qRTPCR	
10074	SMN1-71 m01	1.49736784	0.015650036	SMN Full	30	GM09677	48	qRTPCR	
10075	SMN1-72 m01	1.29316759	0.018238078	SMN Full	30	GM09677	48	qRTPCR	
10080	SMN1-73 m01	1.69830022	0.025080692	SMN Full	30	GM09677	48	qRTPCR	
10081	SMN1-74 m01	1.27166879	0.036458249	SMN Full	30	GM09677	48	qRTPCR	
10082	SMN1-75 m01	1.07874575	0.005588796	SMN Full	30	GM09677	48	qRTPCR	
10084	SMN1-76 m01	0.64394163	0.003158312	SMN Full	30	GM09677	48	qRTPCR	
10087	SMN1-77 m01	2.3171246	0.074077448	SMN Full	30	GM09677	48	qRTPCR	
10168	SMN1-78	0.88257691	0.021764784	SMN Full	30	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
10169	SMN1-79 m01	0.55395231	0.011380667	SMN Full	30	GM09677	48	qRTPCR	
10170	SMN1-80 m01	0.59477427	0.011551901	SMN Full	30	GM09677	48	qRTPCR	
10337	SMN1-81 m01	1.0962431	0.047029705	SMN Full	30	GM09677	48	qRTPCR	
10338	SMN1-82 m01	1.2120668	0.038922609	SMN Full	30	GM09677	48	qRTPCR	
10339	SMN1-83 m01	1.84871964	0.056544727	SMN Full	30	GM09677	48	qRTPCR	
10763	SMN1-84 m01	0.57051055	0.011738491	SMN Full	30	GM09677	48	qRTPCR	
10764	SMN1-85 m01	0.97411173	0.027149571	SMN Full	30	GM09677	48	qRTPCR	
10949	SMN1-86 m01	0.95157537	0.052110672	SMN Full	30	GM09677	48	qRTPCR	
13095	unc-232 m01	1.04684129	0.015842026	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.102105642	SMN Full	30	GM09677	48	qRTPCR	
13091	SMN1-57 m01	1.46995785	0.121570225	SMN del7	30	GM09677	48	qRTPCR	
13092	SMN1-58 m01	0.17144533	0.011346456	SMN del7	30	GM09677	48	qRTPCR	
3650	SMN1-59 m01	1.4911289	0.156833175	SMN del7	30	GM09677	48	qRTPCR	
13093	SMN1-60 m01	0.79320075	0.008292103	SMN del7	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13094	SMN1-61 m01	0.76560985	0.009434592	SMN del7	30	GM09677	48	qRT-PCR	
10065	SMN1-62 m01	1.45604965	0.033297825	SMN del7	30	GM09677	48	qRT-PCR	
10066	SMN1-63 m01	1.36653717	0.04810894	SMN del7	30	GM09677	48	qRT-PCR	
10067	SMN1-64 m01	1.56185404	0.045376171	SMN del7	30	GM09677	48	qRT-PCR	
10068	SMN1-65 m01	1.22542524	0.020611549	SMN del7	30	GM09677	48	qRT-PCR	
10069	SMN1-66 m01	1.10639948	0.039747975	SMN del7	30	GM09677	48	qRT-PCR	
10070	SMN1-67 m01	0.37293785	0.007769646	SMN del7	30	GM09677	48	qRT-PCR	
10071	SMN1-68 m01	0.36124962	0.010638504	SMN del7	30	GM09677	48	qRT-PCR	
10072	SMN1-69 m01	0.71777719	0.011406813	SMN del7	30	GM09677	48	qRT-PCR	
10073	SMN1-70 m01	0.34229191	0.00533162	SMN del7	30	GM09677	48	qRT-PCR	
10074	SMN1-71 m01	0.32463806	0.014610829	SMN del7	30	GM09677	48	qRT-PCR	
10075	SMN1-72 m01	0.345364	0.021178201	SMN del7	30	GM09677	48	qRT-PCR	
10080	SMN1-73 m01	0.48527979	0.00905224	SMN del7	30	GM09677	48	qRT-PCR	
10081	SMN1-74 m01	0.95709314	0.043370186	SMN del7	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10082	SMN1-75 m01	1.44141415	0.080700541	SMN del7	30	GM09677	48	qRT-PCR	
10084	SMN1-76 m01	2.40330798	0.133748372	SMN del7	30	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	0.169335969	0.00891376	SMN del7	30	GM09677	48	qRT-PCR	
10168	SMN1-78 m01	1.62625594	0.057476673	SMN del7	30	GM09677	48	qRT-PCR	
10169	SMN1-79 m01	3.18666709	0.200758267	SMN del7	30	GM09677	48	qRT-PCR	
10170	SMN1-80 m01	2.6833258	0.135337138	SMN del7	30	GM09677	48	qRT-PCR	
10337	SMN1-81 m01	1.04005171	0.01751047	SMN del7	30	GM09677	48	qRT-PCR	
10338	SMN1-82 m01	0.7911597	0.025466287	SMN del7	30	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	0.41530008	0.005389479	SMN del7	30	GM09677	48	qRT-PCR	
10763	SMN1-84 m01	4.8789057	0.59855214	SMN del7	30	GM09677	48	qRT-PCR	
10764	SMN1-85 m01	1.71464609	0.122288358	SMN del7	30	GM09677	48	qRT-PCR	
10949	SMN1-86 m01	1.56872146	0.098014697	SMN del7	30	GM09677	48	qRT-PCR	
13095	unc-232 m01	1.00666292	0.026258805	SMN del7	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.035647339	SMN del7	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10951	SMN1-87 m01	1.53792907	0.046675128	SMN Full	30	GM09677	48	qRTPCR	
10952	SMN1-88 m01	2.68082849	0.623002633	SMN Full	30	GM09677	48	qRTPCR	
10953	SMN1-89 m01	2.06469789	0.351056011	SMN Full	30	GM09677	48	qRTPCR	
10954	SMN1-90 m01	2.13496493	0.600794554	SMN Full	30	GM09677	48	qRTPCR	
11415	SMN1-91 m01	1.00314327	0.031848274	SMN Full	30	GM09677	48	qRTPCR	
11418	SMN1-92 m01	1.32697317	0.480668001	SMN Full	30	GM09677	48	qRTPCR	
11419	SMN1-93 m01	3.11243802	0.974268474	SMN Full	30	GM09677	48	qRTPCR	
11421	SMN1-94 m01	0.78203856	0.044990147	SMN Full	30	GM09677	48	qRTPCR	
11422	SMN1-95 m01	0.846134	0.022459877	SMN Full	30	GM09677	48	qRTPCR	
11423	SMN1-96 m01	0.88240702	0.044881054	SMN Full	30	GM09677	48	qRTPCR	
11440	SMN1-97 m01	0.55888585	0.022952787	SMN Full	30	GM09677	48	qRTPCR	
11441	SMN1-98 m01	1.21129902	0.043288004	SMN Full	30	GM09677	48	qRTPCR	
11443	SMN1-100 m01	0.8537071	0.013419288	SMN Full	30	GM09677	48	qRTPCR	
12369	SMN1-101 m01	1.80766717	0.082703582	SMN Full	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	Ctrl Un	0.94556194	0.07252206	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.019152321	SMN Full	30	GM09677	48	qRTPCR	
10951	SMN1-87 m01	1.02068554	0.030546624	SMN del7	30	GM09677	48	qRTPCR	
10952	SMN1-88 m01	0.70783153	0.030336367	SMN del7	30	GM09677	48	qRTPCR	
10953	SMN1-89 m01	0.49917957	0.126021286	SMN del7	30	GM09677	48	qRTPCR	
10954	SMN1-90 m01	0.74886168	0.061283182	SMN del7	30	GM09677	48	qRTPCR	
11415	SMN1-91 m01	0.96454058	0.015366483	SMN del7	30	GM09677	48	qRTPCR	
11418	SMN1-92 m01	0.86223676	0.243165167	SMN del7	30	GM09677	48	qRTPCR	
11419	SMN1-93 m01	0.83138557	0.057384403	SMN del7	30	GM09677	48	qRTPCR	
11421	SMN1-94 m01	0.50676981	0.026656668	SMN del7	30	GM09677	48	qRTPCR	
11422	SMN1-95 m01	0.35860977	0.019283079	SMN del7	30	GM09677	48	qRTPCR	
11423	SMN1-96 m01	0.61626862	0.040669014	SMN del7	30	GM09677	48	qRTPCR	
11440	SMN1-97 m01	1.00600059	0.036492516	SMN del7	30	GM09677	48	qRTPCR	
11441	SMN1-98 m01	0.41602576	0.020732299	SMN del7	30	GM09677	48	qRTPCR	
11443	SMN1-100 m01	1.21074295	0.039316929	SMN del7	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
12369	SMN1-101 m01	0.45525618	0.04137865	SMN del7	30	GM09677	48	qRT-PCR	
	Ctrl Un	1.01658666	0.023400809	SMN del7	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.036478776	SMN del7	30	GM09677	48	qRT-PCR	
11374	SMN1-27 m01	1.3541409	0.336564981	SMN Full	30	GM09677	48	qRT-PCR	
11375	SMN1-28 m01	1.76018399	0.541941124	SMN Full	30	GM09677	48	qRT-PCR	
11376	SMN1-29 m01	1.53443841	0.031544346	SMN Full	30	GM09677	48	qRT-PCR	
11377	SMN1-30 m01	2.56810902	0.092442327	SMN Full	30	GM09677	48	qRT-PCR	
11378	SMN1-31 m01	2.17436738	0.146065161	SMN Full	30	GM09677	48	qRT-PCR	
11379	SMN1-32 m01	1.46988775	0.048852984	SMN Full	30	GM09677	48	qRT-PCR	
11380	SMN1-33 m01	1.83411953	0.045300205	SMN Full	30	GM09677	48	qRT-PCR	
11381	SMN1-34 m01	2.54311279	0.339760802	SMN Full	30	GM09677	48	qRT-PCR	
11382	SMN1-35 m01	2.59816956	0.209116167	SMN Full	30	GM09677	48	qRT-PCR	
11383	SMN1-36 m01	1.38022942	0.111310724	SMN Full	30	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	2.45406372	0.015507429	SMN Full	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11385	SMN1-38 m01	1.77931624	0.118091219	SMN Full	30	GM09677	48	qRT-PCR	
11386	SMN1-39 m01	1.1937419	0.074370499	SMN Full	30	GM09677	48	qRT-PCR	
11387	SMN1-40 m01	0.94056254	0.017259196	SMN Full	30	GM09677	48	qRT-PCR	
11388	SMN1-41 m01	0.84279639	0.013154444	SMN Full	30	GM09677	48	qRT-PCR	
11389	SMN1-42 m01	0.77788341	0.041022987	SMN Full	30	GM09677	48	qRT-PCR	
11390	SMN1-43 m01	1.03977538	0.070641762	SMN Full	30	GM09677	48	qRT-PCR	
11391	SMN1-44 m01	0.41829423	0.038266657	SMN Full	30	GM09677	48	qRT-PCR	
11392	SMN1-45 m01	0.68465577	0.015860412	SMN Full	30	GM09677	48	qRT-PCR	
11393	SMN1-46 m01	0.43086436	0.025366711	SMN Full	30	GM09677	48	qRT-PCR	
11394	SMN1-47 m01	0.46784099	0.055945015	SMN Full	30	GM09677	48	qRT-PCR	
11395	SMN1-48 m01	0.67399077	0.013589914	SMN Full	30	GM09677	48	qRT-PCR	
11396	SMN1-49 m01	0.69921698	0.013710582	SMN Full	30	GM09677	48	qRT-PCR	
11397	SMN1-50 m01	1.04114616	0.04767673	SMN Full	30	GM09677	48	qRT-PCR	
11398	SMN1-51 m01	1.15862447	0.096298617	SMN Full	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11399	SMN1-52 m01	0.91435924	0.022517246	SMN Full	30	GM09677	48	qRTPCR	
13088	SMN1-53 m03	3.64438659	0.09447468	SMN Full	30	GM09677	48	qRTPCR	
13088	SMN1-54 m01	2.86339328	0.073280747	SMN Full	30	GM09677	48	qRTPCR	
13089	SMN1-55 m01	1.62779362	0.076128297	SMN Full	30	GM09677	48	qRTPCR	
13090	SMN1-56 m01	0.85383458	0.095025247	SMN Full	30	GM09677	48	qRTPCR	
13095	unc-232 m12	1.18400226	0.005998675	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.058926752	SMN Full	30	GM09677	48	qRTPCR	
11374	SMN1-27 m01	1.06246447	0.223952294	SMN del7	30	GM09677	48	qRTPCR	
11375	SMN1-28 m01	1.13793724	0.329381423	SMN del7	30	GM09677	48	qRTPCR	
11376	SMN1-29 m01	1.38627671	0.054105876	SMN del7	30	GM09677	48	qRTPCR	
11377	SMN1-30 m01	0.45463444	0.00574446	SMN del7	30	GM09677	48	qRTPCR	
11378	SMN1-31 m01	0.05417561	0.011156684	SMN del7	30	GM09677	48	qRTPCR	
11379	SMN1-32 m01	0.10447682	0.01597211	SMN del7	30	GM09677	48	qRTPCR	
11380	SMN1-33 m01	0.11632773	0.005090993	SMN del7	30	GM09677	48	qRTPCR	
11381	SMN1-34	0.10081398	0.01173462	SMN	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
11382	SMN1-35 m01	0.17989956	0.018762122	SMN del7	30	GM09677	48	qRTPCR	
11383	SMN1-36 m01	0.32143959	0.038741078	SMN del7	30	GM09677	48	qRTPCR	
11384	SMN1-37 m01	0.29400836	0.068892266	SMN del7	30	GM09677	48	qRTPCR	
11385	SMN1-38 m01	0.55623416	0.047032915	SMN del7	30	GM09677	48	qRTPCR	
11386	SMN1-39 m01	0.59692967	0.055257927	SMN del7	30	GM09677	48	qRTPCR	
11387	SMN1-40 m01	0.78804831	0.011549402	SMN del7	30	GM09677	48	qRTPCR	
11388	SMN1-41 m01	1.33070295	0.073357153	SMN del7	30	GM09677	48	qRTPCR	
11389	SMN1-42 m01	0.71606374	0.041995033	SMN del7	30	GM09677	48	qRTPCR	
11390	SMN1-43 m01	0.88318944	0.068749382	SMN del7	30	GM09677	48	qRTPCR	
11391	SMN1-44 m01	1.386778	0.009682706	SMN del7	30	GM09677	48	qRTPCR	
11392	SMN1-45 m01	2.2815111	0.118189321	SMN del7	30	GM09677	48	qRTPCR	
11393	SMN1-46 m01	2.92927451	0.320642884	SMN del7	30	GM09677	48	qRTPCR	
11394	SMN1-47 m01	4.31592172	0.438583953	SMN del7	30	GM09677	48	qRTPCR	
11395	SMN1-48	1.90809642	0.071990221	SMN	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
11396	SMN1-49 m01	2.67994463	0.213801859	SMN del7	30	GM09677	48	qRTPCR	
11397	SMN1-50 m01	0.17168733	0.012786922	SMN del7	30	GM09677	48	qRTPCR	
11398	SMN1-51 m01	0.86411152	0.053621954	SMN del7	30	GM09677	48	qRTPCR	
11399	SMN1-52 m01	0.34122243	0.009027101	SMN del7	30	GM09677	48	qRTPCR	
13088	SMN1-53 m03	0.00821149	0.001059997	SMN del7	30	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.01377529	0.001357488	SMN del7	30	GM09677	48	qRTPCR	
13089	SMN1-55 m01	0.11531182	0.026449272	SMN del7	30	GM09677	48	qRTPCR	
13090	SMN1-56 m01	1.52221975	0.056733627	SMN del7	30	GM09677	48	qRTPCR	
13095	unc-232 m12	0.89750338	0.04359737	SMN del7	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.09814195	SMN del7	30	GM09677	48	qRTPCR	
13091	SMN1-57 m01	2.22550744	0.375280964	SMN Full	30	GM09677	48	qRTPCR	
13092	SMN1-58 m01	3.59004825	0.153294475	SMN Full	30	GM09677	48	qRTPCR	
3650	SMN1-59 m01	0.41631272	0.020898005	SMN Full	30	GM09677	48	qRTPCR	
13093	SMN1-60	1.87049913	0.049728009	SMN Full	30	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
13094	SMN1-61 m01	1.43951078	0.047768184	SMN Full	30	GM09677	48	qRTPCR	
10065	SMN1-62 m01	0.46776095	0.015139001	SMN Full	30	GM09677	48	qRTPCR	
10066	SMN1-63 m01	0.40403329	0.040650992	SMN Full	30	GM09677	48	qRTPCR	
10067	SMN1-64 m01	0.12463428	0.005379232	SMN Full	30	GM09677	48	qRTPCR	
10068	SMN1-65 m01	0.20342464	0.005559849	SMN Full	30	GM09677	48	qRTPCR	
10069	SMN1-66 m01	0.49714713	0.009471566	SMN Full	30	GM09677	48	qRTPCR	
10070	SMN1-67 m01	0.6947232	0.056749124	SMN Full	30	GM09677	48	qRTPCR	
10071	SMN1-68 m01	1.0257281	0.005700927	SMN Full	30	GM09677	48	qRTPCR	
10072	SMN1-69 m01	1.24261032	0.052198053	SMN Full	30	GM09677	48	qRTPCR	
10073	SMN1-70 m01	1.53006242	0.034576278	SMN Full	30	GM09677	48	qRTPCR	
10074	SMN1-71 m01	1.52477506	0.065247822	SMN Full	30	GM09677	48	qRTPCR	
10075	SMN1-72 m01	1.16556816	0.041237174	SMN Full	30	GM09677	48	qRTPCR	
10080	SMN1-73 m01	1.92866205	0.157278439	SMN Full	30	GM09677	48	qRTPCR	
10081	SMN1-74	1.05657397	0.123335551	SMN Full	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
10082	SMN1-75 m01	0.67896527	0.124110772	SMN Full	30	GM09677	48	qRTPCR	
10084	SMN1-76 m01	0.34948304	0.034704925	SMN Full	30	GM09677	48	qRTPCR	
10087	SMN1-77 m01	2.95799058	0.088276742	SMN Full	30	GM09677	48	qRTPCR	
10168	SMN1-78 m01	0.57988897	0.077568451	SMN Full	30	GM09677	48	qRTPCR	
10169	SMN1-79 m01	0.28124385	0.034620532	SMN Full	30	GM09677	48	qRTPCR	
10170	SMN1-80 m01	0.29122116	0.022647587	SMN Full	30	GM09677	48	qRTPCR	
10337	SMN1-81 m01	1.01380047	0.104112668	SMN Full	30	GM09677	48	qRTPCR	
10338	SMN1-82 m01	1.44452952	0.079106458	SMN Full	30	GM09677	48	qRTPCR	
10339	SMN1-83 m01	2.61693662	0.206978584	SMN Full	30	GM09677	48	qRTPCR	
10763	SMN1-84 m01	0.41707326	0.043311521	SMN Full	30	GM09677	48	qRTPCR	
10764	SMN1-85 m01	1.05592595	0.161385137	SMN Full	30	GM09677	48	qRTPCR	
10949	SMN1-86 m01	1.54974296	0.187600525	SMN Full	30	GM09677	48	qRTPCR	
13095	unc-232 m01	1.02748112	0.115400485	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.179033031	SMN Full	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13091	SMN1-57 m01	0.99508297	0.195997173	SMN del7	30	GM09677	48	qRT-PCR	
13092	SMN1-58 m01	0.10464879	0.015608578	SMN del7	30	GM09677	48	qRT-PCR	
3650	SMN1-59 m01	1.56046891	0.090553149	SMN del7	30	GM09677	48	qRT-PCR	
13093	SMN1-60 m01	1.26287996	0.036077622	SMN del7	30	GM09677	48	qRT-PCR	
13094	SMN1-61 m01	0.98629242	0.043169762	SMN del7	30	GM09677	48	qRT-PCR	
10065	SMN1-62 m01	2.8772299	0.155419129	SMN del7	30	GM09677	48	qRT-PCR	
10066	SMN1-63 m01	2.86355694	0.091393445	SMN del7	30	GM09677	48	qRT-PCR	
10067	SMN1-64 m01	2.50838681	0.094678994	SMN del7	30	GM09677	48	qRT-PCR	
10068	SMN1-65 m01	1.50008698	0.045820284	SMN del7	30	GM09677	48	qRT-PCR	
10069	SMN1-66 m01	2.05257576	0.122750019	SMN del7	30	GM09677	48	qRT-PCR	
10070	SMN1-67 m01	0.40737808	0.006912639	SMN del7	30	GM09677	48	qRT-PCR	
10071	SMN1-68 m01	0.42629131	0.010900547	SMN del7	30	GM09677	48	qRT-PCR	
10072	SMN1-69 m01	0.82293186	0.035520988	SMN del7	30	GM09677	48	qRT-PCR	
10073	SMN1-70 m01	0.27166019	0.030004581	SMN del7	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10074	SMN1-71 m01	0.1993653	0.025617056	SMN del7	30	GM09677	48	qRT-PCR	
10075	SMN1-72 m01	0.18238307	0.003270929	SMN del7	30	GM09677	48	qRT-PCR	
10080	SMN1-73 m01	0.24061599	0.048236443	SMN del7	30	GM09677	48	qRT-PCR	
10081	SMN1-74 m01	1.34676286	0.096395284	SMN del7	30	GM09677	48	qRT-PCR	
10082	SMN1-75 m01	2.51595216	0.25700087	SMN del7	30	GM09677	48	qRT-PCR	
10084	SMN1-76 m01	2.78581042	0.057810176	SMN del7	30	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	0.09253052	0.014373747	SMN del7	30	GM09677	48	qRT-PCR	
10168	SMN1-78 m01	2.29496244	0.08095944	SMN del7	30	GM09677	48	qRT-PCR	
10169	SMN1-79 m01	4.41231707	0.436710044	SMN del7	30	GM09677	48	qRT-PCR	
10170	SMN1-80 m01	2.94329602	0.109976911	SMN del7	30	GM09677	48	qRT-PCR	
10337	SMN1-81 m01	0.90192088	0.210033748	SMN del7	30	GM09677	48	qRT-PCR	
10338	SMN1-82 m01	0.76834669	0.036643623	SMN del7	30	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	0.56329881	0.079029142	SMN del7	30	GM09677	48	qRT-PCR	
10763	SMN1-84 m01	7.8896864	0.887796325	SMN del7	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10764	SMN1-85 m01	2.68703852	0.445181967	SMN del7	30	GM09677	48	qRT-PCR	
10949	SMN1-86 m01	2.15953715	0.290584928	SMN del7	30	GM09677	48	qRT-PCR	
13095	unc-232 m01	1.04551269	0.119898717	SMN del7	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.228647863	SMN del7	30	GM09677	48	qRT-PCR	
10951	SMN1-87 m01	1.58778189	0.268262154	SMN Full	30	GM09677	48	qRT-PCR	
10952	SMN1-88 m01	1.52457897	0.145429236	SMN Full	30	GM09677	48	qRT-PCR	
10953	SMN1-89 m01	1.34730111	0.012667258	SMN Full	30	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	1.50181569	0.016538634	SMN Full	30	GM09677	48	qRT-PCR	
11415	SMN1-91 m01	0.59168909	0.087977045	SMN Full	30	GM09677	48	qRT-PCR	
11418	SMN1-92 m01	0.78134405	0.03932074	SMN Full	30	GM09677	48	qRT-PCR	
11419	SMN1-93 m01	2.33338786	1.508168012	SMN Full	30	GM09677	48	qRT-PCR	
11421	SMN1-94 m01	0.68097078	0.098617361	SMN Full	30	GM09677	48	qRT-PCR	
11422	SMN1-95 m01	0.81225831	0.052695224	SMN Full	30	GM09677	48	qRT-PCR	
11423	SMN1-96 m01	0.65843003	0.045009739	SMN Full	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11440	SMN1-97 m01	0.58128289	0.016284361	SMN Full	30	GM09677	48	qRT-PCR	
11441	SMN1-98 m01	1.20124798	0.016416488	SMN Full	30	GM09677	48	qRT-PCR	
11443	SMN1-100 m01	0.70188432	0.045331214	SMN Full	30	GM09677	48	qRT-PCR	
12369	SMN1-101 m01	1.6889086	0.145081243	SMN Full	30	GM09677	48	qRT-PCR	
13088	SMN1-53 m03	3.66573281	0.613236057	SMN Full	60	GM09677	48	qRT-PCR	
13088	SMN1-53 m03	3.67763326	0.466249427	SMN Full	90	GM09677	48	qRT-PCR	
13095	unc-232 m01	0.873335071	0.186887354	SMN Full	60	GM09677	48	qRT-PCR	
	Ctrl Un	0.9221074	0.02762864	SMN Full	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.096157922	SMN Full	30	GM09677	48	qRT-PCR	
10951	SMN1-87 m01	0.44476338	0.01998328	SMN del7	30	GM09677	48	qRT-PCR	
10952	SMN1-88 m01	0.48556515	0.114903532	SMN del7	30	GM09677	48	qRT-PCR	
10953	SMN1-89 m01	0.3188747	0.030198185	SMN del7	30	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	0.47376804	0.026686436	SMN del7	30	GM09677	48	qRT-PCR	
11415	SMN1-91 m01	0.4986585	0.097505413	SMN del7	30	GM09677	48	qRT-PCR	
11418	SMN1-92 m01	0.37920185	0.039509123	SMN del7	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11419	SMN1-93 m01	0.26285281	0.021205657	SMN del7	30	GM09677	48	qRTPCR	
11421	SMN1-94 m01	0.2079815	0.034629596	SMN del7	30	GM09677	48	qRTPCR	
11422	SMN1-95 m01	0.1781843	0.017475914	SMN del7	30	GM09677	48	qRTPCR	
11423	SMN1-96 m01	0.43584447	0.052049932	SMN del7	30	GM09677	48	qRTPCR	
11440	SMN1-97 m01	1.03284592	0.223289759	SMN del7	30	GM09677	48	qRTPCR	
11441	SMN1-98 m01	0.48612922	0.022087554	SMN del7	30	GM09677	48	qRTPCR	
11443	SMN1-100 m01	1.064444652	0.106529691	SMN del7	30	GM09677	48	qRTPCR	
12369	SMN1-101 m01	0.33710959	0.075041526	SMN del7	30	GM09677	48	qRTPCR	
13088	SMN1-53 m03	0.01253704	0.00583045	SMN del7	60	GM09677	48	qRTPCR	
13088	SMN1-53 m03	0.01632148	0.006108032	SMN del7	90	GM09677	48	qRTPCR	
13095	unc-232 m01	0.96267051	0.223638909	SMN del7	60	GM09677	48	qRTPCR	
	Ctrl Un	0.80815056	0.055070518	SMN del7	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.10346032	SMN del7	30	GM09677	48	qRTPCR	
11384	SMN1-37 m01	2.69941658	0.021378588	SMN Full	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11394	SMN1-47 m01	0.43332587	0.020071176	SMN Full	30	GM09677	48	qRT-PCR	
11395	SMN1-48 m01	0.583706	0.012865645	SMN Full	30	GM09677	48	qRT-PCR	
13088	SMN1-53 m03	4.08908557	0.115183583	SMN Full	30	GM09677	48	qRT-PCR	
13088	SMN1-54 m01	3.57281719	0.080920457	SMN Full	30	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	2.52856173	0.052569466	SMN Full	30	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	2.4253965	0.089651621	SMN Full	30	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	2.05565393	0.04468188	SMN Full	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.02346075	SMN Full	30	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.12518763	0.00463936	SMN del7	30	GM09677	48	qRT-PCR	
11394	SMN1-47 m01	5.85069432	0.414694379	SMN del7	30	GM09677	48	qRT-PCR	
11395	SMN1-48 m01	2.43094522	0.065227949	SMN del7	30	GM09677	48	qRT-PCR	
13088	SMN1-53 m03	0.02034375	0.001372174	SMN del7	30	GM09677	48	qRT-PCR	
13088	SMN1-54 m01	0.0166988	0.001381155	SMN del7	30	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	0.10269955	0.003933416	SMN del7	30	GM09677	48	qRT-PCR	
10339	SMN1-83	0.3270772	0.027916668	SMN	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
10954	SMN1-90 m01	0.78359258	0.061884984	SMN del7	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.069132013	SMN del7	30	GM09677	48	qRTPCR	
13088	SMN1-53 m03	5.05440836	0.177768798	SMN Full	60	GM09677	48	qRTPCR	
13088	SMN1-54 m01	4.49435421	0.392967488	SMN Full	90	GM09677	48	qRTPCR	
13096	unc-293 m01	1.08026356	0.024066164	SMN Full	30	GM09677	48	qRTPCR	
13096	unc-293 m01	1.59238916	0.09230384	SMN Full	60	GM09677	48	qRTPCR	
13096	unc-293 m01	1.49512435	0.174423133	SMN Full	90	GM09677	48	qRTPCR	
	Ctrl Un	0.96649852	0.037476741	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.013089909	SMN Full	30	GM09677	48	qRTPCR	
13088	SMN1-53 m03	0.01680678	0.003625429	SMN del7	60	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.04748237	0.00690858	SMN del7	90	GM09677	48	qRTPCR	
13096	unc-293 m01	1.21701113	0.058986029	SMN del7	30	GM09677	48	qRTPCR	
13096	unc-293 m01	1.45850483	0.074356672	SMN del7	60	GM09677	48	qRTPCR	
13096	unc-293 m01	1.35090755	0.118662442	SMN del7	90	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	Ctrl Un	0.86609519	0.067840092	SMN del7	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.086600155	SMN del7	30	GM09677	48	qRT-PCR	
11394	SMN1-47 m01	1.8602773	0.074379421	SMN	10000	GM10684	48	qRT-PCR	9 day gymnotic delivery
13091	SMN1-57 m01	3.53905896	0.192154141	SMN	10000	GM10684	48	qRT-PCR	9 day gymnotic delivery
10169	SMN1-79 m01	2.41039305	0.257777098	SMN	10000	GM10684	48	qRT-PCR	9 day gymnotic delivery
10170	SMN1-80 m01	1.44773534	0.074504262	SMN	10000	GM10684	48	qRT-PCR	9 day gymnotic delivery
10763	SMN1-84 m01	1.7549978	0.088030953	SMN	10000	GM10684	48	qRT-PCR	9 day gymnotic delivery
11419	SMN1-93 m01	1.92672729	0.05786806	SMN	10000	GM10684	48	qRT-PCR	9 day gymnotic delivery
11423	SMN1-96 m01	1.59552582	0.071828709	SMN	10000	GM10684	48	qRT-PCR	9 day gymnotic delivery
	Ctrl Un	1	0.04269276	SMN	10000	GM10684	48	qRT-PCR	9 day gymnotic delivery
11394	SMN1-47 m02	0.63763464	0.092758711	SMN Full	30	GM03813	48	qRT-PCR	
11395	SMN1-48 m05	0.7379512	0.015664756	SMN Full	30	GM03813	48	qRT-PCR	
13088	SMN1-53 m02	2.33849306	0.080553203	SMN Full	30	GM03813	48	qRT-PCR	
13091	SMN1-57 m02	1.1397891	0.122341913	SMN Full	30	GM03813	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10169	SMN1-79 m02	0.35478075	0.063535552	SMN Full	30	GM03813	48	qRT-PCR	
10170	SMN1-80 m02	0.72556334	0.109643776	SMN Full	30	GM03813	48	qRT-PCR	
10763	SMN1-84 m02	0.947445	0.023066616	SMN Full	30	GM03813	48	qRT-PCR	
11419	SMN1-93 m02	1.60291133	0.139489277	SMN Full	30	GM03813	48	qRT-PCR	
11394	SMN1-47 m01	0.38619183	0.010242864	SMN Full	30	GM03813	48	qRT-PCR	
11395	SMN1-48 m01	0.34233866	0.00771587	SMN Full	30	GM03813	48	qRT-PCR	
13088	SMN1-53 m03	2.78212115	0.788598224	SMN Full	30	GM03813	48	qRT-PCR	
13088	SMN1-54 m01	3.01561695	0.565657757	SMN Full	30	GM03813	48	qRT-PCR	
13091	SMN1-57 m01	2.79789932	0.295447038	SMN Full	30	GM03813	48	qRT-PCR	
10070	SMN1-67 m01	0.28859806	0.014227066	SMN Full	30	GM03813	48	qRT-PCR	
10169	SMN1-79 m01	0.12101525	0.032671291	SMN Full	30	GM03813	48	qRT-PCR	
10170	SMN1-80 m01	0.10276296	0.023566294	SMN Full	30	GM03813	48	qRT-PCR	
10763	SMN1-84 m01	0.20247712	0.013695236	SMN Full	30	GM03813	48	qRT-PCR	
11419	SMN1-93 m01	3.45933108	0.088382999	SMN Full	30	GM03813	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	Ctrl Lipo	1	0.101934256	SMN Full	30	GM03813	48	qRTPCR	
	Ctrl Un	1.21316541	0.10178734	SMN Full	30	GM03813	48	qRTPCR	
11394	SMN1-47 m02	2.55512668	0.064453975	SMN del7	30	GM03813	48	qRTPCR	
11395	SMN1-48 m05	1.83887429	0.20343974	SMN del7	30	GM03813	48	qRTPCR	
13091	SMN1-57 m02	0.95662251	0.055360018	SMN del7	30	GM03813	48	qRTPCR	
10169	SMN1-79 m02	1.37083218	0.03826599	SMN del7	30	GM03813	48	qRTPCR	
10170	SMN1-80 m02	1.06155286	0.082878431	SMN del7	30	GM03813	48	qRTPCR	
10763	SMN1-84 m02	0.73567698	0.084383036	SMN del7	30	GM03813	48	qRTPCR	
11419	SMN1-93 m02	0.51407096	0.065730017	SMN del7	30	GM03813	48	qRTPCR	
11394	SMN1-47 m01	3.12747904	0.409407651	SMN del7	30	GM03813	48	qRTPCR	
11395	SMN1-48 m01	1.08703734	0.202772467	SMN del7	30	GM03813	48	qRTPCR	
13088	SMN1-53 m03	0.01644255	0.000933095	SMN del7	30	GM03813	48	qRTPCR	
13091	SMN1-57 m01	0.51194491	0.072247475	SMN del7	30	GM03813	48	qRTPCR	
10070	SMN1-67 m01	0.09463857	0.006650665	SMN del7	30	GM03813	48	qRTPCR	
10169	SMN1-79 m01	2.32868099	0.394609397	SMN del7	30	GM03813	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10170	SMN1-80 m01	1.71709489	0.093689936	SMN del7	30	GM03813	48	qRTPCR	
10763	SMN1-84 m01	6.86610934	0.928560533	SMN del7	30	GM03813	48	qRTPCR	
	Ctrl Lipo	1	0.142799332	SMN del7	30	GM03813	48	qRTPCR	
	Ctrl Un	1.0913464	0.210259626	SMN del7	30	GM03813	48	qRTPCR	
10087	SMN1-77 m01	1.08670105	0.099685074	SMN Full	40000	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	0.88362515	0.075445022	SMN Full	20000	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	0.77733549	0.058941919	SMN Full	10000	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	0.75253616	0.044026278	SMN Full	5000	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	0.83764301	0.044530691	SMN Full	2500	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	0.78034452	0.047346309	SMN Full	12500	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	0.83138933	0.003093161	SMN Full	600	GM09677	240	qRTPCR	10 day gymnotic delivery
	Ctrl Un	1	0.015284336	SMN Full	0	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	0.92898495	0.086371586	SMN del7	40000	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	0.82167026	0.036470522	SMN del7	20000	GM09677	240	qRTPCR	10 day gymnotic delivery

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10087	SMN1-77 m01	0.99241991	0.067531863	SMN del7	10000	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	1.01262418	0.083197044	SMN del7	5000	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	1.05122381	0.101129872	SMN del7	2500	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	1.01254247	0.020880653	SMN del7	12500	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	0.99066554	0.024027142	SMN del7	600	GM09677	240	qRTPCR	10 day gymnotic delivery
	Ctrl Un	1	0.029239164	SMN del7	0	GM09677	240	qRTPCR	10 day gymnotic delivery
10087	SMN1-77 m01	1.20631602	0.027413191	SMN Full	40000	GM09677	168	qRTPCR	7 day gymnotic delivery
10087	SMN1-77 m01	0.96458295	0.038457568	SMN Full	20000	GM09677	168	qRTPCR	7 day gymnotic delivery
10087	SMN1-77 m01	0.84271394	0.005246139	SMN Full	10000	GM09677	168	qRTPCR	7 day gymnotic delivery
10087	SMN1-77 m01	0.8176466	0.035378749	SMN Full	5000	GM09677	168	qRTPCR	7 day gymnotic delivery
10087	SMN1-77 m01	0.78500175	0.031788265	SMN Full	2500	GM09677	168	qRTPCR	7 day gymnotic delivery
10087	SMN1-77 m01	0.78323982	0.006328598	SMN Full	12500	GM09677	168	qRTPCR	7 day gymnotic delivery
10087	SMN1-77 m01	0.78002335	0.032137979	SMN Full	600	GM09677	168	qRTPCR	7 day gymnotic delivery
	Ctrl Un	1	0.025485524	SMN Full	0	GM09677	168	qRTPCR	7 day gymnotic delivery

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10087	SMN1-77 m01	1.34860952	0.104724899	SMN del7	40000	GM09677	168	qRTPCR	7 day gymnotic delivery
10087	SMN1-77 m01	1.23598306	0.068269411	SMN del7	20000	GM09677	168	qRTPCR	7 day gymnotic delivery
10087	SMN1-77 m01	1.17443077	0.039271851	SMN del7	10000	GM09677	168	qRTPCR	7 day gymnotic delivery
10087	SMN1-77 m01	1.04884533	0.050459532	SMN del7	5000	GM09677	168	qRTPCR	7 day gymnotic delivery
10087	SMN1-77 m01	1.0434675	0.029757786	SMN del7	2500	GM09677	168	qRTPCR	7 day gymnotic delivery
10087	SMN1-77 m01	0.9867599	0.010892606	SMN del7	12500	GM09677	168	qRTPCR	7 day gymnotic delivery
10087	SMN1-77 m01	0.99081562	0.029913134	SMN del7	600	GM09677	168	qRTPCR	7 day gymnotic delivery
	Ctrl Un	1	0.026118212	SMN del7	0	GM09677	168	qRTPCR	7 day gymnotic delivery
11384	SMN1-37 m01	5.06630591	0.212173561	SMN Full	100	GM09677	48	qRTPCR	
11384	SMN1-37 m01	3.39684081	0.316841014	SMN Full	50	GM09677	48	qRTPCR	
11384	SMN1-37 m01	1.9166506	0.126196584	SMN Full	25	GM09677	48	qRTPCR	
11384	SMN1-37 m01	1.33667116	0.076706919	SMN Full	12.5	GM09677	48	qRTPCR	
11384	SMN1-37 m01	1.21562188	0.036464144	SMN Full	6.25	GM09677	48	qRTPCR	
11384	SMN1-37 m01	0.99886255	0.116133577	SMN Full	3.135	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11384	SMN1-37 m01	0.88784903	0.104802096	SMN Full	1.25	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	2.68417642	0.084733141	SMN Full	100	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	3.08450396	0.103620966	SMN Full	50	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	1.58471356	0.069605432	SMN Full	25	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	1.26790869	0.044123931	SMN Full	12.5	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	1.31815948	0.058123172	SMN Full	6.25	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	1.07434336	0.090485243	SMN Full	3.135	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	1.14968454	0.028054774	SMN Full	1.25	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	3.88826283	0.203213699	SMN Full	100	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	4.09013766	0.128899519	SMN Full	50	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	1.8002018	0.0640479	SMN Full	25	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	1.26642231	0.063510122	SMN Full	12.5	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	1.51010511	0.481840418	SMN Full	6.25	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	1.52904616	0.045053833	SMN Full	3.135	GM09677	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10339	SMN1-83 m01	0.9537528	0.049176736	SMN Full	1.25	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	3.28588271	0.24657862	SMN Full	100	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	4.01771228	1.028363019	SMN Full	50	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	1.6481985	0.018726729	SMN Full	25	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	1.10889336	0.050955425	SMN Full	12.5	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	1.0271761	0.031190904	SMN Full	6.25	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	1.02100614	0.030559858	SMN Full	3.135	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	0.97434965	0.040137302	SMN Full	1.25	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.033244759	SMN Full	0	GM09677	48	qRT-PCR	
	Ctrl Un	1.13475618	0.003433601	SMN Full	0	GM09677	48	qRT-PCR	
13095	unc-232 m12	1.26142479	0.03369159	SMN Full	30	GM09677	48	qRT-PCR	
13088	SMN1-54 m01	3.85720287	0.298540194	SMN Full	100	GM09677	48	qRT-PCR	
13088	SMN1-54 m01	4.88916274	0.071201464	SMN Full	50	GM09677	48	qRT-PCR	
13088	SMN1-54 m01	3.55219896	0.081353035	SMN Full	25	GM09677	48	qRT-PCR	
13088	SMN1-54 m01	1.82264748	0.063735864	SMN Full	12.5	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13088	SMN1-54 m01	1.63235578	0.0498193	SMN Full	6.25	GM09677	48	qRT-PCR	
13088	SMN1-54 m01	1.60762417	0.071908227	SMN Full	3.135	GM09677	48	qRT-PCR	
13088	SMN1-54 m01	1.51859224	0.035345986	SMN Full	1.25	GM09677	48	qRT-PCR	
10955	SMN1-141 m01	7.39327443	0.220910503	SMN Full	100	GM09677	48	qRT-PCR	
10955	SMN1-141 m01	8.26519244	0.285763237	SMN Full	50	GM09677	48	qRT-PCR	
10955	SMN1-141 m01	3.63898963	0.073795795	SMN Full	25	GM09677	48	qRT-PCR	
10955	SMN1-141 m01	1.77026921	0.124045627	SMN Full	12.5	GM09677	48	qRT-PCR	
10955	SMN1-141 m01	1.43897801	0.08074245	SMN Full	6.25	GM09677	48	qRT-PCR	
10955	SMN1-141 m01	1.21684596	0.03536615	SMN Full	3.135	GM09677	48	qRT-PCR	
10955	SMN1-141 m01	1.07072346	0.075808329	SMN Full	1.25	GM09677	48	qRT-PCR	
10956	SMN1-142 m01	15.0696515	0.214681957	SMN Full	100	GM09677	48	qRT-PCR	
10956	SMN1-142 m01	15.1681253	0.879497559	SMN Full	50	GM09677	48	qRT-PCR	
10956	SMN1-142 m01	4.51582704	0.167977771	SMN Full	25	GM09677	48	qRT-PCR	
10956	SMN1-142 m01	1.86346641	0.086373094	SMN Full	12.5	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10956	SMN1-142 m01	1.24740086	0.041613839	SMN Full	6.25	GM09677	48	qRT-PCR	
10956	SMN1-142 m01	1.09549482	0.037085098	SMN Full	3.135	GM09677	48	qRT-PCR	
10956	SMN1-142 m01	0.96405604	0.116444587	SMN Full	1.25	GM09677	48	qRT-PCR	
10957	SMN1-143 m01	6.77278426	0.138469559	SMN Full	100	GM09677	48	qRT-PCR	
10957	SMN1-143 m01	6.70673348	0.100888465	SMN Full	50	GM09677	48	qRT-PCR	
10957	SMN1-143 m01	3.03926416	0.026132347	SMN Full	25	GM09677	48	qRT-PCR	
10957	SMN1-143 m01	1.78374644	0.072042469	SMN Full	12.5	GM09677	48	qRT-PCR	
10957	SMN1-143 m01	1.40075553	0.065761642	SMN Full	6.25	GM09677	48	qRT-PCR	
10957	SMN1-143 m01	1.21308984	0.032987163	SMN Full	3.135	GM09677	48	qRT-PCR	
10957	SMN1-143 m01	1.08985212	0.041819898	SMN Full	1.25	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.034703614	SMN Full	0	GM09677	48	qRT-PCR	
	Ctrl Un	1.09110564	0.012034255	SMN Full	0	GM09677	48	qRT-PCR	
13096	unc-293 m01	1.01788869	0.071762463	SMN Full	30	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	10.0476839	0.239306498	SMN Full	100	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	8.71991054	1.375554999	SMN Full	50	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10958	SMN1-144 m01	4.42650473	0.367815831	SMN Full	25	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	2.71018316	0.510810571	SMN Full	12.5	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	1.34908228	0.089496743	SMN Full	6.25	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	1.08434669	0.103599202	SMN Full	3.135	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	1.00207588	0.060623934	SMN Full	1.25	GM09677	48	qRT-PCR	
10959	SMN1-145 m01	1.88121761	0.086651648	SMN Full	100	GM09677	48	qRT-PCR	
10959	SMN1-145 m01	2.41461668	0.314181609	SMN Full	50	GM09677	48	qRT-PCR	
10959	SMN1-145 m01	2.63467341	0.128294152	SMN Full	25	GM09677	48	qRT-PCR	
10959	SMN1-145 m01	1.77168158	0.102127662	SMN Full	12.5	GM09677	48	qRT-PCR	
10959	SMN1-145 m01	1.13369445	0.107837348	SMN Full	6.25	GM09677	48	qRT-PCR	
10959	SMN1-145 m01	1.01514303	0.00573695	SMN Full	3.135	GM09677	48	qRT-PCR	
10959	SMN1-145 m01	0.9813138	0.105371655	SMN Full	1.25	GM09677	48	qRT-PCR	
10961	SMN1-147 m01	2.90967751	0.397830656	SMN Full	100	GM09677	48	qRT-PCR	
10961	SMN1-147 m01	3.2637193	0.425238714	SMN Full	50	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10961	SMN1-147 m01	3.98771642	0.344171071	SMN Full	25	GM09677	48	qRT-PCR	
10961	SMN1-147 m01	2.75802526	0.093333035	SMN Full	12.5	GM09677	48	qRT-PCR	
10961	SMN1-147 m01	1.81299826	0.073659115	SMN Full	6.25	GM09677	48	qRT-PCR	
10961	SMN1-147 m01	1.60247732	0.616101473	SMN Full	3.135	GM09677	48	qRT-PCR	
10961	SMN1-147 m01	0.82841379	0.065552933	SMN Full	1.25	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	3.30604881	0.207407868	SMN Full	100	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	4.15249698	0.196497393	SMN Full	50	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	2.57840381	0.385903248	SMN Full	25	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	1.38893594	0.05576097	SMN Full	12.5	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	1.09846703	0.104813783	SMN Full	6.25	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	1.0285495	0.088571854	SMN Full	3.135	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	0.97605326	0.081241818	SMN Full	1.25	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.045134637	SMN Full	0	GM09677	48	qRT-PCR	
	Ctrl Un	1.0705007	0.063921387	SMN Full	0	GM09677	48	qRT-PCR	
11381	SMN1-34 m01	0.04084074	0.001945025	SMN Full	100	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11381	SMN1-34 m01	0.04896962	0.006588723	SMN Full	50	GM09677	48	qRTPCR	
11381	SMN1-34 m01	0.14874395	0.068415212	SMN Full	25	GM09677	48	qRTPCR	
11381	SMN1-34 m01	0.20490029	0.047408959	SMN Full	12.5	GM09677	48	qRTPCR	
11381	SMN1-34 m01	0.38578855	0.050195494	SMN Full	6.25	GM09677	48	qRTPCR	
11381	SMN1-34 m01	0.59288671	0.116561841	SMN Full	3.135	GM09677	48	qRTPCR	
11381	SMN1-34 m01	0.50517699	0.19042928	SMN Full	1.25	GM09677	48	qRTPCR	
13107	SMN1-109 m01	0.93186123	0.018083326	SMN Full	100	GM09677	48	qRTPCR	
13107	SMN1-109 m01	0.50290135	0.07790185	SMN Full	50	GM09677	48	qRTPCR	
13107	SMN1-109 m01	0.58015291	0.080609864	SMN Full	25	GM09677	48	qRTPCR	
13107	SMN1-109 m01	0.88550514	0.125587632	SMN Full	12.5	GM09677	48	qRTPCR	
13107	SMN1-109 m01	0.63257469	0.085578056	SMN Full	6.25	GM09677	48	qRTPCR	
13107	SMN1-109 m01	0.41688402	0.06039843	SMN Full	3.135	GM09677	48	qRTPCR	
13107	SMN1-109 m01	0.70231954	0.04102474	SMN Full	1.25	GM09677	48	qRTPCR	
10340	SMN1-125 m01	0.42931464	0.008222078	SMN Full	100	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10340	SMN1-125 m01	0.27403634	0.095812398	SMN Full	50	GM09677	48	qRTPCR	
10340	SMN1-125 m01	0.19351494	0.011451309	SMN Full	25	GM09677	48	qRTPCR	
10340	SMN1-125 m01	0.23777484	0.018000375	SMN Full	12.5	GM09677	48	qRTPCR	
10340	SMN1-125 m01	0.52584637	0.161120008	SMN Full	6.25	GM09677	48	qRTPCR	
10340	SMN1-125 m01	0.66479748	0.043912426	SMN Full	3.135	GM09677	48	qRTPCR	
10340	SMN1-125 m01	0.42520961	0.132627407	SMN Full	1.25	GM09677	48	qRTPCR	
10342	SMN1-127 m01	0.14203141	0.021506288	SMN Full	100	GM09677	48	qRTPCR	
10342	SMN1-127 m01	0.08615325	0.039180767	SMN Full	50	GM09677	48	qRTPCR	
10342	SMN1-127 m01	0.30905423	0.18331188	SMN Full	25	GM09677	48	qRTPCR	
10342	SMN1-127 m01	0.21879444	0.021268842	SMN Full	12.5	GM09677	48	qRTPCR	
10342	SMN1-127 m01	0.38881819	0.079672425	SMN Full	6.25	GM09677	48	qRTPCR	
10342	SMN1-127 m01	0.58263021	0.03220219	SMN Full	3.135	GM09677	48	qRTPCR	
10342	SMN1-127 m01	0.62202112	0.088296487	SMN Full	1.25	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.128897	SMN Full	0	GM09677	48	qRTPCR	
	Ctrl Un	0.87449488	0.115103635	SMN Full	0	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11384	SMN1-37 m01	0.06497897	0.008700562	SMN del7	100	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.05259536	0.004353076	SMN del7	50	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.20007531	0.025219551	SMN del7	25	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.31629171	0.011237072	SMN del7	12.5	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.5123567	0.037298009	SMN del7	6.25	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.572806	0.094092392	SMN del7	3.135	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.62941499	0.111156184	SMN del7	1.25	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	0.12686825	0.008403414	SMN del7	100	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	0.09192823	0.007804392	SMN del7	50	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	0.09879707	0.020557828	SMN del7	25	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	0.12014898	0.009156615	SMN del7	12.5	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	0.29711984	0.038689668	SMN del7	6.25	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	0.39793414	0.031715802	SMN del7	3.135	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	0.55277899	0.071092444	SMN del7	1.25	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10339	SMN1-83 m01	0.23764818	0.011632412	SMN del7	100	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.18277366	0.008309779	SMN del7	50	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.19075253	0.00849922	SMN del7	25	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.3092481	0.031935646	SMN del7	12.5	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.36404455	0.034043724	SMN del7	6.25	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.14272055	0.017635279	SMN del7	3.135	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.81829095	0.094647389	SMN del7	1.25	GM09677	48	qRTPCR	
10954	SMN1-90 m01	0.71141754	0.076138792	SMN del7	100	GM09677	48	qRTPCR	
10954	SMN1-90 m01	0.61081897	0.092020925	SMN del7	50	GM09677	48	qRTPCR	
10954	SMN1-90 m01	0.7204336	0.088444023	SMN del7	25	GM09677	48	qRTPCR	
10954	SMN1-90 m01	0.55304302	0.115741101	SMN del7	12.5	GM09677	48	qRTPCR	
10954	SMN1-90 m01	0.56923176	0.08603456	SMN del7	6.25	GM09677	48	qRTPCR	
10954	SMN1-90 m01	0.64724116	0.079697427	SMN del7	3.135	GM09677	48	qRTPCR	
10954	SMN1-90 m01	0.76225277	0.07536244	SMN del7	1.25	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	Ctrl Lipo	1	0.241125065	SMN del7	0	GM09677	48	qRTPCR	
	Ctrl Un	0.88494293	0.015642304	SMN del7	0	GM09677	48	qRTPCR	
13095	unc-232 m12	0.72092908	0.157718406	SMN del7	30	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.10306939	0.006944188	SMN del7	100	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.03836028	0.008283218	SMN del7	50	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.04175557	0.009514029	SMN del7	25	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.08211765	0.015971962	SMN del7	12.5	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.15687385	0.045983653	SMN del7	6.25	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.19430269	0.019784913	SMN del7	3.135	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.19217326	0.004529139	SMN del7	1.25	GM09677	48	qRTPCR	
10955	SMN1-141 m01	0.16312904	0.023353962	SMN del7	100	GM09677	48	qRTPCR	
10955	SMN1-141 m01	0.12884738	0.005221594	SMN del7	50	GM09677	48	qRTPCR	
10955	SMN1-141 m01	0.14269687	0.013223101	SMN del7	25	GM09677	48	qRTPCR	
10955	SMN1-141 m01	0.39766591	0.080135938	SMN del7	12.5	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10955	SMN1-141 m01	0.81645046	0.228032706	SMN del7	6.25	GM09677	48	qRTPCR	
10955	SMN1-141 m01	1.09714022	0.156203348	SMN del7	3.135	GM09677	48	qRTPCR	
10955	SMN1-141 m01	0.82012861	0.031534539	SMN del7	1.25	GM09677	48	qRTPCR	
10956	SMN1-142 m01	0.09564993	0.006151677	SMN del7	100	GM09677	48	qRTPCR	
10956	SMN1-142 m01	0.10159396	0.015598411	SMN del7	50	GM09677	48	qRTPCR	
10956	SMN1-142 m01	0.22340898	0.052131979	SMN del7	25	GM09677	48	qRTPCR	
10956	SMN1-142 m01	0.45271455	0.031626976	SMN del7	12.5	GM09677	48	qRTPCR	
10956	SMN1-142 m01	0.852221536	0.222477276	SMN del7	6.25	GM09677	48	qRTPCR	
10956	SMN1-142 m01	0.95340029	0.093250422	SMN del7	3.135	GM09677	48	qRTPCR	
10956	SMN1-142 m01	1.88229611	0.903846232	SMN del7	1.25	GM09677	48	qRTPCR	
10957	SMN1-143 m01	1.99397052	0.621383476	SMN del7	100	GM09677	48	qRTPCR	
10957	SMN1-143 m01	2.93062375	0.398730535	SMN del7	50	GM09677	48	qRTPCR	
10957	SMN1-143 m01	1.5259383	0.046394838	SMN del7	25	GM09677	48	qRTPCR	
10957	SMN1-143 m01	1.58723142	0.728019654	SMN del7	12.5	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10957	SMN1-143 m01	0.88204105	0.102479011	SMN del7	6.25	GM09677	48	qRT-PCR	
10957	SMN1-143 m01	1.09736864	0.112591826	SMN del7	3.135	GM09677	48	qRT-PCR	
10957	SMN1-143 m01	1.09802198	0.172288461	SMN del7	1.25	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.094360183	SMN del7	0	GM09677	48	qRT-PCR	
	Ctrl Un	1.16909409	0.069645853	SMN del7	0	GM09677	48	qRT-PCR	
13096	unc-293 m01	2.54715886	1.280213074	SMN del7	30	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	0.14418711	0.026372524	SMN del7	100	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	0.07876013	0.009883203	SMN del7	50	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	0.0996249	0.00758507	SMN del7	25	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	0.32107654	0.080292081	SMN del7	12.5	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	0.58170828	0.054568137	SMN del7	6.25	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	0.68123319	0.123612357	SMN del7	3.135	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	0.78840293	0.031470772	SMN del7	1.25	GM09677	48	qRT-PCR	
10959	SMN1-145 m01	1.92115393	#DIV/0!	SMN del7	100	GM09677	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10959	SMN1-145 m01	0.99761526	0.147356914	SMN del7	50	GM09677	48	qRTPCR	
10959	SMN1-145 m01	1.31349889	0.140767746	SMN del7	25	GM09677	48	qRTPCR	
10959	SMN1-145 m01	0.74811237	0.04191162	SMN del7	12.5	GM09677	48	qRTPCR	
10959	SMN1-145 m01	0.64348747	0.009981868	SMN del7	6.25	GM09677	48	qRTPCR	
10959	SMN1-145 m01	0.83876941	0.063333761	SMN del7	3.135	GM09677	48	qRTPCR	
10959	SMN1-145 m01	0.80291282	0.131134246	SMN del7	1.25	GM09677	48	qRTPCR	
10961	SMN1-147 m01	2.73065071	0.052989915	SMN del7	100	GM09677	48	qRTPCR	
10961	SMN1-147 m01	2.95959286	0.071785775	SMN del7	50	GM09677	48	qRTPCR	
10961	SMN1-147 m01	2.18981425	0.278285358	SMN del7	25	GM09677	48	qRTPCR	
10961	SMN1-147 m01	1.22401609	0.179918613	SMN del7	12.5	GM09677	48	qRTPCR	
10961	SMN1-147 m01	0.74298753	0.034948055	SMN del7	6.25	GM09677	48	qRTPCR	
10961	SMN1-147 m01	0.64685971	0.059276084	SMN del7	3.135	GM09677	48	qRTPCR	
10961	SMN1-147 m01	1.088114	0.491464555	SMN del7	1.25	GM09677	48	qRTPCR	
11367	SMN1-149 m01	0.17716882	0.008650731	SMN del7	100	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11367	SMN1-149 m01	0.16783958	0.018279592	SMN del7	50	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	0.30187978	0.071674615	SMN del7	25	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	0.43148292	0.054516223	SMN del7	12.5	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	0.47150073	0.045046095	SMN del7	6.25	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	0.72195499	0.158829963	SMN del7	3.135	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	1.58265038	0.589206427	SMN del7	1.25	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.05391666	SMN del7	0	GM09677	48	qRT-PCR	
	Ctrl Un	1.23961402	0.462082634	SMN del7	0	GM09677	48	qRT-PCR	
11381	SMN1-34 m01	0.04084074	0.001945025	SMN del7	100	GM09677	48	qRT-PCR	
11381	SMN1-34 m01	0.04896962	0.006588723	SMN del7	50	GM09677	48	qRT-PCR	
11381	SMN1-34 m01	0.14874395	0.068415212	SMN del7	25	GM09677	48	qRT-PCR	
11381	SMN1-34 m01	0.20490029	0.047408959	SMN del7	12.5	GM09677	48	qRT-PCR	
11381	SMN1-34 m01	0.38578855	0.050195494	SMN del7	6.25	GM09677	48	qRT-PCR	
11381	SMN1-34 m01	0.59288671	0.116561841	SMN del7	3.135	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11381	SMN1-34 m01	0.50517699	0.19042928	SMN del7	1.25	GM09677	48	qRT-PCR	
13107	SMN1-109 m01	0.93186123	0.018083326	SMN del7	100	GM09677	48	qRT-PCR	
13107	SMN1-109 m01	0.50290135	0.07790185	SMN del7	50	GM09677	48	qRT-PCR	
13107	SMN1-109 m01	0.58015291	0.080609864	SMN del7	25	GM09677	48	qRT-PCR	
13107	SMN1-109 m01	0.88550514	0.125587632	SMN del7	12.5	GM09677	48	qRT-PCR	
13107	SMN1-109 m01	0.63257469	0.085578056	SMN del7	6.25	GM09677	48	qRT-PCR	
13107	SMN1-109 m01	0.41688402	0.06039843	SMN del7	3.135	GM09677	48	qRT-PCR	
13107	SMN1-109 m01	0.70231954	0.04102474	SMN del7	1.25	GM09677	48	qRT-PCR	
10340	SMN1-125 m01	0.42931464	0.00822078	SMN del7	100	GM09677	48	qRT-PCR	
10340	SMN1-125 m01	0.27403634	0.095812398	SMN del7	50	GM09677	48	qRT-PCR	
10340	SMN1-125 m01	0.19351494	0.011451309	SMN del7	25	GM09677	48	qRT-PCR	
10340	SMN1-125 m01	0.23777484	0.018000375	SMN del7	12.5	GM09677	48	qRT-PCR	
10340	SMN1-125 m01	0.52584637	0.161120008	SMN del7	6.25	GM09677	48	qRT-PCR	
10340	SMN1-125 m01	0.66479748	0.043912426	SMN del7	3.135	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10340	SMN1-125 m01	0.42520961	0.132627407	SMN del7	1.25	GM09677	48	qRT-PCR	
10342	SMN1-127 m01	0.14203141	0.021506288	SMN del7	100	GM09677	48	qRT-PCR	
10342	SMN1-127 m01	0.08615325	0.039180767	SMN del7	50	GM09677	48	qRT-PCR	
10342	SMN1-127 m01	0.30905423	0.18331188	SMN del7	25	GM09677	48	qRT-PCR	
10342	SMN1-127 m01	0.21879444	0.021268842	SMN del7	12.5	GM09677	48	qRT-PCR	
10342	SMN1-127 m01	0.38881819	0.079672425	SMN del7	6.25	GM09677	48	qRT-PCR	
10342	SMN1-127 m01	0.58263021	0.03220219	SMN del7	3.135	GM09677	48	qRT-PCR	
10342	SMN1-127 m01	0.62202112	0.088296487	SMN del7	1.25	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.128897	SMN del7	0	GM09677	48	qRT-PCR	
	Ctrl Un	0.87449488	0.115103635	SMN del7	0	GM09677	48	qRT-PCR	
11442	SMN1-99 m01	0.60745293	0.238684758	SMN Full	30	GM03813	48	qRT-PCR	
10087	SMN1-77 m01	1.73607616	0.932259128	SMN Full	30	GM03813	48	qRT-PCR	
10064	SMN1-163 m01	0.63293711	0.133590865	SMN Full	30	GM03813	48	qRT-PCR	
10076	SMN1-164 m01	1.52870424	0.10437615	SMN Full	30	GM03813	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10077	SMN1-165 m01	2.29308713	0.68087844	SMN Full	30	GM03813	48	qRT-PCR	
10083	SMN1-166 m01	0.09990656	0.034973079	SMN Full	30	GM03813	48	qRT-PCR	
10085	SMN1-167 m01	0.07422252	0.033767867	SMN Full	30	GM03813	48	qRT-PCR	
10086	SMN1-168 m01	2.53597656	1.450035013	SMN Full	30	GM03813	48	qRT-PCR	
10088	SMN1-169 m01	1.59415585	0.267560713	SMN Full	30	GM03813	48	qRT-PCR	
10089	SMN1-170 m01	2.8048702	0.69240046	SMN Full	30	GM03813	48	qRT-PCR	
10090	SMN1-110 m01	4.12467891	2.022273339	SMN Full	30	GM03813	48	qRT-PCR	
10091	SMN1-111 m01	4.53500052	1.589427629	SMN Full	30	GM03813	48	qRT-PCR	
10092	SMN1-112 m01	3.68387821	0.997614065	SMN Full	30	GM03813	48	qRT-PCR	
10093	SMN1-113 m01	2.04049614	0.17969628	SMN Full	30	GM03813	48	qRT-PCR	
10094	SMN1-114 m01	1.93932726	0.654238344	SMN Full	30	GM03813	48	qRT-PCR	
10095	SMN1-115 m01	2.04193742	0.671716496	SMN Full	30	GM03813	48	qRT-PCR	
10096	SMN1-116 m01	2.30960769	0.385042107	SMN Full	30	GM03813	48	qRT-PCR	
10097	SMN1-117 m01	1.58359635	0.109779763	SMN Full	30	GM03813	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10339	SMN1-83 m01	2.09230905	0.423357793	SMN Full	30	GM03813	48	qRT-PCR	
10330	SMN1-118 m01	1.21338254	0.12667865	SMN Full	30	GM03813	48	qRT-PCR	
10331	SMN1-119 m01	1.08288988	0.320628146	SMN Full	30	GM03813	48	qRT-PCR	
10332	SMN1-120 m01	3.11484157	1.511756253	SMN Full	30	GM03813	48	qRT-PCR	
10333	SMN1-121 m01	0.77913433	0.251450441	SMN Full	30	GM03813	48	qRT-PCR	
10334	SMN1-122 m01	2.71820434	1.645259939	SMN Full	30	GM03813	48	qRT-PCR	
10335	SMN1-123 m01	1.77921437	0.539691654	SMN Full	30	GM03813	48	qRT-PCR	
10336	SMN1-124 m01	1.95540428	0.510433372	SMN Full	30	GM03813	48	qRT-PCR	
10340	SMN1-125 m01	1.44836239	0.285105229	SMN Full	30	GM03813	48	qRT-PCR	
10341	SMN1-126 m01	1.16434872	0.081868197	SMN Full	30	GM03813	48	qRT-PCR	
10342	SMN1-127 m01	1.20625431	0.226730975	SMN Full	30	GM03813	48	qRT-PCR	
10344	SMN1-128 m01	1.39920509	0.212262107	SMN Full	30	GM03813	48	qRT-PCR	
10345	SMN1-129 m01	0.97802596	0.157831627	SMN Full	30	GM03813	48	qRT-PCR	
10346	SMN1-130 m01	1.5058246	0.12541655	SMN Full	30	GM03813	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13095	unc-232 m12	1.6073063	0.618037004	SMN Full	30	GM03813	48	qRTPCR	
	Ctrl Lipo	1	0.05332148	SMN Full	30	GM03813	48	qRTPCR	
10347	SMN1-131 m01	1.80812609	0.160325835	SMN Full	30	GM03813	48	qRTPCR	
10348	SMN1-132 m01	1.17749024	0.103550442	SMN Full	30	GM03813	48	qRTPCR	
10954	SMN1-90 m01	1.42004841	#VALUE!	SMN Full	30	GM03813	48	qRTPCR	
10942	SMN1-133 m01	0.64911188	0.089024801	SMN Full	30	GM03813	48	qRTPCR	
10943	SMN1-134 m01	0.74688478	0.075568955	SMN Full	30	GM03813	48	qRTPCR	
10944	SMN1-135 m01	1.15720422	0.078529777	SMN Full	30	GM03813	48	qRTPCR	
10945	SMN1-136 m01	1.82338897	0.129704368	SMN Full	30	GM03813	48	qRTPCR	
10946	SMN1-137 m01	1.94178493	0.204739374	SMN Full	30	GM03813	48	qRTPCR	
10947	SMN1-138 m01	1.74504912	0.19025014	SMN Full	30	GM03813	48	qRTPCR	
10948	SMN1-139 m01	1.3426225	0.137525344	SMN Full	30	GM03813	48	qRTPCR	
10950	SMN1-140 m01	1.20675667	0.07407693	SMN Full	30	GM03813	48	qRTPCR	
10955	SMN1-141 m01	4.94270821	0.167810914	SMN Full	30	GM03813	48	qRTPCR	
10956	SMN1-142	7.06109885	0.42174149	SMN Full	30	GM03813	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
10957	SMN1-143 m01	3.88447548	0.277270816	SMN Full	30	GM03813	48	qRTPCR	
10958	SMN1-144 m01	5.75101117	0.472819431	SMN Full	30	GM03813	48	qRTPCR	
10959	SMN1-145 m01	1.63175508	0.143582554	SMN Full	30	GM03813	48	qRTPCR	
10960	SMN1-146 m01	1.02114742	0.033505296	SMN Full	30	GM03813	48	qRTPCR	
10961	SMN1-147 m01	3.84343909	0.362657237	SMN Full	30	GM03813	48	qRTPCR	
10962	SMN1-148 m01	0.56220775	0.133400369	SMN Full	30	GM03813	48	qRTPCR	
11384	SMN1-37 m01	2.67486361	0.303072975	SMN Full	30	GM03813	48	qRTPCR	
11367	SMN1-149 m01	2.81977844	0.14712057	SMN Full	30	GM03813	48	qRTPCR	
11368	SMN1-150 m01	2.53613542	0.114196215	SMN Full	30	GM03813	48	qRTPCR	
11369	SMN1-151 m01	1.87611289	0.176137206	SMN Full	30	GM03813	48	qRTPCR	
11370	SMN1-152 m01	1.32636407	0.024008938	SMN Full	30	GM03813	48	qRTPCR	
11371	SMN1-153 m01	0.60906758	0.067828525	SMN Full	30	GM03813	48	qRTPCR	
11372	SMN1-154 m01	1.59769003	0.092430094	SMN Full	30	GM03813	48	qRTPCR	
11373	SMN1-155 m01	0.990789	0.093004543	SMN Full	30	GM03813	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
11400	SMN1-156 m01	0.21117341	0.047690988	SMN Full	30	GM03813	48	qRTPCR	
11401	SMN1-157 m01	1.8637108	0.09324604	SMN Full	30	GM03813	48	qRTPCR	
11402	SMN1-158 m01	1.38942428	0.114668606	SMN Full	30	GM03813	48	qRTPCR	
11403	SMN1-159 m01	1.37381819	0.069876001	SMN Full	30	GM03813	48	qRTPCR	
11404	SMN1-160 m01	1.48961997	0.020799977	SMN Full	30	GM03813	48	qRTPCR	
13096	unc-293 m01	0.76640425	0.008564961	SMN Full	30	GM03813	48	qRTPCR	
	Ctrl Lipo	1	0.065802125	SMN Full	30	GM03813	48	qRTPCR	
11405	SMN1-161 m01	0.77870721	0.132556624	SMN Full	30	GM03813	48	qRTPCR	
11406	SMN1-162 m01	0.98726096	0.009238158	SMN Full	30	GM03813	48	qRTPCR	
13088	SMN1-54 m01	2.46827382	0.341073223	SMN Full	30	GM03813	48	qRTPCR	
11394	SMN1-47 m01	0.40482214	0.129929055	SMN Full	30	GM03813	48	qRTPCR	
11381	SMN1-34 m01	3.21931748	0.298813668	SMN Full	30	GM03813	48	qRTPCR	
10952	SMN1-88 m01	3.3967123	0.735185908	SMN Full	30	GM03813	48	qRTPCR	
10953	SMN1-89 m01	2.91415498	0.121087816	SMN Full	30	GM03813	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
12369	SMN1-101 m01	2.69438027	0.434775199	SMN Full	30	GM03813	48	qRTPCR	
	EPO-10 m01	2.47562213	0.544552015	SMN Full	30	GM03813	48	qRTPCR	
	EPO-18 m01	2.27770446	0.067866479	SMN Full	30	GM03813	48	qRTPCR	
	Ctrl Lipo	1	0.2500104	SMN Full	30	GM03813	48	qRTPCR	
	Ctrl Un	1.3202777	0.046652725	SMN Full	30	GM03813	48	qRTPCR	
11442	SMN1-99 m01	0.99718687	0.476992825	SMN del7	30	GM03813	48	qRTPCR	
10087	SMN1-77 m01	#VALUE!	#VALUE!	SMN del7	30	GM03813	48	qRTPCR	
10064	SMN1-163 m01	2.01312557	1.640142046	SMN del7	30	GM03813	48	qRTPCR	
10076	SMN1-164 m01	0.19921013	0.039315647	SMN del7	30	GM03813	48	qRTPCR	
10077	SMN1-165 m01	0.59272009	0.406967223	SMN del7	30	GM03813	48	qRTPCR	
10083	SMN1-166 m01	3.80351908	0.487120709	SMN del7	30	GM03813	48	qRTPCR	
10085	SMN1-167 m01	4.75726438	0.73818952	SMN del7	30	GM03813	48	qRTPCR	
10086	SMN1-168 m01	#VALUE!	#VALUE!	SMN del7	30	GM03813	48	qRTPCR	
10088	SMN1-169 m01	1.15377568	0.181354417	SMN del7	30	GM03813	48	qRTPCR	
10089	SMN1-170 m01	0.3903339	0.093415857	SMN del7	30	GM03813	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10090	SMN1-110 m01	0.94893234	0.453588415	SMN del7	30	GM03813	48	qRT-PCR	
10091	SMN1-111 m01	1.33851614	0.681735014	SMN del7	30	GM03813	48	qRT-PCR	
10092	SMN1-112 m01	1.28919719	0.735414719	SMN del7	30	GM03813	48	qRT-PCR	
10093	SMN1-113 m01	0.70942306	0.073147107	SMN del7	30	GM03813	48	qRT-PCR	
10094	SMN1-114 m01	1.30353323	0.927861186	SMN del7	30	GM03813	48	qRT-PCR	
10095	SMN1-115 m01	0.59026034	0.337089049	SMN del7	30	GM03813	48	qRT-PCR	
10096	SMN1-116 m01	0.82448294	0.10247291	SMN del7	30	GM03813	48	qRT-PCR	
10097	SMN1-117 m01	0.52051818	0.094323906	SMN del7	30	GM03813	48	qRT-PCR	
10339	SMN1-83 m01	#VALUE!	#VALUE!	SMN del7	30	GM03813	48	qRT-PCR	
10330	SMN1-118 m01	0.42069338	0.129568649	SMN del7	30	GM03813	48	qRT-PCR	
10331	SMN1-119 m01	2.56464233	0.393931558	SMN del7	30	GM03813	48	qRT-PCR	
10332	SMN1-120 m01	1.01195353	0.736940854	SMN del7	30	GM03813	48	qRT-PCR	
10333	SMN1-121 m01	1.61421904	0.289093411	SMN del7	30	GM03813	48	qRT-PCR	
10334	SMN1-122 m01	2.64621258	0.33206982	SMN del7	30	GM03813	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10335	SMN1-123 m01	#VALUE!	#VALUE!	SMN del7	30	GM03813	48	qRT-PCR	
10336	SMN1-124 m01	0.99042455	0.079659665	SMN del7	30	GM03813	48	qRT-PCR	
10340	SMN1-125 m01	0.21303864	#DIV/0!	SMN del7	30	GM03813	48	qRT-PCR	
10341	SMN1-126 m01	0.34820195	0.0761063	SMN del7	30	GM03813	48	qRT-PCR	
10342	SMN1-127 m01	0.21997966	0.086709096	SMN del7	30	GM03813	48	qRT-PCR	
10344	SMN1-128 m01	0.05974686	0.00487591	SMN del7	30	GM03813	48	qRT-PCR	
10345	SMN1-129 m01	0.08446333	0.019582156	SMN del7	30	GM03813	48	qRT-PCR	
10346	SMN1-130 m01	0.06720175	0.027476918	SMN del7	30	GM03813	48	qRT-PCR	
13095	unc-232 m12	1.46202714	0.553048323	SMN del7	30	GM03813	48	qRT-PCR	
	Ctrl Lipo	1	0.13206188	SMN del7	30	GM03813	48	qRT-PCR	
10347	SMN1-131 m01	0.4698881	0.03490837	SMN del7	30	GM03813	48	qRT-PCR	
10348	SMN1-132 m01	1.03723586	0.073597748	SMN del7	30	GM03813	48	qRT-PCR	
10954	SMN1-90 m01	#VALUE!	#VALUE!	SMN del7	30	GM03813	48	qRT-PCR	
10942	SMN1-133 m01	1.75093653	0.132309167	SMN del7	30	GM03813	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10943	SMN1-134 m01	1.21894268	0.237205832	SMN del7	30	GM03813	48	qRT-PCR	
10944	SMN1-135 m01	0.10219941	0.007829177	SMN del7	30	GM03813	48	qRT-PCR	
10945	SMN1-136 m01	0.12247621	0.021544208	SMN del7	30	GM03813	48	qRT-PCR	
10946	SMN1-137 m01	0.15744957	0.096129423	SMN del7	30	GM03813	48	qRT-PCR	
10947	SMN1-138 m01	0.2689001	0.04976792	SMN del7	30	GM03813	48	qRT-PCR	
10948	SMN1-139 m01	2.20333947	0.110478107	SMN del7	30	GM03813	48	qRT-PCR	
10950	SMN1-140 m01	3.374181	0.126899682	SMN del7	30	GM03813	48	qRT-PCR	
10955	SMN1-141 m01	0.07268975	0.030744292	SMN del7	30	GM03813	48	qRT-PCR	
10956	SMN1-142 m01	0.09347564	0.029405871	SMN del7	30	GM03813	48	qRT-PCR	
10957	SMN1-143 m01	1.08619398	0.129386546	SMN del7	30	GM03813	48	qRT-PCR	
10958	SMN1-144 m01	0.08604226	0.020278104	SMN del7	30	GM03813	48	qRT-PCR	
10959	SMN1-145 m01	0.68552572	0.168686341	SMN del7	30	GM03813	48	qRT-PCR	
10960	SMN1-146 m01	0.90647273	0.105649215	SMN del7	30	GM03813	48	qRT-PCR	
10961	SMN1-147 m01	2.75086744	0.524054531	SMN del7	30	GM03813	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10962	SMN1-148 m01	1.0680935	0.055488036	SMN del7	30	GM03813	48	qRT-PCR	
11384	SMN1-37 m01	#VALUE!	#VALUE!	SMN del7	30	GM03813	48	qRT-PCR	
11367	SMN1-149 m01	0.14953279	0.036807461	SMN del7	30	GM03813	48	qRT-PCR	
11368	SMN1-150 m01	0.54113427	0.085590548	SMN del7	30	GM03813	48	qRT-PCR	
11369	SMN1-151 m01	1.20412884	0.053119288	SMN del7	30	GM03813	48	qRT-PCR	
11370	SMN1-152 m01	2.53650937	0.243841354	SMN del7	30	GM03813	48	qRT-PCR	
11371	SMN1-153 m01	2.72092058	1.045511149	SMN del7	30	GM03813	48	qRT-PCR	
11372	SMN1-154 m01	2.00427529	0.507804529	SMN del7	30	GM03813	48	qRT-PCR	
11373	SMN1-155 m01	1.39469509	0.253381829	SMN del7	30	GM03813	48	qRT-PCR	
11400	SMN1-156 m01	5.01196977	1.293034323	SMN del7	30	GM03813	48	qRT-PCR	
11401	SMN1-157 m01	0.34201867	0.035745023	SMN del7	30	GM03813	48	qRT-PCR	
11402	SMN1-158 m01	4.49352179	0.526632769	SMN del7	30	GM03813	48	qRT-PCR	
11403	SMN1-159 m01	1.23233508	0.353618141	SMN del7	30	GM03813	48	qRT-PCR	
11404	SMN1-160 m01	0.98691766	0.072380865	SMN del7	30	GM03813	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13096	unc-293 m01	0.66207373	0.081435175	SMN del7	30	GM03813	48	qRTPCR	
	Ctrl Lipo	1	0.147420128	SMN del7	30	GM03813	48	qRTPCR	
11442	SMN1-99 m01	0.37124686	0.105051511	SMN Full	60	GM03813	48	qRTPCR	
10087	SMN1-77 m01	2.15352448	0.722861847	SMN Full	60	GM03813	48	qRTPCR	
10064	SMN1-163 m01	0.49869777	0.268089377	SMN Full	60	GM03813	48	qRTPCR	
10076	SMN1-164 m01	0.40070195	0.064566888	SMN Full	60	GM03813	48	qRTPCR	
10077	SMN1-165 m01	0.97366101	0.314616969	SMN Full	60	GM03813	48	qRTPCR	
10083	SMN1-166 m01	0.08456486	0.016716958	SMN Full	60	GM03813	48	qRTPCR	
10085	SMN1-167 m01	#VALUE!	#VALUE!	SMN Full	60	GM03813	48	qRTPCR	
10086	SMN1-168 m01	3.78108397	2.085995603	SMN Full	60	GM03813	48	qRTPCR	
10088	SMN1-169 m01	1.00866595	0.053315974	SMN Full	60	GM03813	48	qRTPCR	
10089	SMN1-170 m01	1.45759048	0.175007568	SMN Full	60	GM03813	48	qRTPCR	
10090	SMN1-110 m01	1.35531718	0.22675629	SMN Full	60	GM03813	48	qRTPCR	
10091	SMN1-111 m01	1.02778829	0.167064943	SMN Full	60	GM03813	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10092	SMN1-112 m01	0.97353224	0.391061587	SMN Full	60	GM03813	48	qRT-PCR	
10093	SMN1-113 m01	1.24559447	0.334537466	SMN Full	60	GM03813	48	qRT-PCR	
10094	SMN1-114 m01	0.71758168	0.123667818	SMN Full	60	GM03813	48	qRT-PCR	
10095	SMN1-115 m01	0.73533664	0.099844414	SMN Full	60	GM03813	48	qRT-PCR	
10096	SMN1-116 m01	0.90841775	0.177472842	SMN Full	60	GM03813	48	qRT-PCR	
10097	SMN1-117 m01	0.73858745	0.187767815	SMN Full	60	GM03813	48	qRT-PCR	
10339	SMN1-83 m01	6.97856719	0.535860484	SMN Full	60	GM03813	48	qRT-PCR	
10330	SMN1-118 m01	0.60869106	0.401208395	SMN Full	60	GM03813	48	qRT-PCR	
10331	SMN1-119 m01	0.35825197	0.071490968	SMN Full	60	GM03813	48	qRT-PCR	
10332	SMN1-120 m01	1.10574094	0.247402856	SMN Full	60	GM03813	48	qRT-PCR	
10333	SMN1-121 m01	0.41101378	0.08237783	SMN Full	60	GM03813	48	qRT-PCR	
10334	SMN1-122 m01	0.69278356	0.309077213	SMN Full	60	GM03813	48	qRT-PCR	
10335	SMN1-123 m01	2.00336141	0.315983723	SMN Full	60	GM03813	48	qRT-PCR	
10336	SMN1-124 m01	1.01096966	0.222112641	SMN Full	60	GM03813	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10340	SMN1-125 m01	1.28313971	0.046302456	SMN Full	60	GM03813	48	qRTPCR	
10341	SMN1-126 m01	0.77986812	0.253270121	SMN Full	60	GM03813	48	qRTPCR	
10342	SMN1-127 m01	0.93977203	0.195752983	SMN Full	60	GM03813	48	qRTPCR	
10344	SMN1-128 m01	0.83007264	0.187985401	SMN Full	60	GM03813	48	qRTPCR	
10345	SMN1-129 m01	0.73630448	0.135935293	SMN Full	60	GM03813	48	qRTPCR	
10346	SMN1-130 m01	1.31262868	0.171862185	SMN Full	60	GM03813	48	qRTPCR	
13095	unc-232 m12	2.32893862	0.731915111	SMN Full	60	GM03813	48	qRTPCR	
	Ctrl Lipo	1	0.171572875	SMN Full	60	GM03813	48	qRTPCR	
10347	SMN1-131 m01	1.45477629	0.327690681	SMN Full	60	GM03813	48	qRTPCR	
10348	SMN1-132 m01	1.05959901	0.051969458	SMN Full	60	GM03813	48	qRTPCR	
10954	SMN1-90 m01	11.4350717	3.386872231	SMN Full	60	GM03813	48	qRTPCR	
10942	SMN1-133 m01	0.72831795	0.01022925	SMN Full	60	GM03813	48	qRTPCR	
10943	SMN1-134 m01	0.83719006	0.058426854	SMN Full	60	GM03813	48	qRTPCR	
10944	SMN1-135 m01	1.43103588	0.195151151	SMN Full	60	GM03813	48	qRTPCR	
10945	SMN1-136	1.89000575	0.201099036	SMN Full	60	GM03813	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
10946	SMN1-137 m01	1.7713431	0.243485311	SMN Full	60	GM03813	48	qRTPCR	
10947	SMN1-138 m01	1.67262811	0.112207771	SMN Full	60	GM03813	48	qRTPCR	
10948	SMN1-139 m01	1.62544998	0.123554852	SMN Full	60	GM03813	48	qRTPCR	
10950	SMN1-140 m01	0.96909303	0.003513528	SMN Full	60	GM03813	48	qRTPCR	
10955	SMN1-141 m01	6.36949063	0.62640389	SMN Full	60	GM03813	48	qRTPCR	
10956	SMN1-142 m01	10.4940251	0.851490894	SMN Full	60	GM03813	48	qRTPCR	
10957	SMN1-143 m01	4.65097036	0.28384382	SMN Full	60	GM03813	48	qRTPCR	
10958	SMN1-144 m01	7.61488579	0.136297703	SMN Full	60	GM03813	48	qRTPCR	
10959	SMN1-145 m01	1.29371502	0.084392475	SMN Full	60	GM03813	48	qRTPCR	
10960	SMN1-146 m01	1.01464966	0.228517595	SMN Full	60	GM03813	48	qRTPCR	
10961	SMN1-147 m01	2.62718334	0.456459819	SMN Full	60	GM03813	48	qRTPCR	
10962	SMN1-148 m01	0.36668805	0.1111797398	SMN Full	60	GM03813	48	qRTPCR	
11384	SMN1-37 m01	16.8241438	2.851068036	SMN Full	60	GM03813	48	qRTPCR	
11367	SMN1-149	1.69269051	0.090159355	SMN Full	60	GM03813	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
11368	SMN1-150 m01	1.88029177	0.099664657	SMN Full	60	GM03813	48	qRTPCR	
11369	SMN1-151 m01	1.3708809	0.19467374	SMN Full	60	GM03813	48	qRTPCR	
11370	SMN1-152 m01	1.21512658	0.251597877	SMN Full	60	GM03813	48	qRTPCR	
11371	SMN1-153 m01	0.56519242	0.057328455	SMN Full	60	GM03813	48	qRTPCR	
11372	SMN1-154 m01	1.24701644	0.113465066	SMN Full	60	GM03813	48	qRTPCR	
11373	SMN1-155 m01	0.6425592	0.079920176	SMN Full	60	GM03813	48	qRTPCR	
11400	SMN1-156 m01	0.48311866	0.281370772	SMN Full	60	GM03813	48	qRTPCR	
11401	SMN1-157 m01	2.02496667	0.216383117	SMN Full	60	GM03813	48	qRTPCR	
11402	SMN1-158 m01	1.2934357	0.085559594	SMN Full	60	GM03813	48	qRTPCR	
11403	SMN1-159 m01	1.56807321	0.0835293	SMN Full	60	GM03813	48	qRTPCR	
11404	SMN1-160 m01	1.26231685	0.068451668	SMN Full	60	GM03813	48	qRTPCR	
13096	unc-293 m01	0.98427659	0.101401932	SMN Full	60	GM03813	48	qRTPCR	
	Ctrl Lipo	1	0.120992679	SMN Full	60	GM03813	48	qRTPCR	
11405	SMN1-161 m01	3.75743665	0.868245962	SMN Full	60	GM03813	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11406	SMN1-162 m01	2.35107199	0.306527091	SMN Full	60	GM03813	48	qRTPCR	
13088	SMN1-54 m01	14.4334548	1.485386398	SMN Full	60	GM03813	48	qRTPCR	
11394	SMN1-47 m01	0.36157004	0.056194271	SMN Full	60	GM03813	48	qRTPCR	
11381	SMN1-34 m01	10.1594702	0.931835232	SMN Full	60	GM03813	48	qRTPCR	
10952	SMN1-88 m01	5.99874935	1.378424007	SMN Full	60	GM03813	48	qRTPCR	
10953	SMN1-89 m01	4.53950654	0.959045358	SMN Full	60	GM03813	48	qRTPCR	
12369	SMN1-101 m01	8.69933693	1.539951964	SMN Full	60	GM03813	48	qRTPCR	
	EPO-10 m01	7.0669842	2.057612233	SMN Full	60	GM03813	48	qRTPCR	
	EPO-18 m01	2.90076135	1.128102743	SMN Full	60	GM03813	48	qRTPCR	
	Ctrl Lipo	1	0.235988263	SMN Full	60	GM03813	48	qRTPCR	
	Ctrl Un	5.84614895	1.657929817	SMN Full	60	GM03813	48	qRTPCR	
11442	SMN1-99 m01	1.05767197	0.11324727	SMN Full	30	GM09677	48	qRTPCR	
10087	SMN1-77 m01	2.28368516	0.273614746	SMN Full	30	GM09677	48	qRTPCR	
10064	SMN1-163 m01	0.7978951	0.057972984	SMN Full	30	GM09677	48	qRTPCR	
10076	SMN1-164 m01	0.91026818	0.16080178	SMN Full	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10077	SMN1-165 m01	1.20166763	0.208597381	SMN Full	30	GM09677	48	qRT-PCR	
10083	SMN1-166 m01	0.23640308	0.0244498126	SMN Full	30	GM09677	48	qRT-PCR	
10085	SMN1-167 m01	0.05473458	0.004803382	SMN Full	30	GM09677	48	qRT-PCR	
10086	SMN1-168 m01	1.52393409	0.063795338	SMN Full	30	GM09677	48	qRT-PCR	
10088	SMN1-169 m01	1.77968182	0.704284835	SMN Full	30	GM09677	48	qRT-PCR	
10089	SMN1-170 m01	2.12423405	0.59535874	SMN Full	30	GM09677	48	qRT-PCR	
10090	SMN1-110 m01	1.25805948	0.064188099	SMN Full	30	GM09677	48	qRT-PCR	
10091	SMN1-111 m01	1.0365813	0.096514217	SMN Full	30	GM09677	48	qRT-PCR	
10092	SMN1-112 m01	0.95023302	0.170524441	SMN Full	30	GM09677	48	qRT-PCR	
10093	SMN1-113 m01	1.10741335	0.066385791	SMN Full	30	GM09677	48	qRT-PCR	
10094	SMN1-114 m01	0.90975275	0.011898718	SMN Full	30	GM09677	48	qRT-PCR	
10095	SMN1-115 m01	0.61792274	0.036380062	SMN Full	30	GM09677	48	qRT-PCR	
10096	SMN1-116 m01	1.54552844	0.504495865	SMN Full	30	GM09677	48	qRT-PCR	
10097	SMN1-117 m01	0.83224135	0.214545638	SMN Full	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10339	SMN1-83 m01	2.25084024	0.124396695	SMN Full	30	GM09677	48	qRT-PCR	
10330	SMN1-118 m01	1.8949807	0.137448926	SMN Full	30	GM09677	48	qRT-PCR	
10331	SMN1-119 m01	1.08063386	0.145518583	SMN Full	30	GM09677	48	qRT-PCR	
10332	SMN1-120 m01	1.9022125	0.082767731	SMN Full	30	GM09677	48	qRT-PCR	
10333	SMN1-121 m01	1.7123379	0.057943519	SMN Full	30	GM09677	48	qRT-PCR	
10334	SMN1-122 m01	2.35227184	0.196949483	SMN Full	30	GM09677	48	qRT-PCR	
10335	SMN1-123 m01	2.73161163	0.821920903	SMN Full	30	GM09677	48	qRT-PCR	
10336	SMN1-124 m01	1.14929745	0.032266183	SMN Full	30	GM09677	48	qRT-PCR	
10340	SMN1-125 m01	1.39488238	0.092009685	SMN Full	30	GM09677	48	qRT-PCR	
10341	SMN1-126 m01	1.74109485	0.153571692	SMN Full	30	GM09677	48	qRT-PCR	
10342	SMN1-127 m01	1.67768769	0.107635453	SMN Full	30	GM09677	48	qRT-PCR	
10344	SMN1-128 m01	1.00583895	0.187672914	SMN Full	30	GM09677	48	qRT-PCR	
10345	SMN1-129 m01	1.03795516	0.156496035	SMN Full	30	GM09677	48	qRT-PCR	
10346	SMN1-130 m01	1.4385732	0.263259158	SMN Full	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13095	unc-232 m12	1.15576513	0.15925985	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.09263974	SMN Full	30	GM09677	48	qRTPCR	
10347	SMN1-131 m01	1.27765593	0.086800675	SMN Full	30	GM09677	48	qRTPCR	
10348	SMN1-132 m01	1.3149732	0.072667694	SMN Full	30	GM09677	48	qRTPCR	
10954	SMN1-90 m01	1.93175058	0.089309016	SMN Full	30	GM09677	48	qRTPCR	
10942	SMN1-133 m01	1.00106665	0.090044174	SMN Full	30	GM09677	48	qRTPCR	
10943	SMN1-134 m01	1.25263787	0.099844735	SMN Full	30	GM09677	48	qRTPCR	
10944	SMN1-135 m01	1.64430874	0.043494129	SMN Full	30	GM09677	48	qRTPCR	
10945	SMN1-136 m01	1.97488576	0.124701688	SMN Full	30	GM09677	48	qRTPCR	
10946	SMN1-137 m01	2.58700172	0.061090116	SMN Full	30	GM09677	48	qRTPCR	
10947	SMN1-138 m01	1.20774237	0.149403436	SMN Full	30	GM09677	48	qRTPCR	
10948	SMN1-139 m01	1.26504251	0.23186868	SMN Full	30	GM09677	48	qRTPCR	
10950	SMN1-140 m01	1.1671447	0.091181692	SMN Full	30	GM09677	48	qRTPCR	
10955	SMN1-141 m01	3.77038231	0.31410442	SMN Full	30	GM09677	48	qRTPCR	
10956	SMN1-142	4.84179177	0.484478019	SMN Full	30	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
10957	SMN1-143 m01	3.19275814	0.449738635	SMN Full	30	GM09677	48	qRTPCR	
10958	SMN1-144 m01	4.05584193	0.342905419	SMN Full	30	GM09677	48	qRTPCR	
10959	SMN1-145 m01	2.19247635	0.07563888	SMN Full	30	GM09677	48	qRTPCR	
10960	SMN1-146 m01	1.14795167	0.260343909	SMN Full	30	GM09677	48	qRTPCR	
10961	SMN1-147 m01	3.40348648	0.179898146	SMN Full	30	GM09677	48	qRTPCR	
10962	SMN1-148 m01	0.71686874	0.02859678	SMN Full	30	GM09677	48	qRTPCR	
11384	SMN1-37 m01	2.49638427	0.133860951	SMN Full	30	GM09677	48	qRTPCR	
11367	SMN1-149 m01	1.80168566	0.183679606	SMN Full	30	GM09677	48	qRTPCR	
11368	SMN1-150 m01	1.55272143	0.062123043	SMN Full	30	GM09677	48	qRTPCR	
11369	SMN1-151 m01	1.52371167	0.261283532	SMN Full	30	GM09677	48	qRTPCR	
11370	SMN1-152 m01	1.16583308	0.11586437	SMN Full	30	GM09677	48	qRTPCR	
11371	SMN1-153 m01	0.84391077	0.069881453	SMN Full	30	GM09677	48	qRTPCR	
11372	SMN1-154 m01	1.09252713	0.15465206	SMN Full	30	GM09677	48	qRTPCR	
11373	SMN1-155 m01	0.97901164	0.060003672	SMN Full	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
11400	SMN1-156 m01	0.25360242	0.037067821	SMN Full	30	GM09677	48	qRTPCR	
11401	SMN1-157 m01	2.31993783	0.182264516	SMN Full	30	GM09677	48	qRTPCR	
11402	SMN1-158 m01	0.88135916	0.055446489	SMN Full	30	GM09677	48	qRTPCR	
11403	SMN1-159 m01	1.28317352	0.064141678	SMN Full	30	GM09677	48	qRTPCR	
11404	SMN1-160 m01	1.15567054	0.066040939	SMN Full	30	GM09677	48	qRTPCR	
13096	unc-293 m01	1.33079613	0.473931905	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.138833665	SMN Full	30	GM09677	48	qRTPCR	
11405	SMN1-161 m01	1.13190119	0.046557167	SMN Full	30	GM09677	48	qRTPCR	
11406	SMN1-162 m01	0.94785179	0.046487703	SMN Full	30	GM09677	48	qRTPCR	
13088	SMN1-54 m01	3.00224684	0.071512184	SMN Full	30	GM09677	48	qRTPCR	
11394	SMN1-47 m01	0.48529261	0.012864886	SMN Full	30	GM09677	48	qRTPCR	
11381	SMN1-34 m01	2.71914762	0.108759907	SMN Full	30	GM09677	48	qRTPCR	
10952	SMN1-88 m01	1.90924382	0.059699542	SMN Full	30	GM09677	48	qRTPCR	
10953	SMN1-89 m01	1.71675591	0.060991838	SMN Full	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
12369	SMN1-101 m01	1.72466998	0.053807142	SMN Full	30	GM09677	48	qRTPCR	
	EPO-10 m01	1.20152017	0.025105928	SMN Full	30	GM09677	48	qRTPCR	
	EPO-18 m01	1.13559561	0.067236986	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.027759454	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Un	1.12243902	0.05357349	SMN Full	30	GM09677	48	qRTPCR	
11442	SMN1-99 m01	0.83001834	0.039674404	SMN del7	30	GM09677	48	qRTPCR	
10087	SMN1-77 m01	0.14987698	0.020155811	SMN del7	30	GM09677	48	qRTPCR	
10064	SMN1-163 m01	0.60191248	0.044649897	SMN del7	30	GM09677	48	qRTPCR	
10076	SMN1-164 m01	0.13664277	0.017482733	SMN del7	30	GM09677	48	qRTPCR	
10077	SMN1-165 m01	0.11678361	0.007546315	SMN del7	30	GM09677	48	qRTPCR	
10083	SMN1-166 m01	1.55836756	0.147989112	SMN del7	30	GM09677	48	qRTPCR	
10085	SMN1-167 m01	2.57826108	0.672787596	SMN del7	30	GM09677	48	qRTPCR	
10086	SMN1-168 m01	1.16206917	0.154759296	SMN del7	30	GM09677	48	qRTPCR	
10088	SMN1-169 m01	0.42784631	0.047692014	SMN del7	30	GM09677	48	qRTPCR	
10089	SMN1-170 m01	0.54643476	0.198440196	SMN del7	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10090	SMN1-110 m01	0.51099799	0.079820361	SMN del7	30	GM09677	48	qRTPCR	
10091	SMN1-111 m01	0.36631019	0.089455979	SMN del7	30	GM09677	48	qRTPCR	
10092	SMN1-112 m01	0.33623511	0.061594783	SMN del7	30	GM09677	48	qRTPCR	
10093	SMN1-113 m01	0.43517916	0.133086085	SMN del7	30	GM09677	48	qRTPCR	
10094	SMN1-114 m01	0.42177584	0.039454401	SMN del7	30	GM09677	48	qRTPCR	
10095	SMN1-115 m01	0.45166256	0.045273713	SMN del7	30	GM09677	48	qRTPCR	
10096	SMN1-116 m01	0.59598741	0.064640362	SMN del7	30	GM09677	48	qRTPCR	
10097	SMN1-117 m01	0.8960201	0.605146387	SMN del7	30	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.33166378	0.029008231	SMN del7	30	GM09677	48	qRTPCR	
10330	SMN1-118 m01	0.46451931	0.163291851	SMN del7	30	GM09677	48	qRTPCR	
10331	SMN1-119 m01	1.17945983	0.348534902	SMN del7	30	GM09677	48	qRTPCR	
10332	SMN1-120 m01	0.27051872	0.039942245	SMN del7	30	GM09677	48	qRTPCR	
10333	SMN1-121 m01	0.58906997	0.0624576	SMN del7	30	GM09677	48	qRTPCR	
10334	SMN1-122 m01	1.13608961	0.350456445	SMN del7	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10335	SMN1-123 m01	0.32404307	0.016598159	SMN del7	30	GM09677	48	qRT-PCR	
10336	SMN1-124 m01	1.03368937	0.609810593	SMN del7	30	GM09677	48	qRT-PCR	
10340	SMN1-125 m01	0.84368667	0.266885198	SMN del7	30	GM09677	48	qRT-PCR	
10341	SMN1-126 m01	0.14276208	0.008984738	SMN del7	30	GM09677	48	qRT-PCR	
10342	SMN1-127 m01	0.1153661	0.022433332	SMN del7	30	GM09677	48	qRT-PCR	
10344	SMN1-128 m01	0.11184112	0.030144041	SMN del7	30	GM09677	48	qRT-PCR	
10345	SMN1-129 m01	0.13557855	0.030299249	SMN del7	30	GM09677	48	qRT-PCR	
10346	SMN1-130 m01	0.05862167	0.011575453	SMN del7	30	GM09677	48	qRT-PCR	
13095	unc-232 m12	0.47752506	0.098591453	SMN del7	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.25395028	SMN del7	30	GM09677	48	qRT-PCR	
10347	SMN1-131 m01	0.57301004	0.138720724	SMN del7	30	GM09677	48	qRT-PCR	
10348	SMN1-132 m01	1.07511943	0.159709568	SMN del7	30	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	0.69024775	0.063261928	SMN del7	30	GM09677	48	qRT-PCR	
10942	SMN1-133 m01	2.24286282	0.259101685	SMN del7	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10943	SMN1-134 m01	2.37403718	0.018534239	SMN del7	30	GM09677	48	qRT-PCR	
10944	SMN1-135 m01	0.13077017	0.028676006	SMN del7	30	GM09677	48	qRT-PCR	
10945	SMN1-136 m01	0.41160583	0.10837426	SMN del7	30	GM09677	48	qRT-PCR	
10946	SMN1-137 m01	0.30053439	0.088101006	SMN del7	30	GM09677	48	qRT-PCR	
10947	SMN1-138 m01	0.531192	0.045844587	SMN del7	30	GM09677	48	qRT-PCR	
10948	SMN1-139 m01	2.19972272	0.587358179	SMN del7	30	GM09677	48	qRT-PCR	
10950	SMN1-140 m01	3.10770158	0.595337778	SMN del7	30	GM09677	48	qRT-PCR	
10955	SMN1-141 m01	0.21752532	#DIV/0!	SMN del7	30	GM09677	48	qRT-PCR	
10956	SMN1-142 m01	0.2408982	0.073557085	SMN del7	30	GM09677	48	qRT-PCR	
10957	SMN1-143 m01	1.54825334	0.44563566	SMN del7	30	GM09677	48	qRT-PCR	
10958	SMN1-144 m01	0.33554241	0.168573522	SMN del7	30	GM09677	48	qRT-PCR	
10959	SMN1-145 m01	1.129316	0.278641339	SMN del7	30	GM09677	48	qRT-PCR	
10960	SMN1-146 m01	1.39571103	0.064349924	SMN del7	30	GM09677	48	qRT-PCR	
10961	SMN1-147 m01	2.10475633	0.532797376	SMN del7	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10962	SMN1-148 m01	3.09828675	0.746027783	SMN del7	30	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	0.18047757	0.019763197	SMN del7	30	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	0.44655156	0.091784495	SMN del7	30	GM09677	48	qRT-PCR	
11368	SMN1-150 m01	0.51336937	0.073759546	SMN del7	30	GM09677	48	qRT-PCR	
11369	SMN1-151 m01	0.87553725	0.059695491	SMN del7	30	GM09677	48	qRT-PCR	
11370	SMN1-152 m01	1.73076321	0.35658645	SMN del7	30	GM09677	48	qRT-PCR	
11371	SMN1-153 m01	3.43646534	0.818612424	SMN del7	30	GM09677	48	qRT-PCR	
11372	SMN1-154 m01	3.1089364	0.908728632	SMN del7	30	GM09677	48	qRT-PCR	
11373	SMN1-155 m01	2.78408234	0.975010487	SMN del7	30	GM09677	48	qRT-PCR	
11400	SMN1-156 m01	5.82710399	0.028031838	SMN del7	30	GM09677	48	qRT-PCR	
11401	SMN1-157 m01	1.02236222	0.306023245	SMN del7	30	GM09677	48	qRT-PCR	
11402	SMN1-158 m01	1.88822781	0.370679129	SMN del7	30	GM09677	48	qRT-PCR	
11403	SMN1-159 m01	1.30112817	0.093453016	SMN del7	30	GM09677	48	qRT-PCR	
11404	SMN1-160 m01	1.7757207	0.582587498	SMN del7	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
13096	unc-293 m01	1.50106438	0.23127888	SMN del7	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.05875681	SMN del7	30	GM09677	48	qRTPCR	
11405	SMN1-161 m01	0.81296144	0.053983479	SMN del7	30	GM09677	48	qRTPCR	
11406	SMN1-162 m01	1.11553135	0.035831812	SMN del7	30	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.08349887	0.010709396	SMN del7	30	GM09677	48	qRTPCR	
11394	SMN1-47 m01	3.94789393	0.424479249	SMN del7	30	GM09677	48	qRTPCR	
11381	SMN1-34 m01	0.17890991	0.025617211	SMN del7	30	GM09677	48	qRTPCR	
10952	SMN1-88 m01	0.88130653	0.078767758	SMN del7	30	GM09677	48	qRTPCR	
10953	SMN1-89 m01	0.44782052	0.036112493	SMN del7	30	GM09677	48	qRTPCR	
12369	SMN1-101 m01	0.38247939	0.023518034	SMN del7	30	GM09677	48	qRTPCR	
	EPO-10 m01	0.697491	0.01489629	SMN del7	30	GM09677	48	qRTPCR	
	EPO-18 m01	1.12927792	0.051996253	SMN del7	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.040372479	SMN del7	30	GM09677	48	qRTPCR	
	Ctrl Un	0.98192236	0.096938792	SMN del7	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11442	SMN1-99 m01	0.56668372	0.130759703	SMN Full	30	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	2.33593735	0.128481413	SMN Full	30	GM09677	48	qRT-PCR	
10064	SMN1-163 m01	0.60258819	0.063839935	SMN Full	30	GM09677	48	qRT-PCR	
10076	SMN1-164 m01	0.5764615	0.050710559	SMN Full	30	GM09677	48	qRT-PCR	
10077	SMN1-165 m01	0.79436011	0.110760388	SMN Full	30	GM09677	48	qRT-PCR	
10083	SMN1-166 m01	0.30239894	0.009292662	SMN Full	30	GM09677	48	qRT-PCR	
10085	SMN1-167 m01	0.0703509	0.004505243	SMN Full	30	GM09677	48	qRT-PCR	
10086	SMN1-168 m01	0.82909723	0.067396919	SMN Full	30	GM09677	48	qRT-PCR	
10088	SMN1-169 m01	1.01097645	0.048786742	SMN Full	30	GM09677	48	qRT-PCR	
10089	SMN1-170 m01	0.99116139	0.101796819	SMN Full	30	GM09677	48	qRT-PCR	
10090	SMN1-110 m01	0.62510754	0.063688353	SMN Full	30	GM09677	48	qRT-PCR	
10091	SMN1-111 m01	0.80108355	0.069256097	SMN Full	30	GM09677	48	qRT-PCR	
10092	SMN1-112 m01	0.75091753	0.108949561	SMN Full	30	GM09677	48	qRT-PCR	
10093	SMN1-113 m01	0.80422449	0.062834188	SMN Full	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10094	SMN1-114 m01	0.67514929	0.012132471	SMN Full	30	GM09677	48	qRT-PCR	
10095	SMN1-115 m01	0.73608873	0.106645018	SMN Full	30	GM09677	48	qRT-PCR	
10096	SMN1-116 m01	0.72824982	0.019220631	SMN Full	30	GM09677	48	qRT-PCR	
10097	SMN1-117 m01	1.06599464	0.172119172	SMN Full	30	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	1.96223187	0.066968255	SMN Full	30	GM09677	48	qRT-PCR	
10330	SMN1-118 m01	1.9831033	0.166619361	SMN Full	30	GM09677	48	qRT-PCR	
10331	SMN1-119 m01	1.30302471	0.093813365	SMN Full	30	GM09677	48	qRT-PCR	
10332	SMN1-120 m01	2.01307558	0.496266595	SMN Full	30	GM09677	48	qRT-PCR	
10333	SMN1-121 m01	1.64011344	0.254366293	SMN Full	30	GM09677	48	qRT-PCR	
10334	SMN1-122 m01	2.17003254	0.083439321	SMN Full	30	GM09677	48	qRT-PCR	
10335	SMN1-123 m01	1.65021929	0.425712813	SMN Full	30	GM09677	48	qRT-PCR	
10336	SMN1-124 m01	1.60574511	0.321190592	SMN Full	30	GM09677	48	qRT-PCR	
10340	SMN1-125 m01	1.39712772	0.096998085	SMN Full	30	GM09677	48	qRT-PCR	
10341	SMN1-126 m01	1.62249905	0.162423086	SMN Full	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10342	SMN1-127 m01	0.9156302	0.246317113	SMN Full	30	GM09677	48	qRT-PCR	
10344	SMN1-128 m01	1.39325565	0.163332878	SMN Full	30	GM09677	48	qRT-PCR	
10345	SMN1-129 m01	0.90782165	0.041889678	SMN Full	30	GM09677	48	qRT-PCR	
10346	SMN1-130 m01	1.1217169	0.022147103	SMN Full	30	GM09677	48	qRT-PCR	
13095	unc-232 m12	0.94164842	0.029884959	SMN Full	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.053883803	SMN Full	30	GM09677	48	qRT-PCR	
10347	SMN1-131 m01	0.67222436	0.13236255	SMN Full	30	GM09677	48	qRT-PCR	
10348	SMN1-132 m01	0.74538511	0.00051687	SMN Full	30	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	1.49306782	0.098444041	SMN Full	30	GM09677	48	qRT-PCR	
10942	SMN1-133 m01	0.88441927	0.094008544	SMN Full	30	GM09677	48	qRT-PCR	
10943	SMN1-134 m01	0.79932852	0.076352986	SMN Full	30	GM09677	48	qRT-PCR	
10944	SMN1-135 m01	1.10122606	0.157213622	SMN Full	30	GM09677	48	qRT-PCR	
10945	SMN1-136 m01	1.29688437	0.044072318	SMN Full	30	GM09677	48	qRT-PCR	
10946	SMN1-137 m01	1.79426511	0.238087274	SMN Full	30	GM09677	48	qRT-PCR	
10947	SMN1-138	1.10494227	0.094367636	SMN Full	30	GM09677	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
10948	SMN1-139 m01	0.68886676	0.119063499	SMN Full	30	GM09677	48	qRTPCR	
10950	SMN1-140 m01	1.36511579	0.564973007	SMN Full	30	GM09677	48	qRTPCR	
10955	SMN1-141 m01	2.47823125	0.332315996	SMN Full	30	GM09677	48	qRTPCR	
10956	SMN1-142 m01	3.49121087	0.29170436	SMN Full	30	GM09677	48	qRTPCR	
10957	SMN1-143 m01	2.10894653	0.16868009	SMN Full	30	GM09677	48	qRTPCR	
10958	SMN1-144 m01	2.12580262	0.109200736	SMN Full	30	GM09677	48	qRTPCR	
10959	SMN1-145 m01	1.59635337	0.035935478	SMN Full	30	GM09677	48	qRTPCR	
10960	SMN1-146 m01	0.9430475	0.020357137	SMN Full	30	GM09677	48	qRTPCR	
10961	SMN1-147 m01	2.6993122	0.052253519	SMN Full	30	GM09677	48	qRTPCR	
10962	SMN1-148 m01	0.65648464	0.063211554	SMN Full	30	GM09677	48	qRTPCR	
11384	SMN1-37 m01	2.21468266	0.082942629	SMN Full	30	GM09677	48	qRTPCR	
11367	SMN1-149 m01	1.96496218	0.339897934	SMN Full	30	GM09677	48	qRTPCR	
11368	SMN1-150 m01	1.07739082	0.158777804	SMN Full	30	GM09677	48	qRTPCR	
11369	SMN1-151 m01	1.01792256	0.04774769	SMN Full	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
11370	SMN1-152 m01	0.85548419	0.095903615	SMN Full	30	GM09677	48	qRTPCR	
11371	SMN1-153 m01	0.5714109	0.069065208	SMN Full	30	GM09677	48	qRTPCR	
11372	SMN1-154 m01	0.87841249	0.126993849	SMN Full	30	GM09677	48	qRTPCR	
11373	SMN1-155 m01	0.79024835	0.06455943	SMN Full	30	GM09677	48	qRTPCR	
11400	SMN1-156 m01	0.28615995	0.012813687	SMN Full	30	GM09677	48	qRTPCR	
11401	SMN1-157 m01	1.68376982	0.231762196	SMN Full	30	GM09677	48	qRTPCR	
11402	SMN1-158 m01	0.87025001	0.02018517	SMN Full	30	GM09677	48	qRTPCR	
11403	SMN1-159 m01	0.89725988	0.035306873	SMN Full	30	GM09677	48	qRTPCR	
11404	SMN1-160 m01	0.95691826	0.042228756	SMN Full	30	GM09677	48	qRTPCR	
13096	unc-293 m01	0.85525153	0.032144033	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.048353123	SMN Full	30	GM09677	48	qRTPCR	
11405	SMN1-161 m01	1.43510265	0.03161019	SMN Full	30	GM09677	48	qRTPCR	
11406	SMN1-162 m01	0.88787486	0.018765428	SMN Full	30	GM09677	48	qRTPCR	
13088	SMN1-54 m01	3.40212317	0.308884544	SMN Full	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11394	SMN1-47 m01	0.54281012	0.02769981	SMN Full	30	GM09677	48	qRT-PCR	
11381	SMN1-34 m01	2.37698713	0.052270132	SMN Full	30	GM09677	48	qRT-PCR	
10952	SMN1-88 m01	1.25880913	0.212997068	SMN Full	30	GM09677	48	qRT-PCR	
10953	SMN1-89 m01	1.45023479	0.063669206	SMN Full	30	GM09677	48	qRT-PCR	
12369	SMN1-101 m01	2.03233944	0.230003871	SMN Full	30	GM09677	48	qRT-PCR	
	EPO-10 m01	1.10819777	0.134843995	SMN Full	30	GM09677	48	qRT-PCR	
	EPO-18 m01	1.01478998	0.095729492	SMN Full	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.155131077	SMN Full	30	GM09677	48	qRT-PCR	
	Ctrl Un	1.01440851	0.041840928	SMN Full	30	GM09677	48	qRT-PCR	
11442	SMN1-99 m01	0.90815793	0.015600726	SMN del7	30	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	0.18771169	0.039671544	SMN del7	30	GM09677	48	qRT-PCR	
10064	SMN1-163 m01	0.59630344	0.134484787	SMN del7	30	GM09677	48	qRT-PCR	
10076	SMN1-164 m01	0.14990172	0.0186459	SMN del7	30	GM09677	48	qRT-PCR	
10077	SMN1-165 m01	0.23092313	0.013791047	SMN del7	30	GM09677	48	qRT-PCR	
10083	SMN1-166 m01	1.2629957	0.016833042	SMN del7	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10085	SMN1-167 m01	1.88407833	0.090740749	SMN del7	30	GM09677	48	qRTPCR	
10086	SMN1-168 m01	0.90295882	0.173911573	SMN del7	30	GM09677	48	qRTPCR	
10088	SMN1-169 m01	0.56352156	0.059373079	SMN del7	30	GM09677	48	qRTPCR	
10089	SMN1-170 m01	0.55941156	0.124597504	SMN del7	30	GM09677	48	qRTPCR	
10090	SMN1-110 m01	0.29580326	0.013587154	SMN del7	30	GM09677	48	qRTPCR	
10091	SMN1-111 m01	0.37974329	0.088802592	SMN del7	30	GM09677	48	qRTPCR	
10092	SMN1-112 m01	0.32326084	0.082019393	SMN del7	30	GM09677	48	qRTPCR	
10093	SMN1-113 m01	0.38305034	0.019362531	SMN del7	30	GM09677	48	qRTPCR	
10094	SMN1-114 m01	0.46565068	0.082055011	SMN del7	30	GM09677	48	qRTPCR	
10095	SMN1-115 m01	0.72069429	0.194054875	SMN del7	30	GM09677	48	qRTPCR	
10096	SMN1-116 m01	0.94265473	0.388132923	SMN del7	30	GM09677	48	qRTPCR	
10097	SMN1-117 m01	1.12417997	0.492089989	SMN del7	30	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.32896874	0.015915617	SMN del7	30	GM09677	48	qRTPCR	
10330	SMN1-118 m01	0.47341856	0.120418329	SMN del7	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10331	SMN1-119 m01	1.80156929	0.129459683	SMN del7	30	GM09677	48	qRTPCR	
10332	SMN1-120 m01	0.59521844	0.05633146	SMN del7	30	GM09677	48	qRTPCR	
10333	SMN1-121 m01	1.79753721	0.20936729	SMN del7	30	GM09677	48	qRTPCR	
10334	SMN1-122 m01	1.27535214	0.40962679	SMN del7	30	GM09677	48	qRTPCR	
10335	SMN1-123 m01	0.8129618	0.113069582	SMN del7	30	GM09677	48	qRTPCR	
10336	SMN1-124 m01	1.20651711	0.311522416	SMN del7	30	GM09677	48	qRTPCR	
10340	SMN1-125 m01	0.55134922	0.071049282	SMN del7	30	GM09677	48	qRTPCR	
10341	SMN1-126 m01	0.52936079	0.040573444	SMN del7	30	GM09677	48	qRTPCR	
10342	SMN1-127 m01	0.27288663	0.072421112	SMN del7	30	GM09677	48	qRTPCR	
10344	SMN1-128 m01	0.6471026	0.13547974	SMN del7	30	GM09677	48	qRTPCR	
10345	SMN1-129 m01	0.29566339	0.030275299	SMN del7	30	GM09677	48	qRTPCR	
10346	SMN1-130 m01	0.34085569	0.041496944	SMN del7	30	GM09677	48	qRTPCR	
13095	unc-232 m12	1.06026821	0.101503248	SMN del7	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.052626562	SMN del7	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10347	SMN1-131 m01	0.48068548	0.091741075	SMN del7	30	GM09677	48	qRT-PCR	
10348	SMN1-132 m01	1.03293314	0.149962363	SMN del7	30	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	0.87858958	0.003919376	SMN del7	30	GM09677	48	qRT-PCR	
10942	SMN1-133 m01	2.09761536	0.388430083	SMN del7	30	GM09677	48	qRT-PCR	
10943	SMN1-134 m01	1.89208907	0.211105914	SMN del7	30	GM09677	48	qRT-PCR	
10944	SMN1-135 m01	0.15803006	0.018049924	SMN del7	30	GM09677	48	qRT-PCR	
10945	SMN1-136 m01	0.43083044	0.080705805	SMN del7	30	GM09677	48	qRT-PCR	
10946	SMN1-137 m01	0.41539665	0.052944301	SMN del7	30	GM09677	48	qRT-PCR	
10947	SMN1-138 m01	0.45708389	0.105769782	SMN del7	30	GM09677	48	qRT-PCR	
10948	SMN1-139 m01	2.41401399	0.487998898	SMN del7	30	GM09677	48	qRT-PCR	
10950	SMN1-140 m01	2.76629924	0.131247877	SMN del7	30	GM09677	48	qRT-PCR	
10955	SMN1-141 m01	0.16606492	0.014684689	SMN del7	30	GM09677	48	qRT-PCR	
10956	SMN1-142 m01	0.43445373	0.086640277	SMN del7	30	GM09677	48	qRT-PCR	
10957	SMN1-143 m01	1.40486864	0.412260433	SMN del7	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10958	SMN1-144 m01	0.29510513	0.069351104	SMN del7	30	GM09677	48	qRTPCR	
10959	SMN1-145 m01	3.22311066	1.352082071	SMN del7	30	GM09677	48	qRTPCR	
10960	SMN1-146 m01	1.73097799	0.074486242	SMN del7	30	GM09677	48	qRTPCR	
10961	SMN1-147 m01	3.82106819	0.166029374	SMN del7	30	GM09677	48	qRTPCR	
10962	SMN1-148 m01	3.12332905	0.058447123	SMN del7	30	GM09677	48	qRTPCR	
11384	SMN1-37 m01	0.22008251	0.049019052	SMN del7	30	GM09677	48	qRTPCR	
11367	SMN1-149 m01	0.36883015	0.034172847	SMN del7	30	GM09677	48	qRTPCR	
11368	SMN1-150 m01	0.68779533	0.066526515	SMN del7	30	GM09677	48	qRTPCR	
11369	SMN1-151 m01	0.80645256	0.13493948	SMN del7	30	GM09677	48	qRTPCR	
11370	SMN1-152 m01	1.91641891	0.380475076	SMN del7	30	GM09677	48	qRTPCR	
11371	SMN1-153 m01	1.89734274	0.229673535	SMN del7	30	GM09677	48	qRTPCR	
11372	SMN1-154 m01	2.88289945	0.850000837	SMN del7	30	GM09677	48	qRTPCR	
11373	SMN1-155 m01	2.06374966	0.535967959	SMN del7	30	GM09677	48	qRTPCR	
11400	SMN1-156 m01	4.50803486	0.660771688	SMN del7	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11401	SMN1-157 m01	0.59138591	0.084506813	SMN del7	30	GM09677	48	qRT-PCR	
11402	SMN1-158 m01	3.44851423	0.162086823	SMN del7	30	GM09677	48	qRT-PCR	
11403	SMN1-159 m01	0.91604006	0.205091449	SMN del7	30	GM09677	48	qRT-PCR	
11404	SMN1-160 m01	1.43917921	0.330910692	SMN del7	30	GM09677	48	qRT-PCR	
13096	unc-293 m01	1.31596316	0.687964776	SMN del7	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.015247612	SMN del7	30	GM09677	48	qRT-PCR	
11405	SMN1-161 m01	1.16321433	0.07664856	SMN del7	30	GM09677	48	qRT-PCR	
11406	SMN1-162 m01	1.31795661	0.141433537	SMN del7	30	GM09677	48	qRT-PCR	
13088	SMN1-54 m01	0.09875383	0.002680295	SMN del7	30	GM09677	48	qRT-PCR	
11394	SMN1-47 m01	4.41043278	0.263674611	SMN del7	30	GM09677	48	qRT-PCR	
11381	SMN1-34 m01	0.20423987	0.038880992	SMN del7	30	GM09677	48	qRT-PCR	
10952	SMN1-88 m01	0.90029851	0.269942361	SMN del7	30	GM09677	48	qRT-PCR	
10953	SMN1-89 m01	0.62291818	0.195337362	SMN del7	30	GM09677	48	qRT-PCR	
12369	SMN1-101 m01	0.47034618	0.07574209	SMN del7	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	EPO-10 m01	1.0186228	0.28599999	SMN del7	30	GM09677	48	qRT-PCR	
	EPO-18 m01	1.26596539	0.003509738	SMN del7	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.098475246	SMN del7	30	GM09677	48	qRT-PCR	
	Ctrl Un	1.12059118	0.189105022	SMN del7	30	GM09677	48	qRT-PCR	
11442	SMN1-99 m01	0.811116351	0.043942024	SMN Full	30	GM09677	48	qRT-PCR	
10087	SMN1-77 m01	2.41557556	0.225148224	SMN Full	30	GM09677	48	qRT-PCR	
10064	SMN1-163 m01	0.9775188	0.07225815	SMN Full	30	GM09677	48	qRT-PCR	
10076	SMN1-164 m01	1.64994674	0.10088955	SMN Full	30	GM09677	48	qRT-PCR	
10077	SMN1-165 m01	1.65516628	0.076613667	SMN Full	30	GM09677	48	qRT-PCR	
10083	SMN1-166 m01	0.280387	0.039179606	SMN Full	30	GM09677	48	qRT-PCR	
10085	SMN1-167 m01	0.09710685	0.012720227	SMN Full	30	GM09677	48	qRT-PCR	
10086	SMN1-168 m01	2.27412583	0.11665946	SMN Full	30	GM09677	48	qRT-PCR	
10088	SMN1-169 m01	1.43347874	0.079838776	SMN Full	30	GM09677	48	qRT-PCR	
10089	SMN1-170 m01	2.25368267	0.144447463	SMN Full	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10090	SMN1-110 m01	2.25384849	0.281986031	SMN Full	30	GM09677	48	qRT-PCR	
10091	SMN1-111 m01	2.5154949	0.15391528	SMN Full	30	GM09677	48	qRT-PCR	
10092	SMN1-112 m01	2.38140743	0.224076868	SMN Full	30	GM09677	48	qRT-PCR	
10093	SMN1-113 m01	2.45923426	0.139627423	SMN Full	30	GM09677	48	qRT-PCR	
10094	SMN1-114 m01	1.69856635	0.039572327	SMN Full	30	GM09677	48	qRT-PCR	
10095	SMN1-115 m01	1.25291015	0.080562144	SMN Full	30	GM09677	48	qRT-PCR	
10096	SMN1-116 m01	1.63858874	0.033525426	SMN Full	30	GM09677	48	qRT-PCR	
10097	SMN1-117 m01	1.5475686	0.015749097	SMN Full	30	GM09677	48	qRT-PCR	
10339	SMN1-83 m01	2.32354091	0.234417163	SMN Full	30	GM09677	48	qRT-PCR	
10330	SMN1-118 m01	2.09096912	0.054273984	SMN Full	30	GM09677	48	qRT-PCR	
10331	SMN1-119 m01	1.09354575	0.153522263	SMN Full	30	GM09677	48	qRT-PCR	
10332	SMN1-120 m01	2.64326226	0.266647817	SMN Full	30	GM09677	48	qRT-PCR	
10333	SMN1-121 m01	2.34441965	0.112756145	SMN Full	30	GM09677	48	qRT-PCR	
10334	SMN1-122 m01	3.44824108	0.140134627	SMN Full	30	GM09677	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10335	SMN1-123 m01	3.97144914	0.379111066	SMN Full	30	GM09677	48	qRT-PCR	
10336	SMN1-124 m01	1.25620659	0.041173123	SMN Full	30	GM09677	48	qRT-PCR	
10340	SMN1-125 m01	2.54075069	0.056870834	SMN Full	30	GM09677	48	qRT-PCR	
10341	SMN1-126 m01	2.84972744	0.186858031	SMN Full	30	GM09677	48	qRT-PCR	
10342	SMN1-127 m01	2.38911287	0.370142882	SMN Full	30	GM09677	48	qRT-PCR	
10344	SMN1-128 m01	1.68623322	0.158129572	SMN Full	30	GM09677	48	qRT-PCR	
10345	SMN1-129 m01	1.55104631	0.110750148	SMN Full	30	GM09677	48	qRT-PCR	
10346	SMN1-130 m01	1.72280748	0.071835676	SMN Full	30	GM09677	48	qRT-PCR	
13095	unc-232 m12	1.3622846	0.1048249	SMN Full	30	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.023541051	SMN Full	30	GM09677	48	qRT-PCR	
10347	SMN1-131 m01	1.43138315	0.079806105	SMN Full	30	GM09677	48	qRT-PCR	
10348	SMN1-132 m01	1.38051327	0.017562804	SMN Full	30	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	2.19992665	0.229838671	SMN Full	30	GM09677	48	qRT-PCR	
10942	SMN1-133 m01	0.92197375	0.063349564	SMN Full	30	GM09677	48	qRT-PCR	
10943	SMN1-134	1.19921907	0.198997172	SMN Full	30	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
10944	SMN1-135 m01	2.01663402	0.106303431	SMN Full	30	GM09677	48	qRTPCR	
10945	SMN1-136 m01	2.26018078	0.30314746	SMN Full	30	GM09677	48	qRTPCR	
10946	SMN1-137 m01	2.65637885	0.165745871	SMN Full	30	GM09677	48	qRTPCR	
10947	SMN1-138 m01	1.69393573	0.069285118	SMN Full	30	GM09677	48	qRTPCR	
10948	SMN1-139 m01	1.30428714	0.061433463	SMN Full	30	GM09677	48	qRTPCR	
10950	SMN1-140 m01	1.09324571	0.068839209	SMN Full	30	GM09677	48	qRTPCR	
10955	SMN1-141 m01	7.06609134	0.181594362	SMN Full	30	GM09677	48	qRTPCR	
10956	SMN1-142 m01	14.3604855	0.703316364	SMN Full	30	GM09677	48	qRTPCR	
10957	SMN1-143 m01	5.8876156	0.341543434	SMN Full	30	GM09677	48	qRTPCR	
10958	SMN1-144 m01	8.08283543	0.200138001	SMN Full	30	GM09677	48	qRTPCR	
10959	SMN1-145 m01	2.66558244	0.062818612	SMN Full	30	GM09677	48	qRTPCR	
10960	SMN1-146 m01	1.40619139	0.165341262	SMN Full	30	GM09677	48	qRTPCR	
10961	SMN1-147 m01	3.89543793	0.093295857	SMN Full	30	GM09677	48	qRTPCR	
10962	SMN1-148 m01	0.4939407	0.025903638	SMN Full	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
11384	SMN1-37 m01	5.07783374	0.821261273	SMN Full	30	GM09677	48	qRTPCR	
11367	SMN1-149 m01	3.2759279	0.140918632	SMN Full	30	GM09677	48	qRTPCR	
11368	SMN1-150 m01	1.99439163	0.051735061	SMN Full	30	GM09677	48	qRTPCR	
11369	SMN1-151 m01	1.51324657	0.204363349	SMN Full	30	GM09677	48	qRTPCR	
11370	SMN1-152 m01	1.28515093	0.213530293	SMN Full	30	GM09677	48	qRTPCR	
11371	SMN1-153 m01	1.80402419	0.097005128	SMN Full	30	GM09677	48	qRTPCR	
11372	SMN1-154 m01	1.66039469	0.182107977	SMN Full	30	GM09677	48	qRTPCR	
11373	SMN1-155 m01	1.0305783	0.028870073	SMN Full	30	GM09677	48	qRTPCR	
11400	SMN1-156 m01	0.17467731	0.018816982	SMN Full	30	GM09677	48	qRTPCR	
11401	SMN1-157 m01	3.3333211	0.279671555	SMN Full	30	GM09677	48	qRTPCR	
11402	SMN1-158 m01	1.70623682	0.230457471	SMN Full	30	GM09677	48	qRTPCR	
11403	SMN1-159 m01	1.07973453	0.02394033	SMN Full	30	GM09677	48	qRTPCR	
11404	SMN1-160 m01	1.38457378	0.091844171	SMN Full	30	GM09677	48	qRTPCR	
13096	unc-293	1.08174786	0.007034015	SMN Full	30	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
	Ctrl Lipo	1	0.042985157	SMN Full	30	GM09677	48	qRTPCR	
11405	SMN1-161 m01	2.85069791	1.184249888	SMN Full	30	GM09677	48	qRTPCR	
11406	SMN1-162 m01	1.5457627	0.520076419	SMN Full	30	GM09677	48	qRTPCR	
13088	SMN1-54 m01	3.44721705	0.217271997	SMN Full	30	GM09677	48	qRTPCR	
11394	SMN1-47 m01	0.33368176	0.033438086	SMN Full	30	GM09677	48	qRTPCR	
11381	SMN1-34 m01	2.79916791	0.222774991	SMN Full	30	GM09677	48	qRTPCR	
10952	SMN1-88 m01	2.01106331	0.038778474	SMN Full	30	GM09677	48	qRTPCR	
10953	SMN1-89 m01	2.28953759	0.122706108	SMN Full	30	GM09677	48	qRTPCR	
12369	SMN1-101 m01	2.24524453	0.098703985	SMN Full	30	GM09677	48	qRTPCR	
	EPO-10 m01	1.59339511	0.063413085	SMN Full	30	GM09677	48	qRTPCR	
	EPO-18 m01	1.08061998	0.038047746	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.057269483	SMN Full	30	GM09677	48	qRTPCR	
	Ctrl Un	0.60309537	0.280501847	SMN Full	30	GM09677	48	qRTPCR	
11442	SMN1-99 m01	1.87388944	0.37243506	SMN del7	30	GM09677	48	qRTPCR	
10087	SMN1-77	0.15613676	0.020191111	SMN	30	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
10064	SMN1-163 m01	1.21377997	0.104827003	SMN del7	30	GM09677	48	qRTPCR	
10076	SMN1-164 m01	0.37556027	0.073051472	SMN del7	30	GM09677	48	qRTPCR	
10077	SMN1-165 m01	0.23343008	0.049195687	SMN del7	30	GM09677	48	qRTPCR	
10083	SMN1-166 m01	2.81888694	0.047266025	SMN del7	30	GM09677	48	qRTPCR	
10085	SMN1-167 m01	5.20789891	1.26159287	SMN del7	30	GM09677	48	qRTPCR	
10086	SMN1-168 m01	2.42079005	0.125460855	SMN del7	30	GM09677	48	qRTPCR	
10088	SMN1-169 m01	0.87825483	0.117185888	SMN del7	30	GM09677	48	qRTPCR	
10089	SMN1-170 m01	1.28696876	0.229043004	SMN del7	30	GM09677	48	qRTPCR	
10090	SMN1-110 m01	0.80762379	0.067826812	SMN del7	30	GM09677	48	qRTPCR	
10091	SMN1-111 m01	0.70499238	0.049135469	SMN del7	30	GM09677	48	qRTPCR	
10092	SMN1-112 m01	0.71602044	0.029023227	SMN del7	30	GM09677	48	qRTPCR	
10093	SMN1-113 m01	0.72167379	0.05747778	SMN del7	30	GM09677	48	qRTPCR	
10094	SMN1-114 m01	0.54173539	0.056454125	SMN del7	30	GM09677	48	qRTPCR	
10095	SMN1-115	0.9045253	0.119134128	SMN	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
10096	SMN1-116 m01	1.09420516	0.091762926	SMN del7	30	GM09677	48	qRTPCR	
10097	SMN1-117 m01	0.76736259	0.045471956	SMN del7	30	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.25046283	0.04324439	SMN del7	30	GM09677	48	qRTPCR	
10330	SMN1-118 m01	1.49599415	0.731558802	SMN del7	30	GM09677	48	qRTPCR	
10331	SMN1-119 m01	4.09297711	0.130360987	SMN del7	30	GM09677	48	qRTPCR	
10332	SMN1-120 m01	0.95598958	0.068147527	SMN del7	30	GM09677	48	qRTPCR	
10333	SMN1-121 m01	3.31258736	0.476753361	SMN del7	30	GM09677	48	qRTPCR	
10334	SMN1-122 m01	4.41829307	0.712405663	SMN del7	30	GM09677	48	qRTPCR	
10335	SMN1-123 m01	3.3106985	2.45512246	SMN del7	30	GM09677	48	qRTPCR	
10336	SMN1-124 m01	0.45123328	0.096974509	SMN del7	30	GM09677	48	qRTPCR	
10340	SMN1-125 m01	0.5109367	0.045925287	SMN del7	30	GM09677	48	qRTPCR	
10341	SMN1-126 m01	0.41799945	0.029392592	SMN del7	30	GM09677	48	qRTPCR	
10342	SMN1-127 m01	0.17718727	0.017414179	SMN del7	30	GM09677	48	qRTPCR	
10344	SMN1-128	0.17676491	0.036812101	SMN	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
10345	SMN1-129 m01	0.20328696	0.029935681	SMN del7	30	GM09677	48	qRTPCR	
10346	SMN1-130 m01	0.19866733	0.020140344	SMN del7	30	GM09677	48	qRTPCR	
13095	unc-232 m12	0.951117088	0.133802534	SMN del7	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.08800877	SMN del7	30	GM09677	48	qRTPCR	
10347	SMN1-131 m01	0.31471383	0.012354432	SMN del7	30	GM09677	48	qRTPCR	
10348	SMN1-132 m01	1.09240033	0.058222114	SMN del7	30	GM09677	48	qRTPCR	
10954	SMN1-90 m01	0.86126965	0.082973107	SMN del7	30	GM09677	48	qRTPCR	
10942	SMN1-133 m01	3.07861743	0.405974775	SMN del7	30	GM09677	48	qRTPCR	
10943	SMN1-134 m01	3.14639814	0.398151128	SMN del7	30	GM09677	48	qRTPCR	
10944	SMN1-135 m01	0.1416366	0.041552545	SMN del7	30	GM09677	48	qRTPCR	
10945	SMN1-136 m01	0.09616882	0.018462581	SMN del7	30	GM09677	48	qRTPCR	
10946	SMN1-137 m01	0.06242446	0.017326987	SMN del7	30	GM09677	48	qRTPCR	
10947	SMN1-138 m01	0.24560933	0.009709532	SMN del7	30	GM09677	48	qRTPCR	
10948	SMN1-139	2.42854253	0.778654141	SMN	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
10950	SMN1-140 m01	3.22093612	0.501945741	SMN del7	30	GM09677	48	qRTPCR	
10955	SMN1-141 m01	0.06722355	0.011262807	SMN del7	30	GM09677	48	qRTPCR	
10956	SMN1-142 m01	0.09867146	0.01138516	SMN del7	30	GM09677	48	qRTPCR	
10957	SMN1-143 m01	1.8652232	0.229765838	SMN del7	30	GM09677	48	qRTPCR	
10958	SMN1-144 m01	0.03532186	0.008274233	SMN del7	30	GM09677	48	qRTPCR	
10959	SMN1-145 m01	1.35592654	0.206647516	SMN del7	30	GM09677	48	qRTPCR	
10960	SMN1-146 m01	1.33738603	0.136176114	SMN del7	30	GM09677	48	qRTPCR	
10961	SMN1-147 m01	2.91445776	0.216045923	SMN del7	30	GM09677	48	qRTPCR	
10962	SMN1-148 m01	1.40813148	0.181502547	SMN del7	30	GM09677	48	qRTPCR	
11384	SMN1-37 m01	0.16908965	0.004202113	SMN del7	30	GM09677	48	qRTPCR	
11367	SMN1-149 m01	0.14118625	0.006683874	SMN del7	30	GM09677	48	qRTPCR	
11368	SMN1-150 m01	0.33433773	0.077861084	SMN del7	30	GM09677	48	qRTPCR	
11369	SMN1-151 m01	0.5453545	0.030111245	SMN del7	30	GM09677	48	qRTPCR	
11370	SMN1-152 m01	1.61213427	0.203636633	SMN del7	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
11371	SMN1-153 m01	6.15815871	0.471941252	SMN del7	30	GM09677	48	qRTPCR	
11372	SMN1-154 m01	1.44214064	0.133231148	SMN del7	30	GM09677	48	qRTPCR	
11373	SMN1-155 m01	1.5280276	0.072536876	SMN del7	30	GM09677	48	qRTPCR	
11400	SMN1-156 m01	7.212274	0.329570439	SMN del7	30	GM09677	48	qRTPCR	
11401	SMN1-157 m01	0.59619379	0.028497617	SMN del7	30	GM09677	48	qRTPCR	
11402	SMN1-158 m01	4.35153168	0.177837011	SMN del7	30	GM09677	48	qRTPCR	
11403	SMN1-159 m01	0.60699311	0.076016095	SMN del7	30	GM09677	48	qRTPCR	
11404	SMN1-160 m01	0.9860087	0.099500234	SMN del7	30	GM09677	48	qRTPCR	
13096	unc-293 m01	1.02600158	0.152674045	SMN del7	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.132643444	SMN del7	30	GM09677	48	qRTPCR	
11405	SMN1-161 m01	2.54148147	1.6730075	SMN del7	30	GM09677	48	qRTPCR	
11406	SMN1-162 m01	1.27457213	0.298863328	SMN del7	30	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.06092259	0.016300657	SMN del7	30	GM09677	48	qRTPCR	
11394	SMN1-47	3.99048608	0.77263625	SMN	30	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01			del7					
11381	SMN1-34 m01	0.17598603	0.035243981	SMN del7	30	GM09677	48	qRTPCR	
10952	SMN1-88 m01	0.72163208	0.072324583	SMN del7	30	GM09677	48	qRTPCR	
10953	SMN1-89 m01	0.52061827	0.125327557	SMN del7	30	GM09677	48	qRTPCR	
12369	SMN1-101 m01	0.43391642	0.089047741	SMN del7	30	GM09677	48	qRTPCR	
	EPO-10 m01	0.80549969	0.084094246	SMN del7	30	GM09677	48	qRTPCR	
	EPO-18 m01	1.83383744	0.091072028	SMN del7	30	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.069925643	SMN del7	30	GM09677	48	qRTPCR	
	Ctrl Un	1.62974476	0.83438518	SMN del7	30	GM09677	48	qRTPCR	
11442	SMN1-99 m01	0.74128669	0.013273883	SMN Full	60	GM09677	48	qRTPCR	
10087	SMN1-77 m01	3.20702858	0.3841936	SMN Full	60	GM09677	48	qRTPCR	
10064	SMN1-163 m01	1.23904317	0.33514277	SMN Full	60	GM09677	48	qRTPCR	
10076	SMN1-164 m01	1.12268429	0.037503658	SMN Full	60	GM09677	48	qRTPCR	
10077	SMN1-165 m01	1.41680673	0.121049611	SMN Full	60	GM09677	48	qRTPCR	
10083	SMN1-166 m01	0.25589063	0.013206404	SMN Full	60	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
10085	SMN1-167 m01	0.11669442	0.008014612	SMN Full	60	GM09677	48	qRTPCR	
10086	SMN1-168 m01	5.4110456	1.710962864	SMN Full	60	GM09677	48	qRTPCR	
10088	SMN1-169 m01	1.64446413	0.289444373	SMN Full	60	GM09677	48	qRTPCR	
10089	SMN1-170 m01	1.14561312	0.362969134	SMN Full	60	GM09677	48	qRTPCR	
10090	SMN1-110 m01	7.07077606	4.231239127	SMN Full	60	GM09677	48	qRTPCR	
10091	SMN1-111 m01	1.38713801	0.073075004	SMN Full	60	GM09677	48	qRTPCR	
10092	SMN1-112 m01	1.2265034	0.05426638	SMN Full	60	GM09677	48	qRTPCR	
10093	SMN1-113 m01	1.38674702	0.047680262	SMN Full	60	GM09677	48	qRTPCR	
10094	SMN1-114 m01	1.28163135	0.055764542	SMN Full	60	GM09677	48	qRTPCR	
10095	SMN1-115 m01	1.15251192	0.078238097	SMN Full	60	GM09677	48	qRTPCR	
10096	SMN1-116 m01	1.5619683	0.235392284	SMN Full	60	GM09677	48	qRTPCR	
10097	SMN1-117 m01	2.32926373	1.034069353	SMN Full	60	GM09677	48	qRTPCR	
10339	SMN1-83 m01	5.99822174	0.492134175	SMN Full	60	GM09677	48	qRTPCR	
10330	SMN1-118	1.62778209	0.221839867	SMN Full	60	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
10331	SMN1-119 m01	1.2328502	0.080518975	SMN Full	60	GM09677	48	qRTPCR	
10332	SMN1-120 m01	2.17950185	0.138531676	SMN Full	60	GM09677	48	qRTPCR	
10333	SMN1-121 m01	2.2450465	0.575063477	SMN Full	60	GM09677	48	qRTPCR	
10334	SMN1-122 m01	2.6516627	0.089008315	SMN Full	60	GM09677	48	qRTPCR	
10335	SMN1-123 m01	2.52624189	0.113915113	SMN Full	60	GM09677	48	qRTPCR	
10336	SMN1-124 m01	1.31867981	0.269260446	SMN Full	60	GM09677	48	qRTPCR	
10340	SMN1-125 m01	2.3016125	0.159330638	SMN Full	60	GM09677	48	qRTPCR	
10341	SMN1-126 m01	1.42669932	0.076555315	SMN Full	60	GM09677	48	qRTPCR	
10342	SMN1-127 m01	1.84019478	0.146041491	SMN Full	60	GM09677	48	qRTPCR	
10344	SMN1-128 m01	1.11339649	0.061334325	SMN Full	60	GM09677	48	qRTPCR	
10345	SMN1-129 m01	1.00156421	0.025641704	SMN Full	60	GM09677	48	qRTPCR	
10346	SMN1-130 m01	1.19099781	0.117390823	SMN Full	60	GM09677	48	qRTPCR	
13095	unc-232 m12	1.06180461	0.058261605	SMN Full	60	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.066915331	SMN Full	60	GM09677	48	qRTPCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10347	SMN1-131 m01	1.40329157	0.132940154	SMN Full	60	GM09677	48	qRT-PCR	
10348	SMN1-132 m01	1.17289903	0.026812092	SMN Full	60	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	5.70881901	2.207043013	SMN Full	60	GM09677	48	qRT-PCR	
10942	SMN1-133 m01	1.00946631	0.020654487	SMN Full	60	GM09677	48	qRT-PCR	
10943	SMN1-134 m01	1.08324786	0.017685874	SMN Full	60	GM09677	48	qRT-PCR	
10944	SMN1-135 m01	1.9574364	0.127149784	SMN Full	60	GM09677	48	qRT-PCR	
10945	SMN1-136 m01	2.22127614	0.091628577	SMN Full	60	GM09677	48	qRT-PCR	
10946	SMN1-137 m01	2.5430633	0.100848776	SMN Full	60	GM09677	48	qRT-PCR	
10947	SMN1-138 m01	1.62903696	0.025965241	SMN Full	60	GM09677	48	qRT-PCR	
10948	SMN1-139 m01	1.6574767	0.154818659	SMN Full	60	GM09677	48	qRT-PCR	
10950	SMN1-140 m01	1.04437633	0.047323044	SMN Full	60	GM09677	48	qRT-PCR	
10955	SMN1-141 m01	5.43436346	0.491065425	SMN Full	60	GM09677	48	qRT-PCR	
10956	SMN1-142 m01	14.8681027	0.275698978	SMN Full	60	GM09677	48	qRT-PCR	
10957	SMN1-143 m01	4.92548125	0.830389203	SMN Full	60	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10958	SMN1-144 m01	6.77429795	0.053289887	SMN Full	60	GM09677	48	qRT-PCR	
10959	SMN1-145 m01	2.03484877	0.092430736	SMN Full	60	GM09677	48	qRT-PCR	
10960	SMN1-146 m01	1.19334264	0.031590768	SMN Full	60	GM09677	48	qRT-PCR	
10961	SMN1-147 m01	3.62045461	0.417339116	SMN Full	60	GM09677	48	qRT-PCR	
10962	SMN1-148 m01	0.66721391	0.04938164	SMN Full	60	GM09677	48	qRT-PCR	
11384	SMN1-37 m01	4.53775736	0.205642342	SMN Full	60	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	2.33143995	0.187585868	SMN Full	60	GM09677	48	qRT-PCR	
11368	SMN1-150 m01	1.9816772	0.143994018	SMN Full	60	GM09677	48	qRT-PCR	
11369	SMN1-151 m01	1.21897471	0.004526784	SMN Full	60	GM09677	48	qRT-PCR	
11370	SMN1-152 m01	1.13271313	0.035793987	SMN Full	60	GM09677	48	qRT-PCR	
11371	SMN1-153 m01	1.08423997	0.02408717	SMN Full	60	GM09677	48	qRT-PCR	
11372	SMN1-154 m01	1.25586008	0.033541199	SMN Full	60	GM09677	48	qRT-PCR	
11373	SMN1-155 m01	1.09894107	0.030788234	SMN Full	60	GM09677	48	qRT-PCR	
11400	SMN1-156 m01	0.54295093	0.058706093	SMN Full	60	GM09677	48	qRT-PCR	

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SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11401	SMN1-157 m01	2.76482469	0.220174428	SMN Full	60	GM09677	48	qRTPCR	
11402	SMN1-158 m01	1.41386535	0.077886552	SMN Full	60	GM09677	48	qRTPCR	
11403	SMN1-159 m01	1.46913166	0.149793248	SMN Full	60	GM09677	48	qRTPCR	
11404	SMN1-160 m01	1.43092045	0.037148845	SMN Full	60	GM09677	48	qRTPCR	
13096	unc-293 m01	0.91997668	0.02805883	SMN Full	60	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.030901466	SMN Full	60	GM09677	48	qRTPCR	
11405	SMN1-161 m01	3.23740534	1.005687203	SMN Full	60	GM09677	48	qRTPCR	
11406	SMN1-162 m01	1.2650589	0.300584718	SMN Full	60	GM09677	48	qRTPCR	
13088	SMN1-54 m01	3.48924031	0.805352864	SMN Full	60	GM09677	48	qRTPCR	
11394	SMN1-47 m01	0.2963453	0.018181719	SMN Full	60	GM09677	48	qRTPCR	
11381	SMN1-34 m01	4.46938282	0.152804558	SMN Full	60	GM09677	48	qRTPCR	
10952	SMN1-88 m01	3.64477732	0.627201657	SMN Full	60	GM09677	48	qRTPCR	
10953	SMN1-89 m01	2.77375423	0.0751968	SMN Full	60	GM09677	48	qRTPCR	
12369	SMN1-101 m01	2.23216428	0.067610621	SMN Full	60	GM09677	48	qRTPCR	
	EPO-10	2.80473629	0.28098213	SMN Full	60	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	m01								
	EPO-18 m01	1.29087214	0.03247116	SMN Full	60	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.159438395	SMN Full	60	GM09677	48	qRTPCR	
	Ctrl Un	1.43046436	0.085913492	SMN Full	60	GM09677	48	qRTPCR	
11442	SMN1-99 m01	1.10582561	0.519703281	SMN del7	60	GM09677	48	qRTPCR	
10087	SMN1-77 m01	0.18412136	0.027837589	SMN del7	60	GM09677	48	qRTPCR	
10064	SMN1-163 m01	0.58302062	0.031314351	SMN del7	60	GM09677	48	qRTPCR	
10076	SMN1-164 m01	0.26797262	0.045442132	SMN del7	60	GM09677	48	qRTPCR	
10077	SMN1-165 m01	0.1466259	0.015524987	SMN del7	60	GM09677	48	qRTPCR	
10083	SMN1-166 m01	1.70462486	0.222869951	SMN del7	60	GM09677	48	qRTPCR	
10085	SMN1-167 m01	2.0273409	0.696558388	SMN del7	60	GM09677	48	qRTPCR	
10086	SMN1-168 m01	1.19955703	0.234100212	SMN del7	60	GM09677	48	qRTPCR	
10088	SMN1-169 m01	0.40031917	0.078189504	SMN del7	60	GM09677	48	qRTPCR	
10089	SMN1-170 m01	0.52955859	0.107445859	SMN del7	60	GM09677	48	qRTPCR	
10090	SMN1-110 m01	3.67116068	3.246016875	SMN del7	60	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10091	SMN1-111 m01	0.36475068	0.069915409	SMN del7	60	GM09677	48	qRTPCR	
10092	SMN1-112 m01	0.30938101	0.036873874	SMN del7	60	GM09677	48	qRTPCR	
10093	SMN1-113 m01	0.36457672	0.035880503	SMN del7	60	GM09677	48	qRTPCR	
10094	SMN1-114 m01	0.26908344	0.043691076	SMN del7	60	GM09677	48	qRTPCR	
10095	SMN1-115 m01	0.36418836	0.087382922	SMN del7	60	GM09677	48	qRTPCR	
10096	SMN1-116 m01	1.34194182	0.866353474	SMN del7	60	GM09677	48	qRTPCR	
10097	SMN1-117 m01	0.40832376	0.032327721	SMN del7	60	GM09677	48	qRTPCR	
10339	SMN1-83 m01	0.32029029	0.03352509	SMN del7	60	GM09677	48	qRTPCR	
10330	SMN1-118 m01	0.3336211	0.039955243	SMN del7	60	GM09677	48	qRTPCR	
10331	SMN1-119 m01	1.12990619	0.213125089	SMN del7	60	GM09677	48	qRTPCR	
10332	SMN1-120 m01	0.53518508	0.036815311	SMN del7	60	GM09677	48	qRTPCR	
10333	SMN1-121 m01	0.83358837	0.027742199	SMN del7	60	GM09677	48	qRTPCR	
10334	SMN1-122 m01	1.32282802	0.129049169	SMN del7	60	GM09677	48	qRTPCR	
10335	SMN1-123 m01	0.50333749	0.149844513	SMN del7	60	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10336	SMN1-124 m01	0.211158818	0.039246173	SMN del7	60	GM09677	48	qRT-PCR	
10340	SMN1-125 m01	0.264446968	0.040989218	SMN del7	60	GM09677	48	qRT-PCR	
10341	SMN1-126 m01	0.33048635	0.049295134	SMN del7	60	GM09677	48	qRT-PCR	
10342	SMN1-127 m01	0.15901346	0.030056583	SMN del7	60	GM09677	48	qRT-PCR	
10344	SMN1-128 m01	0.223335443	0.039950358	SMN del7	60	GM09677	48	qRT-PCR	
10345	SMN1-129 m01	0.21288501	0.018182191	SMN del7	60	GM09677	48	qRT-PCR	
10346	SMN1-130 m01	0.13137869	0.009635282	SMN del7	60	GM09677	48	qRT-PCR	
13095	unc-232 m12	0.50040829	0.155826391	SMN del7	60	GM09677	48	qRT-PCR	
	Ctrl Lipo	1	0.286614249	SMN del7	60	GM09677	48	qRT-PCR	
10347	SMN1-131 m01	0.3962837	0.07574928	SMN del7	60	GM09677	48	qRT-PCR	
10348	SMN1-132 m01	0.62169833	0.041472633	SMN del7	60	GM09677	48	qRT-PCR	
10954	SMN1-90 m01	0.77825545	0.069953762	SMN del7	60	GM09677	48	qRT-PCR	
10942	SMN1-133 m01	2.29234237	0.386569692	SMN del7	60	GM09677	48	qRT-PCR	
10943	SMN1-134 m01	1.22600659	0.099476432	SMN del7	60	GM09677	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
10944	SMN1-135 m01	0.20136018	0.021779105	SMN del7	60	GM09677	48	qRTPCR	
10945	SMN1-136 m01	0.22892439	0.040783367	SMN del7	60	GM09677	48	qRTPCR	
10946	SMN1-137 m01	0.46306369	0.144437737	SMN del7	60	GM09677	48	qRTPCR	
10947	SMN1-138 m01	1.20132677	0.868689734	SMN del7	60	GM09677	48	qRTPCR	
10948	SMN1-139 m01	28.7785895	15.39702199	SMN del7	60	GM09677	48	qRTPCR	
10950	SMN1-140 m01	7.40686537	6.327122769	SMN del7	60	GM09677	48	qRTPCR	
10955	SMN1-141 m01	1.3969898	1.153412167	SMN del7	60	GM09677	48	qRTPCR	
10956	SMN1-142 m01	0.2034766	0.044803581	SMN del7	60	GM09677	48	qRTPCR	
10957	SMN1-143 m01	1.55675257	0.246106153	SMN del7	60	GM09677	48	qRTPCR	
10958	SMN1-144 m01	0.31959667	0.057419906	SMN del7	60	GM09677	48	qRTPCR	
10959	SMN1-145 m01	2.02943876	0.842900636	SMN del7	60	GM09677	48	qRTPCR	
10960	SMN1-146 m01	1.41870118	0.579100078	SMN del7	60	GM09677	48	qRTPCR	
10961	SMN1-147 m01	1.69718888	0.344963638	SMN del7	60	GM09677	48	qRTPCR	
10962	SMN1-148 m01	3.47280353	2.094705579	SMN del7	60	GM09677	48	qRTPCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
11384	SMN1-37 m01	0.15183315	0.027066093	SMN del7	60	GM09677	48	qRT-PCR	
11367	SMN1-149 m01	0.27422566	0.02378103	SMN del7	60	GM09677	48	qRT-PCR	
11368	SMN1-150 m01	0.42987103	0.118142251	SMN del7	60	GM09677	48	qRT-PCR	
11369	SMN1-151 m01	0.80330607	0.188450903	SMN del7	60	GM09677	48	qRT-PCR	
11370	SMN1-152 m01	1.00958066	0.061820097	SMN del7	60	GM09677	48	qRT-PCR	
11371	SMN1-153 m01	4.24521963	0.907982904	SMN del7	60	GM09677	48	qRT-PCR	
11372	SMN1-154 m01	14.0987349	11.49720462	SMN del7	60	GM09677	48	qRT-PCR	
11373	SMN1-155 m01	1.81780758	0.623207783	SMN del7	60	GM09677	48	qRT-PCR	
11400	SMN1-156 m01	8.8357819	3.147002334	SMN del7	60	GM09677	48	qRT-PCR	
11401	SMN1-157 m01	0.69824047	0.273668375	SMN del7	60	GM09677	48	qRT-PCR	
11402	SMN1-158 m01	2.77739744	0.184778945	SMN del7	60	GM09677	48	qRT-PCR	
11403	SMN1-159 m01	1.01575871	0.600775173	SMN del7	60	GM09677	48	qRT-PCR	
11404	SMN1-160 m01	0.86749256	0.052724981	SMN del7	60	GM09677	48	qRT-PCR	
13096	unc-293 m01	1.59597031	0.802672625	SMN del7	60	GM09677	48	qRT-PCR	

Appendix A: Table 5

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Target	[oligo]	cell line	Time (hr)	Assay Type	Note
	Ctrl Lipo	1	0.307492364	SMN del7	60	GM09677	48	qRTPCR	
11405	SMN1-161 m01	5.68787938	4.344059239	SMN del7	60	GM09677	48	qRTPCR	
11406	SMN1-162 m01	1.26053035	0.256436858	SMN del7	60	GM09677	48	qRTPCR	
13088	SMN1-54 m01	0.16374355	0.014592044	SMN del7	60	GM09677	48	qRTPCR	
11394	SMN1-47 m01	8.43332406	1.18745211	SMN del7	60	GM09677	48	qRTPCR	
11381	SMN1-34 m01	0.16471233	0.018640468	SMN del7	60	GM09677	48	qRTPCR	
10952	SMN1-88 m01	1.01641307	0.223670999	SMN del7	60	GM09677	48	qRTPCR	
10953	SMN1-89 m01	0.4665284	0.041017918	SMN del7	60	GM09677	48	qRTPCR	
12369	SMN1-101 m01	0.4183577	0.059059361	SMN del7	60	GM09677	48	qRTPCR	
	EPO-10 m01	0.9212779	0.312413391	SMN del7	60	GM09677	48	qRTPCR	
	EPO-18 m01	2.39878515	0.321417519	SMN del7	60	GM09677	48	qRTPCR	
	Ctrl Lipo	1	0.288265988	SMN del7	60	GM09677	48	qRTPCR	
	Ctrl Un	1.86825265	0.250098757	SMN del7	60	GM09677	48	qRTPCR	

[illegible]

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	Ctrl Un	0.948 90886	0.051 92626	SMN	0	GM03813	qRTPCR	5aza-rC	10	uM			
	Ctrl Un	1.036 7145	0.013 3192	SMN	0	GM03813	qRTPCR	5aza-rC	5	uM			
	Ctrl Un	1.068 04716	0.029 53011	SMN	0	GM03813	qRTPCR	5aza-rC	1	uM			
	Ctrl Un	1.101 91704	0.013 42905	SMN	0	GM03813	qRTPCR	5aza-rC	0.5	uM			
	Ctrl Un	1.036 33028	0.017 91786	SMN	0	GM03813	qRTPCR	5aza-rC	0.1	uM			
	Ctrl Un	1.054 10312	0.021 08369	SMN	0	GM03813	qRTPCR	5aza-rC	0.05	uM			
	Ctrl Un	1.133 79147	0.027 47487	SMN	0	GM03813	qRTPCR	5aza-rC	0.01	uM			
13088	SMN1- 53 m03	3.145 14441	0.098 38557	SMN	30	GM03813	qRTPCR	5aza-rC	10	uM			
13088	SMN1- 53 m03	2.913 78997	0.108 13399	SMN	30	GM03813	qRTPCR	5aza-rC	5	uM			
13088	SMN1- 53 m03	2.929 62926	0.082 60546	SMN	30	GM03813	qRTPCR	5aza-rC	1	uM			
13088	SMN1- 53 m03	2.986 85716	0.095 41197	SMN	30	GM03813	qRTPCR	5aza-rC	0.5	uM			
13088	SMN1- 53 m03	3.096 50047	0.111 35157	SMN	30	GM03813	qRTPCR	5aza-rC	0.1	uM			
13088	SMN1- 53 m03	2.956 32332	0.079 5303	SMN	30	GM03813	qRTPCR	5aza-rC	0.05	uM			
13088	SMN1-	2.752	0.094	SMN	30	GM03813	qRTPCR	5aza-rC	0.01	uM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	53 m03	70767	1681										
13088	SMN1-53 m03	2.674 12135	0.140 86231	SMN	30	GM03813	qRTPCR	5aza-rC	0	uM			
10087	SMN1-77 m01	1.923 08	0.058 60922	SMN	30	GM03813	qRTPCR	5aza-rC	10	uM			
10087	SMN1-77 m01	1.734 7136	0.035 0468	SMN	30	GM03813	qRTPCR	5aza-rC	5	uM			
10087	SMN1-77 m01	1.601 99088	0.012 42694	SMN	30	GM03813	qRTPCR	5aza-rC	1	uM			
10087	SMN1-77 m01	1.547 27667	0.051 36152	SMN	30	GM03813	qRTPCR	5aza-rC	0.5	uM			
10087	SMN1-77 m01	1.470 60312	0.039 537	SMN	30	GM03813	qRTPCR	5aza-rC	0.1	uM			
10087	SMN1-77 m01	1.423 88256	0.018 14721	SMN	30	GM03813	qRTPCR	5aza-rC	0.05	uM			
10087	SMN1-77 m01	1.380 9276	0.043 76613	SMN	30	GM03813	qRTPCR	5aza-rC	0.01	uM			
10087	SMN1-77 m01	1.335 20497	0.085 26542	SMN	30	GM03813	qRTPCR	5aza-rC	0	uM			
11419	SMN1-93 m01	1.662 37696	0.091 82698	SMN	30	GM03813	qRTPCR	5aza-rC	10	uM			
11419	SMN1-93 m01	1.517 83277	0.060 66192	SMN	30	GM03813	qRTPCR	5aza-rC	5	uM			
11419	SMN1-93 m01	1.567 98279	0.111 06937	SMN	30	GM03813	qRTPCR	5aza-rC	1	uM			
11419	SMN1-93 m01	1.510 0183	0.061 11726	SMN	30	GM03813	qRTPCR	5aza-rC	0.5	uM			
11419	SMN1-	1.572	0.097	SMN	30	GM03813	qRTPCR	5aza-rC	0.1	uM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	93 m01	37056	19743										
11419	SMN1-93 m01	1.616 19115	0.087 16888	SMN	30	GM03813	qRTPCR	5aza-rC	0.05	uM			
11419	SMN1-93 m01	1.616 79854	0.039 42636	SMN	30	GM03813	qRTPCR	5aza-rC	0.01	uM			
11419	SMN1-93 m01	1.512 41028	0.116 01773	SMN	30	GM03813	qRTPCR	5aza-rC	0	uM			
	Ctrl Un	0.986 54472	0.052 00244	SMN	0	GM03813	qRTPCR	5aza-rC	0	uM			
	Ctrl Un	1.121 60994	0.024 84253	SMN	0	GM03813	qRTPCR	5aza-dC	10	uM			
	Ctrl Un	0.960 0307	0.025 27686	SMN	0	GM03813	qRTPCR	5aza-dC	5	uM			
	Ctrl Un	0.999 99154	0.023 00838	SMN	0	GM03813	qRTPCR	5aza-dC	1	uM			
	Ctrl Un	0.928 55326	0.042 57254	SMN	0	GM03813	qRTPCR	5aza-dC	0.5	uM			
	Ctrl Un	0.943 97056	0.055 23091	SMN	0	GM03813	qRTPCR	5aza-dC	0.1	uM			
	Ctrl Un	0.953 32744	0.024 37269	SMN	0	GM03813	qRTPCR	5aza-dC	0.05	uM			
	Ctrl Un	0.975 92365	0.028 89654	SMN	0	GM03813	qRTPCR	5aza-dC	0.01	uM			
13088	SMN1-53 m03	3.849 0588	0.174 16396	SMN	30	GM03813	qRTPCR	5aza-dC	10	uM			
13088	SMN1-53 m03	3.453 46941	0.157 62832	SMN	30	GM03813	qRTPCR	5aza-dC	5	uM			
13088	SMN1-	3.082	0.031	SMN	30	GM03813	qRTPCR	5aza-dC	1	uM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	53 m03	44854	49394										
13088	SMN1-53 m03	3.070 40276	0.154 52073	SMN	30	GM03813	qRTPCR	5aza-dC	0.5	uM			
13088	SMN1-53 m03	2.800 31292	0.045 05611	SMN	30	GM03813	qRTPCR	5aza-dC	0.1	uM			
13088	SMN1-53 m03	2.751 166	0.014 13941	SMN	30	GM03813	qRTPCR	5aza-dC	0.05	uM			
13088	SMN1-53 m03	2.734 46856	0.110 11864	SMN	30	GM03813	qRTPCR	5aza-dC	0.01	uM			
13088	SMN1-53 m03	2.674 53466	0.066 64967	SMN	30	GM03813	qRTPCR	5aza-dC	0	uM			
10087	SMN1-77 m01	2.352 76348	0.051 75939	SMN	30	GM03813	qRTPCR	5aza-dC	10	uM			
10087	SMN1-77 m01	2.148 53422	0.030 33785	SMN	30	GM03813	qRTPCR	5aza-dC	5	uM			
10087	SMN1-77 m01	2.007 35209	0.058 1863	SMN	30	GM03813	qRTPCR	5aza-dC	1	uM			
10087	SMN1-77 m01	1.862 13013	0.076 25986	SMN	30	GM03813	qRTPCR	5aza-dC	0.5	uM			
10087	SMN1-77 m01	1.780 97639	0.060 66029	SMN	30	GM03813	qRTPCR	5aza-dC	0.1	uM			
10087	SMN1-77 m01	1.812 05411	0.104 94238	SMN	30	GM03813	qRTPCR	5aza-dC	0.05	uM			
10087	SMN1-77 m01	1.740 27757	0.070 29565	SMN	30	GM03813	qRTPCR	5aza-dC	0.01	uM			
10087	SMN1-77 m01	1.525 95591	0.046 80201	SMN	30	GM03813	qRTPCR	5aza-dC	0	uM			
11419	SMN1-	2.117	0.176	SMN	30	GM03813	qRTPCR	5aza-dC	10	uM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	93 m01	63113	73471										
11419	SMN1-93 m01	1.849 13941	0.179 42183	SMN	30	GM03813	qRTPCR	5aza-dC	5	uM			
11419	SMN1-93 m01	1.642 97194	0.095 03467	SMN	30	GM03813	qRTPCR	5aza-dC	1	uM			
11419	SMN1-93 m01	1.622 61112	0.055 8071	SMN	30	GM03813	qRTPCR	5aza-dC	0.5	uM			
11419	SMN1-93 m01	1.538 81623	0.042 6226	SMN	30	GM03813	qRTPCR	5aza-dC	0.1	uM			
11419	SMN1-93 m01	1.514 48822	0.046 18763	SMN	30	GM03813	qRTPCR	5aza-dC	0.05	uM			
11419	SMN1-93 m01	1.681 85394	0.011 0823	SMN	30	GM03813	qRTPCR	5aza-dC	0.01	uM			
11419	SMN1-93 m01	1.664 11353	0.019 52251	SMN del7	30	GM03813	qRTPCR	5aza-dC	0	uM			
	Ctrl Un	1.013 45528	0.038 4336	SMN del7	0	GM03813	qRTPCR	5aza-dC	0	uM			
	Ctrl Un	1.115 36211	0.077 95413	SMN del7	0	GM03813	qRTPCR	5aza-rC	10	uM			
	Ctrl Un	1.152 22619	0.027 23302	SMN del7	0	GM03813	qRTPCR	5aza-rC	5	uM			
	Ctrl Un	1.186 51886	0.066 97689	SMN del7	0	GM03813	qRTPCR	5aza-rC	1	uM			
	Ctrl Un	1.131 32797	0.057 85634	SMN del7	0	GM03813	qRTPCR	5aza-rC	0.5	uM			
	Ctrl Un	1.076 72231	0.097 25993	SMN del7	0	GM03813	qRTPCR	5aza-rC	0.1	uM			
	Ctrl Un	1.156	0.033	SMN	0	GM03813	qRTPCR	5aza-rC	0.05	uM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
		33867	648	del7									
	Ctrl Un	1.172 93905	0.004 34664	SMN del7	0	GM03813	qRTPCR	5aza-rC	0.01	uM			
13088	SMN1- 53 m03	0.010 023	0.002 64294	SMN del7	30	GM03813	qRTPCR	5aza-rC	10	uM			
13088	SMN1- 53 m03	0.006 85641	0.002 87858	SMN del7	30	GM03813	qRTPCR	5aza-rC	5	uM			
13088	SMN1- 53 m03	0.015 23506	0.003 97769	SMN del7	30	GM03813	qRTPCR	5aza-rC	1	uM			
13088	SMN1- 53 m03	0.009 51193	0.001 69088	SMN del7	30	GM03813	qRTPCR	5aza-rC	0.5	uM			
13088	SMN1- 53 m03	0.014 34478	0.004 07735	SMN del7	30	GM03813	qRTPCR	5aza-rC	0.1	uM			
13088	SMN1- 53 m03	0.009 3286	0.001 15618	SMN del7	30	GM03813	qRTPCR	5aza-rC	0.05	uM			
13088	SMN1- 53 m03	0.007 77325	0.001 77723	SMN del7	30	GM03813	qRTPCR	5aza-rC	0.01	uM			
13088	SMN1- 53 m03	0.009 57709	0.001 46883	SMN del7	30	GM03813	qRTPCR	5aza-rC	0	uM			
10087	SMN1- 77 m01	0.022 24619	0.004 77737	SMN del7	30	GM03813	qRTPCR	5aza-rC	10	uM			
10087	SMN1- 77 m01	0.019 85308	0.003 03346	SMN del7	30	GM03813	qRTPCR	5aza-rC	5	uM			
10087	SMN1- 77 m01	0.015 48537	0.002 25689	SMN del7	30	GM03813	qRTPCR	5aza-rC	1	uM			
10087	SMN1- 77 m01	0.013 55173	0.001 77769	SMN del7	30	GM03813	qRTPCR	5aza-rC	0.5	uM			
10087	SMN1- 77 m01	0.016	0.001	SMN	30	GM03813	qRTPCR	5aza-rC	0.1	uM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	77 m01	31313	34399	del7									
10087	SMN1-77 m01	0.015 19774	0.001 69323	SMN del7	30	GM03813	qRTPCR	5aza-rC	0.05	uM			
10087	SMN1-77 m01	0.013 15108	0.000 58046	SMN del7	30	GM03813	qRTPCR	5aza-rC	0.01	uM			
10087	SMN1-77 m01	0.016 91304	0.001 73442	SMN del7	30	GM03813	qRTPCR	5aza-rC	0	uM			
11419	SMN1-93 m01	0.086 70607	0.011 69335	SMN del7	30	GM03813	qRTPCR	5aza-rC	10	uM			
11419	SMN1-93 m01	0.096 31891	0.000 77132	SMN del7	30	GM03813	qRTPCR	5aza-rC	5	uM			
11419	SMN1-93 m01	0.101 42297	0.002 90946	SMN del7	30	GM03813	qRTPCR	5aza-rC	1	uM			
11419	SMN1-93 m01	0.091 51616	0.002 19793	SMN del7	30	GM03813	qRTPCR	5aza-rC	0.5	uM			
11419	SMN1-93 m01	0.099 18084	0.005 02672	SMN del7	30	GM03813	qRTPCR	5aza-rC	0.1	uM			
11419	SMN1-93 m01	0.110 94903	0.002 39292	SMN del7	30	GM03813	qRTPCR	5aza-rC	0.05	uM			
11419	SMN1-93 m01	0.106 60837	0.000 87275	SMN del7	30	GM03813	qRTPCR	5aza-rC	0.01	uM			
11419	SMN1-93 m01	0.100 53646	0.007 62819	SMN del7	30	GM03813	qRTPCR	5aza-rC	0	uM			
	Ctrl Un	1.059 30308	0.039 64601	SMN del7	0	GM03813	qRTPCR	5aza-rC	0	uM			
	Ctrl Un	1.190 23699	0.069 73989	SMN del7	0	GM03813	qRTPCR	5aza-dC	10	uM			
	Ctrl Un	0.969	0.070	SMN	0	GM03813	qRTPCR	5aza-dC	5	uM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
		51765	99994	del7									
	Ctrl Un	0.975 40833	0.028 55834	SMN del7	0	GM03813	qRTPCR	5aza-dC	1	uM			
	Ctrl Un	0.905 35152	0.139 76718	SMN del7	0	GM03813	qRTPCR	5aza-dC	0.5	uM			
	Ctrl Un	0.864 9919	0.119 5257	SMN del7	0	GM03813	qRTPCR	5aza-dC	0.1	uM			
	Ctrl Un	0.906 58476	0.033 94592	SMN del7	0	GM03813	qRTPCR	5aza-dC	0.05	uM			
	Ctrl Un	0.963 90519	0.056 6844	SMN del7	0	GM03813	qRTPCR	5aza-dC	0.01	uM			
13088	SMN1- 53 m03	0.012 49915	0.002 20564	SMN del7	30	GM03813	qRTPCR	5aza-dC	10	uM			
13088	SMN1- 53 m03	0.009 90022	0.001 58222	SMN del7	30	GM03813	qRTPCR	5aza-dC	5	uM			
13088	SMN1- 53 m03	0.010 55991	0.003 16607	SMN del7	30	GM03813	qRTPCR	5aza-dC	1	uM			
13088	SMN1- 53 m03	0.010 33609	0.001 93316	SMN del7	30	GM03813	qRTPCR	5aza-dC	0.5	uM			
13088	SMN1- 53 m03	0.006 26954	0.001 11029	SMN del7	30	GM03813	qRTPCR	5aza-dC	0.1	uM			
13088	SMN1- 53 m03	0.008 18259	0.001 90283	SMN del7	30	GM03813	qRTPCR	5aza-dC	0.05	uM			
13088	SMN1- 53 m03	0.010 57994	0.002 59775	SMN del7	30	GM03813	qRTPCR	5aza-dC	0.01	uM			
13088	SMN1- 53 m03	0.009 13725	0.001 28232	SMN del7	30	GM03813	qRTPCR	5aza-dC	0	uM			
10087	SMN1-	0.018	0.000	SMN	30	GM03813	qRTPCR	5aza-dC	10	uM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	77 m01	68683	40091	del7									
10087	SMN1-77 m01	0.016	0.001	SMN del7	30	GM03813	qRTPCR	5aza-dC	5	uM			
10087	SMN1-77 m01	56083	16899	del7									
10087	SMN1-77 m01	0.014	0.000	SMN del7	30	GM03813	qRTPCR	5aza-dC	1	uM			
10087	SMN1-77 m01	52977	5516	del7									
10087	SMN1-77 m01	0.014	0.001	SMN del7	30	GM03813	qRTPCR	5aza-dC	0.5	uM			
10087	SMN1-77 m01	90873	31616	del7									
10087	SMN1-77 m01	0.015	0.001	SMN del7	30	GM03813	qRTPCR	5aza-dC	0.1	uM			
10087	SMN1-77 m01	64309	64242	del7									
10087	SMN1-77 m01	0.016	0.002	SMN del7	30	GM03813	qRTPCR	5aza-dC	0.05	uM			
10087	SMN1-77 m01	77367	26877	del7									
10087	SMN1-77 m01	0.013	0.000	SMN del7	30	GM03813	qRTPCR	5aza-dC	0.01	uM			
10087	SMN1-77 m01	48978	73481	del7									
10087	SMN1-77 m01	0.013	0.001	SMN del7	30	GM03813	qRTPCR	5aza-dC	0	uM			
10087	SMN1-77 m01	57098	64376	del7									
11419	SMN1-93 m01	0.146	0.009	SMN del7	30	GM03813	qRTPCR	5aza-dC	10	uM			
11419	SMN1-93 m01	40808	04035	del7									
11419	SMN1-93 m01	0.144	0.007	SMN del7	30	GM03813	qRTPCR	5aza-dC	5	uM			
11419	SMN1-93 m01	62817	81638	del7									
11419	SMN1-93 m01	0.117	0.000	SMN del7	30	GM03813	qRTPCR	5aza-dC	1	uM			
11419	SMN1-93 m01	3942	88146	del7									
11419	SMN1-93 m01	0.122	0.005	SMN del7	30	GM03813	qRTPCR	5aza-dC	0.5	uM			
11419	SMN1-93 m01	22578	37149	del7									
11419	SMN1-93 m01	0.108	0.004	SMN del7	30	GM03813	qRTPCR	5aza-dC	0.1	uM			
11419	SMN1-93 m01	96272	63692	del7									
11419	SMN1-93 m01	0.122	0.009	SMN del7	30	GM03813	qRTPCR	5aza-dC	0.05	uM			
11419	SMN1-93 m01	65829	30432	del7									
11419	SMN1-93 m01	0.118	0.003	SMN del7	30	GM03813	qRTPCR	5aza-dC	0.01	uM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	93 m01	74495	42997	del7									
11419	SMN1-93 m01	0.097	0.003	SMN del7	30	GM03813	qRTPCR	5aza-dC	0	uM			
	Ctrl Un	0.940	0.050	SMN del7	0	GM03813	qRTPCR	5aza-dC	0	uM			
11394	SMN1-47 m01	1.773	0.056	SMN	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11395	SMN1-48 m01	1.212	0.038	SMN	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11394	SMN1-47 m01	1.629	0.007	SMN	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
11395	SMN1-48 m01	1.309	0.062	SMN	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
11394	SMN1-47 m01	1.015	0.014	SMN	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
11395	SMN1-48 m01	1.034	0.014	SMN	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
13088	SMN1-53 m03	1.939	0.096	SMN	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
13088	SMN1-54 m01	1.715	0.035	SMN	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
10169	SMN1-79 m01	0.575	0.026	SMN	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
10170	SMN1-80 m01	0.334	0.009	SMN	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
10763	SMN1-84 m01	0.551	0.049	SMN	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
11394	SMN1-	0.483	0.025	SMN	30	GM09677	qRTPCR	SMN1-57	30	nM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	47 m01	01739	34669					m01					
11395	SMN1-48 m01	0.757 3052	0.031 78786	SMN	30	GM09677	qRTPCR	SMN1-57 m01	30	nM			
10169	SMN1-79 m01	0.302 62071	0.026 14779	SMN	30	GM09677	qRTPCR	SMN1-57 m01	30	nM			
10170	SMN1-80 m01	0.241 53527	0.022 02247	SMN	30	GM09677	qRTPCR	SMN1-57 m01	30	nM			
10763	SMN1-84 m01	0.597 49334	0.026 84332	SMN	30	GM09677	qRTPCR	SMN1-57 m01	30	nM			
11394	SMN1-47 m01	0.051 09188	0.006 19445	SMN del7	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11395	SMN1-48 m01	0.030 24995	0.006 38187	SMN del7	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11394	SMN1-47 m01	0.045 32971	0.004 17238	SMN del7	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
11395	SMN1-48 m01	0.085 27683	0.008 1459	SMN del7	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
11394	SMN1-47 m01	2.238 4051	0.108 56948	SMN del7	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
11395	SMN1-48 m01	0.795 30003	0.024 73036	SMN del7	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
13088	SMN1-53 m03	0.108 05536	0.012 12223	SMN del7	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
13088	SMN1-54 m01	0.119 32989	0.005 32665	SMN del7	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
10169	SMN1-79 m01	1.732 83517	0.171 05216	SMN del7	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
10170	SMN1-	2.006	0.100	SMN	30	GM09677	qRTPCR	SMN1-93	30	nM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	80 m01	88333	99911	del7				m01					
10763	SMN1-84 m01	2.753 54608	0.237 98447	SMN del7	30	GM09677	qRTPCR	SMN1-93 m01	30	nM			
11394	SMN1-47 m01	2.155 01895	0.077 27011	SMN del7	30	GM09677	qRTPCR	SMN1-57 m01	30	nM			
11395	SMN1-48 m01	1.298 36689	0.120 39448	SMN del7	30	GM09677	qRTPCR	SMN1-57 m01	30	nM			
10169	SMN1-79 m01	4.319 80421	0.149 86525	SMN del7	30	GM09677	qRTPCR	SMN1-57 m01	30	nM			
10170	SMN1-80 m01	5.174 12191	0.036 79871	SMN del7	30	GM09677	qRTPCR	SMN1-57 m01	30	nM			
10763	SMN1-84 m01	3.231 33015	0.098 14992	SMN del7	30	GM09677	qRTPCR	SMN1-57 m01	30	nM			
11394	SMN1-47 m01	2.016 2316	0.175 79357	SMN	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11395	SMN1-48 m01	1.707 79592	0.160 76092	SMN	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11384	SMN1-37 m01	2.130 8672	0.285 86252	SMN	30	GM09677	qRTPCR	SMN1-77 m01	30	nM			
11384	SMN1-37 m01	3.859 51505	1.021 65704	SMN	30	GM09677	qRTPCR	SMN1-90 m01	30	nM			
10087	SMN1-77 m01	2.656 91646	0.263 87337	SMN	30	GM09677	qRTPCR	SMN1-90 m01	30	nM			
11384	SMN1-37 m01	2.655 20142	0.125 96498	SMN	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM
11384	SMN1-37 m01	1.443 75777	0.309 07655	SMN	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM
10087	SMN1-	1.504	0.261	SMN	30	GM09677	qRTPCR	SMN1-47	30	nM	SMN1-53	30	nM

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	77 m01	42187	31663					m01			m03		
10087	SMN1-77 m01	1.379 13168	0.220 39405	SMN	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM
10954	SMN1-90 m01	2.179 58526	0.374 48891	SMN	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM
10954	SMN1-90 m01	1.857 11526	0.327 94008	SMN	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM
11394	SMN1-47 m01	1.633 28715	0.261 26992	SMN	30	GM09677	qRTPCR	SMN1-53 m03	30	nM	unc-293 m01	30	nM
11395	SMN1-48 m01	1.569 15446	0.116 72019	SMN	30	GM09677	qRTPCR	SMN1-53 m03	30	nM	unc-293 m01	30	nM
11394	SMN1-47 m01	0.018 35766	0.007 42043	SMN del7	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11395	SMN1-48 m01	0.009 19298	0.003 84953	SMN del7	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11384	SMN1-37 m01	0.033 96535	0.009 86471	SMN del7	30	GM09677	qRTPCR	SMN1-77 m01	30	nM			
11384	SMN1-37 m01	0.082 33201	0.015 38199	SMN del7	30	GM09677	qRTPCR	SMN1-90 m01	30	nM			
10087	SMN1-77 m01	0.063 83505	0.021 23364	SMN del7	30	GM09677	qRTPCR	SMN1-90 m01	30	nM			
11384	SMN1-37 m01	0.020 22004	0.001 08701	SMN del7	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM
11384	SMN1-37 m01	0.009 75344	0.003 13602	SMN del7	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM
10087	SMN1-77 m01	0.008 37303	0.000 16767	SMN del7	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM
10087	SMN1-	0.010	0.003	SMN	30	GM09677	qRTPCR	SMN1-48	30	nM	SMN1-53	30	nM

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	77 m01	61265	16924	del7				m01			m03		
10954	SMN1-90 m01	0.025 08374	0.002 78869	SMN del7	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM
10954	SMN1-90 m01	0.009 16924	0.002 15956	SMN del7	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM
11394	SMN1-47 m01	0.018 09811	0.004 96135	SMN del7	30	GM09677	qRTPCR	SMN1-53 m03	30	nM	unc-293 m01	30	nM
11395	SMN1-48 m01	0.013 15026	0.004 05211	SMN del7	30	GM09677	qRTPCR	SMN1-53 m03	30	nM	unc-293 m01	30	nM
11384	SMN1-37 m01	4.145 99747	0.075 97496	SMN	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11394	SMN1-47 m01	2.427 13841	0.020 8461	SMN	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11395	SMN1-48 m01	2.536 86027	0.047 58036	SMN	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
10087	SMN1-77 m01	3.961 94388	0.066 14935	SMN	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
10339	SMN1-83 m01	4.343 67865	0.021 53163	SMN	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
10954	SMN1-90 m01	5.252 75132	0.170 50622	SMN	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11384	SMN1-37 m01	3.710 77913	0.123 51431	SMN	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
11394	SMN1-47 m01	2.232 99977	0.029 11702	SMN	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
11395	SMN1-48 m01	2.110 56838	0.037 5565	SMN	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
10087	SMN1-	4.093	0.116	SMN	30	GM09677	qRTPCR	SMN1-54	30	nM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	77 m01	89564	75101					m01					
10339	SMN1-83 m01	3.596	0.068	SMN	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
10954	SMN1-90 m01	3.756	0.232	SMN	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
11394	SMN1-47 m01	1.176	0.047	SMN	30	GM09677	qRTPCR	SMN1-37 m01	30	nM			
11395	SMN1-48 m01	2.456	0.076	SMN	30	GM09677	qRTPCR	SMN1-37 m01	30	nM			
10087	SMN1-77 m01	4.304	0.166	SMN	30	GM09677	qRTPCR	SMN1-37 m01	30	nM			
10339	SMN1-83 m01	5.928	0.298	SMN	30	GM09677	qRTPCR	SMN1-37 m01	30	nM			
10954	SMN1-90 m01	6.388	0.191	SMN	30	GM09677	qRTPCR	SMN1-37 m01	30	nM			
11394	SMN1-47 m01	0.558	0.067	SMN	30	GM09677	qRTPCR	SMN1-77 m01	30	nM			
11395	SMN1-48 m01	1.080	0.067	SMN	30	GM09677	qRTPCR	SMN1-77 m01	30	nM			
10339	SMN1-83 m01	3.758	0.238	SMN	30	GM09677	qRTPCR	SMN1-77 m01	30	nM			
10954	SMN1-90 m01	4.013	0.368	SMN	30	GM09677	qRTPCR	SMN1-77 m01	30	nM			
11394	SMN1-47 m01	1.067	0.004	SMN	30	GM09677	qRTPCR	SMN1-83 m01	30	nM			
11395	SMN1-48 m01	1.882	0.069	SMN	30	GM09677	qRTPCR	SMN1-83 m01	30	nM			
11384	SMN1-	0.029	0.006	SMN	30	GM09677	qRTPCR	SMN1-53	30	nM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	37 m01	62051	3917	del7				m03					
11394	SMN1-47 m01	0.028 63023	0.003 87127	SMN del7	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11395	SMN1-48 m01	0.023 6993	0.001 28742	SMN del7	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
10087	SMN1-77 m01	0.026 16448	0.002 36867	SMN del7	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
10339	SMN1-83 m01	0.031 97793	0.001 92236	SMN del7	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
10954	SMN1-90 m01	0.022 81249	0.005 06162	SMN del7	30	GM09677	qRTPCR	SMN1-53 m03	30	nM			
11384	SMN1-37 m01	0.023 13364	0.001 83724	SMN del7	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
11394	SMN1-47 m01	0.014 82174	0.001 06281	SMN del7	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
11395	SMN1-48 m01	0.028 77398	0.001 4165	SMN del7	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
10087	SMN1-77 m01	0.031 05581	0.003 06981	SMN del7	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
10339	SMN1-83 m01	0.030 33051	0.002 78744	SMN del7	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
10954	SMN1-90 m01	0.023 08799	0.006 74818	SMN del7	30	GM09677	qRTPCR	SMN1-54 m01	30	nM			
11394	SMN1-47 m01	6.530 63944	0.621 43926	SMN del7	30	GM09677	qRTPCR	SMN1-37 m01	30	nM			
11395	SMN1-48 m01	2.199 81471	0.014 77772	SMN del7	30	GM09677	qRTPCR	SMN1-37 m01	30	nM			
10087	SMN1-	0.041	0.002	SMN	30	GM09677	qRTPCR	SMN1-37	30	nM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	77 m01	62187	32615	del7				m01					
10339	SMN1-83 m01	0.055 14879	0.002 53418	SMN del7	30	GM09677	qRTPCR	SMN1-37 m01	30	nM			
10954	SMN1-90 m01	0.083 1398	0.019 91898	SMN del7	30	GM09677	qRTPCR	SMN1-37 m01	30	nM			
11394	SMN1-47 m01	5.790 49221	0.758 7558	SMN del7	30	GM09677	qRTPCR	SMN1-77 m01	30	nM			
11395	SMN1-48 m01	3.596 13526	0.453 73346	SMN del7	30	GM09677	qRTPCR	SMN1-77 m01	30	nM			
10339	SMN1-83 m01	0.251 88595	0.022 92161	SMN del7	30	GM09677	qRTPCR	SMN1-77 m01	30	nM			
10954	SMN1-90 m01	0.095 38983	0.006 59607	SMN del7	30	GM09677	qRTPCR	SMN1-77 m01	30	nM			
11394	SMN1-47 m01	5.085 87511	0.213 0986	SMN del7	30	GM09677	qRTPCR	SMN1-83 m01	30	nM			
11395	SMN1-48 m01	0.281 23652	0.030 91891	SMN del7	30	GM09677	qRTPCR	SMN1-83 m01	30	nM			
10339	SMN1-83 m01	3.562 9077	0.020 28662	SMN	30	GM09677	qRTPCR	SMN1-90 m01	30	nM			
11394	SMN1-47 m01	0.636 27958	0.006 05457	SMN	30	GM09677	qRTPCR	SMN1-90 m01	30	nM			
11395	SMN1-48 m01	1.254 66796	0.063 69191	SMN	30	GM09677	qRTPCR	SMN1-90 m01	30	nM			
13088	SMN1-53 m03	5.067 93009	0.302 52172	SMN	30	GM09677	qRTPCR	unc-293 m01	30	nM			
13088	SMN1-54 m01	4.466 41736	0.280 44597	SMN	30	GM09677	qRTPCR	unc-293 m01	30	nM			
11384	SMN1-	5.037	0.294	SMN	30	GM09677	qRTPCR	unc-293	30	nM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	37 m01	04694	86482					m01					
10087	SMN1-77 m01	2.740 49121	0.275 31372	SMN	30	GM09677	qRTPCR	unc-293 m01	30	nM			
10339	SMN1-83 m01	3.490 25943	0.011 78177	SMN	30	GM09677	qRTPCR	unc-293 m01	30	nM			
10954	SMN1-90 m01	2.880 2887	0.049 59427	SMN	30	GM09677	qRTPCR	unc-293 m01	30	nM			
11384	SMN1-37 m01	4.753 3598	0.321 03162	SMN	30	GM09677	qRTPCR	SMN1-77 m01	30	nM	SMN1-83 m01	30	nM
11384	SMN1-37 m01	5.651 89819	0.295 56802	SMN	30	GM09677	qRTPCR	SMN1-77 m01	30	nM	SMN1-90 m01	30	nM
11384	SMN1-37 m01	6.014 90124	0.173 31336	SMN	30	GM09677	qRTPCR	SMN1-83 m01	30	nM	SMN1-90 m01	30	nM
10087	SMN1-77 m01	4.444 7551	0.088 96458	SMN	30	GM09677	qRTPCR	SMN1-83 m01	30	nM	SMN1-90 m01	30	nM
11384	SMN1-37 m01	3.414 56679	0.054 74137	SMN	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM
11384	SMN1-37 m01	2.596 55022	0.044 28349	SMN	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM
11384	SMN1-37 m01	2.818 40342	0.059 78105	SMN	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-54 m01	30	nM
11384	SMN1-37 m01	2.489 27348	0.029 50485	SMN	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-54 m01	30	nM
10087	SMN1-77 m01	3.047 5297	0.062 1069	SMN	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM
10087	SMN1-77 m01	3.111 15736	0.169 21418	SMN	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM
10087	SMN1-	3.028	0.053	SMN	30	GM09677	qRTPCR	SMN1-48	30	nM	SMN1-54	30	nM

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	77 m01	52549	97185					m01			m01		
10087	SMN1-77 m01	2.894 0824	0.232 61271	SMN	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-54 m01	30	nM
10954	SMN1-90 m01	4.193 43028	0.078 93861	SMN	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM
10954	SMN1-90 m01	3.026 37119	0.122 56163	SMN	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM
10954	SMN1-90 m01	3.022 20427	0.026 3004	SMN	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-54 m01	30	nM
10954	SMN1-90 m01	2.524 64505	0.023 50823	SMN	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-54 m01	30	nM
10339	SMN1-83 m01	0.370 84716	0.006 07458	SMN del7	30	GM09677	qRTPCR	SMN1-90 m01	30	nM			
11394	SMN1-47 m01	7.799 92768	0.066 4015	SMN del7	30	GM09677	qRTPCR	SMN1-90 m01	30	nM			
11395	SMN1-48 m01	3.732 99885	0.306 36405	SMN del7	30	GM09677	qRTPCR	SMN1-90 m01	30	nM			
13088	SMN1-53 m03	0.033 76088	0.003 61436	SMN del7	30	GM09677	qRTPCR	unc-293 m01	30	nM			
13088	SMN1-54 m01	0.029 98051	0.005 98089	SMN del7	30	GM09677	qRTPCR	unc-293 m01	30	nM			
11384	SMN1-37 m01	0.079 83821	0.002 01965	SMN del7	30	GM09677	qRTPCR	unc-293 m01	30	nM			
10087	SMN1-77 m01	0.134 96564	0.002 11544	SMN del7	30	GM09677	qRTPCR	unc-293 m01	30	nM			
10339	SMN1-83 m01	0.219 27647	0.008 84032	SMN del7	30	GM09677	qRTPCR	unc-293 m01	30	nM			
10954	SMN1-	0.673	0.041	SMN	30	GM09677	qRTPCR	unc-293	30	nM			

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	90 m01	81963	36937	del7				m01					
11384	SMN1-37 m01	0.038 19572	0.004 35806	SMN del7	30	GM09677	qRTPCR	SMN1-77 m01	30	nM	SMN1-83 m01	30	nM
11384	SMN1-37 m01	0.051 20095	0.004 54166	SMN del7	30	GM09677	qRTPCR	SMN1-77 m01	30	nM	SMN1-90 m01	30	nM
11384	SMN1-37 m01	0.079 28237	0.008 25721	SMN del7	30	GM09677	qRTPCR	SMN1-83 m01	30	nM	SMN1-90 m01	30	nM
10087	SMN1-77 m01	0.164 84634	0.010 15905	SMN del7	30	GM09677	qRTPCR	SMN1-83 m01	30	nM	SMN1-90 m01	30	nM
11384	SMN1-37 m01	0.025 75108	0.000 94995	SMN del7	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM
11384	SMN1-37 m01	0.022 0704	0.002 64208	SMN del7	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM
11384	SMN1-37 m01	0.024 24402	0.002 62483	SMN del7	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-54 m01	30	nM
11384	SMN1-37 m01	0.040 74225	0.003 90149	SMN del7	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-54 m01	30	nM
10087	SMN1-77 m01	0.024 91523	0.003 93743	SMN del7	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM
10087	SMN1-77 m01	0.026 02505	0.001 02729	SMN del7	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM
10087	SMN1-77 m01	0.022 38013	0.001 34454	SMN del7	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-54 m01	30	nM
10087	SMN1-77 m01	0.035 14381	0.006 24866	SMN del7	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-54 m01	30	nM
10954	SMN1-90 m01	0.035 98881	0.002 38386	SMN del7	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM
10954	SMN1-	0.034	0.003	SMN	30	GM09677	qRTPCR	SMN1-48	30	nM	SMN1-53	30	nM

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	90 m01	79284	47436	del7				m01			m03		
10954	SMN1-90 m01	0.029	0.001	SMN del7	30	GM09677	qRTPCR	SMN1-47 m01	30	nM	SMN1-54 m01	30	nM
10954	SMN1-90 m01	0.044	0.002	SMN del7	30	GM09677	qRTPCR	SMN1-48 m01	30	nM	SMN1-54 m01	30	nM
11394	SMN1-47 m01	2.932	0.284	SMN Full	30	GM03813	qRTPCR	SMN1-53 m03	2				
11395	SMN1-48 m01	4.255	0.185	SMN Full	30	GM03813	qRTPCR	SMN1-53 m03	2				
13091	SMN1-57 m01	2.579	0.210	SMN Full	30	GM03813	qRTPCR	SMN1-53 m03	2				
10169	SMN1-79 m01	4.167	0.179	SMN Full	30	GM03813	qRTPCR	SMN1-53 m03	2				
10170	SMN1-80 m01	3.931	0.041	SMN Full	30	GM03813	qRTPCR	SMN1-53 m03	2				
10763	SMN1-84 m01	3.315	0.042	SMN Full	30	GM03813	qRTPCR	SMN1-53 m03	2				
11419	SMN1-93 m01	5.127	0.724	SMN Full	30	GM03813	qRTPCR	SMN1-53 m03	2				
11394	SMN1-47 m01	2.655	0.541	SMN Full	30	GM03813	qRTPCR	SMN1-53 m03	1				
11395	SMN1-48 m01	2.438	0.232	SMN Full	30	GM03813	qRTPCR	SMN1-53 m03	1				
11394	SMN1-47 m01	2.823	0.145	SMN Full	30	GM03813	qRTPCR	SMN1-54 m01	1				
11395	SMN1-48 m01	3.023	0.218	SMN Full	30	GM03813	qRTPCR	SMN1-54 m01	1				
13088	SMN1-	4.085	0.353	SMN	30	GM03813	qRTPCR	unc-293	2				

Appendix B: Table 6

SeqID	Oligo Name	Avg RQ	Avg RQ SE	Targ et	[oligo]	cell line	Assay Type	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units
	53 m02	30259	16274	Full				m01					
11394	SMN1-47 m01	0.034 81705	0.008 93227	SMN del7	30	GM03813	qRTPCR	SMN1-53 m03	2				
10169	SMN1-79 m01	1.245 09089	0.182 38866	SMN del7	30	GM03813	qRTPCR	SMN1-53 m03	2				
10170	SMN1-80 m01	0.057 89038	0.006 78869	SMN del7	30	GM03813	qRTPCR	SMN1-53 m03	2				
10763	SMN1-84 m01	0.017 49812	0.007 30538	SMN del7	30	GM03813	qRTPCR	SMN1-53 m03	2				
11394	SMN1-47 m01	0.016 09125	0.005 21425	SMN del7	30	GM03813	qRTPCR	SMN1-54 m01	1				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
11394	SMN1-47 m01	187.313138 2	43.6469 3799	SMN	10000	GM10684	216							9 day gymnotic delivery
13091	SMN1-57 m01	112.442799 8	10.9262 4256	SMN	10000	GM10684	216							9 day gymnotic delivery
10169	SMN1-79 m01	126.265114 7	25.7636 4993	SMN	10000	GM10684	216							9 day gymnotic delivery
10170	SMN1-80 m01	116.788031 9	15.3733 9077	SMN	10000	GM10684	216							9 day gymnotic delivery
10763	SMN1-84 m01	173.970287	56.3490 3079	SMN	10000	GM10684	216							9 day gymnotic delivery
11419	SMN1-93 m01	168.148405 1	11.0296 7581	SMN	10000	GM10684	216							9 day gymnotic delivery
11423	SMN1-96 m01	143.968183 2	16.8636 9193	SMN	10000	GM10684	216							9 day gymnotic delivery
	Ctrl Un	100	4.94913 3557	SMN	0	GM10684	216							9 day gymnotic delivery
11394	SMN1-47 m01	84.2683173	13.2099 5601	SMN	30	GM03813	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
13091	SMN1-57 m01	109.6862057	11.75820155	SMN	30	GM03813	48							
10169	SMN1-79 m01	57.51478525	6.187112927	SMN	30	GM03813	48							
10170	SMN1-80 m01	55.90174709	2.228261222	SMN	30	GM03813	48							
10763	SMN1-84 m01	59.67222944	7.45998743	SMN	30	GM03813	48							
10070	SMN1-67 m01	120.6101756	5.400113597	SMN	30	GM03813	48							
	Ctrl Lipo	100	8.829454543	SMN	0	GM03813	48							
	Ctrl Un	93.25582151	2.305925874	SMN	0	GM03813	48							
11394	SMN1-47 m01	122.7722836	5.427315515	SMN	30	GM03814	48							
13091	SMN1-57 m01	99.71392257	8.67824611	SMN	30	GM03814	48							
10169	SMN1-79 m01	98.10272057	4.911735745	SMN	30	GM03814	48							
10170	SMN1-80 m01	81.02504424	1.401511819	SMN	30	GM03814	48							
10763	SMN1-84 m01	105.1325104	3.738011077	SMN	30	GM03814	48							
10070	SMN1-67 m01	118.8409179	4.165131399	SMN	30	GM03814	48							
	Ctrl	100	1.88719	SMN	0	GM03	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	Lipo		7484			814								
	Ctrl Un	106.7167457	3.276692776	SMN	0	GM03814	48							
11394	SMN1-47 m01	127.5336934	9.28043039	SMN	30	GM03815	48							
13091	SMN1-57 m01	105.7522507	5.593295765	SMN	30	GM03815	48							
10169	SMN1-79 m01	91.02333961	9.153973663	SMN	30	GM03815	48							
10170	SMN1-80 m01	69.83766983	9.737419648	SMN	30	GM03815	48							
10763	SMN1-84 m01	109.1323998	17.65477226	SMN	30	GM03815	48							
10070	SMN1-67 m01	120.9840419	10.83225593	SMN	30	GM03815	48							
	Ctrl Lipo	100	14.00862871	SMN	0	GM03815	48							
	Ctrl Un	96.0184852	11.88607186	SMN	0	GM03815	48							
11394	SMN1-47 m01	96.52569542	0.55322678	SMN	30	GM00232	48							
13091	SMN1-57 m01	96.24883365	7.027245536	SMN	30	GM00232	48							
10169	SMN1-79 m01	64.64349993	6.04748272	SMN	30	GM00232	48							
10170	SMN1-80 m01	51.28511335	7.579171795	SMN	30	GM00232	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10763	SMN1-84 m01	72.26562448	6.837934976	SMN	30	GM00232	48							
10070	SMN1-67 m01	130.7503148	10.63777753	SMN	30	GM00232	48							
	Ctrl Lipo	100	4.615563038	SMN	0	GM00232	48							
	Ctrl Un	106.6568122	12.91825741	SMN	0	GM00232	48							
11394	SMN1-47 m01	90.35067408	2.923375471	SMN	30	GM22592	48							
13091	SMN1-57 m01	91.60787509	8.087676793	SMN	30	GM22592	48							
10169	SMN1-79 m01	70.67008003	16.25717494	SMN	30	GM22592	48							
10170	SMN1-80 m01	58.9687565	5.975225151	SMN	30	GM22592	48							
10763	SMN1-84 m01	67.53566449	8.144771631	SMN	30	GM22592	48							
10070	SMN1-67 m01	120.9523148	8.556718718	SMN	30	GM22592	48							
	Ctrl Lipo	100	3.080041565	SMN	0	GM22592	48							
	Ctrl Un	98.55893671	8.022220991	SMN	0	GM22592	48							
11394	SMN1-47 m01	88.41860921	10.68651422	SMN	30	GM09677	48							
13091	SMN1-	115.167660	5.22808	SMN	30	GM09	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	57 m01	4	2344			677								
10169	SMN1-79 m01	58.35206004	3.252554167	SMN	30	GM09 677	48							
10170	SMN1-80 m01	53.18251847	4.959795069	SMN	30	GM09 677	48							
10763	SMN1-84 m01	61.24254545	1.224093056	SMN	30	GM09 677	48							
10070	SMN1-67 m01	128.4833603	14.65955978	SMN	30	GM09 677	48							
	Ctrl Lipo	100	4.393159001	SMN	0	GM09 677	48							
	Ctrl Un	88.47225251	2.730317119	SMN	0	GM09 677	48							
11394	SMN1-47 m01	92.77447534	2.004361791	SMN	30	GM03 813	24							
11395	SMN1-48 m01	95.46354749	14.15612668	SMN	30	GM03 813	24							
13088	SMN1-54 m01	171.4033577	8.209640005	SMN	30	GM03 813	24							
13088	SMN1-54 m01	129.3943149	13.7739916	SMN	30	GM03 813	24							
13091	SMN1-57 m01	94.684338	7.277858941	SMN	30	GM03 813	24							
10070	SMN1-67 m01	109.9615428	17.74508171	SMN	30	GM03 813	24							
10169	SMN1-79 m01	79.6693439	17.92480163	SMN	30	GM03 813	24							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10170	SMN1-80 m01	60.94236955	15.40672056	SMN	30	GM03813	24							
10763	SMN1-84 m01	86.52731514	6.746367335	SMN	30	GM03813	24							
11419	SMN1-93 m01	109.0143818	19.74352677	SMN	30	GM03813	24							
	Ctrl Lipo	100	22.56173368	SMN	0	GM03813	24							
	Ctrl Un	76.24141817	15.93701953	SMN	0	GM03813	24							
11394	SMN1-47 m01	76.32816476	11.76174122	SMN	30	GM03814	24							
11395	SMN1-48 m01	81.50999768	17.79371956	SMN	30	GM03814	24							
13088	SMN1-54 m01	118.3429949	3.830016211	SMN	30	GM03814	24							
13088	SMN1-54 m01	98.58114158	25.24675814	SMN	30	GM03814	24							
13091	SMN1-57 m01	86.77393808	3.225864389	SMN	30	GM03814	24							
10070	SMN1-67 m01	99.85396407	20.50014614	SMN	30	GM03814	24							
10169	SMN1-79 m01	76.86320491	11.51963531	SMN	30	GM03814	24							
10170	SMN1-80 m01	76.48795019	17.91116512	SMN	30	GM03814	24							
10763	SMN1-	58.9142966	16.1219	SMN	30	GM03	24							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	84 m01	5	8224			814								
11419	SMN1-93 m01	94.25918919	23.04216025	SMN	30	GM03814	24							
	Ctrl Lipo	100	4.432488328	SMN	0	GM03814	24							
	Ctrl Un	85.83601568	15.15277672	SMN	0	GM03814	24							
11394	SMN1-47 m01	79.70871716	4.681583273	SMN	30	GM00232	24							
11395	SMN1-48 m01	69.802263	15.52208554	SMN	30	GM00232	24							
13088	SMN1-54 m01	115.2573585	5.59924848	SMN	30	GM00232	24							
13088	SMN1-54 m01	94.18000598	32.47457231	SMN	30	GM00232	24							
13091	SMN1-57 m01	87.22184074	8.510552213	SMN	30	GM00232	24							
10070	SMN1-67 m01	96.13695073	11.82984869	SMN	30	GM00232	24							
10169	SMN1-79 m01	61.37679838	25.53876353	SMN	30	GM00232	24							
10170	SMN1-80 m01	73.98275893	15.89515293	SMN	30	GM00232	24							
10763	SMN1-84 m01	78.9256132	6.100637282	SMN	30	GM00232	24							
11419	SMN1-93 m01	110.7707325	1.900558891	SMN	30	GM00232	24							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	Ctrl Lipo	100	12.2018 5983	SMN	0	GM00 232	24							
	Ctrl Un	92.2388166 1	15.0626 1486	SMN	0	GM00 232	24							
11394	SMN1- 47 m01	51.4931024 3	23.0870 0157	SMN	30	GM22 592	24							
11395	SMN1- 48 m01	71.6811027 1	17.0776 0131	SMN	30	GM22 592	24							
13088	SMN1- 54 m01	74.3057944 2	28.8141 3349	SMN	30	GM22 592	24							
13088	SMN1- 54 m01	86.5730796	16.5678 5105	SMN	30	GM22 592	24							
13091	SMN1- 57 m01	83.0638481 4	20.4094 7288	SMN	30	GM22 592	24							
10070	SMN1- 67 m01	103.145965 1	27.7938 8179	SMN	30	GM22 592	24							
10169	SMN1- 79 m01	88.3226960 3	33.3234 3731	SMN	30	GM22 592	24							
10170	SMN1- 80 m01	99.6677674	38.8921 6027	SMN	30	GM22 592	24							
10763	SMN1- 84 m01	99.0802321 8	41.2988 2335	SMN	30	GM22 592	24							
11419	SMN1- 93 m01	111.158982 8	9.16146 2441	SMN	30	GM22 592	24							
	Ctrl Lipo	100	6.00354 1375	SMN	0	GM22 592	24							
	Ctrl Un	102.353520	21.4998	SMN	0	GM22	24							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
		8	176			592								
11394	SMN1-47 m01	88.0385071 3	10.1167 9972	SMN	30	GM09 677	24							
11395	SMN1-48 m01	72.1900200 2	12.5846 2115	SMN	30	GM09 677	24							
13088	SMN1-54 m01	144.652183 2	14.7947 3368	SMN	30	GM09 677	24							
13088	SMN1-54 m01	104.303505 4	8.59290 1227	SMN	30	GM09 677	24							
13091	SMN1-57 m01	88.8771953 5	5.28126 9142	SMN	30	GM09 677	24							
10070	SMN1-67 m01	103.538959 4	9.35666 7348	SMN	30	GM09 677	24							
10169	SMN1-79 m01	53.5178187 4	4.44822 145	SMN	30	GM09 677	24							
10170	SMN1-80 m01	73.2625562 5	2.55438 6219	SMN	30	GM09 677	24							
10763	SMN1-84 m01	76.7606080 8	10.6040 2341	SMN	30	GM09 677	24							
11419	SMN1-93 m01	96.0943661 9	7.94364 2032	SMN	30	GM09 677	24							
	Ctrl Lipo	100	17.0703 3688	SMN	0	GM09 677	24							
	Ctrl Un	91.2993413 4	4.19904 7869	SMN	0	GM09 677	24							
11394	SMN1-47 m01	79.3852267 8	6.85648 1119	SMN	30	GM03 813	72							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
11395	SMN1-48 m01	96.59918336	19.63494074	SMN	30	GM03813	72							
13088	SMN1-54 m01	204.0292193	40.19982235	SMN	30	GM03813	72							
13088	SMN1-54 m01	219.214278	24.02727621	SMN	30	GM03813	72							
13091	SMN1-57 m01	123.9300988	15.02050245	SMN	30	GM03813	72							
10070	SMN1-67 m01	117.8414458	8.470992058	SMN	30	GM03813	72							
10169	SMN1-79 m01	29.22098129	10.76832281	SMN	30	GM03813	72							
10170	SMN1-80 m01	35.65877155	3.860403008	SMN	30	GM03813	72							
10763	SMN1-84 m01	38.70275082	7.369133838	SMN	30	GM03813	72							
11419	SMN1-93 m01	169.5228249	7.097277507	SMN	30	GM03813	72							
	Ctrl Lipo	100	4.697264435	SMN	0	GM03813	72							
	Ctrl Un	106.3914308	10.63028163	SMN	0	GM03813	72							
11394	SMN1-47 m01	108.5096333	10.44764422	SMN	30	GM03814	72							
11395	SMN1-48 m01	130.4557557	7.588943664	SMN	30	GM03814	72							
13088	SMN1-	178.025689	4.43408	SMN	30	GM03	72							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	54 m01	9	3478			814								
13088	SMN1-54 m01	168.2518983	19.54899424	SMN	30	GM03814	72							
13091	SMN1-57 m01	110.3122155	27.96303191	SMN	30	GM03814	72							
10070	SMN1-67 m01	110.2601263	11.60606798	SMN	30	GM03814	72							
10169	SMN1-79 m01	56.0048371	7.913173551	SMN	30	GM03814	72							
10170	SMN1-80 m01	60.19019999	9.477538778	SMN	30	GM03814	72							
10763	SMN1-84 m01	90.29208341	22.90334102	SMN	30	GM03814	72							
11419	SMN1-93 m01	107.7635192	8.716683278	SMN	30	GM03814	72							
	Ctrl Lipo	100	6.380468706	SMN	0	GM03814	72							
	Ctrl Un	89.61346064	6.407810844	SMN	0	GM03814	72							
11394	SMN1-47 m01	71.55786498	2.067738718	SMN	30	GM00232	72							
11395	SMN1-48 m01	96.24298626	8.373248804	SMN	30	GM00232	72							
13088	SMN1-54 m01	242.8171835	18.54852061	SMN	30	GM00232	72							
13088	SMN1-54 m01	174.2550787	26.79689845	SMN	30	GM00232	72							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
13091	SMN1-57 m01	120.688384 3	3.19010 9155	SMN	30	GM00 232	72							
10070	SMN1-67 m01	122.794098 4	10.0350 626	SMN	30	GM00 232	72							
10169	SMN1-79 m01	35.5674409 3	9.85128 8262	SMN	30	GM00 232	72							
10170	SMN1-80 m01	39.2373287 7	2.11743 705	SMN	30	GM00 232	72							
10763	SMN1-84 m01	39.9468358 5	8.13496 9086	SMN	30	GM00 232	72							
11419	SMN1-93 m01	122.150081 5	5.65052 0137	SMN	30	GM00 232	72							
	Ctrl Lipo	100	12.3247 4091	SMN	0	GM00 232	72							
	Ctrl Un	91.1249822 3	13.0180 1346	SMN	0	GM00 232	72							
11394	SMN1-47 m01	103.270922 6	10.9581 6121	SMN	30	GM22 592	72							
11395	SMN1-48 m01	127.985623 2	20.8545 3966	SMN	30	GM22 592	72							
13088	SMN1-54 m01	332.891559 5	26.0992 3535	SMN	30	GM22 592	72							
13088	SMN1-54 m01	238.687400 9	55.2531 9119	SMN	30	GM22 592	72							
13091	SMN1-57 m01	145.479304	10.2577 2741	SMN	30	GM22 592	72							
10070	SMN1-	118.434952	7.04740	SMN	30	GM22	72							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	67 m01	2	0879			592								
10169	SMN1-79 m01	55.70298085	12.21657309	SMN	30	GM22592	72							
10170	SMN1-80 m01	62.42195907	5.157718521	SMN	30	GM22592	72							
10763	SMN1-84 m01	85.0047662	22.36733885	SMN	30	GM22592	72							
11419	SMN1-93 m01	158.147634	9.472136318	SMN	30	GM22592	72							
	Ctrl Lipo	100	7.140209675	SMN	0	GM22592	72							
	Ctrl Un	85.47872417	12.81143586	SMN	0	GM22592	72							
11394	SMN1-47 m01	95.43338812	6.058852137	SMN	30	GM09677	72							
11395	SMN1-48 m01	115.0404937	15.90399183	SMN	30	GM09677	72							
13088	SMN1-54 m01	374.3302628	11.30525561	SMN	30	GM09677	72							
13088	SMN1-54 m01	299.3015998	18.99699908	SMN	30	GM09677	72							
13091	SMN1-57 m01	177.1459161	26.27882422	SMN	30	GM09677	72							
10070	SMN1-67 m01	153.5955083	20.95281966	SMN	30	GM09677	72							
10169	SMN1-79 m01	51.81616325	4.497625538	SMN	30	GM09677	72							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10170	SMN1-80 m01	63.22854646	6.020688805	SMN	30	GM09677	72							
10763	SMN1-84 m01	49.72870023	1.667306917	SMN	30	GM09677	72							
11419	SMN1-93 m01	161.7477626	2.396599484	SMN	30	GM09677	72							
	Ctrl Lipo	100	1.04424907	SMN	0	GM09677	72							
	Ctrl Un	87.27564996	12.26075137	SMN	0	GM09677	72							
11394	SMN1-47 m01	260.7810001	63.1153116	SMN	30	GM03813	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	266.8937333	10.10530835	SMN	30	GM03813	48	SMN1-53 m03	30	nM				
13091	SMN1-57 m01	138.7284492	14.28586019	SMN	30	GM03813	48	SMN1-53 m03	30	nM				
10070	SMN1-67 m01	212.0614774	59.29880914	SMN	30	GM03813	48	SMN1-53 m03	30	nM				
10169	SMN1-79 m01	135.3818622	18.74996151	SMN	30	GM03813	48	SMN1-53 m03	30	nM				
10170	SMN1-80 m01	89.08809789	24.8325483	SMN	30	GM03813	48	SMN1-53 m03	30	nM				
10763	SMN1-84 m01	149.6156726	51.74518585	SMN	30	GM03813	48	SMN1-53 m03	30	nM				
11419	SMN1-93 m01	231.0655029	43.33607284	SMN	30	GM03813	48	SMN1-53 m03	30	nM				
11394	SMN1-	285.016347	24.2610	SMN	30	GM03	48	SMN1-	30	nM				

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SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	47 m01	1	3112			813		54 m01						
11395	SMN1-48 m01	294.0930965	19.51747386	SMN	30	GM03813	48	SMN1-54 m01	30	nM				
13091	SMN1-57 m01	126.0381654	0.64462045	SMN	30	GM03813	48	SMN1-54 m01	30	nM				
10070	SMN1-67 m01	209.6952732	14.554729	SMN	30	GM03813	48	SMN1-54 m01	30	nM				
10169	SMN1-79 m01	159.948513	20.14231085	SMN	30	GM03813	48	SMN1-54 m01	30	nM				
10170	SMN1-80 m01	116.3886837	9.2741632	SMN	30	GM03813	48	SMN1-54 m01	30	nM				
10763	SMN1-84 m01	192.2738451	18.70681528	SMN	30	GM03813	48	SMN1-54 m01	30	nM				
11419	SMN1-93 m01	216.5730582	7.652054583	SMN	30	GM03813	48	SMN1-54 m01	30	nM				
13088	SMN1-54 m01	186.381388	8.195474318	SMN	30	GM03813	48							
13088	SMN1-54 m01	165.731076	14.94915205	SMN	30	GM03813	48							
	Ctrl Lipo	100	6.418805011	SMN	0	GM03813	48							
	Ctrl Un	94.42564593	11.1796209	SMN	0	GM03813	48							
11394	SMN1-47 m01	247.4984674	14.12386732	SMN	30	GM03814	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	308.0546378	9.748462356	SMN	30	GM03814	48	SMN1-53 m03	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
13091	SMN1-57 m01	136.9772314	32.75189812	SMN	30	GM03814	48	SMN1-53 m03	30	nM				
10070	SMN1-67 m01	157.9565027	25.16448097	SMN	30	GM03814	48	SMN1-53 m03	30	nM				
10169	SMN1-79 m01	155.4239183	38.01906743	SMN	30	GM03814	48	SMN1-53 m03	30	nM				
10170	SMN1-80 m01	125.8443897	18.10166591	SMN	30	GM03814	48	SMN1-53 m03	30	nM				
10763	SMN1-84 m01	138.4168596	30.14851537	SMN	30	GM03814	48	SMN1-53 m03	30	nM				
11419	SMN1-93 m01	192.6186617	32.57899069	SMN	30	GM03814	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	167.3850998	43.4993728	SMN	30	GM03814	48	SMN1-54 m01	30	nM				
11395	SMN1-48 m01	183.9218325	47.72907204	SMN	30	GM03814	48	SMN1-54 m01	30	nM				
13091	SMN1-57 m01	133.1597889	22.48287624	SMN	30	GM03814	48	SMN1-54 m01	30	nM				
10070	SMN1-67 m01	104.5052561	13.67616753	SMN	30	GM03814	48	SMN1-54 m01	30	nM				
10169	SMN1-79 m01	183.9745458	45.07483989	SMN	30	GM03814	48	SMN1-54 m01	30	nM				
10170	SMN1-80 m01	124.822064	46.19508612	SMN	30	GM03814	48	SMN1-54 m01	30	nM				
10763	SMN1-84 m01	163.337248	30.09685009	SMN	30	GM03814	48	SMN1-54 m01	30	nM				
11419	SMN1-	190.898951	30.9509	SMN	30	GM03	48	SMN1-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	93 m01	9	1518			814		54 m01						
13088	SMN1-54 m01	145.430691	66.2268 2254	SMN	30	GM03 814	48							
13088	SMN1-54 m01	136.615984 8	28.9746 1899	SMN	30	GM03 814	48							
	Ctrl Lipo	100	18.1799 2444	SMN	0	GM03 814	48							
	Ctrl Un	107.199861 1	18.6380 8833	SMN	0	GM03 814	48							
11394	SMN1-47 m01	265.430924 4	11.0226 4827	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	285.581405 8	0.68131 966	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
13091	SMN1-57 m01	121.160371 4	32.6993 3288	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
10070	SMN1-67 m01	199.037698	30.1687 9844	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
10169	SMN1-79 m01	120.372242 7	8.74891 7453	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
10170	SMN1-80 m01	79.9433442	10.6980 6922	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
10763	SMN1-84 m01	161.859708 7	13.5189 2452	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
11419	SMN1-93 m01	195.505544 2	45.4345 9641	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	211.377018 1	23.7878 0469	SMN	30	GM00 232	48	SMN1-54 m01	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
11395	SMN1-48 m01	250.524894 3	43.5763 8596	SMN	30	GM00 232	48	SMN1-54 m01	30	nM				
13091	SMN1-57 m01	118.575632 7	14.4141 5745	SMN	30	GM00 232	48	SMN1-54 m01	30	nM				
10070	SMN1-67 m01	175.043762 1	52.3335 4564	SMN	30	GM00 232	48	SMN1-54 m01	30	nM				
10169	SMN1-79 m01	137.988530 1	10.4691 719	SMN	30	GM00 232	48	SMN1-54 m01	30	nM				
10170	SMN1-80 m01	104.974800 9	30.3304 7967	SMN	30	GM00 232	48	SMN1-54 m01	30	nM				
10763	SMN1-84 m01	162.629923 9	22.1876 1714	SMN	30	GM00 232	48	SMN1-54 m01	30	nM				
11419	SMN1-93 m01	171.121678 2	23.0402 413	SMN	30	GM00 232	48	SMN1-54 m01	30	nM				
13088	SMN1-54 m01	162.628751 9	10.2278 9882	SMN	30	GM00 232	48							
13088	SMN1-54 m01	107.560368 2	6.30346 2984	SMN	30	GM00 232	48							
	Ctrl Lipo	100	13.5285 799	SMN	0	GM00 232	48							
	Ctrl Un	81.6736613 2	18.9476 0331	SMN	0	GM00 232	48							
11394	SMN1-47 m01	259.810268 9	26.0791 8678	SMN	30	GM22 592	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	272.309335	29.8699 5135	SMN	30	GM22 592	48	SMN1-53 m03	30	nM				
13091	SMN1-	142.207866	11.6257	SMN	30	GM22	48	SMN1-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	57 m01	9	9653			592		53 m03						
10070	SMN1-67 m01	208.463570 3	55.3390 9153	SMN	30	GM22 592	48	SMN1-53 m03	30	nM				
10169	SMN1-79 m01	148.497594 3	16.9051 6925	SMN	30	GM22 592	48	SMN1-53 m03	30	nM				
10170	SMN1-80 m01	111.608377 2	9.77681 6404	SMN	30	GM22 592	48	SMN1-53 m03	30	nM				
10763	SMN1-84 m01	205.640762	5.26684 5718	SMN	30	GM22 592	48	SMN1-53 m03	30	nM				
11419	SMN1-93 m01	190.506102 6	62.6765 5665	SMN	30	GM22 592	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	240.788008	4.35969 651	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
11395	SMN1-48 m01	198.246847 3	90.8430 6257	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
13091	SMN1-57 m01	119.566732 1	3.33495 9389	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
10070	SMN1-67 m01	166.530782 3	31.7425 7233	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
10169	SMN1-79 m01	160.159988 1	12.7450 0258	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
10170	SMN1-80 m01	82.1187508 3	24.6870 9581	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
10763	SMN1-84 m01	154.019558	19.7404 1367	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
11419	SMN1-93 m01	208.909898 1	7.36991 7645	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
13088	SMN1-54 m01	148.111182	10.3591 5653	SMN	30	GM22 592	48							
13088	SMN1-54 m01	140.596416	36.2074 9258	SMN	30	GM22 592	48							
	Ctrl Lipo	100	1.45547 2416	SMN	0	GM22 592	48							
	Ctrl Un	93.1985933 6	2.65695 7551	SMN	0	GM22 592	48							
11394	SMN1-47 m01	271.945905 9	20.1291 6912	SMN	30	GM09 677	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	275.957939 6	17.0072 9467	SMN	30	GM09 677	48	SMN1-53 m03	30	nM				
13091	SMN1-57 m01	117.051889 2	26.4255 7894	SMN	30	GM09 677	48	SMN1-53 m03	30	nM				
10070	SMN1-67 m01	199.070031 3	9.66442 5722	SMN	30	GM09 677	48	SMN1-53 m03	30	nM				
10169	SMN1-79 m01	236.145619 6	31.3533 2308	SMN	30	GM09 677	48	SMN1-53 m03	30	nM				
10170	SMN1-80 m01	93.3333333 1	11.5329 874	SMN	30	GM09 677	48	SMN1-53 m03	30	nM				
10763	SMN1-84 m01	150.706259 3	13.9780 9579	SMN	30	GM09 677	48	SMN1-53 m03	30	nM				
11419	SMN1-93 m01	207.402571 9	8.41538 2319	SMN	30	GM09 677	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	226.102481 4	45.9227 6339	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
11395	SMN1-	255.722808	13.2683	SMN	30	GM09	48	SMN1-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	48 m01	6	4859			677		54 m01						
13091	SMN1-57 m01	114.745214 5	23.2481 6108	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
10070	SMN1-67 m01	187.641227 4	17.8693 2618	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
10169	SMN1-79 m01	296.939271 2	86.5553 1249	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
10170	SMN1-80 m01	98.4660443 8	38.8433 4724	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
10763	SMN1-84 m01	142.393402 9	43.3636 5142	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
11419	SMN1-93 m01	195.240592 9	26.1936 0828	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
13088	SMN1-54 m01	187.716441 2	18.3836 2405	SMN	30	GM09 677	48							
13088	SMN1-54 m01	176.178390 3	5.45990 6221	SMN	30	GM09 677	48							
	Ctrl Lipo	100	3.75370 2953	SMN	0	GM09 677	48							
	Ctrl Un	106.072544 5	11.1244 7417	SMN	0	GM09 677	48							
11394	SMN1-47 m01	267.770334 7	13.5110 123	SMN	30	GM03 813	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	309.518783 4	5.12853 9351	SMN	30	GM03 813	48	SMN1-53 m03	30	nM				
13091	SMN1-57 m01	177.515794 5	6.19315 2304	SMN	30	GM03 813	48	SMN1-53 m03	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10070	SMN1-67 m01	296.175188 2	16.9997 5486	SMN	30	GM03 813	48	SMN1-53 m03	30	nM				
10169	SMN1-79 m01	218.173998 4	23.7096 0452	SMN	30	GM03 813	48	SMN1-53 m03	30	nM				
10170	SMN1-80 m01	169.405522 8	7.84838 9576	SMN	30	GM03 813	48	SMN1-53 m03	30	nM				
10763	SMN1-84 m01	206.176582 3	9.56452 1579	SMN	30	GM03 813	48	SMN1-53 m03	30	nM				
11419	SMN1-93 m01	241.417259 4	74.0045 3895	SMN	30	GM03 813	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	233.795738 7	13.3621 1387	SMN	30	GM03 813	48	SMN1-54 m01	30	nM				
11395	SMN1-48 m01	263.247065 5	11.8483 1849	SMN	30	GM03 813	48	SMN1-54 m01	30	nM				
13091	SMN1-57 m01	158.626445 8	3.22176 4347	SMN	30	GM03 813	48	SMN1-54 m01	30	nM				
10070	SMN1-67 m01	211.693914 8	8.23514 0905	SMN	30	GM03 813	48	SMN1-54 m01	30	nM				
10169	SMN1-79 m01	198.195951 6	12.2421 2951	SMN	30	GM03 813	48	SMN1-54 m01	30	nM				
10170	SMN1-80 m01	140.840335 9	9.74261 7387	SMN	30	GM03 813	48	SMN1-54 m01	30	nM				
10763	SMN1-84 m01	193.082127 6	11.5220 5048	SMN	30	GM03 813	48	SMN1-54 m01	30	nM				
11419	SMN1-93 m01	243.244076 1	26.5576 1137	SMN	30	GM03 813	48	SMN1-54 m01	30	nM				
13088	SMN1-	185.101706	19.0031	SMN	30	GM03	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	54 m01	9	5084			813								
13088	SMN1-54 m01	134.3951103	45.99392328	SMN	30	GM03813	48							
13088	SMN1-54 m01	229.1106868	20.04334402	SMN	60	GM03813	48							
13088	SMN1-54 m01	143.7130216	35.30558535	SMN	60	GM03813	48							
13088	SMN1-54 m01	227.1906114	95.14113064	SMN	30	GM03813	48	unc-293 m01	30	nM				
13088	SMN1-54 m01	165.3423465	19.7415068	SMN	30	GM03813	48	unc-293 m01	30	nM				
	Ctrl Lipo	100	5.335670928	SMN	0	GM03813	48							
	Ctrl Un	109.7590355	4.484564408	SMN	0	GM03813	48							
11394	SMN1-47 m01	180.9662966	9.555899021	SMN	30	GM03814	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	193.6571974	53.21843035	SMN	30	GM03814	48	SMN1-53 m03	30	nM				
13091	SMN1-57 m01	114.914036	9.92084154	SMN	30	GM03814	48	SMN1-53 m03	30	nM				
10070	SMN1-67 m01	159.2605767	4.245321576	SMN	30	GM03814	48	SMN1-53 m03	30	nM				
10169	SMN1-79 m01	176.3580097	15.73209487	SMN	30	GM03814	48	SMN1-53 m03	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10170	SMN1-80 m01	141.321145 5	21.2131 3341	SMN	30	GM03 814	48	SMN1-53 m03	30	nM				
10763	SMN1-84 m01	131.262032 3	21.8608 3292	SMN	30	GM03 814	48	SMN1-53 m03	30	nM				
11419	SMN1-93 m01	171.444667 1	12.3744 2364	SMN	30	GM03 814	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	175.426292	9.66756 6951	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
11395	SMN1-48 m01	189.821072 1	1.71269 3691	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
13091	SMN1-57 m01	117.372731 4	7.41525 2688	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
10070	SMN1-67 m01	147.164189	16.7142 8826	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
10169	SMN1-79 m01	155.951332 2	13.8550 5736	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
10170	SMN1-80 m01	119.368385 7	12.4152 6297	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
10763	SMN1-84 m01	128.496682 3	13.7997 5502	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
11419	SMN1-93 m01	160.993329	14.8341 8054	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
13088	SMN1-54 m01	153.888745	1.37225 2066	SMN	30	GM03 814	48							
13088	SMN1-54 m01	134.448235 3	12.0893 8204	SMN	30	GM03 814	48							
13088	SMN1-	161.295208	11.2478	SMN	30	GM03	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	54 m01	3	5756			814								
13088	SMN1-54 m01	151.5046465	11.10819675	SMN	30	GM03 814	48							
13088	SMN1-54 m01	184.3453869	8.833826813	SMN	30	GM03 814	48	unc-293 m01	30	nM				
13088	SMN1-54 m01	163.8042809	13.52957827	SMN	30	GM03 814	48	unc-293 m01	30	nM				
	Ctrl Lipo	100	3.862163426	SMN	0	GM03 814	48							
	Ctrl Un	106.7189913	15.43997676	SMN	0	GM03 814	48							
11394	SMN1-47 m01	263.3891997	15.13481637	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	209.9327687	15.39958408	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
13091	SMN1-57 m01	138.5660657	10.64384121	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
10070	SMN1-67 m01	196.1617149	9.049014615	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
10169	SMN1-79 m01	109.7410536	20.59782582	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
10170	SMN1-80 m01	95.86478234	8.763638808	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				
10763	SMN1-84 m01	128.7869289	36.6036626	SMN	30	GM00 232	48	SMN1-53 m03	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
11419	SMN1-93 m01	183.0799837	6.704266943	SMN	30	GM00232	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	229.7695139	21.8247503	SMN	30	GM00232	48	SMN1-54 m01	30	nM				
11395	SMN1-48 m01	244.0892315	5.616201343	SMN	30	GM00232	48	SMN1-54 m01	30	nM				
13091	SMN1-57 m01	146.1808404	8.106219615	SMN	30	GM00232	48	SMN1-54 m01	30	nM				
10070	SMN1-67 m01	204.0695758	19.97966281	SMN	30	GM00232	48	SMN1-54 m01	30	nM				
10169	SMN1-79 m01	141.0191342	6.874545734	SMN	30	GM00232	48	SMN1-54 m01	30	nM				
10170	SMN1-80 m01	114.7338671	10.85289615	SMN	30	GM00232	48	SMN1-54 m01	30	nM				
10763	SMN1-84 m01	147.6241403	9.82436282	SMN	30	GM00232	48	SMN1-54 m01	30	nM				
11419	SMN1-93 m01	167.8738602	4.56373034	SMN	30	GM00232	48	SMN1-54 m01	30	nM				
13088	SMN1-54 m01	217.863522	11.60532656	SMN	30	GM00232	48							
13088	SMN1-54 m01	122.4086013	17.92266544	SMN	30	GM00232	48							
13088	SMN1-54 m01	204.2081143	30.56033012	SMN	30	GM00232	48							
13088	SMN1-54 m01	129.1262027	31.81232816	SMN	30	GM00232	48							
13088	SMN1-	217.807888	16.0334	SMN	30	GM00	48	unc-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	54 m01	3	6823			232		293 m01						
13088	SMN1-54 m01	153.4412306	8.890852385	SMN	30	GM00232	48	unc-293 m01	30	nM				
	Ctrl Lipo	100	6.761629592	SMN	0	GM00232	48							
	Ctrl Un	98.26603127	6.064063358	SMN	0	GM00232	48							
11394	SMN1-47 m01	256.7841887	12.07500321	SMN	30	GM22592	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	242.0019593	8.277263941	SMN	30	GM22592	48	SMN1-53 m03	30	nM				
13091	SMN1-57 m01	153.1767347	18.48962973	SMN	30	GM22592	48	SMN1-53 m03	30	nM				
10070	SMN1-67 m01	242.1428431	23.21158961	SMN	30	GM22592	48	SMN1-53 m03	30	nM				
10169	SMN1-79 m01	166.5472176	4.640871691	SMN	30	GM22592	48	SMN1-53 m03	30	nM				
10170	SMN1-80 m01	120.3100699	3.612488754	SMN	30	GM22592	48	SMN1-53 m03	30	nM				
10763	SMN1-84 m01	169.6493911	7.221967399	SMN	30	GM22592	48	SMN1-53 m03	30	nM				
11419	SMN1-93 m01	199.0900139	15.06506215	SMN	30	GM22592	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	219.571192	19.74854879	SMN	30	GM22592	48	SMN1-54 m01	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
11395	SMN1-48 m01	249.181399	29.0419 383	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
13091	SMN1-57 m01	132.763195 5	8.92899 8838	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
10070	SMN1-67 m01	206.347379 4	19.6710 1107	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
10169	SMN1-79 m01	164.516427 1	14.0095 5691	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
10170	SMN1-80 m01	125.034643	13.1401 2826	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
10763	SMN1-84 m01	162.622615 7	17.3229 3012	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
11419	SMN1-93 m01	208.604737 1	30.7861 7445	SMN	30	GM22 592	48	SMN1-54 m01	30	nM				
13088	SMN1-54 m01	186.410359 4	19.4828 0431	SMN	30	GM22 592	48							
13088	SMN1-54 m01	137.103984 2	21.2040 8858	SMN	30	GM22 592	48							
13088	SMN1-54 m01	201.306996 5	1.46637 1524	SMN	30	GM22 592	48							
13088	SMN1-54 m01	155.354693	17.7880 3418	SMN	30	GM22 592	48							
13088	SMN1-54 m01	229.277966 3	12.7649 8706	SMN	30	GM22 592	48	unc-293 m01	30	nM				
13088	SMN1-54 m01	192.959283 6	16.0208 7607	SMN	30	GM22 592	48	unc-293	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
								m01						
	Ctrl Lipo	100	6.31936 2149	SMN	0	GM22 592	48							
	Ctrl Un	112.191266 2	11.4172 735	SMN	0	GM22 592	48							
11394	SMN1- 47 m01	344.316988	29.1160 3665	SMN	30	GM09 677	48	SMN1- 53 m03	30	nM				
11395	SMN1- 48 m01	382.363306 6	21.5953 7778	SMN	30	GM09 677	48	SMN1- 53 m03	30	nM				
13091	SMN1- 57 m01	186.476462 2	4.50803 9586	SMN	30	GM09 677	48	SMN1- 53 m03	30	nM				
10070	SMN1- 67 m01	345.966270 3	9.72790 2656	SMN	30	GM09 677	48	SMN1- 53 m03	30	nM				
10169	SMN1- 79 m01	215.776437 5	2.89773 1248	SMN	30	GM09 677	48	SMN1- 53 m03	30	nM				
10170	SMN1- 80 m01	156.460343 6	4.96821 3123	SMN	30	GM09 677	48	SMN1- 53 m03	30	nM				
10763	SMN1- 84 m01	186.792310 4	38.0157 7691	SMN	30	GM09 677	48	SMN1- 53 m03	30	nM				
11419	SMN1- 93 m01	354.314246 5	4.02182 2699	SMN	30	GM09 677	48	SMN1- 53 m03	30	nM				
11394	SMN1- 47 m01	308.540373	19.2780 7951	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				
11395	SMN1- 48 m01	332.587526 7	30.2077 0293	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				
13091	SMN1- 57 m01	164.425952 6	16.9552 4984	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10070	SMN1-67 m01	257.962829 5	26.9644 1743	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
10169	SMN1-79 m01	182.725015 7	5.17715 0817	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
10170	SMN1-80 m01	140.890286 8	16.8142 5437	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
10763	SMN1-84 m01	160.674167	23.7151 9014	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
11419	SMN1-93 m01	286.605949 8	10.3914 4849	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
13088	SMN1-54 m01	242.885193 3	28.8338 7457	SMN	30	GM09 677	48							
13088	SMN1-54 m01	196.326276 8	9.19228 1606	SMN	30	GM09 677	48							
13088	SMN1-54 m01	242.776253 3	11.4221 9147	SMN	30	GM09 677	48							
13088	SMN1-54 m01	198.766792 2	9.31387 7514	SMN	30	GM09 677	48							
13088	SMN1-54 m01	281.608592 3	23.1878 9547	SMN	30	GM09 677	48	unc-293 m01	30	nM				
13088	SMN1-54 m01	231.246464 9	3.90749 4972	SMN	30	GM09 677	48	unc-293 m01	30	nM				
	Ctrl Lipo	100	6.75877 5023	SMN	0	GM09 677	48							
	Ctrl Un	107.957282	5.44956	SMN	0	GM09	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
			3135			677								
11394	SMN1-47 m01	169.996224 2	54.7707 7224	SMN	30	GM00 321	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	206.110085 7	48.7047 3514	SMN	30	GM00 321	48	SMN1-53 m03	30	nM				
10169	SMN1-79 m01	162.786201 6	9.12441 7167	SMN	30	GM00 321	48	SMN1-53 m03	30	nM				
10170	SMN1-80 m01	118.094000 1	15.1968 8728	SMN	30	GM00 321	48	SMN1-53 m03	30	nM				
10763	SMN1-84 m01	129.947452 5	39.7119 416	SMN	30	GM00 321	48	SMN1-53 m03	30	nM				
11419	SMN1-93 m01	172.132509 2	35.7159 8191	SMN	30	GM00 321	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	156.396807	17.5827 4339	SMN	30	GM00 321	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	198.482886 5	13.7414 1421	SMN	30	GM00 321	48	SMN1-53 m03	30	nM				
10169	SMN1-79 m01	178.665704 4	12.6478 2581	SMN	30	GM00 321	48	SMN1-53 m03	30	nM				
10170	SMN1-80 m01	141.810720 1	4.83516 6003	SMN	30	GM00 321	48	SMN1-53 m03	30	nM				
10763	SMN1-84 m01	113.818763 4	42.8529 6644	SMN	30	GM00 321	48	SMN1-53 m03	30	nM				
11419	SMN1-93 m01	154.083947 5	7.82821 8526	SMN	30	GM00 321	48	SMN1-53 m03	30	nM				
13088	SMN1-54 m01	126.114016 9	36.4872 1359	SMN	30	GM00 321	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
13088	SMN1-54 m01	106.846023 9	9.89826 1651	SMN	30	GM00 321	48							
	Ctrl Lipo	100	20.1524 9396	SMN	0	GM00 321	48							
	Ctrl Un	99.0322493 4	11.0206 8125	SMN	0	GM00 321	48							
11394	SMN1-47 m02	126.552290 5	8.92581 3016	SMN	30	GM03 813	48							
11395	SMN1-48 m05	137.835525 7	6.60417 2068	SMN	30	GM03 813	48							
13088	SMN1-53 m02	215.423272 9	4.18503 0467	SMN	30	GM03 813	48							
13091	SMN1-57 m02	139.904184 5	17.9827 6801	SMN	30	GM03 813	48							
10169	SMN1-79 m02	80.0152228 5	3.20878 9172	SMN	30	GM03 813	48							
10170	SMN1-80 m02	104.902781 8	7.37945 2998	SMN	30	GM03 813	48							
10763	SMN1-84 m02	117.872491 5	8.67267 7348	SMN	30	GM03 813	48							
11419	SMN1-93 m02	125.817990 6	29.4227 9354	SMN	30	GM03 813	48							
11394	SMN1-47 m01	102.904401 7	16.5737 1676	SMN	30	GM03 813	48							
11395	SMN1-48 m01	118.436715 3	2.83442 2697	SMN	30	GM03 813	48							
13088	SMN1-	203.133034	2.02630	SMN	30	GM03	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	54 m01	6	4808			813								
13088	SMN1-54 m01	177.9777764	12.89399624	SMN	30	GM03813	48							
13091	SMN1-57 m01	128.6495166	9.597004916	SMN	30	GM03813	48							
10070	SMN1-67 m01	154.1330357	4.487120381	SMN	30	GM03813	48							
10169	SMN1-79 m01	55.51118468	7.150544063	SMN	30	GM03813	48							
10170	SMN1-80 m01	59.96981341	6.115298097	SMN	30	GM03813	48							
10763	SMN1-84 m01	66.62100589	9.045089295	SMN	30	GM03813	48							
11419	SMN1-93 m01	150.5043366	16.69522713	SMN	30	GM03813	48							
11394	SMN1-47 m02	244.0307132	21.78835051	SMN	30	GM03813	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m05	225.4734281	70.23035621	SMN	30	GM03813	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	316.031249	8.504085462	SMN	30	GM03813	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	372.0214635	60.08393945	SMN	30	GM03813	48	SMN1-53 m03	30	nM				
	Ctrl Lipo	100	7.161941326	SMN	0	GM03813	48							
	Ctrl Un	118.5568205	14.47017987	SMN	0	GM03813	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
11394	SMN1-47 m02	90.033766	22.2812 0495	SMN	30	GM09 677	48							
11395	SMN1-48 m05	128.008122 5	18.5395 4294	SMN	30	GM09 677	48							
13088	SMN1-53 m02	244.748344 2	18.6368 3793	SMN	30	GM09 677	48							
13091	SMN1-57 m02	160.800448 4	16.0078 0502	SMN	30	GM09 677	48							
10169	SMN1-79 m02	61.5656907 6	11.7413 9338	SMN	30	GM09 677	48							
10170	SMN1-80 m02	85.3038450 1	0.81872 7676	SMN	30	GM09 677	48							
10763	SMN1-84 m02	130.459624 1	10.4295 8772	SMN	30	GM09 677	48							
11419	SMN1-93 m02	174.505331 9	13.0476 7813	SMN	30	GM09 677	48							
11394	SMN1-47 m01	86.8197229 1	2.05737 7192	SMN	30	GM09 677	48							
11395	SMN1-48 m01	108.442478 3	10.7423 4853	SMN	30	GM09 677	48							
13088	SMN1-54 m01	232.190234 5	24.3717 8906	SMN	30	GM09 677	48							
13088	SMN1-54 m01	143.813146 7	16.0071 7246	SMN	30	GM09 677	48							
13091	SMN1-57 m01	139.293681 4	5.50040 0569	SMN	30	GM09 677	48							
10070	SMN1-	143.578923	8.92614	SMN	30	GM09	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	67 m01	5	8545			677								
10169	SMN1-79 m01	45.0024495	3.80694 7422	SMN	30	GM09 677	48							
10170	SMN1-80 m01	42.0750953 5	13.9671 8413	SMN	30	GM09 677	48							
10763	SMN1-84 m01	51.5152899 6	6.31638 6967	SMN	30	GM09 677	48							
11419	SMN1-93 m01	132.344045 5	40.0948 8984	SMN	30	GM09 677	48							
11394	SMN1-47 m02	209.031559 4	34.7655 8333	SMN	30	GM09 677	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m05	154.494537	75.5902 6865	SMN	30	GM09 677	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	286.518059 9	15.4596 5533	SMN	30	GM09 677	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	301.807054 8	62.5181 2181	SMN	30	GM09 677	48	SMN1-53 m03	30	nM				
	Ctrl Lipo	100	12.8317 5529	SMN	0	GM09 677	48							
	Ctrl Un	96.8840108 3	17.9353 5029	SMN	0	GM09 677	48							
11394	SMN1-47 m02	147.083765 3	5.13720 3261	SMN	30	GM00 232	48							
11395	SMN1-48 m05	167.422131 3	8.64829 5729	SMN	30	GM00 232	48							
13088	SMN1-53 m02	229.048299 1	16.1541 6714	SMN	30	GM00 232	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
13091	SMN1-57 m02	130.732097 1	13.3499 8639	SMN	30	GM00 232	48							
10169	SMN1-79 m02	81.8672331 6	10.2829 1787	SMN	30	GM00 232	48							
10170	SMN1-80 m02	102.219329 1	7.97730 8961	SMN	30	GM00 232	48							
10763	SMN1-84 m02	131.000654 2	8.80202 0603	SMN	30	GM00 232	48							
11419	SMN1-93 m02	115.635348 7	8.80414 148	SMN	30	GM00 232	48							
11394	SMN1-47 m01	143.723571 6	36.7668 9915	SMN	30	GM00 232	48							
11395	SMN1-48 m01	144.967606 5	3.46797 1955	SMN	30	GM00 232	48							
13088	SMN1-54 m01	308.976438 2	54.9897 2252	SMN	30	GM00 232	48							
13088	SMN1-54 m01	182.473410 7	8.81466 5571	SMN	30	GM00 232	48							
13091	SMN1-57 m01	157.904188	43.2883 8552	SMN	30	GM00 232	48							
10070	SMN1-67 m01	162.640665 2	10.5744 3349	SMN	30	GM00 232	48							
10169	SMN1-79 m01	74.1094351 1	5.41620 3761	SMN	30	GM00 232	48							
10170	SMN1-80 m01	66.4660779 6	7.44689 0726	SMN	30	GM00 232	48							
10763	SMN1-	75.9409879	3.63311	SMN	30	GM00	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	84 m01		4664			232								
11419	SMN1-93 m01	156.9179012	10.12079452	SMN	30	GM00232	48							
11394	SMN1-47 m02	238.8540547	28.28968929	SMN	30	GM00232	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m05	239.5385809	24.97736551	SMN	30	GM00232	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	342.5882705	14.79874652	SMN	30	GM00232	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	400.7167927	28.88122237	SMN	30	GM00232	48	SMN1-53 m03	30	nM				
	Ctrl Lipo	100	15.10590842	SMN	0	GM00232	48							
	Ctrl Un	120.5277771	13.7942099	SMN	0	GM00232	48							
11394	SMN1-47 m02	115.0514912	8.549166314	SMN	30	GM22592	48							
11395	SMN1-48 m05	134.9651236	12.32385693	SMN	30	GM22592	48							
13088	SMN1-53 m02	206.5334695	19.72309475	SMN	30	GM22592	48							
13091	SMN1-57 m02	154.2725322	16.26206505	SMN	30	GM22592	48							
10169	SMN1-79 m02	76.73846737	8.012403799	SMN	30	GM22592	48							
10170	SMN1-80 m02	95.59491384	5.822818022	SMN	30	GM22592	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10763	SMN1-84 m02	115.7188496	6.769715464	SMN	30	GM22592	48							
11419	SMN1-93 m02	122.2945785	25.69284425	SMN	30	GM22592	48							
11394	SMN1-47 m01	110.9510851	2.633988282	SMN	30	GM22592	48							
11395	SMN1-48 m01	121.4370531	9.179057189	SMN	30	GM22592	48							
13088	SMN1-54 m01	232.0454398	22.19979354	SMN	30	GM22592	48							
13088	SMN1-54 m01	161.1507777	15.66399454	SMN	30	GM22592	48							
13091	SMN1-57 m01	133.2573568	5.466033463	SMN	30	GM22592	48							
10070	SMN1-67 m01	134.8525367	1.979038751	SMN	30	GM22592	48							
10169	SMN1-79 m01	59.19206971	4.78443798	SMN	30	GM22592	48							
10170	SMN1-80 m01	41.48792813	9.030546741	SMN	30	GM22592	48							
10763	SMN1-84 m01	67.68606377	4.533176368	SMN	30	GM22592	48							
11419	SMN1-93 m01	137.2074396	7.595568307	SMN	30	GM22592	48							
11394	SMN1-47 m02	263.9141425	15.073073	SMN	30	GM22592	48	SMN1-53 m03	30	nM				
11395	SMN1-	222.068908	22.5288	SMN	30	GM22	48	SMN1-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	48 m05	4	1038			592		53 m03						
11394	SMN1-47 m01	308.6195056	30.37373327	SMN	30	GM22592	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	324.2658286	25.3912316	SMN	30	GM22592	48	SMN1-53 m03	30	nM				
	Ctrl Lipo	100	10.72332707	SMN	0	GM22592	48							
	Ctrl Un	92.28090431	15.99715922	SMN	0	GM22592	48							
11381	SMN1-34 m01	266.1010351	8.576208449	SMN	30	GM09677	48							
11382	SMN1-35 m01	174.1843122	9.876658342	SMN	30	GM09677	48							
11384	SMN1-37 m01	314.9269174	44.28918647	SMN	30	GM09677	48							
13088	SMN1-54 m01	234.0780919	13.11265041	SMN	30	GM09677	48							
13088	SMN1-54 m01	238.7435794	41.29804911	SMN	30	GM09677	48							
13092	SMN1-58 m01	259.3952644	18.29820996	SMN	30	GM09677	48							
10087	SMN1-77 m01	357.5076186	41.88916014	SMN	30	GM09677	48							
10339	SMN1-83 m01	262.7965115	13.7897363	SMN	30	GM09677	48							
10952	SMN1-88 m01	276.9889932	10.40083586	SMN	30	GM09677	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10953	SMN1-89 m01	232.3062475	25.70363687	SMN	30	GM09677	48							
10954	SMN1-90 m01	311.8738131	27.86801601	SMN	30	GM09677	48							
11419	SMN1-93 m01	146.161142	38.15640574	SMN	30	GM09677	48							
12369	SMN1-101 m01	256.0230323	31.52233699	SMN	30	GM09677	48							
13095	unc-232 m01	194.9119438	24.11290814	SMN	30	GM09677	48							
	Ctrl Lipo	100	11.07515076	SMN	0	GM09677	48							
	Ctrl Un	89.31850912	3.057159805	SMN	0	GM09677	48							
11393	SMN1-46 m01	253.4557248	23.33374049	SMN	30	GM09677	48	SMN1-34 m01	30	nM				
11394	SMN1-47 m01	149.7906772	11.590122	SMN	30	GM09677	48	SMN1-34 m01	30	nM				
11395	SMN1-48 m01	267.4489472	22.10870204	SMN	30	GM09677	48	SMN1-34 m01	30	nM				
10084	SMN1-76 m01	232.7920219	15.68435913	SMN	30	GM09677	48	SMN1-34 m01	30	nM				
10169	SMN1-79 m01	68.97283797	4.681859133	SMN	30	GM09677	48	SMN1-34 m01	30	nM				
10170	SMN1-	65.9649138	3.24037	SMN	30	GM09	48	SMN1-	30	nM				

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SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	80 m01	5	8402			677		34 m01						
10763	SMN1-84 m01	187.781913 1	21.3800 5315	SMN	30	GM09 677	48	SMN1-34 m01	30	nM				
13095	unc-232 m01	347.342112 3	6.33946 1734	SMN	30	GM09 677	48	SMN1-34 m01	30	nM				
11393	SMN1-46 m01	80.4085091 3	15.9940 5468	SMN	30	GM09 677	48	SMN1-35 m01	30	nM				
11394	SMN1-47 m01	73.5831052 4	11.4327 5864	SMN	30	GM09 677	48	SMN1-35 m01	30	nM				
11395	SMN1-48 m01	109.660118 9	6.35184 2709	SMN	30	GM09 677	48	SMN1-35 m01	30	nM				
10084	SMN1-76 m01	133.254119 5	9.11183 3857	SMN	30	GM09 677	48	SMN1-35 m01	30	nM				
10169	SMN1-79 m01	48.5161284 4	2.83077 0061	SMN	30	GM09 677	48	SMN1-35 m01	30	nM				
10170	SMN1-80 m01	60.1055554 8	5.16353 1837	SMN	30	GM09 677	48	SMN1-35 m01	30	nM				
10763	SMN1-84 m01	57.9586207 9	6.70089 1036	SMN	30	GM09 677	48	SMN1-35 m01	30	nM				
13095	unc-232 m01	219.698743 4	20.0933 1599	SMN	30	GM09 677	48	SMN1-35 m01	30	nM				
11393	SMN1-46 m01	127.244442 2	9.27707 9056	SMN	30	GM09 677	48	SMN1-37 m01	30	nM				
11394	SMN1-47 m01	134.643496 7	2.80872 7435	SMN	30	GM09 677	48	SMN1-37 m01	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
11395	SMN1-48 m01	301.7170718	18.44881367	SMN	30	GM09677	48	SMN1-37 m01	30	nM				
10084	SMN1-76 m01	225.5487222	2.599808728	SMN	30	GM09677	48	SMN1-37 m01	30	nM				
10169	SMN1-79 m01	57.77806317	8.385172531	SMN	30	GM09677	48	SMN1-37 m01	30	nM				
10170	SMN1-80 m01	60.09608405	2.144153192	SMN	30	GM09677	48	SMN1-37 m01	30	nM				
10763	SMN1-84 m01	163.1802862	23.86345926	SMN	30	GM09677	48	SMN1-37 m01	30	nM				
13095	unc-232 m01	279.9704092	7.895824306	SMN	30	GM09677	48	SMN1-37 m01	30	nM				
11393	SMN1-46 m01	327.4542383	29.79783494	SMN	30	GM09677	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	340.9640282	63.80467341	SMN	30	GM09677	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	467.0604003	23.38817389	SMN	30	GM09677	48	SMN1-53 m03	30	nM				
10084	SMN1-76 m01	159.9744393	30.7127566	SMN	30	GM09677	48	SMN1-53 m03	30	nM				
10169	SMN1-79 m01	176.0658821	13.66531954	SMN	30	GM09677	48	SMN1-53 m03	30	nM				
10170	SMN1-80 m01	126.703895	11.56623408	SMN	30	GM09677	48	SMN1-53 m03	30	nM				
10763	SMN1-84 m01	150.5711773	1.066165278	SMN	30	GM09677	48	SMN1-53 m03	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
13095	unc-232 m01	237.782576 9	25.5344 3885	SMN	30	GM09 677	48	SMN1- 53 m03	30	nM				
11393	SMN1-46 m01	351.259642	29.0634 0325	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				
11394	SMN1-47 m01	317.534109 9	65.1465 3138	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				
11395	SMN1-48 m01	409.315744 2	40.5648 7597	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				
10084	SMN1-76 m01	168.810375 4	44.6303 9296	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				
10169	SMN1-79 m01	219.501270 5	14.8190 0065	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				
10170	SMN1-80 m01	165.827103 6	22.3867 6092	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				
10763	SMN1-84 m01	152.135667 4	12.6044 1383	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				
13095	unc-232 m01	214.382692 3	31.1477 8407	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				
11393	SMN1-46 m01	195.878203 6	20.1232 8992	SMN	30	GM09 677	48	SMN1- 58 m01	30	nM				
11394	SMN1-47 m01	199.764084 7	62.4599 4953	SMN	30	GM09 677	48	SMN1- 58 m01	30	nM				
11395	SMN1-48 m01	236.605290 4	30.9953 6438	SMN	30	GM09 677	48	SMN1- 58 m01	30	nM				
10084	SMN1-	138.317712	17.0404	SMN	30	GM09	48	SMN1-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	76 m01	4	7355			677		58 m01						
10169	SMN1-79 m01	80.4222872 2	4.83256 6581	SMN	30	GM09 677	48	SMN1-58 m01	30	nM				
10170	SMN1-80 m01	63.4029999 6	7.83887 3489	SMN	30	GM09 677	48	SMN1-58 m01	30	nM				
10763	SMN1-84 m01	166.718944 4	17.4686 0633	SMN	30	GM09 677	48	SMN1-58 m01	30	nM				
13095	unc-232 m01	246.312016 4	17.1485 2451	SMN	30	GM09 677	48	SMN1-58 m01	30	nM				
11393	SMN1-46 m01	102.736463 2	15.2731 1625	SMN	30	GM09 677	48	SMN1-77 m01	30	nM				
11394	SMN1-47 m01	99.0491312 5	7.75009 1272	SMN	30	GM09 677	48	SMN1-77 m01	30	nM				
11395	SMN1-48 m01	206.088415 7	50.1392 8608	SMN	30	GM09 677	48	SMN1-77 m01	30	nM				
10084	SMN1-76 m01	129.785595 8	44.0666 538	SMN	30	GM09 677	48	SMN1-77 m01	30	nM				
10169	SMN1-79 m01	46.6663897 4	2.70290 3651	SMN	30	GM09 677	48	SMN1-77 m01	30	nM				
10170	SMN1-80 m01	45.4193817 4	9.51146 5496	SMN	30	GM09 677	48	SMN1-77 m01	30	nM				
10763	SMN1-84 m01	66.0799921 4	30.8086 0679	SMN	30	GM09 677	48	SMN1-77 m01	30	nM				
13095	unc-232 m01	332.715543 5	90.5618 9738	SMN	30	GM09 677	48	SMN1-77 m01	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
11393	SMN1-46 m01	127.3612987	14.35364673	SMN	30	GM09677	48	SMN1-83 m01	30	nM				
11394	SMN1-47 m01	113.1825501	26.13819201	SMN	30	GM09677	48	SMN1-83 m01	30	nM				
11395	SMN1-48 m01	274.9202985	84.30692648	SMN	30	GM09677	48	SMN1-83 m01	30	nM				
10084	SMN1-76 m01	137.9757751	41.57183759	SMN	30	GM09677	48	SMN1-83 m01	30	nM				
10169	SMN1-79 m01	54.8963944	5.173456821	SMN	30	GM09677	48	SMN1-83 m01	30	nM				
10170	SMN1-80 m01	51.00058692	5.485326926	SMN	30	GM09677	48	SMN1-83 m01	30	nM				
10763	SMN1-84 m01	70.05852863	33.18768903	SMN	30	GM09677	48	SMN1-83 m01	30	nM				
13095	unc-232 m01	291.5327357	76.69342051	SMN	30	GM09677	48	SMN1-83 m01	30	nM				
11393	SMN1-46 m01	97.54879308	7.984224225	SMN	30	GM09677	48	SMN1-88 m01	30	nM				
11394	SMN1-47 m01	99.49338097	9.425222184	SMN	30	GM09677	48	SMN1-88 m01	30	nM				
11395	SMN1-48 m01	134.5138915	2.673078157	SMN	30	GM09677	48	SMN1-88 m01	30	nM				
10084	SMN1-76 m01	89.46574217	14.96122915	SMN	30	GM09677	48	SMN1-88 m01	30	nM				
10169	SMN1-79 m01	52.09301003	6.029231725	SMN	30	GM09677	48	SMN1-88 m01	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10170	SMN1-80 m01	50.3966695 1	5.46831 6304	SMN	30	GM09 677	48	SMN1-88 m01	30	nM				
10763	SMN1-84 m01	72.0589696 6	7.69322 5228	SMN	30	GM09 677	48	SMN1-88 m01	30	nM				
13095	unc-232 m01	258.297008 8	23.7510 3379	SMN	30	GM09 677	48	SMN1-88 m01	30	nM				
11393	SMN1-46 m01	111.394592 7	8.46935 469	SMN	30	GM09 677	48	SMN1-89 m01	30	nM				
11394	SMN1-47 m01	134.080098 4	5.88947 3238	SMN	30	GM09 677	48	SMN1-89 m01	30	nM				
11395	SMN1-48 m01	154.537184 4	14.6630 4426	SMN	30	GM09 677	48	SMN1-89 m01	30	nM				
10084	SMN1-76 m01	81.3209120 4	7.79318 9178	SMN	30	GM09 677	48	SMN1-89 m01	30	nM				
10169	SMN1-79 m01	59.1510850 5	3.86115 7383	SMN	30	GM09 677	48	SMN1-89 m01	30	nM				
10170	SMN1-80 m01	51.0603399 8	2.62541 4902	SMN	30	GM09 677	48	SMN1-89 m01	30	nM				
10763	SMN1-84 m01	52.0353148 8	2.58605 7521	SMN	30	GM09 677	48	SMN1-89 m01	30	nM				
13095	unc-232 m01	205.812243	8.65520 3726	SMN	30	GM09 677	48	SMN1-89 m01	30	nM				
11393	SMN1-46 m01	60.3801076 1	26.9371 3023	SMN	30	GM09 677	48	SMN1-90 m01	30	nM				
11394	SMN1-	73.2106465	11.8663	SMN	30	GM09	48	SMN1-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	47 m01	6	6634			677		90 m01						
11395	SMN1-48 m01	120.3004223	5.290432429	SMN	30	GM09 677	48	SMN1-90 m01	30	nM				
10084	SMN1-76 m01	61.03305458	12.52840288	SMN	30	GM09 677	48	SMN1-90 m01	30	nM				
10169	SMN1-79 m01	61.30531982	5.100035632	SMN	30	GM09 677	48	SMN1-90 m01	30	nM				
10170	SMN1-80 m01	47.41271214	4.412147652	SMN	30	GM09 677	48	SMN1-90 m01	30	nM				
10763	SMN1-84 m01	68.78599778	7.306852898	SMN	30	GM09 677	48	SMN1-90 m01	30	nM				
13095	unc-232 m01	298.9231904	17.81604307	SMN	30	GM09 677	48	SMN1-90 m01	30	nM				
11393	SMN1-46 m01	51.15182114	4.185687029	SMN	30	GM09 677	48	SMN1-93 m01	30	nM				
11394	SMN1-47 m01	71.91986489	4.189373926	SMN	30	GM09 677	48	SMN1-93 m01	30	nM				
11395	SMN1-48 m01	107.0025374	1.965050508	SMN	30	GM09 677	48	SMN1-93 m01	30	nM				
10084	SMN1-76 m01	79.16390805	14.57997892	SMN	30	GM09 677	48	SMN1-93 m01	30	nM				
10169	SMN1-79 m01	48.26655805	4.637123169	SMN	30	GM09 677	48	SMN1-93 m01	30	nM				
10170	SMN1-80 m01	43.71205821	8.823085289	SMN	30	GM09 677	48	SMN1-93 m01	30	nM				
10763	SMN1-	58.1934661	2.55342	SMN	30	GM09	48	SMN1-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	84 m01	6	1271			677		93 m01						
13095	unc-232 m01	158.630481 4	25.1374 2548	SMN	30	GM09 677	48	SMN1- 93 m01	30	nM				
11393	SMN1-46 m01	158.016327 5	7.48470 2879	SMN	30	GM09 677	48	SMN1- 101 m01	30	nM				
11394	SMN1-47 m01	116.494958 7	15.4102 7215	SMN	30	GM09 677	48	SMN1- 101 m01	30	nM				
11395	SMN1-48 m01	130.169789 8	9.62441 8302	SMN	30	GM09 677	48	SMN1- 101 m01	30	nM				
10084	SMN1-76 m01	94.1593563 3	20.2004 2447	SMN	30	GM09 677	48	SMN1- 101 m01	30	nM				
10169	SMN1-79 m01	86.9072706 4	2.66146 9839	SMN	30	GM09 677	48	SMN1- 101 m01	30	nM				
10170	SMN1-80 m01	77.3542077 4	7.76721 7871	SMN	30	GM09 677	48	SMN1- 101 m01	30	nM				
10763	SMN1-84 m01	79.3870998 1	1.68477 9761	SMN	30	GM09 677	48	SMN1- 101 m01	30	nM				
13095	unc-232	219.107799 5	29.3581 6139	SMN	30	GM09 677	48	SMN1- 101	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	m01							m01						
11384	SMN1-37 m01	200.0471527	13.53724885	SMN	30	GM09677	48							
11394	SMN1-47 m01	80.1277224	8.20792981	SMN	30	GM09677	48							
11395	SMN1-48 m01	93.87926117	5.052411604	SMN	30	GM09677	48							
13088	SMN1-54 m01	249.7926835	12.36417064	SMN	30	GM09677	48							
13088	SMN1-54 m01	182.0980332	9.470894217	SMN	30	GM09677	48							
10087	SMN1-77 m01	355.1911972	41.85596589	SMN	30	GM09677	48							
10339	SMN1-83 m01	237.2426712	10.11437826	SMN	30	GM09677	48							
10954	SMN1-90 m01	182.6483319	16.52600897	SMN	30	GM09677	48							
11384	SMN1-37 m01	211.4749202	3.289018632	SMN	30	GM09677	48	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	258.7686434	28.04307123	SMN	30	GM09677	48	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	322.4456162	33.95533387	SMN	30	GM09677	48	SMN1-53 m03	30	nM				
10087	SMN1-77 m01	279.6837708	36.25789452	SMN	30	GM09677	48	SMN1-53 m03	30	nM				
10339	SMN1-83 m01	298.0343763	2.704395802	SMN	30	GM09677	48	SMN1-53 m03	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10954	SMN1-90 m01	281.9254251	7.270012805	SMN	30	GM09677	48	SMN1-53 m03	30	nM				
11384	SMN1-37 m01	246.6395044	18.18532411	SMN	30	GM09677	48	SMN1-54 m01	30	nM				
11394	SMN1-47 m01	285.8234231	14.24897686	SMN	30	GM09677	48	SMN1-54 m01	30	nM				
11395	SMN1-48 m01	243.2192137	11.99467347	SMN	30	GM09677	48	SMN1-54 m01	30	nM				
10087	SMN1-77 m01	214.9368614	15.26263125	SMN	30	GM09677	48	SMN1-54 m01	30	nM				
10339	SMN1-83 m01	188.6702007	6.990461422	SMN	30	GM09677	48	SMN1-54 m01	30	nM				
10954	SMN1-90 m01	227.316235	7.643374884	SMN	30	GM09677	48	SMN1-54 m01	30	nM				
11394	SMN1-47 m01	132.5679343	5.409470692	SMN	30	GM09677	48	SMN1-37 m01	30	nM				
11395	SMN1-48 m01	250.0145895	13.70921214	SMN	30	GM09677	48	SMN1-37 m01	30	nM				
10087	SMN1-77 m01	377.3697441	58.11703004	SMN	30	GM09677	48	SMN1-37 m01	30	nM				
10339	SMN1-83 m01	303.2461466	57.47350558	SMN	30	GM09677	48	SMN1-37 m01	30	nM				
10954	SMN1-90 m01	204.3185375	13.70239844	SMN	30	GM09677	48	SMN1-37 m01	30	nM				
11394	SMN1-47 m01	123.6244575	14.91544556	SMN	30	GM09677	48	SMN1-77 m01	30	nM				
11395	SMN1-	197.639371	22.7015	SMN	30	GM09	48	SMN1-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	48 m01	6	3004			677		77 m01						
10339	SMN1-83 m01	376.4251584	32.45275792	SMN	30	GM09 677	48	SMN1-77 m01	30	nM				
10954	SMN1-90 m01	418.6174953	45.1816772	SMN	30	GM09 677	48	SMN1-77 m01	30	nM				
11394	SMN1-47 m01	143.9588406	12.98314569	SMN	30	GM09 677	48	SMN1-83 m01	30	nM				
11395	SMN1-48 m01	243.6528705	11.65345244	SMN	30	GM09 677	48	SMN1-83 m01	30	nM				
10339	SMN1-83 m01	274.033755	37.19873905	SMN	30	GM09 677	48	SMN1-90 m01	30	nM				
11394	SMN1-47 m01	129.2883101	13.14829025	SMN	30	GM09 677	48	SMN1-90 m01	30	nM				
11395	SMN1-48 m01	154.1086243	2.007404147	SMN	30	GM09 677	48	SMN1-90 m01	30	nM				
13088	SMN1-54 m01	395.3696421	20.97039108	SMN	30	GM09 677	48	unc-293 m01	30	nM				
13088	SMN1-54 m01	267.8543619	53.15744466	SMN	30	GM09 677	48	unc-293 m01	30	nM				
11384	SMN1-37 m01	311.263051	10.38744086	SMN	30	GM09 677	48	unc-293 m01	30	nM				
10087	SMN1-77 m01	493.4551625	16.62538624	SMN	30	GM09 677	48	unc-293 m01	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10339	SMN1-83 m01	363.630871 3	45.7648 2124	SMN	30	GM09 677	48	unc-293 m01	30	nM				
10954	SMN1-90 m01	325.174367 8	28.6220 7369	SMN	30	GM09 677	48	unc-293 m01	30	nM				
11384	SMN1-37 m01	332.155504 6	14.6526 1106	SMN	30	GM09 677	48	SMN1-77 m01	30	nM	SMN1-83 m01	30	nM	
11384	SMN1-37 m01	281.805113 8	20.2063 893	SMN	30	GM09 677	48	SMN1-77 m01	30	nM	SMN1-90 m01	30	nM	
11384	SMN1-37 m01	274.282241 9	12.3909 572	SMN	30	GM09 677	48	SMN1-83 m01	30	nM	SMN1-90 m01	30	nM	
10087	SMN1-77 m01	341.070952	13.0929 2861	SMN	30	GM09 677	48	SMN1-83 m01	30	nM	SMN1-90 m01	30	nM	
11384	SMN1-37 m01	318.317982 5	12.2682 1967	SMN	30	GM09 677	48	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM	
11384	SMN1-37 m01	433.864760 6	19.2173 564	SMN	30	GM09 677	48	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM	
11384	SMN1-37 m01	320.425777	32.5110 8915	SMN	30	GM09 677	48	SMN1-47 m01	30	nM	SMN1-54 m01	30	nM	
11384	SMN1-37 m01	347.077126 4	4.21106 2011	SMN	30	GM09 677	48	SMN1-48 m01	30	nM	SMN1-54 m01	30	nM	
10087	SMN1-77 m01	333.874176 8	17.8523 1294	SMN	30	GM09 677	48	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM	
10087	SMN1-77 m01	491.014485 2	149.260 7269	SMN	30	GM09 677	48	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM	
10087	SMN1-	339.767545	47.8706	SMN	30	GM09	48	SMN1-	30	nM	SMN1-	30	nM	

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	77 m01	9	3089			677		47 m01			54 m01			
10087	SMN1-77 m01	494.8456358	98.86423232	SMN	30	GM09 677	48	SMN1-48 m01	30	nM	SMN1-54 m01	30	nM	
10954	SMN1-90 m01	485.9393901	64.50230323	SMN	30	GM09 677	48	SMN1-47 m01	30	nM	SMN1-53 m03	30	nM	
10954	SMN1-90 m01	570.545191	84.54230941	SMN	30	GM09 677	48	SMN1-48 m01	30	nM	SMN1-53 m03	30	nM	
10954	SMN1-90 m01	322.4814988	13.04472971	SMN	30	GM09 677	48	SMN1-47 m01	30	nM	SMN1-54 m01	30	nM	
10954	SMN1-90 m01	486.6710018	17.67224981	SMN	30	GM09 677	48	SMN1-48 m01	30	nM	SMN1-54 m01	30	nM	
13088	SMN1-54 m01	282.4977957	54.0564563	SMN	60	GM09 677	48							
13088	SMN1-54 m01	189.3914304	38.06613473	SMN	90	GM09 677	48							
13095	unc-232 m01	151.3940636	21.34019565	SMN	30	GM09 677	48							
13095	unc-232 m01	212.9787191	7.951522571	SMN	60	GM09 677	48							
13095	unc-232 m01	328.8483763	100.4999128	SMN	90	GM09 677	48							
11394	SMN1-47 m01	568.6799086	46.30542506	SMN	30	GM09 677	48	SMN1-53 m03	30	nM	unc-293 m01	30	nM	

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	Ctrl Lipo	100	8.78014 3796	SMN	0	GM09 677	48							
	Ctrl Un	108.656507 1	9.66796 4387	SMN	0	GM09 677	48							
13088	SMN1- 54 m01	379.644654 9	25.7085 785	SMN	30	GM09 677	48							
13088	SMN1- 54 m01	276.066850 4	4.22134 9055	SMN	30	GM09 677	48							
11384	SMN1- 37 m01	341.772862 1	39.8118 1567	SMN	30	GM09 677	48							
10087	SMN1- 77 m01	495.211666 5	26.6937 3234	SMN	30	GM09 677	48							
10339	SMN1- 83 m01	372.290566 6	17.2671 5487	SMN	30	GM09 677	48							
10954	SMN1- 90 m01	453.789090 9	25.4856 6994	SMN	30	GM09 677	48							
11394	SMN1- 47 m01	595.687887 1	67.0106 4045	SMN	30	GM09 677	48	SMN1- 53 m03	30	nM				
11395	SMN1- 48 m01	739.378946 7	90.4649 8588	SMN	30	GM09 677	48	SMN1- 53 m03	30	nM				
11394	SMN1- 47 m01	472.242942 1	48.3095 2456	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				
11395	SMN1- 48 m01	509.233984	29.0235 539	SMN	30	GM09 677	48	SMN1- 54 m01	30	nM				
10087	SMN1- 77 m01	618.042137 3	59.4182 2739	SMN	30	GM09 677	48	SMN1- 37 m01	30	nM				
10339	SMN1-	339.844923	28.9530	SMN	30	GM09	48	SMN1-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	83 m01	2	0391			677		37 m01						
10954	SMN1-90 m01	275.491606 2	11.8168 9193	SMN	30	GM09 677	48	SMN1-37 m01	30	nM				
10339	SMN1-83 m01	561.323535 1	38.0366 786	SMN	30	GM09 677	48	SMN1-77 m01	30	nM				
10954	SMN1-90 m01	432.961094	110.921 3377	SMN	30	GM09 677	48	SMN1-77 m01	30	nM				
10339	SMN1-83 m01	257.851302 7	42.2657 7702	SMN	30	GM09 677	48	SMN1-90 m01	30	nM				
13088	SMN1-54 m01	517.961929 2	75.4187 4164	SMN	30	GM09 677	48	unc-293 m01	30	nM				
10087	SMN1-77 m01	640.464634 5	137.183 6183	SMN	30	GM09 677	48	unc-293 m01	30	nM				
13088	SMN1-54 m01	354.082039 9	27.2577 0189	SMN	60	GM09 677	48							
10087	SMN1-77 m01	820.516761 4	88.6576 0235	SMN	60	GM09 677	48							
13095	unc-232 m01	284.028946 1	63.2870 865	SMN	30	GM09 677	48							
13095	unc-232 m01	367.569389 1	60.3328 7211	SMN	60	GM09 677	48							
	Ctrl Lipo	100	10.6305 8695	SMN	0	GM09 677	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	Ctrl Un	124.2067538	28.36545552	SMN	0	GM09 677	48							
13088	SMN1-54 m01	370.8043214	50.53867618	SMN	30	GM09 677	72							
13088	SMN1-54 m01	273.9128838	26.38304345	SMN	30	GM09 677	72							
11384	SMN1-37 m01	318.5666517	19.90255302	SMN	30	GM09 677	72							
10087	SMN1-77 m01	534.8105282	42.35615758	SMN	30	GM09 677	72							
10339	SMN1-83 m01	330.1331094	31.11938802	SMN	30	GM09 677	72							
10954	SMN1-90 m01	309.8933935	31.3776324	SMN	30	GM09 677	72							
11394	SMN1-47 m01	596.7632403	140.3104489	SMN	30	GM09 677	72	SMN1-53 m03	30	nM				
11395	SMN1-48 m01	628.9768013	42.91822594	SMN	30	GM09 677	72	SMN1-53 m03	30	nM				
11394	SMN1-47 m01	500.558544	55.61664468	SMN	30	GM09 677	72	SMN1-54 m01	30	nM				
11395	SMN1-48 m01	704.4618428	65.222309	SMN	30	GM09 677	72	SMN1-54 m01	30	nM				
10087	SMN1-77 m01	470.2212479	57.62134547	SMN	30	GM09 677	72	SMN1-37 m01	30	nM				
10339	SMN1-83 m01	442.8568257	65.49815867	SMN	30	GM09 677	72	SMN1-37 m01	30	nM				
10954	SMN1-	259.494126	12.9166	SMN	30	GM09	72	SMN1-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	90 m01	9	0616			677		37 m01						
10339	SMN1-83 m01	653.923634 9	52.6975 7081	SMN	30	GM09 677	72	SMN1-77 m01	30	nM				
10954	SMN1-90 m01	603.452802 9	115.035 1266	SMN	30	GM09 677	72	SMN1-77 m01	30	nM				
10339	SMN1-83 m01	368.264716 8	125.074 98	SMN	30	GM09 677	72	SMN1-90 m01	30	nM				
13088	SMN1-54 m01	507.772736 4	55.1237 9327	SMN	30	GM09 677	72	unc-293 m01	30	nM				
10087	SMN1-77 m01	547.915440 3	97.3001 137	SMN	30	GM09 677	72	unc-293 m01	30	nM				
13088	SMN1-54 m01	427.413786 9	55.5286 143	SMN	60	GM09 677	72							
10087	SMN1-77 m01	942.389937 4	216.611 4614	SMN	60	GM09 677	72							
13095	unc-232 m01	263.587402 3	35.2280 7163	SMN	30	GM09 677	72							
13095	unc-232 m01	316.252109 3	37.3441 6821	SMN	60	GM09 677	72							
	Ctrl Lipo	100	10.5045 6875	SMN	0	GM09 677	72							
	Ctrl Un	119.612036 9	19.9853 5571	SMN	0	GM09 677	72							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
13088	SMN1-54 m01	151.4974756	7.613343668	SMN	30	GM09677	48							
11394	SMN1-47 m01	82.5286402	3.416852087	SMN	30	GM09677	48							
11395	SMN1-48 m01	102.5291494	4.632463628	SMN	30	GM09677	48							
11419	SMN1-93 m01	144.008129	9.541650469	SMN	30	GM09677	48							
13088	SMN1-54 m01	154.4507759	8.865438928	SMN	30	GM09677	48							
13088	SMN1-54 m01	159.0116199	4.988511393	SMN	60	GM09677	48							
13096	unc-293 m01	122.8462826	2.842755021	SMN	30	GM09677	48							
13096	unc-293 m01	163.032365	6.049227708	SMN	60	GM09677	48							
11394	SMN1-47 m01	183.831114	8.745187507	SMN	30	GM09677	48	SMN1-54 m01	30	nM				
11395	SMN1-48 m01	220.9028221	11.25896406	SMN	30	GM09677	48	SMN1-54 m01	30	nM				
11419	SMN1-93 m01	225.658457	8.040041773	SMN	30	GM09677	48	SMN1-54 m01	30	nM				
11394	SMN1-47 m01	209.3616258	11.738718	SMN	30	GM09677	48	SMN1-54 m01	30	nM				
11395	SMN1-	231.119321	5.01320	SMN	30	GM09	48	SMN1-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	48 m01	7	0401			677		54 m01						
11419	SMN1-93 m01	227.1321039	12.85074012	SMN	30	GM09 677	48	SMN1-54 m01	30	nM				
13097	SMN1-102 m01	120.9527161	4.134339633	SMN	30	GM09 677	48							SMN1-47-53 dimer
13102	SMN1-103 m01	97.78963368	0.847725114	SMN	30	GM09 677	48							SMN1-48-53 dimer
13099	SMN1-104 m01	89.54908525	6.234592144	SMN	30	GM09 677	48							SMN1-93-53 dimer
13088	SMN1-54 m01	157.3780546	5.435038168	SMN	30	GM09 677	48							
	Ctrl Lipo	100	2.186877372	SMN	0	GM09 677	48							
	Ctrl Un	104.1957198	12.02131215	SMN	0	GM09 677	48							
13088	SMN1-54 m01	172.4397436	30.42042479	SMN	30	GM03 813	48							
11394	SMN1-47 m01	86.03256426	2.94451449	SMN	30	GM03 813	48							
11395	SMN1-48 m01	131.036647	50.76091941	SMN	30	GM03 813	48							
11419	SMN1-93 m01	136.7878998	6.096642576	SMN	30	GM03 813	48							
13088	SMN1-	180.554753	3.99029	SMN	30	GM03	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
13099	SMN1-104 m01	130.6899612	5.923768846	SMN	30	GM03813	48							SMN1-93-53 dimer
13088	SMN1-54 m01	203.1074669	11.5707393	SMN	30	GM03813	48	unc-293 m01	30	nM				
	Ctrl Lipo	100	3.270822071	SMN	0	GM03813	48							
	Ctrl Un	101.2312298	1.192687328	SMN	0	GM03813	48							
13088	SMN1-54 m01	130.0478058	8.014051145	SMN	30	GM03814	48							
11394	SMN1-47 m01	109.5289367	7.128162012	SMN	30	GM03814	48							
11395	SMN1-48 m01	116.5687214	6.048427437	SMN	30	GM03814	48							
11419	SMN1-93 m01	112.7951985	4.187360309	SMN	30	GM03814	48							
13088	SMN1-54 m01	134.041925	8.588180624	SMN	30	GM03814	48							
13088	SMN1-54 m01	141.1741043	5.667153663	SMN	30	GM03814	48							
13095	unc-232 m01	113.7772159	15.73744431	SMN	30	GM03814	48							
13095	unc-232	165.772599	14.34662471	SMN	30	GM03814	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	m01													
11394	SMN1-47 m01	186.496891	4.47316 5508	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
11395	SMN1-48 m01	166.700574 9	33.6270 7877	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
11419	SMN1-93 m01	205.176676 3	6.04734 1076	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
11394	SMN1-47 m01	185.731356 6	7.96239 3046	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
11395	SMN1-48 m01	182.641078 4	14.3564 2077	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
11419	SMN1-93 m01	201.260866 6	11.1352 1087	SMN	30	GM03 814	48	SMN1-54 m01	30	nM				
13097	SMN1-102 m01	157.138168 6	4.39694 7394	SMN	30	GM03 814	48							SMN1-47-53 dimer
13102	SMN1-103 m01	139.178869	4.77739 1537	SMN	30	GM03 814	48							SMN1-48-53 dimer
13099	SMN1-104 m01	104.879096 4	7.36305 4575	SMN	30	GM03 814	48							SMN1-93-53 dimer
13088	SMN1-54 m01	164.154552 7	1.84377 6064	SMN	30	GM03 814	48	unc-293 m01	30	nM				
	Ctrl Lipo	100	7.51738 0555	SMN	0	GM03 814	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	Ctrl Un	112.552505 5	6.79958 3446	SMN	0	GM03 814	48							
13088	SMN1- 53 m12	196.796617 3	6.44159 921	SMN	30	GM03 813	48							
13088	SMN1- 54 m01	196.379450 7	4.47978 5305	SMN	30	GM03 813	48							
11384	SMN1- 37 m01	233.413000 9	14.6870 6347	SMN	30	GM03 813	48							
10087	SMN1- 77 m01	353.982016 3	23.4524 5704	SMN	30	GM03 813	48							
10339	SMN1- 83 m01	243.355489 3	24.2047 7706	SMN	30	GM03 813	48							
10954	SMN1- 90 m01	177.810790 6	12.1669 1483	SMN	30	GM03 813	48							
11394	SMN1- 47 m01	325.892287 5	25.3755 7888	SMN	30	GM03 813	48	SMN1- 53 m03	30	nM				
11395	SMN1- 48 m01	361.972239	7.69829 6188	SMN	30	GM03 813	48	SMN1- 53 m03	30	nM				
11394	SMN1- 47 m01	214.176909 1	15.7449 9729	SMN	30	GM03 813	48	SMN1- 54 m01	30	nM				
11395	SMN1- 48 m01	276.464197 8	10.8960 3225	SMN	30	GM03 813	48	SMN1- 54 m01	30	nM				
10087	SMN1- 77 m01	318.571836 5	20.1474 535	SMN	30	GM03 813	48	SMN1- 37 m01	30	nM				
10339	SMN1- 83 m01	216.723148 7	3.69529 9528	SMN	30	GM03 813	48	SMN1- 37 m01	30	nM				
10954	SMN1-	205.519143	20.4563	SMN	30	GM03	48	SMN1-	30	nM				

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	90 m01	7	4284			813		37 m01						
10339	SMN1-83 m01	270.4212441	32.84757247	SMN	30	GM03813	48	SMN1-77 m01	30	nM				
10954	SMN1-90 m01	296.697045	25.68739603	SMN	30	GM03813	48	SMN1-77 m01	30	nM				
10339	SMN1-83 m01	245.5082199	11.95517859	SMN	30	GM03813	48	SMN1-90 m01	30	nM				
13088	SMN1-54 m01	125.4215754	79.71251983	SMN	30	GM03813	48	unc-293 m01	30	nM				
10087	SMN1-77 m01	392.0614361	53.72650681	SMN	30	GM03813	48	unc-293 m01	30	nM				
13088	SMN1-54 m01	226.8104246	52.47561012	SMN	60	GM03813	48							
10087	SMN1-77 m01	635.1936282	83.50352608	SMN	60	GM03813	48							
13096	unc-293 m01	148.2760972	10.69516242	SMN	30	GM03813	48							
13096	unc-293 m01	199.7931872	4.762753564	SMN	60	GM03813	48							
	Ctrl Lipo	100	6.587866729	SMN	0	GM03813	48							
	Ctrl Un	99.20509855	2.474999996	SMN	0	GM03813	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
13088	SMN1-54 m01	175.919651 5	8.59397 5405	SMN	30	GM09 677	48							
11384	SMN1-37 m01	219.511807 6	16.0251 6376	SMN	30	GM09 677	48							
10087	SMN1-77 m01	385.845156 2	2.23325 8922	SMN	30	GM09 677	48							
10339	SMN1-83 m01	224.780444 5	39.9488 6826	SMN	30	GM09 677	48							
10954	SMN1-90 m01	191.461594 2	0.73912 4406	SMN	30	GM09 677	48							
13088	SMN1-54 m01	158.886672 4	23.6013 9242	SMN	30	GM09 677	48							
11384	SMN1-37 m01	239.047422 3	16.0584 8354	SMN	30	GM09 677	48							
10087	SMN1-77 m01	341.763443 8	40.3376 4151	SMN	30	GM09 677	48							
10339	SMN1-83 m01	236.414813 1	9.67593 5641	SMN	30	GM09 677	48							
10954	SMN1-90 m01	167.231348 8	5.61656 9437	SMN	30	GM09 677	48							
10087	SMN1-77 m01	166.043823 9	4.72260 2967	SMN	30	GM09 677	48							
10087	SMN1-77 m01	123.415318 3	9.39429 0699	SMN	60	GM09 677	48							
10087	SMN1-77 m01	395.429572 8	5.98950 2565	SMN	60	GM09 677	48							
13096	unc-	148.530911	1.23954	SMN	30	GM09	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	293 m01	7	7901			677								
	Ctrl Lipo	100	3.74664 1299	SMN	0	GM09 677	48							
	Ctrl Un	114.487088 6	5.27641 4309	SMN	0	GM09 677	48							
13088	SMN1-54 m01	151.481329 9	6.06253 1771	SMN	30	GM03 813	48							
11384	SMN1-37 m01	199.710720 7	4.70454 4546	SMN	30	GM03 813	48							
10087	SMN1-77 m01	307.446023 1	9.26101 9008	SMN	30	GM03 813	48							
10339	SMN1-83 m01	221.123847 7	16.3283 0037	SMN	30	GM03 813	48							
10954	SMN1-90 m01	171.756951 2	4.81175 5485	SMN	30	GM03 813	48							
13088	SMN1-54 m01	167.912468 9	7.67713 6696	SMN	30	GM03 813	48							
11384	SMN1-37 m01	237.095974 4	13.3240 8561	SMN	30	GM03 813	48							
10087	SMN1-77 m01	235.736246 7	24.3149 6311	SMN	30	GM03 813	48							
	Ctrl Lipo	100	3.22855 3658	SMN	0	GM03 813	48							
	Ctrl Un	105.588758 3	8.21872 6998	SMN	0	GM03 813	48							
13088	SMN1-	122.334041	11.4866	SMN	30	GM09	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	54 m01	4	3137			677								
11384	SMN1-37 m01	188.4023749	25.99367708	SMN	30	GM09 677	48							
10087	SMN1-77 m01	352.3342661	9.785348021	SMN	30	GM09 677	48							
10339	SMN1-83 m01	231.90303	14.90561498	SMN	30	GM09 677	48							
10954	SMN1-90 m01	180.9606799	4.593257168	SMN	30	GM09 677	48							
13088	SMN1-54 m01	158.444272	2.923904257	SMN	30	GM09 677	48							
11384	SMN1-37 m01	240.3674014	16.12151395	SMN	30	GM09 677	48							
10087	SMN1-77 m01	310.8566324	5.171055677	SMN	30	GM09 677	48							
10339	SMN1-83 m01	221.4277632	13.16066354	SMN	30	GM09 677	48							
10954	SMN1-90 m01	170.3625519	12.27139953	SMN	30	GM09 677	48							
10087	SMN1-77 m01	157.3393043	7.764553285	SMN	15	GM09 677	48							
10087	SMN1-77 m01	135.0633981	1.427534096	SMN	30	GM09 677	48							
10087	SMN1-77 m01	112.6465714	7.938596073	SMN	60	GM09 677	48							
13096	unc-293	136.2019635	21.35219926	SMN	30	GM09 677	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	m01													
	Ctrl Lipo	100	3.98783 3087	SMN	0	GM09 677	48							
	Ctrl Un	92.2164860 6	7.21777 5705	SMN	0	GM09 677	48							
13088	SMN1- 54 m01	214.239067 5	13.8126 7769	SMN	30	GM03 813	48							
11384	SMN1- 37 m01	275.043962 1	15.8990 5736	SMN	30	GM03 813	48							
10087	SMN1- 77 m01	427.600754 1	#DIV/0!	SMN	30	GM03 813	48							
10339	SMN1- 83 m01	293.418671 2	21.6357 5799	SMN	30	GM03 813	48							
10954	SMN1- 90 m01	244.432502 1	3.40607 2946	SMN	30	GM03 813	48							
13088	SMN1- 54 m01	206.206877 1	28.6022 6235	SMN	30	GM03 813	48							
11384	SMN1- 37 m01	291.149281 1	16.8665 8837	SMN	30	GM03 813	48							
10087	SMN1- 77 m01	311.106837 1	20.3558 9866	SMN	30	GM03 813	48							
10339	SMN1- 83 m01	234.145330 6	9.13500 3549	SMN	30	GM03 813	48							
10954	SMN1- 90 m01	192.938250 7	5.46185 4501	SMN	30	GM03 813	48							
10087	SMN1- 77 m01	217.817361 7	9.16062 2763	SMN	15	GM03 813	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	Ctrl Lipo	100	20.9973 6457	SMN	0	GM03 813	48							
	Ctrl Un	123.348619 7	0.96195 9215	SMN	0	GM03 813	48							
11381	SMN1-34 m01	246.060347	5.82032 5653	SMN	30	GM03 813	48							
10952	SMN1-88 m01	196.198436 4	7.56282 0235	SMN	30	GM03 813	48							
10953	SMN1-89 m01	191.283735 3	5.25675 0304	SMN	30	GM03 813	48							
12369	SMN1-101 m01	172.785057 9	11.8820 1759	SMN	30	GM03 813	48							
13105	SMN1-107 m01	174.540874 6	5.78728 4064	SMN	30	GM03 813	48							
13107	SMN1-109 m01	196.759801 4	8.81286 7014	SMN	30	GM03 813	48							
10090	SMN1-110 m01	140.267937 2	6.33696 9406	SMN	30	GM03 813	48							
10091	SMN1-111 m01	123.911813 6	13.9711 1908	SMN	30	GM03 813	48							
10092	SMN1-112	141.084454 7	4.72232 8253	SMN	30	GM03 813	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	m01													
10093	SMN1-113 m01	151.473804 1	6.36650 8373	SMN	30	GM03 813	48							
10094	SMN1-114 m01	153.194237 4	15.7473 0978	SMN	30	GM03 813	48							
10095	SMN1-115 m01	112.025741 8	15.2801 8019	SMN	30	GM03 813	48							
10096	SMN1-116 m01	135.770585 4	5.79558 9034	SMN	30	GM03 813	48							
10097	SMN1-117 m01	140.987056 7	13.7505 682	SMN	30	GM03 813	48							
10330	SMN1-118 m01	196.225218 1	5.76040 0388	SMN	30	GM03 813	48							
10332	SMN1-120 m01	183.079905 3	1.42196 0355	SMN	30	GM03 813	48							
10333	SMN1-121 m01	154.643198 5	4.37815 006	SMN	30	GM03 813	48							
10334	SMN1-122	150.099566 6	7.09727 5601	SMN	30	GM03 813	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	m01													
10335	SMN1-123 m01	199.4006359	5.232405701	SMN	30	GM03813	48							
10340	SMN1-125 m01	216.3786572	26.05055644	SMN	30	GM03813	48							
10341	SMN1-126 m01	198.3434352	12.45973339	SMN	30	GM03813	48							
10342	SMN1-127 m01	267.2959059	16.59960914	SMN	30	GM03813	48							
10944	SMN1-135 m01	186.1831282	37.5339576	SMN	30	GM03813	48							
10945	SMN1-136 m01	183.3688838	60.89995338	SMN	30	GM03813	48							
10946	SMN1-137 m01	195.9514466	10.45161645	SMN	30	GM03813	48							
10955	SMN1-141 m01	199.9003121	6.654755144	SMN	30	GM03813	48							
10956	SMN1-142	314.8823104	39.60521588	SMN	30	GM03813	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	m01													
10957	SMN1-143 m01	318.128633 1	12.6021 1999	SMN	30	GM03 813	48							
10958	SMN1-144 m01	402.790961 1	27.5549 4479	SMN	30	GM03 813	48							
10959	SMN1-145 m01	215.349109 5	3.22223 7598	SMN	30	GM03 813	48							
10961	SMN1-147 m01	214.814454 7	15.2318 8228	SMN	30	GM03 813	48							
11367	SMN1-149 m01	221.158987 8	13.2857 2905	SMN	30	GM03 813	48							
11368	SMN1-150 m01	190.457728 5	1.90795 6989	SMN	30	GM03 813	48							
11401	SMN1-157 m01	190.441432 1	13.6565 2949	SMN	30	GM03 813	48							
11405	SMN1-161 m01	193.141227 4	5.78471 9874	SMN	30	GM03 813	48							
11384	SMN1-37 m01	260.858395 2	8.91596 5838	SMN	30	GM03 813	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10087	SMN1-77 m01	366.4887397	9.38303154	SMN	30	GM03813	48							
10339	SMN1-83 m01	244.1263311	11.28436092	SMN	30	GM03813	48							
10954	SMN1-90 m01	188.1129772	9.736900116	SMN	30	GM03813	48							
13088	SMN1-54 m01	206.6873942	10.08156665	SMN	30	GM03813	48							
10087	SMN1-77 m01	519.9480411	5.273153634	SMN	60	GM03813	48							
11394	SMN1-47 m01	104.3166706	2.151555911	SMN	30	GM03813	48							
	EPO-10 m01	194.2399846	8.428930335	SMN	30	GM03813	48							
	EPO-18 m01	148.9358222	8.219133664	SMN	30	GM03813	48							
13096	unc-293 m01	145.3094854	1.966302542	SMN	30	GM03813	48							
13095	unc-232 m12	132.7710924	5.322917696	SMN	30	GM03813	48							
	Ctrl Lipo	100	4.214452226	SMN	30	GM03813	48							
	Ctrl Un	110.2931151	9.711085411	SMN	30	GM03813	48							
11381	SMN1-	200.116565	19.2930	SMN	30	GM09	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	34 m01	8	2004			677								
10952	SMN1-88 m01	208.5865674	28.19873402	SMN	30	GM09 677	48							
10953	SMN1-89 m01	213.7968794	5.269881498	SMN	30	GM09 677	48							
12369	SMN1-101 m01	210.9966499	12.73168976	SMN	30	GM09 677	48							
13105	SMN1-107 m01	206.1038335	14.96657146	SMN	30	GM09 677	48							
13107	SMN1-109 m01	243.858238	13.40396791	SMN	30	GM09 677	48							
10090	SMN1-110 m01	162.7725802	3.123514089	SMN	30	GM09 677	48							
10091	SMN1-111 m01	153.7558299	2.495565984	SMN	30	GM09 677	48							
10092	SMN1-112 m01	181.7400468	5.349662869	SMN	30	GM09 677	48							
10093	SMN1-113 m01	198.0325876	12.3193778	SMN	30	GM09 677	48							
10094	SMN1-	165.382056	5.71441	SMN	30	GM09	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	114 m01		5493			677								
10095	SMN1-115 m01	126.9752849	8.347302338	SMN	30	GM09677	48							
10096	SMN1-116 m01	123.1078624	9.003590778	SMN	30	GM09677	48							
10097	SMN1-117 m01	152.8612342	1.626979545	SMN	30	GM09677	48							
10330	SMN1-118 m01	191.5812619	3.300362398	SMN	30	GM09677	48							
10332	SMN1-120 m01	234.3745925	11.63620425	SMN	30	GM09677	48							
10333	SMN1-121 m01	175.6060918	5.790851325	SMN	30	GM09677	48							
10334	SMN1-122 m01	128.4275612	5.242476716	SMN	30	GM09677	48							
10335	SMN1-123 m01	197.068391	31.73422118	SMN	30	GM09677	48							
10340	SMN1-	343.760322	39.8812	SMN	30	GM09	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	125 m01	2	6272			677								
10341	SMN1-126 m01	221.040792	32.5557 3942	SMN	30	GM09 677	48							
10342	SMN1-127 m01	308.213764 9	39.7183 7769	SMN	30	GM09 677	48							
10944	SMN1-135 m01	148.859471 9	20.6230 8278	SMN	30	GM09 677	48							
10945	SMN1-136 m01	159.418365 9	1.63651 6837	SMN	30	GM09 677	48							
10946	SMN1-137 m01	223.934332 6	4.01434 9329	SMN	30	GM09 677	48							
10955	SMN1-141 m01	199.737784 3	6.69281 3458	SMN	30	GM09 677	48							
10956	SMN1-142 m01	350.958565 7	24.3466 8935	SMN	30	GM09 677	48							
10957	SMN1-143 m01	336.800982 4	28.7270 4689	SMN	30	GM09 677	48							
10958	SMN1-	432.162523	13.3206	SMN	30	GM09	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	144 m01	5	9449			677								
10959	SMN1-145 m01	217.8856196	11.40143967	SMN	30	GM09 677	48							
10961	SMN1-147 m01	171.2825928	8.289810496	SMN	30	GM09 677	48							
11367	SMN1-149 m01	273.5138766	26.12851486	SMN	30	GM09 677	48							
11368	SMN1-150 m01	199.32215	6.586245772	SMN	30	GM09 677	48							
11401	SMN1-157 m01	216.6194041	5.718062521	SMN	30	GM09 677	48							
11405	SMN1-161 m01	201.2780235	13.83361274	SMN	30	GM09 677	48							
11384	SMN1-37 m01	263.6988246	13.25729084	SMN	30	GM09 677	48							
10087	SMN1-77 m01	567.2034546	23.95110459	SMN	30	GM09 677	48							
10339	SMN1-83 m01	308.437665	21.56682726	SMN	30	GM09 677	48							
10954	SMN1-	236.116772	17.1629	SMN	30	GM09	48							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	90 m01		7887			677								
13088	SMN1-54 m01	217.6064925	18.03601518	SMN	30	GM09 677	48							
10087	SMN1-77 m01	710.735513	2.098288031	SMN	30	GM09 677	48							
11394	SMN1-47 m01	96.98210016	3.169998374	SMN	30	GM09 677	48							
	EPO-10 m01	272.4923353	19.22355834	SMN	30	GM09 677	48							
	EPO-18 m01	155.0805654	12.46522909	SMN	30	GM09 677	48							
13096	unc-293 m01	153.5758988	15.92357071	SMN	30	GM09 677	48							
13095	unc-232 m12	139.8807935	3.959404111	SMN	30	GM09 677	48							
	Ctrl Lipo	100	1.024685803	SMN	30	GM09 677	48							
	Ctrl Un	102.2281346	4.272544109	SMN	30	GM09 677	48							
11384	SMN1-37 m01	104.2825495	4.302933177	SMN	30	GM09 677	12							
10087	SMN1-77 m01	113.2866152	4.557229672	SMN	30	GM09 677	12							
10339	SMN1-83 m01	104.5688948	7.766091617	SMN	30	GM09 677	12							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10954	SMN1-90 m01	110.4527329	5.753074354	SMN	30	GM09677	12							
13088	SMN1-54 m01	115.0698919	5.502332917	SMN	30	GM09677	12							
13096	unc-293 m01	106.5874646	4.996011717	SMN	30	GM09677	12							
	Ctrl Lipo	100	2.622316447	SMN	30	GM09677	12							
	Ctrl Un	98.73426792	2.731450415	SMN	30	GM09677	12							
11381	SMN1-34 m01	99.83300323	6.254105951	SMN	30	GM09677	12							
10340	SMN1-125 m01	104.2294736	10.45053575	SMN	30	GM09677	12							
10342	SMN1-127 m01	97.56691242	6.371359203	SMN	30	GM09677	12							
10955	SMN1-141 m01	105.7455917	5.785188824	SMN	30	GM09677	12							
10956	SMN1-142 m01	102.7146801	5.341817973	SMN	30	GM09677	12							
10957	SMN1-143	111.5415857	0.883328775	SMN	30	GM09677	12							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	m01													
10958	SMN1-144 m01	96.94232699	1.796857944	SMN	30	GM09677	12							
11367	SMN1-149 m01	100.6413069	1.702644189	SMN	30	GM09677	12							
11384	SMN1-37 m01	124.0635862	0.720165067	SMN	30	GM09677	24							
10087	SMN1-77 m01	142.5381459	7.342667685	SMN	30	GM09677	24							
10339	SMN1-83 m01	123.4804611	4.512237083	SMN	30	GM09677	24							
10954	SMN1-90 m01	120.4918524	5.527678898	SMN	30	GM09677	24							
13088	SMN1-54 m01	129.5483911	2.582886464	SMN	30	GM09677	24							
13096	unc-293 m01	116.4320128	2.943784563	SMN	30	GM09677	24							
	Ctrl Lipo	100	0.96878073	SMN	30	GM09677	24							
	Ctrl Un	100.9044669	5.062298317	SMN	30	GM09677	24							
11381	SMN1-34 m01	131.6032491	2.521082247	SMN	30	GM09677	24							
10340	SMN1-	132.276494	4.43185	SMN	30	GM09	24							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	125 m01	6	5965			677								
10342	SMN1-127 m01	131.0941184	3.86700279	SMN	30	GM09677	24							
10955	SMN1-141 m01	135.126845	2.466284523	SMN	30	GM09677	24							
10956	SMN1-142 m01	147.5274776	1.996170884	SMN	30	GM09677	24							
10957	SMN1-143 m01	146.5545403	3.926841627	SMN	30	GM09677	24							
10958	SMN1-144 m01	142.2203992	2.236827725	SMN	30	GM09677	24							
11367	SMN1-149 m01	126.2967197	3.406663573	SMN	30	GM09677	24							
11384	SMN1-37 m01	354.8236134	14.35307717	SMN	30	GM09677	72							
10087	SMN1-77 m01	417.5559494	5.875157196	SMN	30	GM09677	72							
10339	SMN1-83 m01	310.7125283	7.940733124	SMN	30	GM09677	72							
10954	SMN1-	299.727845	1.40548	SMN	30	GM09	72							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	90 m01	8	6305			677								
13088	SMN1-54 m01	272.0778604	15.68568882	SMN	30	GM09 677	72							
13096	unc-293 m01	141.1642274	4.01698458	SMN	30	GM09 677	72							
	Ctrl Lipo	100	1.435349732	SMN	30	GM09 677	72							
	Ctrl Un	103.7512405	0.590262102	SMN	30	GM09 677	72							
11381	SMN1-34 m01	266.828526	5.410141181	SMN	30	GM09 677	72							
10340	SMN1-125 m01	306.3854442	12.14927647	SMN	30	GM09 677	72							
10342	SMN1-127 m01	356.0450663	11.64481415	SMN	30	GM09 677	72							
10955	SMN1-141 m01	259.1592824	11.40490637	SMN	30	GM09 677	72							
10956	SMN1-142 m01	401.4008572	24.34623768	SMN	30	GM09 677	72							
10957	SMN1-143 m01	447.6151832	18.21256525	SMN	30	GM09 677	72							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10958	SMN1-144 m01	546.626693 9	16.8836 3313	SMN	30	GM09 677	72							
11367	SMN1-149 m01	208.589540 4	7.78572 0491	SMN	30	GM09 677	72							
11384	SMN1-37 m01	427.484981 1	10.6073 1203	SMN	30	GM09 677	96							
10087	SMN1-77 m01	433.066111 2	12.9013 0774	SMN	30	GM09 677	96							
10339	SMN1-83 m01	354.478314 3	18.2226 9474	SMN	30	GM09 677	96							
10954	SMN1-90 m01	366.086447 3	19.4291 8258	SMN	30	GM09 677	96							
13088	SMN1-54 m01	309.619003 5	6.32664 7169	SMN	30	GM09 677	96							
13096	unc-293 m01	139.250990 4	11.1923 281	SMN	30	GM09 677	96							
	Ctrl Lipo	100	7.32272 1777	SMN	30	GM09 677	96							
	Ctrl Un	99.1607785 2	3.89827 1673	SMN	30	GM09 677	96							
11381	SMN1-34 m01	257.304646 2	6.43242 7611	SMN	30	GM09 677	96							
10340	SMN1-125	243.998406 6	6.28739 7026	SMN	30	GM09 677	96							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	m01													
10342	SMN1-127 m01	317.786365 3	9.02141 1179	SMN	30	GM09 677	96							
10955	SMN1-141 m01	292.277454 1	14.6593 3032	SMN	30	GM09 677	96							
10956	SMN1-142 m01	433.172557 9	44.3310 1358	SMN	30	GM09 677	96							
10957	SMN1-143 m01	435.258019 1	14.2342 8264	SMN	30	GM09 677	96							
10958	SMN1-144 m01	568.861916 2	14.7625 4804	SMN	30	GM09 677	96							
11367	SMN1-149 m01	228.246217 9	10.7481 843	SMN	30	GM09 677	96							
11384	SMN1-37 m01	475.202876 9	15.3874 8204	SMN	30	GM09 677	120							
10087	SMN1-77 m01	356.001906 1	34.5578 9792	SMN	30	GM09 677	120							
10339	SMN1-83 m01	287.040729 9	11.9439 1767	SMN	30	GM09 677	120							
10954	SMN1-90 m01	453.971147 2	29.5574 1546	SMN	30	GM09 677	120							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
13088	SMN1-54 m01	267.965546 2	15.6565 8001	SMN	30	GM09 677	120							
13096	unc-293 m01	146.432825 2	8.12151 9161	SMN	30	GM09 677	120							
	Ctrl Lipo	100	2.09254 5395	SMN	30	GM09 677	120							
	Ctrl Un	109.768766	8.34832 936	SMN	30	GM09 677	120							
11381	SMN1-34 m01	276.803601 4	13.5928 2326	SMN	30	GM09 677	120							
10340	SMN1-125 m01	259.253828	13.0063 7987	SMN	30	GM09 677	120							
10342	SMN1-127 m01	324.803436 9	29.6672 022	SMN	30	GM09 677	120							
10955	SMN1-141 m01	383.916828 8	20.3543 1281	SMN	30	GM09 677	120							
10956	SMN1-142 m01	538.551315 3	19.2309 9829	SMN	30	GM09 677	120							
10957	SMN1-143 m01	559.316991 4	4.28481 2982	SMN	30	GM09 677	120							
10958	SMN1-	685.623746	27.6133	SMN	30	GM09	120							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	144 m01	4	979			677								
11367	SMN1-149 m01	263.0261034	22.31550442	SMN	30	GM09 677	120							
11384	SMN1-37 m01	417.1337189	8.825342836	SMN	30	GM09 677	144							
10087	SMN1-77 m01	326.2780422	19.61536392	SMN	30	GM09 677	144							
10339	SMN1-83 m01	269.5887276	11.28550249	SMN	30	GM09 677	144							
10954	SMN1-90 m01	438.5523906	5.687972266	SMN	30	GM09 677	144							
13088	SMN1-54 m01	319.3324164	20.64281192	SMN	30	GM09 677	144							
13096	unc-293 m01	124.5993204	3.130011099	SMN	30	GM09 677	144							
	Ctrl Lipo	100	3.720359821	SMN	30	GM09 677	144							
	Ctrl Un	103.6635296	2.571906761	SMN	30	GM09 677	144							
11381	SMN1-34 m01	206.4504913	22.59143237	SMN	30	GM09 677	144							
10340	SMN1-125 m01	189.2113429	23.7929527	SMN	30	GM09 677	144							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
10342	SMN1-127 m01	251.780822 8	27.4824 4912	SMN	30	GM09 677	144							
10955	SMN1-141 m01	350.553037 2	13.4877 1671	SMN	30	GM09 677	144							
10956	SMN1-142 m01	457.912742 3	40.4848 3228	SMN	30	GM09 677	144							
10957	SMN1-143 m01	441.564591 1	13.3206 4749	SMN	30	GM09 677	144							
10958	SMN1-144 m01	492.565680 3	12.1590 6825	SMN	30	GM09 677	144							
11367	SMN1-149 m01	181.311968 4	11.3949 7331	SMN	30	GM09 677	144							
11384	SMN1-37 m01	340.189810 5	6.97334 24	SMN	30	GM09 677	168							
10087	SMN1-77 m01	260.336402 2	14.9916 91	SMN	30	GM09 677	168							
10339	SMN1-83 m01	205.415658 4	4.54660 9026	SMN	30	GM09 677	168							
10954	SMN1-90 m01	383.454351	7.36945 1908	SMN	30	GM09 677	168							
13088	SMN1-	309.320767	7.67072	SMN	30	GM09	168							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	54 m01		7078			677								
13096	unc-293 m01	101.3340126	3.668480582	SMN	30	GM09 677	168							
	Ctrl Lipo	100	1.628765533	SMN	30	GM09 677	168							
	Ctrl Un	99.24241694	3.580487523	SMN	30	GM09 677	168							
11381	SMN1-34 m01	167.8889332	4.062192788	SMN	30	GM09 677	168							
10340	SMN1-125 m01	147.2469599	10.02472788	SMN	30	GM09 677	168							
10342	SMN1-127 m01	191.8478217	15.66029444	SMN	30	GM09 677	168							
10955	SMN1-141 m01	317.5938402	23.82885743	SMN	30	GM09 677	168							
10956	SMN1-142 m01	376.0249497	12.85381169	SMN	30	GM09 677	168							
10957	SMN1-143 m01	377.2732885	8.301632964	SMN	30	GM09 677	168							
10958	SMN1-144	392.1544529	8.791129966	SMN	30	GM09 677	168							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	m01													
11367	SMN1-149 m01	156.4052045	6.97947014	SMN	30	GM09 677	168							
11384	SMN1-37 m01	390.9615937	39.8928501	SMN	30	GM09 677	216							
10087	SMN1-77 m01	253.4102719	10.35106947	SMN	30	GM09 677	216							
10339	SMN1-83 m01	174.3477066	7.562154652	SMN	30	GM09 677	216							
10954	SMN1-90 m01	504.3685069	8.76542754	SMN	30	GM09 677	216							
13088	SMN1-54 m01	269.5824302	19.20845332	SMN	30	GM09 677	216							
13096	unc-293 m01	159.9447029	44.33750451	SMN	30	GM09 677	216							
	Ctrl Lipo	100	16.60800005	SMN	30	GM09 677	216							
	Ctrl Un	97.88267744	11.72625917	SMN	30	GM09 677	216							
11381	SMN1-34 m01	143.670673	7.822849447	SMN	30	GM09 677	216							
10340	SMN1-125 m01	155.4096268	7.322292061	SMN	30	GM09 677	216							
10342	SMN1-	116.420535	24.9292	SMN	30	GM09	216							

Appendix C: Table 7

SeqID	Oligo Name	%SMN over lipo only control	%SMN CVV	Targ et	[oligo]	cell line	Time (hr)	2nd Drug	[2nd]	units	3rd Drug	[3rd]	units	Note
	127 m01	9	4415			677								
10955	SMN1-141 m01	418.787548	21.2437 4777	SMN	30	GM09 677	216							
10956	SMN1-142 m01	369.752451 2	36.9974 3388	SMN	30	GM09 677	216							
10957	SMN1-143 m01	317.642402	19.4770 1989	SMN	30	GM09 677	216							
10958	SMN1-144 m01	272.870529 2	27.1926 9947	SMN	30	GM09 677	216							
11367	SMN1-149 m01	131.269774 9	23.9437 1891	SMN	30	GM09 677	216							

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CLAIMS

What is claimed is:

1. A single stranded oligonucleotide having a sequence 5'-X-Y-Z, wherein X is any nucleotide, Y is a nucleotide sequence of 6 nucleotides in length that is not a seed
5 sequence of a human microRNA, and Z is a nucleotide sequence of 1-23 nucleotides in length, wherein the single stranded oligonucleotide is complementary with at least 8 consecutive nucleotides of a PRC2-associated region of an SMN gene.

2. The single stranded oligonucleotide of claim 1, wherein the oligonucleotide does not comprise three or more consecutive guanosine nucleotides.

10 3. The single stranded oligonucleotide of claim 1, wherein the oligonucleotide does not comprise four or more consecutive guanosine nucleotides.

4. The single stranded oligonucleotide of any one of claims 1 to 3, wherein the oligonucleotide is 8 to 30 nucleotides in length.

15 5. The single stranded oligonucleotide of any one of claims 1 to 3, wherein the oligonucleotide is 8 to 10 nucleotides in length and all but 1, 2, or 3 of the nucleotides of the complementary sequence of the PRC2-associated region are cytosine or guanosine nucleotides.

6. The single stranded oligonucleotide of any one of claims 1 to 5, wherein at least one nucleotide of the oligonucleotide is a nucleotide analogue.

20 7. The single stranded oligonucleotide of claim 7, wherein the at least one nucleotide analogue results in an increase in T_m of the oligonucleotide in a range of 1 to 5 °C compared with an oligonucleotide that does not have the at least one nucleotide analogue.

8. The single stranded oligonucleotide of any one of claims 1 to 7, wherein at least one nucleotide of the oligonucleotide comprises a 2' O-methyl.

25 9. The single stranded oligonucleotide of any one of claims 1 to 9, wherein each nucleotide of the oligonucleotide comprises a 2' O-methyl.

10. The single stranded oligonucleotide of any one of claims 1 to 9, wherein the oligonucleotide comprises at least one ribonucleotide, at least one deoxyribonucleotide, or at least one bridged nucleotide.

11. The single strand oligonucleotide of claim 10, wherein the bridged nucleotide is a LNA nucleotide, a cEt nucleotide or a ENA modified nucleotide.

12. The single stranded oligonucleotide of any one of claims 1 to 6, wherein each nucleotide of the oligonucleotide is a LNA nucleotide.

5 13. The single stranded oligonucleotide of any one of claims 1 to 6, wherein the nucleotides of the oligonucleotide comprise alternating deoxyribonucleotides and 2'-fluoro-deoxyribonucleotides.

14. The single stranded oligonucleotide of any one of claims 1 to 6, wherein the nucleotides of the oligonucleotide comprise alternating deoxyribonucleotides and 2'-O-
10 methyl nucleotides.

15. The single stranded oligonucleotide of any one of claims 1 to 6, wherein the nucleotides of the oligonucleotide comprise alternating deoxyribonucleotides and ENA nucleotide analogues.

16. The single stranded oligonucleotide of any one of claims 1 to 6, wherein the
15 nucleotides of the oligonucleotide comprise alternating deoxyribonucleotides and LNA nucleotides.

17. The single stranded oligonucleotide of any one of claims 13 to 16, wherein the 5' nucleotide of the oligonucleotide is a deoxyribonucleotide.

18. The single stranded oligonucleotide of any one of claims 1 to 6, wherein the
20 nucleotides of the oligonucleotide comprise alternating LNA nucleotides and 2'-O-methyl nucleotides.

19. The single stranded oligonucleotide of claim 19, wherein the 5' nucleotide of the oligonucleotide is a LNA nucleotide.

20. The single stranded oligonucleotide of any one of claims 1 to 8, wherein the
25 nucleotides of the oligonucleotide comprise deoxyribonucleotides flanked by at least one LNA nucleotide on each of the 5' and 3' ends of the deoxyribonucleotides.

21. The single stranded oligonucleotide of any one of claims 1 to 20, further comprising phosphorothioate internucleotide linkages between at least two nucleotides.

22. The single stranded oligonucleotide of claim 21, further comprising phosphorothioate internucleotide linkages between all nucleotides.

23. The single stranded oligonucleotide of any one of claims 1 to 22, wherein the nucleotide at the 3' position of the oligonucleotide has a 3' hydroxyl group.

5 24. The single stranded oligonucleotide of any one of claims 1 to 22, wherein the nucleotide at the 3' position of the oligonucleotide has a 3' thiophosphate.

25. The single stranded oligonucleotide of any one of claims 1 to 24, further comprising a biotin moiety conjugated to the 5' nucleotide.

10 26. A single stranded oligonucleotide comprising a region of complementarity that is complementary with at least 8 consecutive nucleotides of a PRC2-associated region of an SMN gene, wherein the oligonucleotide has at least one of:

a) a sequence that is 5'X-Y-Z, wherein X is any nucleotide and wherein X is anchored at the 5' end of the oligonucleotide, Y is a nucleotide sequence of 6 nucleotides in length that is not a human seed sequence of a microRNA, and Z is a nucleotide sequence of 1 to 23
15 nucleotides in length;

b) a sequence that does not comprise three or more consecutive guanosine nucleotides;

c) a sequence that has less than a threshold level of sequence identity with every sequence of nucleotides, of equivalent length, that are between 50 kilobases upstream of a 5'-
20 end of an off-target gene and 50 kilobases downstream of a 3'-end of the off-target gene;

d) a sequence that is complementary to a PRC2-associated region that encodes an RNA that forms a secondary structure comprising at least two single stranded loops; and/or

e) a sequence that has greater than 60% G-C content.

27. The single stranded oligonucleotide of claim 26, wherein the oligonucleotide
25 has the sequence 5'X-Y-Z and wherein the oligonucleotide is 8-50 nucleotides in length.

28. A composition comprising a single stranded oligonucleotide of any one of claims 1 to 27 and a carrier.

29. A composition comprising a single stranded oligonucleotide of any one of claims 1 to 27 in a buffered solution.

30. A composition of claim 29, wherein the oligonucleotide is conjugated to the carrier.

5 31. The composition of claim 30, wherein the carrier is a peptide.

32. The composition of claim 30, wherein the carrier is a steroid.

33. A pharmaceutical composition comprising a composition of any one of claims 28 to 32 and a pharmaceutically acceptable carrier.

10 34. A kit comprising a container housing the composition of any one of claims 28 to 33.

35. A method of increasing expression of SMN1 or SMN2 in a cell, the method comprising delivering the single stranded oligonucleotide of any one of claims 1 to 28 into the cell.

15 36. The method of claim 35, wherein delivery of the single stranded oligonucleotide into the cell results in a level of expression of SMN1 or SMN2 that is at least 50% greater than a level of expression of SMN1 or SMN2 in a control cell that does not comprise the single stranded oligonucleotide.

20 37. A method increasing levels of SMN1 or SMN2 in a subject, the method comprising administering the single stranded oligonucleotide of any one of claims 1 to 27 to the subject.

38. A method of treating a condition associated with decreased levels of SMN1 or SMN2 in a subject, the method comprising administering the single stranded oligonucleotide of any one of claims 1 to 27 to the subject.

39. The method of claim 38, wherein the condition is Spinal muscular atrophy.

25 40. A method of increasing expression of SMN protein in a cell, the method comprising:

delivering to the cell a first single stranded oligonucleotide complementary with at least 8 consecutive nucleotides of a PRC2-associated region of SMN2 and a second single

stranded oligonucleotide complementary with a splice control sequence of a precursor mRNA of SMN2, in amounts sufficient to increase expression of a mature mRNA of SMN2 that comprises exon 7 in the cell.

41. The method of claim 40, wherein the splice control sequence resides in an
5 exon of SMN2.

42. The method of claim 41, wherein the exon is exon 7 or exon 8.

43. The method of claim 40, wherein the splice control sequence traverses an
intron-exon junction of SMN2.

44. The method of claim 43, wherein the intron-exon junction is the intron 6/exon
10 7 junction or the intron 7/exon 8 junction.

45. The method of claim 40, wherein the splice control sequence resides in an
intron of SMN2.

46. The method of claim 45, wherein the intron is intron 6 or intron 7.

47. The method of any one of claims 40 to 46, wherein the first single stranded
15 oligonucleotide has a sequence 5'-X-Y-Z, wherein X is any nucleotide, Y is a nucleotide
sequence of 6 nucleotides in length that is not a seed sequence of a human microRNA, and Z
is a nucleotide sequence of 1-23 nucleotides in length.

48. The method of any one of claims 40 to 47, wherein the first single stranded
oligonucleotide does not comprise three or more consecutive guanosine nucleotides.

20 49. The method of any one of claims 40 to 47, wherein the first single stranded
oligonucleotide does not comprise four or more consecutive guanosine nucleotides.

50. The method of any one of claims 40 to 49, wherein the first single stranded
oligonucleotide is 8 to 30 nucleotides in length.

51. The method of any one of claims 40 to 50, wherein the first single stranded
25 oligonucleotide is 8 to 10 nucleotides in length and all but 1, 2, or 3 of the nucleotides of the
complementary sequence of the PRC2-associated region are cytosine or guanosine
nucleotides.

52. The method of any one of claims 40 to 51, wherein the first single stranded oligonucleotide and the second single stranded oligonucleotide are delivered to the cell simultaneously.

53. The method of any one of claims 40 to 52, wherein the cell is in a subject and the step of delivering to the cell comprises administering the first single stranded oligonucleotide and the second single stranded oligonucleotide to the subject as a co-formulation.

54. The method of any one of claims 40 to 53, wherein the first single stranded oligonucleotide is covalently linked to the second single stranded oligonucleotide through a linker.

55. The method of claim 54, wherein the linker comprises an oligonucleotide, a peptide, a low pH-labile bond, or a disulfide bond.

56. The method of claim 54 or 55, wherein the linker comprises an oligonucleotide, optionally wherein the oligonucleotide comprises 1 to 10 thymidines or uridines.

57. The method of any one of claims 54 to 56, wherein the linker is more susceptible to cleavage in a mammalian extract than the first and second single stranded oligonucleotides.

58. The method of any one of claims 40 to 53, wherein the first single stranded oligonucleotide is not covalently linked to the second single stranded oligonucleotide.

59. The method of any one of claims 40 to 51 and 58, wherein the first single stranded oligonucleotide and the second single stranded oligonucleotide are delivered to the cell separately.

60. The method of any one of claims 40 to 59, wherein at least one nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide is a nucleotide analogue.

61. The method of claim 60, wherein the at least one nucleotide analogue results in an increase in T_m of the oligonucleotide in a range of 1 to 5 °C compared with an oligonucleotide that does not have the at least one nucleotide analogue.

62. The method of any one of claims 40 to 61, wherein at least one nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide comprises a 2' O-methyl.

63. The method of any one of claims 40 to 62, wherein each nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide comprises a 2' O-methyl.

64. The method of any one of claims 40 to 62, wherein the first single stranded oligonucleotide or second single stranded oligonucleotide comprises at least one ribonucleotide, at least one deoxyribonucleotide, or at least one bridged nucleotide.

65. The method of claim 64, wherein the bridged nucleotide is a LNA nucleotide, a cEt nucleotide or a ENA modified nucleotide.

66. The method of any one of claims 40 to 60, wherein each nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide is a LNA nucleotide.

67. The method of any one of claims 40 to 60, wherein the nucleotides of the first single stranded oligonucleotide or second single stranded oligonucleotide comprise alternating deoxyribonucleotides and 2'-fluoro-deoxyribonucleotides.

68. The method of any one of claims 40 to 60, wherein the nucleotides of the first single stranded oligonucleotide or second single stranded oligonucleotide comprise alternating deoxyribonucleotides and 2'-O-methyl nucleotides.

69. The method of any one of claims 40 to 60, wherein the nucleotides of the first single stranded oligonucleotide or second single stranded oligonucleotide comprise alternating deoxyribonucleotides and ENA nucleotide analogues.

70. The method of any one of claims 40 to 60, wherein the nucleotides of the first single stranded oligonucleotide or second single stranded oligonucleotide comprise alternating deoxyribonucleotides and LNA nucleotides.

71. The method of any one of claims 67 to 70, wherein the 5' nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide is a deoxyribonucleotide.

72. The method of any one of claims 40 to 60, wherein the nucleotides of the first single stranded oligonucleotide or second single stranded oligonucleotide comprise alternating LNA nucleotides and 2'-O-methyl nucleotides.

73. The method of claim 72, wherein the 5' nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide is a LNA nucleotide.

74. The method of any one of claims 40 to 60, wherein the nucleotides of the first single stranded oligonucleotide or second single stranded oligonucleotide comprise deoxyribonucleotides flanked by at least one LNA nucleotide on each of the 5' and 3' ends of the deoxyribonucleotides.

75. The method of any one of claims 40 to 74, wherein the first single stranded oligonucleotide or second single stranded oligonucleotide further comprises phosphorothioate internucleotide linkages between at least two nucleotides.

76. The method of claim 75, wherein the first single stranded oligonucleotide or second single stranded oligonucleotide further comprises phosphorothioate internucleotide linkages between all nucleotides.

77. The method of any one of claims 40 to 76, wherein the nucleotide at the 3' position of the first single stranded oligonucleotide or second single stranded oligonucleotide has a 3' hydroxyl group.

78. The method of any one of claims 40 to 76, wherein the nucleotide at the 3' position of the first single stranded oligonucleotide or second single stranded oligonucleotide has a 3' thiophosphate.

79. The method of any one of claims 40 to 78, a biotin moiety is conjugated to the 5' or 3' nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide.

80. A method of treating spinal muscular atrophy in a subject, the method comprising:

administering to the subject a first single stranded oligonucleotide complementary with at least 8 consecutive nucleotides of a PRC2-associated region of SMN2 and a second

single stranded oligonucleotide complementary with a splice control sequence of a precursor mRNA of SMN2, in amounts sufficient to increase expression of SMN protein in the subject.

81. A composition comprising:

a first single stranded oligonucleotide complementary with at least 8 consecutive
5 nucleotides of a PRC2-associated region of SMN2, and a second single stranded
oligonucleotide complementary to a splice control sequence of a precursor mRNA of SMN2.

82. The composition of claim 81, wherein the splice control sequence resides in an exon of SMN2.

83. The composition of claim 82, wherein the exon is exon 7 or exon 8.

10 84. The composition of claim 81, wherein the splice control sequence traverses an
intron-exon junction of SMN2.

85. The composition of claim 84, wherein the intron-exon junction is the intron
6/exon 7 junction or the intron 7/exon 8 junction.

15 86. The composition of claim 81, wherein the splice control sequence resides in an
intron of SMN2.

87. The composition of claim 86, wherein the intron is intron 6 or intron 7.

88. The composition of any one of claims 81 to 87, wherein the splice control
sequence comprises at least one hnRNAP binding sequence.

20 89. The composition of any one of claims 81 to 88, wherein the first single
stranded oligonucleotide has a sequence 5'-X-Y-Z, wherein X is any nucleotide, Y is a
nucleotide sequence of 6 nucleotides in length that is not a seed sequence of a human
microRNA, and Z is a nucleotide sequence of 1-23 nucleotides in length.

90. The composition of any one of claims 81 to 89, wherein the first single
stranded oligonucleotide does not comprise three or more consecutive guanosine nucleotides.

25 91. The composition of any one of claims 81 to 90, wherein the first single
stranded oligonucleotide does not comprise four or more consecutive guanosine nucleotides.

92. The composition of any one of claims 81 to 91, wherein the first single
stranded oligonucleotide is 8 to 30 nucleotides in length.

93. The composition of any one of claims 81 to 92, wherein the first single stranded oligonucleotide is 8 to 10 nucleotides in length and all but 1, 2, or 3 of the nucleotides of the complementary sequence of the PRC2-associated region are cytosine or guanosine nucleotides.

5 94. The composition of any one of claims 81 to 93, wherein the first single stranded oligonucleotide is covalently linked to the second single stranded oligonucleotide through a linker.

95. The composition of claim 94, wherein the linker comprises an oligonucleotide, a peptide, a low pH-labile bond, or a disulfide bond.

10 96. The composition of claim 94 or 95, wherein the linker comprises an oligonucleotide, optionally wherein the oligonucleotide comprises 1 to 10 thymidines or uridines.

15 97. The composition of any one of claims 94 to 96, wherein the linker is more susceptible to cleavage in a mammalian extract than the first and second single stranded oligonucleotides.

98. The composition of any one of claims 81 to 93, wherein the first single stranded oligonucleotide is not covalently linked to the second single stranded oligonucleotide.

99. The composition of any one of claims 81 to 98 further comprising a carrier.

20 100. The composition of claim 99, wherein the carrier is a pharmaceutically acceptable carrier.

101. The composition of any one of claims 81 to 100, wherein at least one nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide is a nucleotide analogue.

25 102. The composition of claim 101, wherein the at least one nucleotide analogue results in an increase in T_m of the oligonucleotide in a range of 1 to 5 °C compared with an oligonucleotide that does not have the at least one nucleotide analogue.

103. The composition of any one of claims 81 to 101, wherein at least one nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide comprises a 2' O-methyl.

104. The composition of any one of claims 81 to 103, wherein each nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide comprises a 2' O-methyl.

105. The composition of any one of claims 81 to 103, wherein the first single stranded oligonucleotide or second single stranded oligonucleotide comprises at least one ribonucleotide, at least one deoxyribonucleotide, or at least one bridged nucleotide.

106. The composition of claim 105, wherein the bridged nucleotide is a LNA nucleotide, a cEt nucleotide or a ENA modified nucleotide.

107. The composition of any one of claims 81 to 101, wherein each nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide is a LNA nucleotide.

108. The composition of any one of claims 81 to 101, wherein the nucleotides of the first single stranded oligonucleotide or second single stranded oligonucleotide comprise alternating deoxyribonucleotides and 2'-fluoro-deoxyribonucleotides.

109. The composition of any one of claims 81 to 101, wherein the nucleotides of the first single stranded oligonucleotide or second single stranded oligonucleotide comprise alternating deoxyribonucleotides and 2'-O-methyl nucleotides.

110. The composition of any one of claims 81 to 101, wherein the nucleotides of the first single stranded oligonucleotide or second single stranded oligonucleotide comprise alternating deoxyribonucleotides and ENA nucleotide analogues.

111. The composition of any one of claims 81 to 101, wherein the nucleotides of the first single stranded oligonucleotide or second single stranded oligonucleotide comprise alternating deoxyribonucleotides and LNA nucleotides.

112. The composition of any one of claims 108 to 111, wherein the 5' nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide is a deoxyribonucleotide.

113. The composition of any one of claims 81 to 101, wherein the nucleotides of the first single stranded oligonucleotide or second single stranded oligonucleotide comprise alternating LNA nucleotides and 2'-O-methyl nucleotides.

114. The composition of claim 113, wherein the 5' nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide is a LNA nucleotide.

115. The composition of any one of claims 81 to 101, wherein the nucleotides of the first single stranded oligonucleotide or second single stranded oligonucleotide comprise deoxyribonucleotides flanked by at least one LNA nucleotide on each of the 5' and 3' ends of the deoxyribonucleotides.

116. The composition of any one of claims 81 to 115, wherein the first single stranded oligonucleotide or second single stranded oligonucleotide further comprises phosphorothioate internucleotide linkages between at least two nucleotides.

117. The composition of claim 115 or 116, wherein the first single stranded oligonucleotide or second single stranded oligonucleotide further comprises phosphorothioate internucleotide linkages between all nucleotides.

118. The composition of any one of claims 81 to 117, wherein the nucleotide at the 3' position of the first single stranded oligonucleotide or second single stranded oligonucleotide has a 3' hydroxyl group.

119. The composition of any one of claims 81 to 118, wherein the nucleotide at the 3' position of the first single stranded oligonucleotide or second single stranded oligonucleotide has a 3' thiophosphate.

120.. The composition of any one of claims 81 to 119, further comprising a biotin moiety conjugated to the 5' or 3' nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide.

121. A compound comprising the general formula A-B-C,

wherein A is a single stranded oligonucleotide complementary with at least 8 consecutive nucleotides of a PRC2-associated region of a gene, B is a linker, and C is a single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA of the gene.

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122. The compound of claim 121, wherein B comprises an oligonucleotide, peptide, low pH labile bond, or disulfide bond.

123. The compound of claim 121 or 122, wherein the splice control sequence resides in an exon of the gene.

5 124. The compound of claim 121 or 122, wherein the splice control sequence traverses an intron-exon junction of the gene.

125. The compound of claim 121 or 122, wherein the splice control sequence resides in an intron of the gene.

10 126. The compound of any one of claims 121 to 125, wherein the splice control sequence comprises at least one hnRNAP binding sequence.

127. The compound of any one of claims 121 to 126, wherein hybridization of an oligonucleotide having the sequence of C with the splice control sequence of the precursor mRNA in a cell results in inclusion of a particular exon in a mature mRNA that arises from processing of the precursor mRNA in the cell.

15 128. The compound of any one of claims 121 to 126, wherein hybridization of an oligonucleotide having the sequence of C with the splice control sequence of the precursor mRNA in a cell results in exclusion of a particular intron or exon in a mature mRNA that arises from processing of the precursor mRNA in the cell.

129. The compound of any one of claims 121 to 128, wherein the gene is SMN2.

20 130. The compound of claim 129, wherein the splice control sequence resides in intron 6, intron 7, exon 7, exon 8 or at the junction of intron 7 and exon 8.

131. The compound of any one of claims 121 to 130, wherein A has a sequence 5'-X-Y-Z, wherein X is any nucleotide, Y is a nucleotide sequence of 6 nucleotides in length that is not a seed sequence of a human microRNA, and Z is a nucleotide sequence of 1-23
25 nucleotides in length.

132. The compound of any one of claims 121 to 131, wherein A does not comprise three or more consecutive guanosine nucleotides.

133. The compound of any one of claims 121 to 132, wherein A does not comprise four or more consecutive guanosine nucleotides.

134. The compound of any one of claims 121 to 133, wherein A is 8 to 30 nucleotides in length.

5 135. The compound of any one of claims 121 to 134, wherein A is 8 to 10 nucleotides in length and all but 1, 2, or 3 of the nucleotides of the complementary sequence of the PRC2-associated region are cytosine or guanosine nucleotides.

136. The compound of any one of claims 121 to 135, wherein the B is an oligonucleotide comprising 1 to 10 thymidines or uridines.

10 137. The compound of any one of claims 121 to 136, wherein B is more susceptible to cleavage in a mammalian extract than A and C.

138. The compound of any one of claims 121 to 137, wherein at least one nucleotide of the compound is a nucleotide analogue.

139. The compound of any one of claims 121 to 138, wherein at least one
15 nucleotide of the compound comprises a 2' O-methyl.

140. The compound of any one of claims 121 to 139, wherein each nucleotide of the compound comprises a 2' O-methyl.

141. The compound of any one of claims 121 to 140, wherein the compound comprises at least one ribonucleotide, at least one deoxyribonucleotide, or at least one
20 bridged nucleotide.

142.. The compound of claim 141, wherein the bridged nucleotide is a LNA nucleotide, a cEt nucleotide or a ENA modified nucleotide.

143. A kit comprising:

a first container housing first single stranded oligonucleotide complementary with at
25 least 8 consecutive nucleotides of a PRC2-associated region of a gene; and

a second container housing a second single stranded oligonucleotide complementary to a splice control sequence of a precursor mRNA of the gene.

144. The kit of claim 143, wherein the splice control sequence resides in an exon of the gene.

145. The kit of claim 143, wherein the splice control sequence traverses an intron-exon junction of the gene.

5 146. The kit of claim 145, wherein the splice control sequence resides in an intron of the gene.

147. The kit of any one of claims 143 to 146, wherein the splice control sequence comprises at least one hnRNAP binding sequence.

10 148. The kit of any one of claims 143 to 147, wherein hybridization of an oligonucleotide having the sequence of C with the splice control sequence of the precursor mRNA in a cell results in inclusion of a particular exon in a mature mRNA that arises from processing of the precursor mRNA in the cell.

15 149. The kit of any one of claims 143 to 149, wherein hybridization of an oligonucleotide having the sequence of C with the splice control sequence of the precursor mRNA in a cell results in exclusion of a particular intron or exon in a mature mRNA that arises from processing of the precursor mRNA in the cell.

150. The kit of any one of claims 143 to 149, wherein the gene is SMN2.

151. The kit of claim 150, wherein the splice control sequence resides in intron 6, intron 7, exon 7, exon 8 or at the junction of intron 7 and exon 8.

20 152. The kit of any one of claims 143 to 151, wherein the first single stranded oligonucleotide has a sequence 5'-X-Y-Z, wherein X is any nucleotide, Y is a nucleotide sequence of 6 nucleotides in length that is not a seed sequence of a human microRNA, and Z is a nucleotide sequence of 1-23 nucleotides in length.

25 153. The kit of any one of claims 143 to 152, wherein the first single stranded oligonucleotide does not comprise three or more consecutive guanosine nucleotides.

154. The kit of any one of claims 143 to 153, wherein the first single stranded oligonucleotide does not comprise four or more consecutive guanosine nucleotides.

155. The kit of any one of claims 143 to 154, wherein the first single stranded oligonucleotide is 8 to 30 nucleotides in length.

156. The kit of any one of claims 143 to 155, wherein the first single stranded oligonucleotide is 8 to 10 nucleotides in length and all but 1, 2, or 3 of the nucleotides of the complementary sequence of the PRC2-associated region are cytosine or guanosine nucleotides.

157. The kit of any one of claims 143 to 156, wherein at least one nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide is a nucleotide analogue.

158. The kit of any one of claims 143 to 157, wherein at least one nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide comprises a 2' O-methyl.

159. The kit of any one of claims 143 to 158, wherein each nucleotide of the first single stranded oligonucleotide or second single stranded oligonucleotide comprises a 2' O-methyl.

160. The kit of any one of claims 143 to 159, wherein the first single stranded oligonucleotide or second single stranded oligonucleotide comprises at least one ribonucleotide, at least one deoxyribonucleotide, or at least one bridged nucleotide.

161. The kit of claim 160, wherein the bridged nucleotide is a LNA nucleotide, a cEt nucleotide or a ENA modified nucleotide.

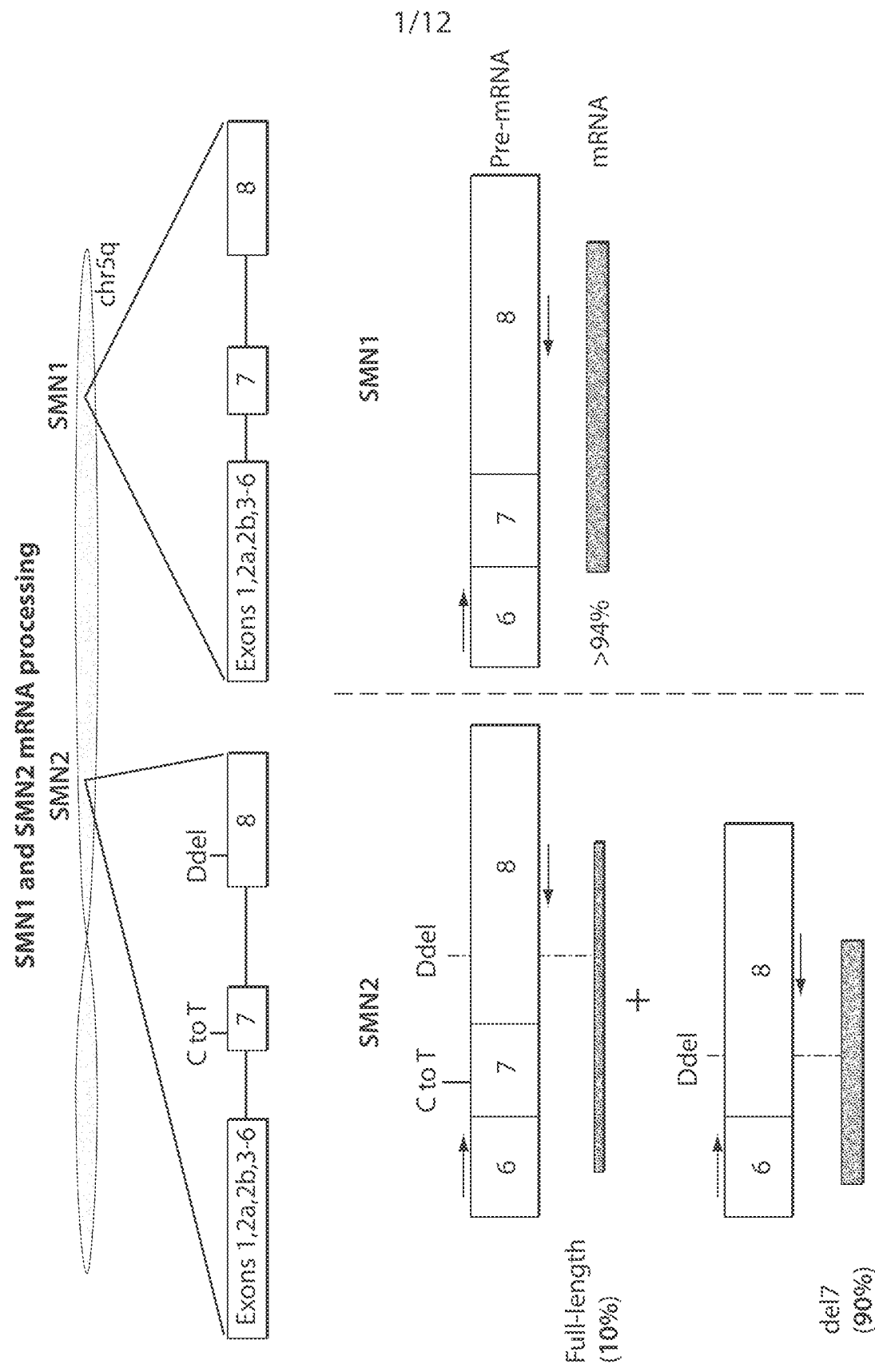
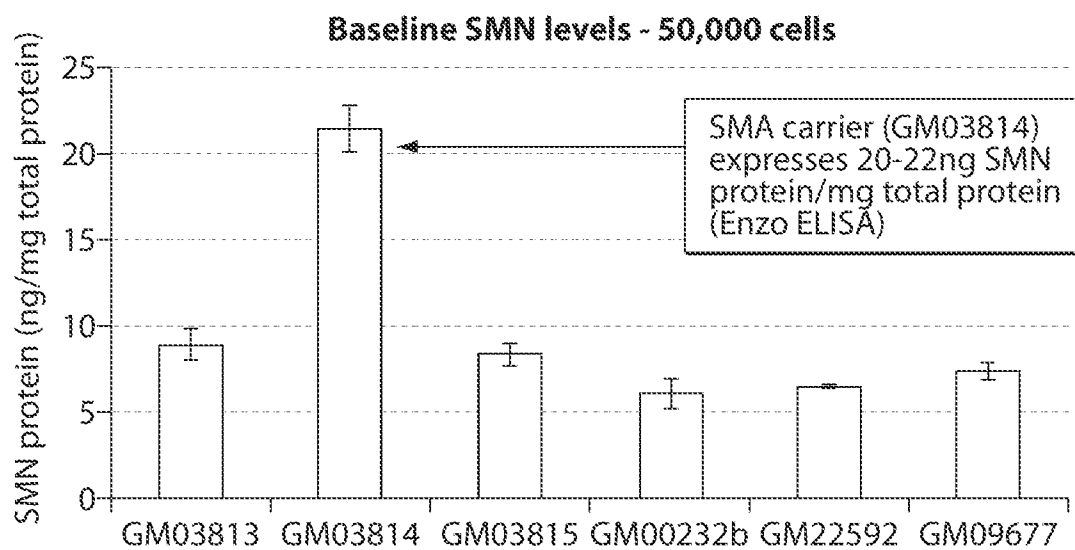


Fig. 1

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SMA Classification and protein expression levels

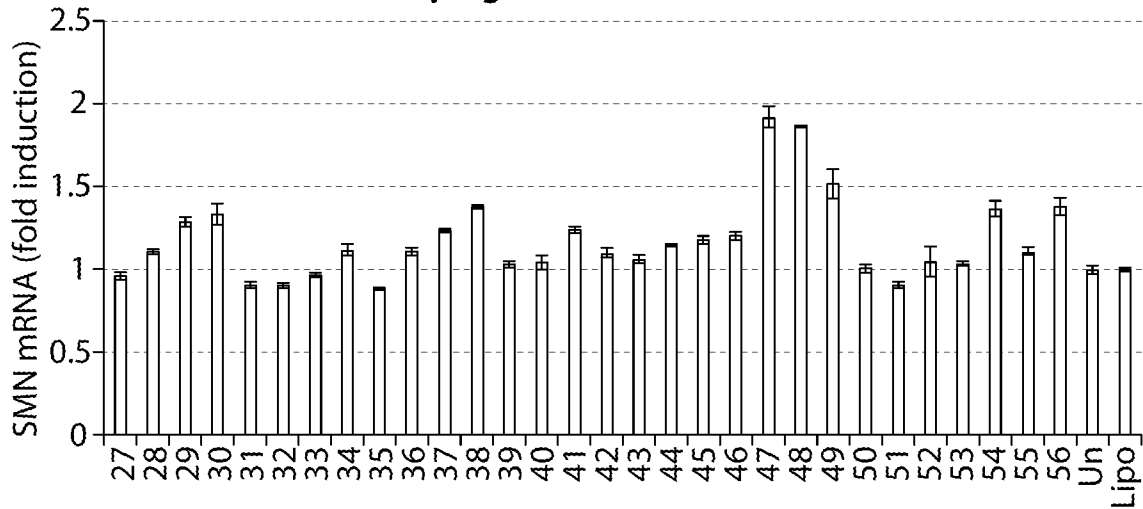
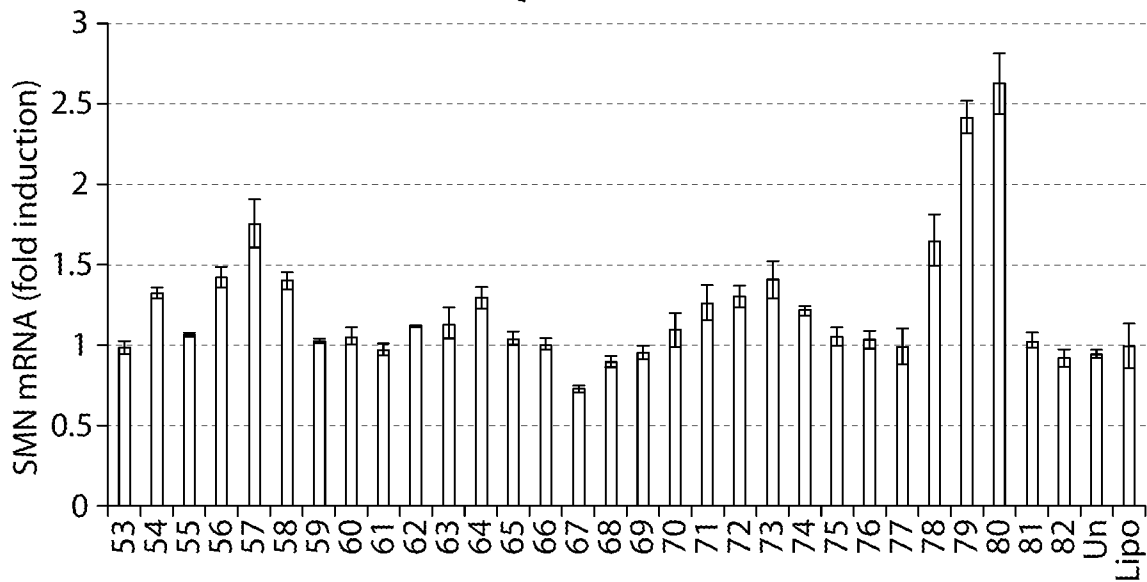
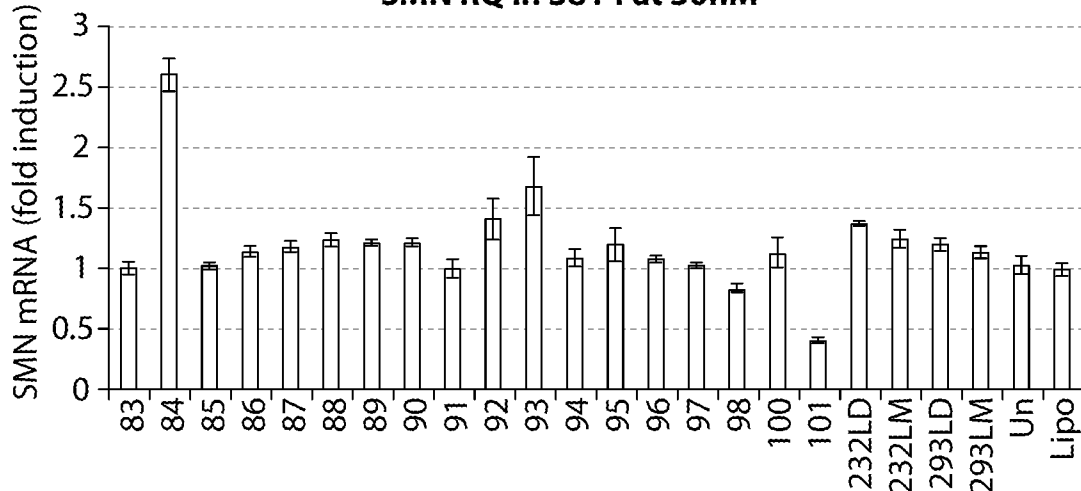
	Age	Sex	Race	SMN2 Copy #	Deletions in SMN1	SMA Type
GM03813	3	M	W	2	Homo, Ex7	I
GM00232b		M	W	1	Homo, Ex7	I
GM22592	1	M		3	Homo, Ex7	II
GM09677	2	M	W	2	Homo, Ex7	I
GM03814		F	W	2	Hetero, Ex7	Clinically Normal Mother
GM03815		M	W		NA	Clinically Normal Father

**Fig. 2**

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Family 553

3814 - female carrier

SMN upregulation in 3814 - 30nM**SMN RQ in 3814 at 30nM****SMN RQ in 3814 at 30nM****Fig. 3**

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Family 553

3813 3yr male SMA

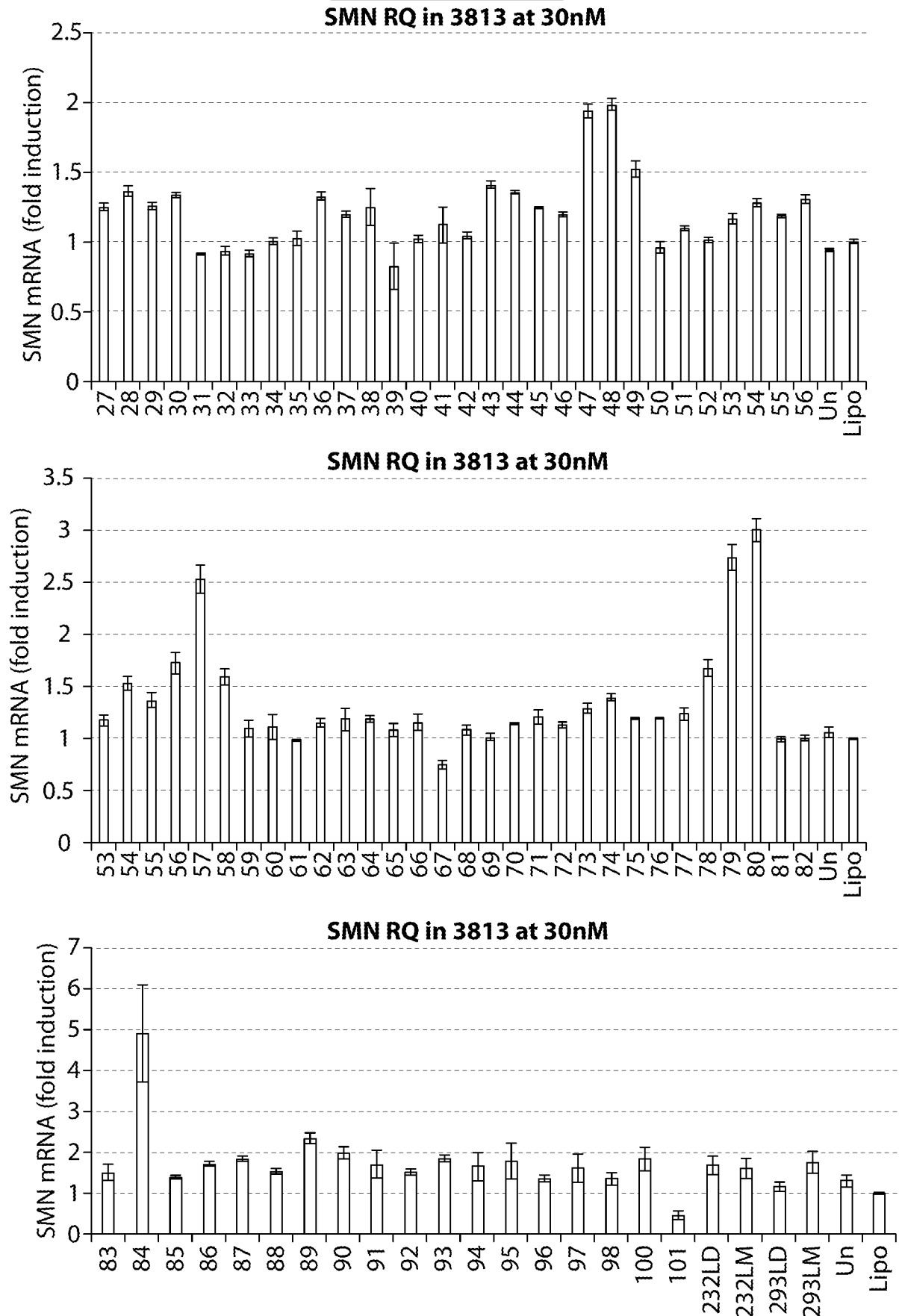


Fig. 4

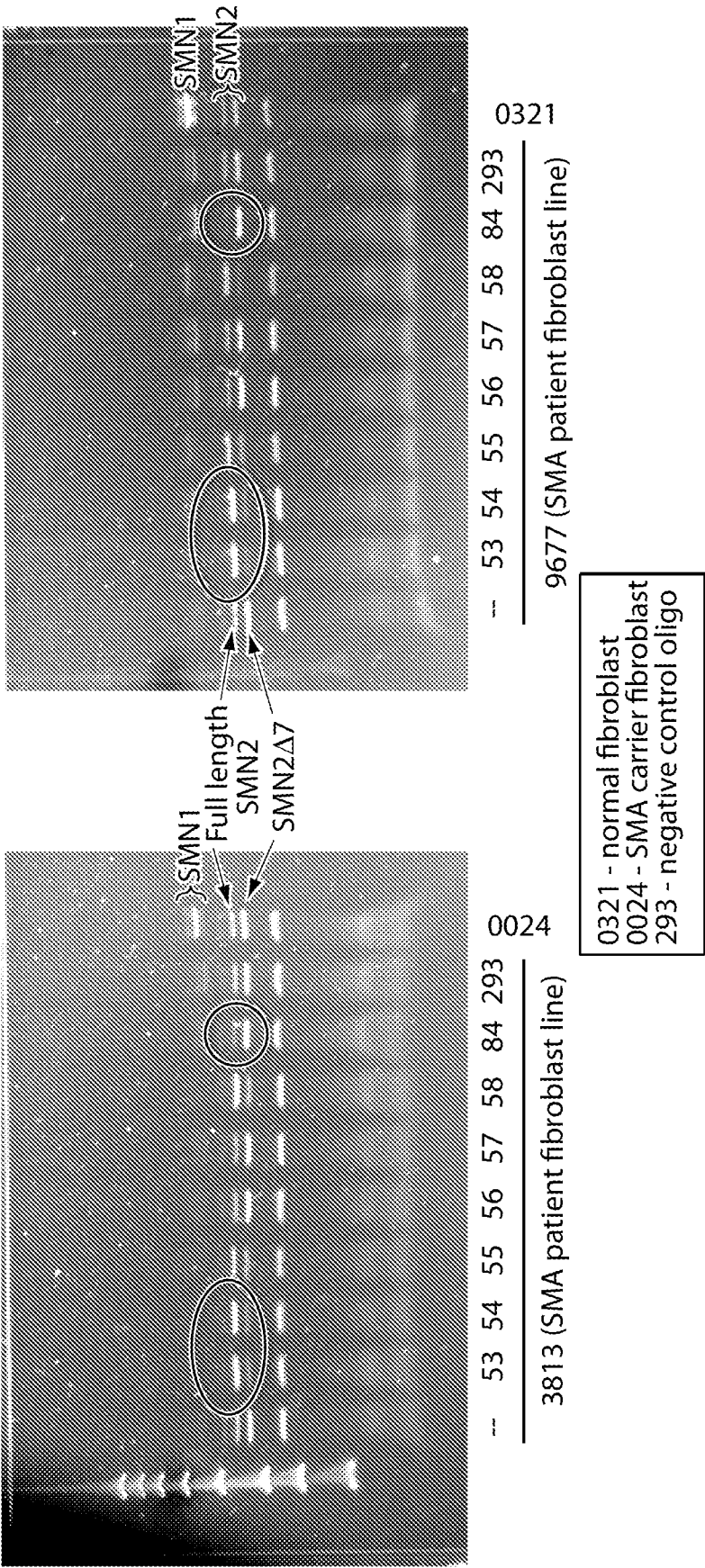


Fig. 5

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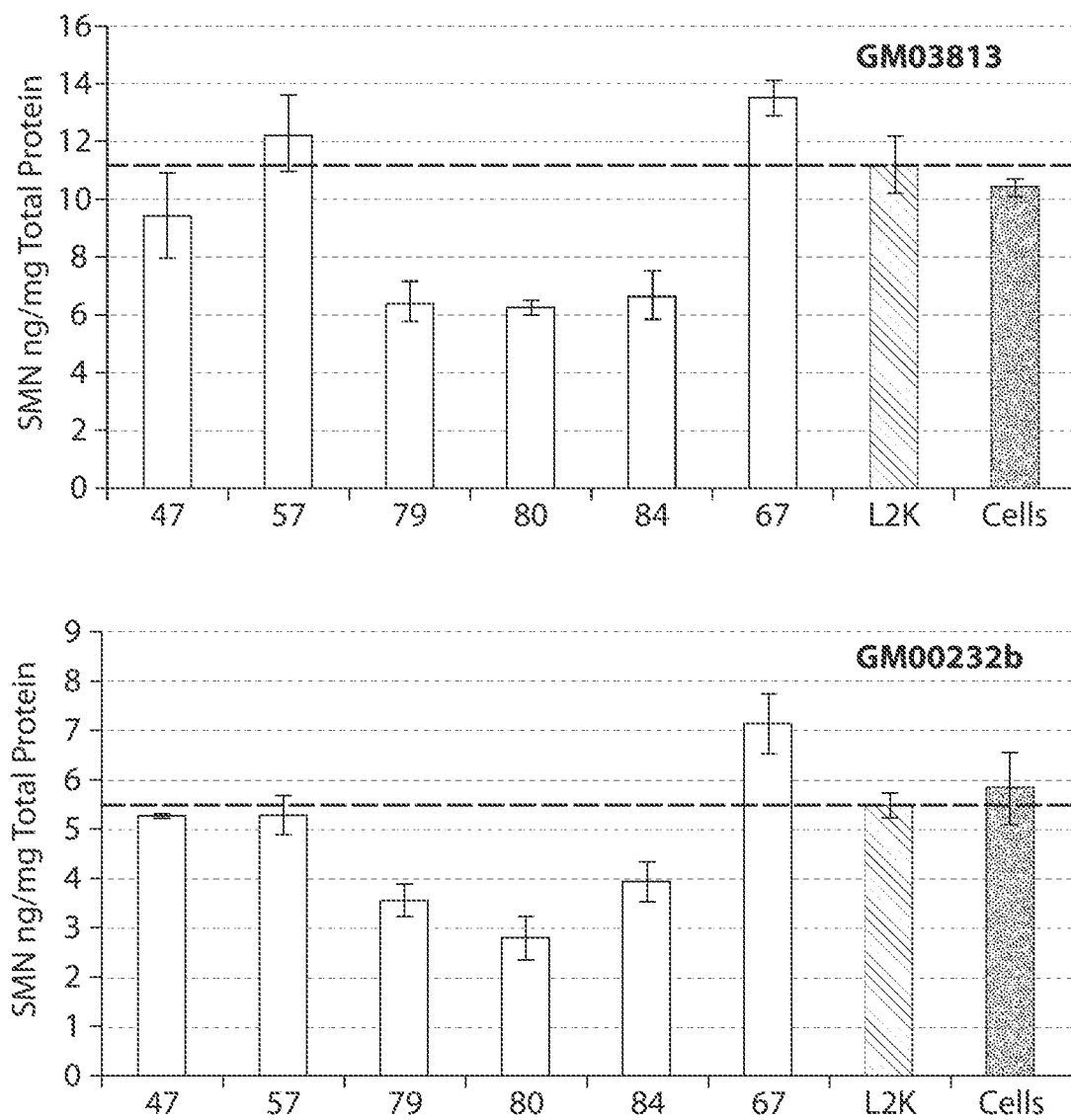


Fig. 6-1

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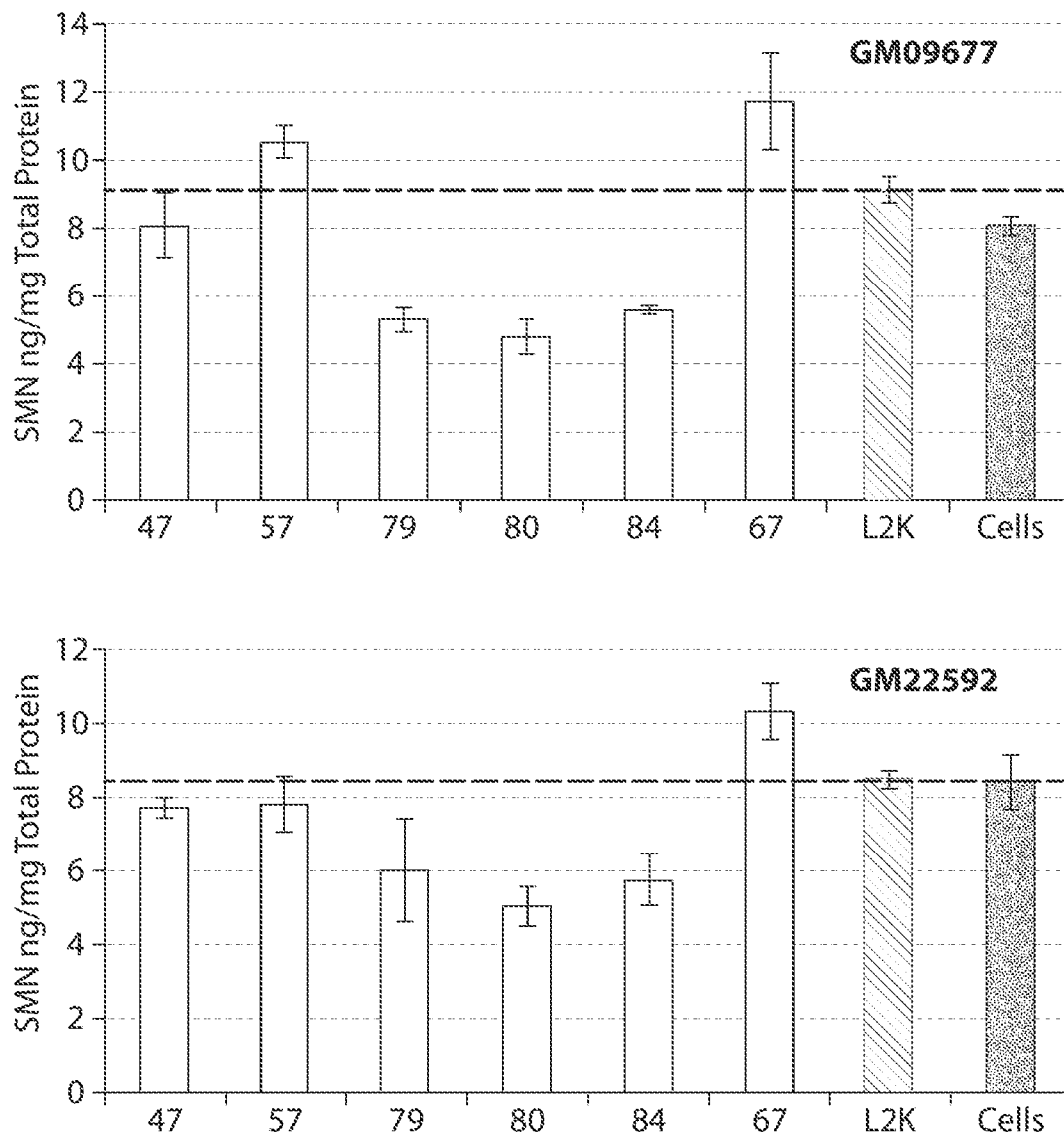


Fig. 6-2

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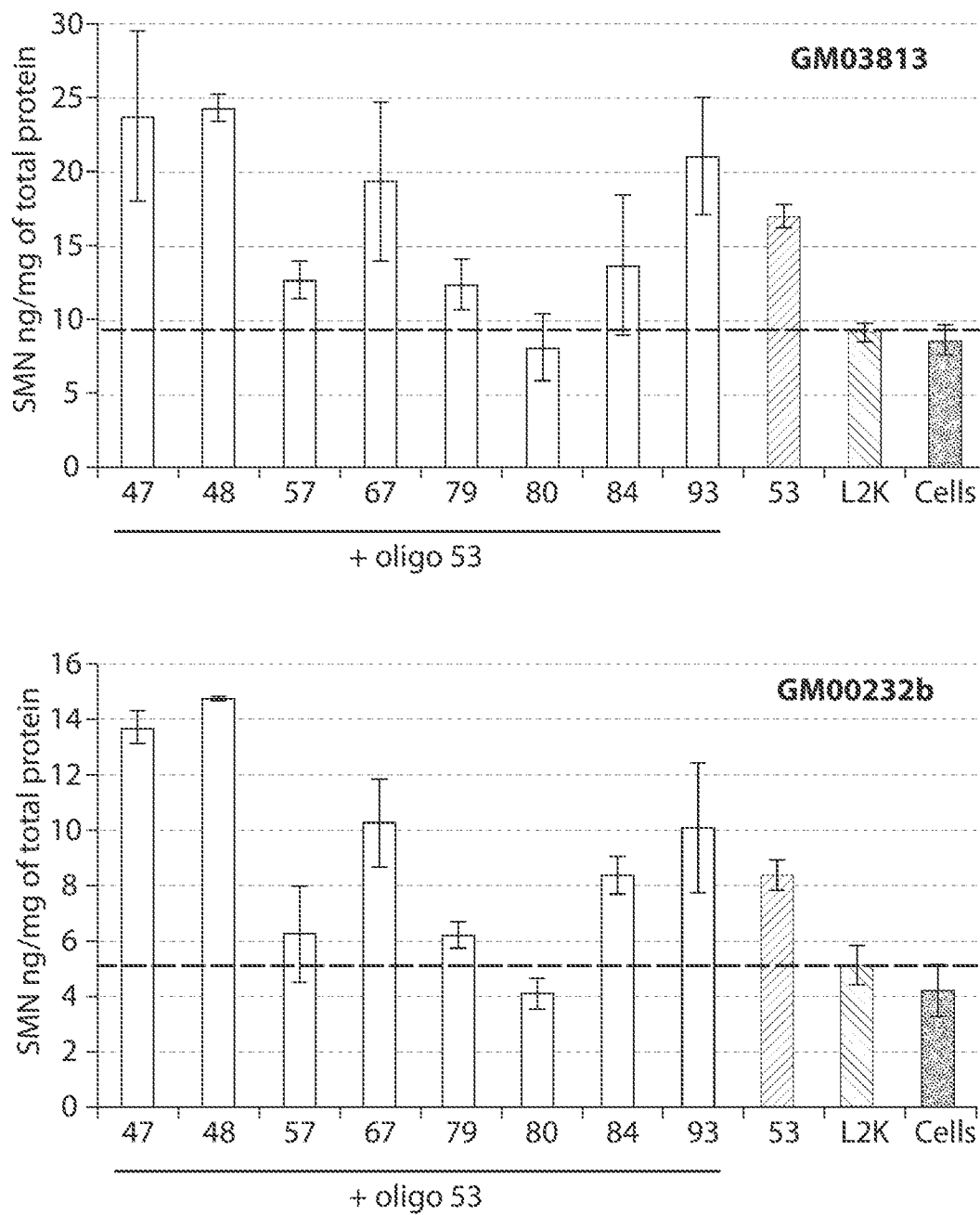


Fig. 7-1

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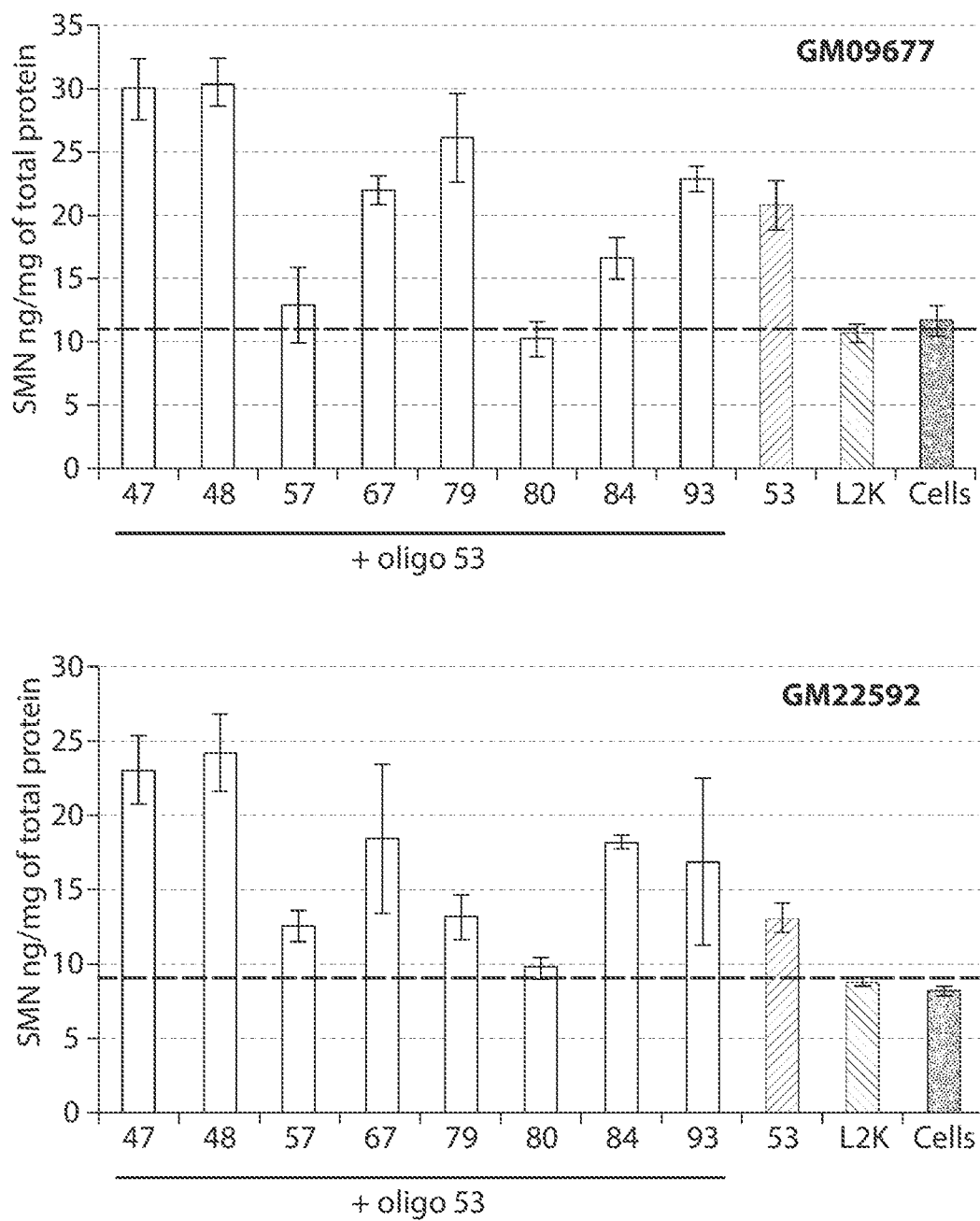


Fig. 7-2

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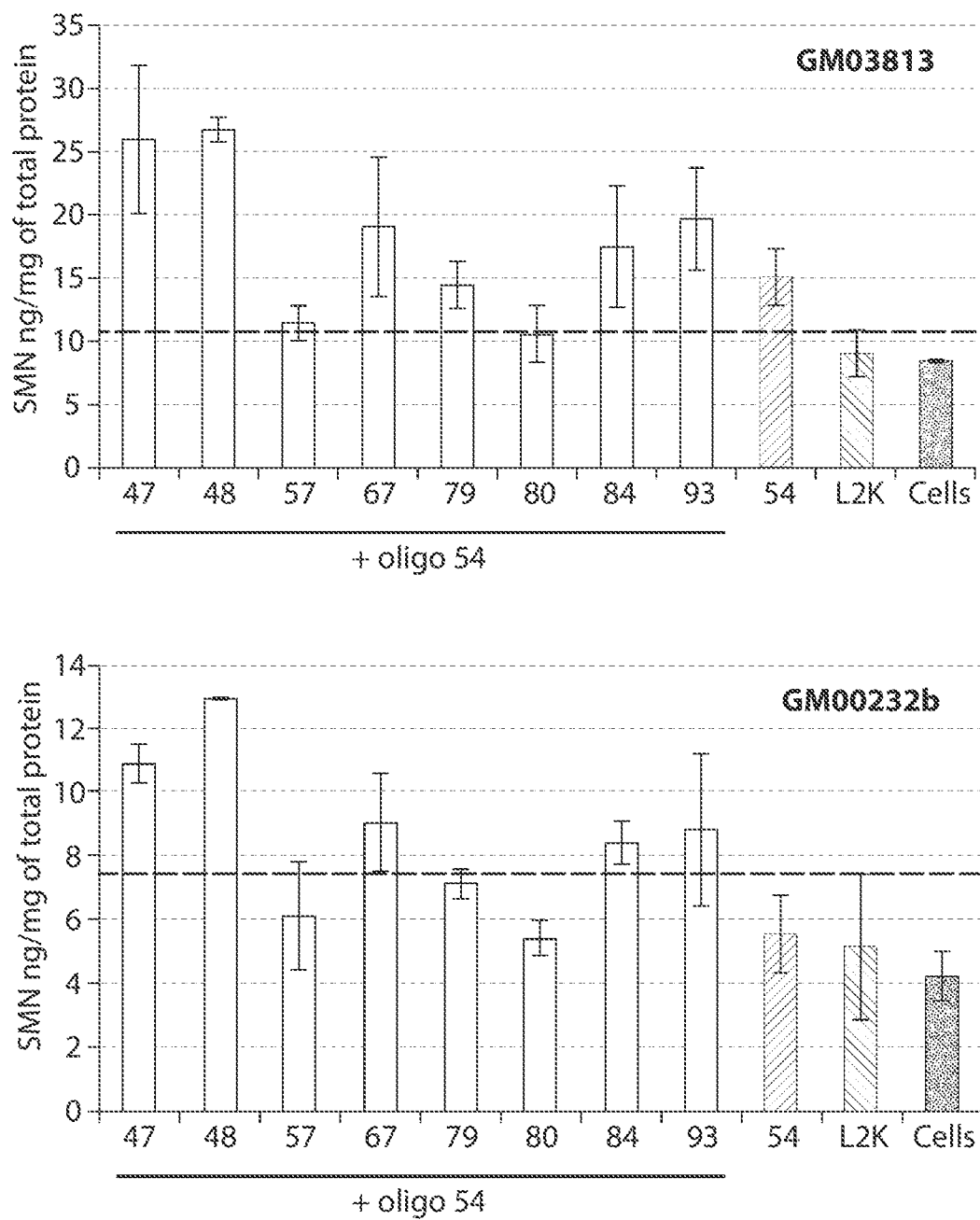


Fig. 8-1

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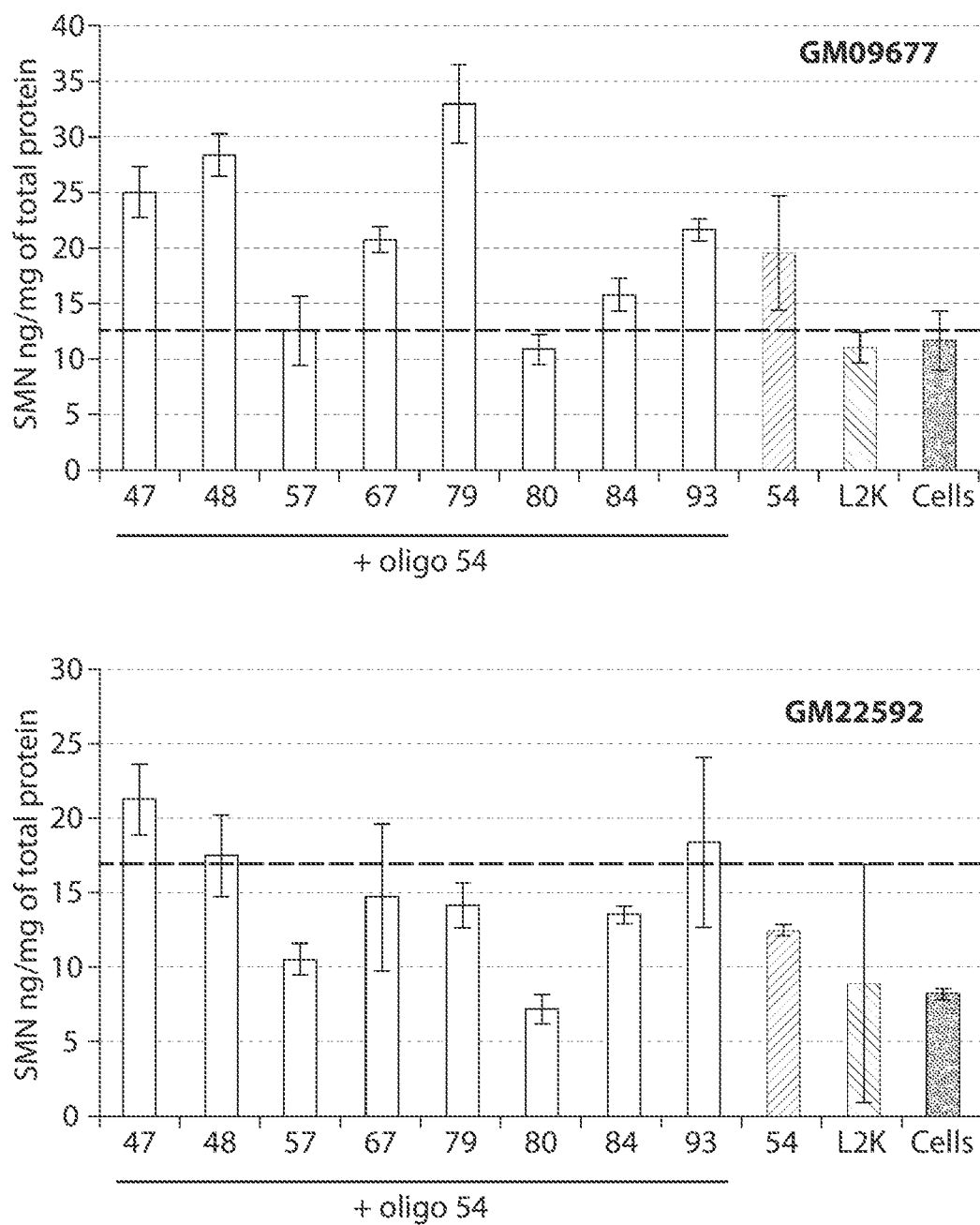


Fig. 8-2

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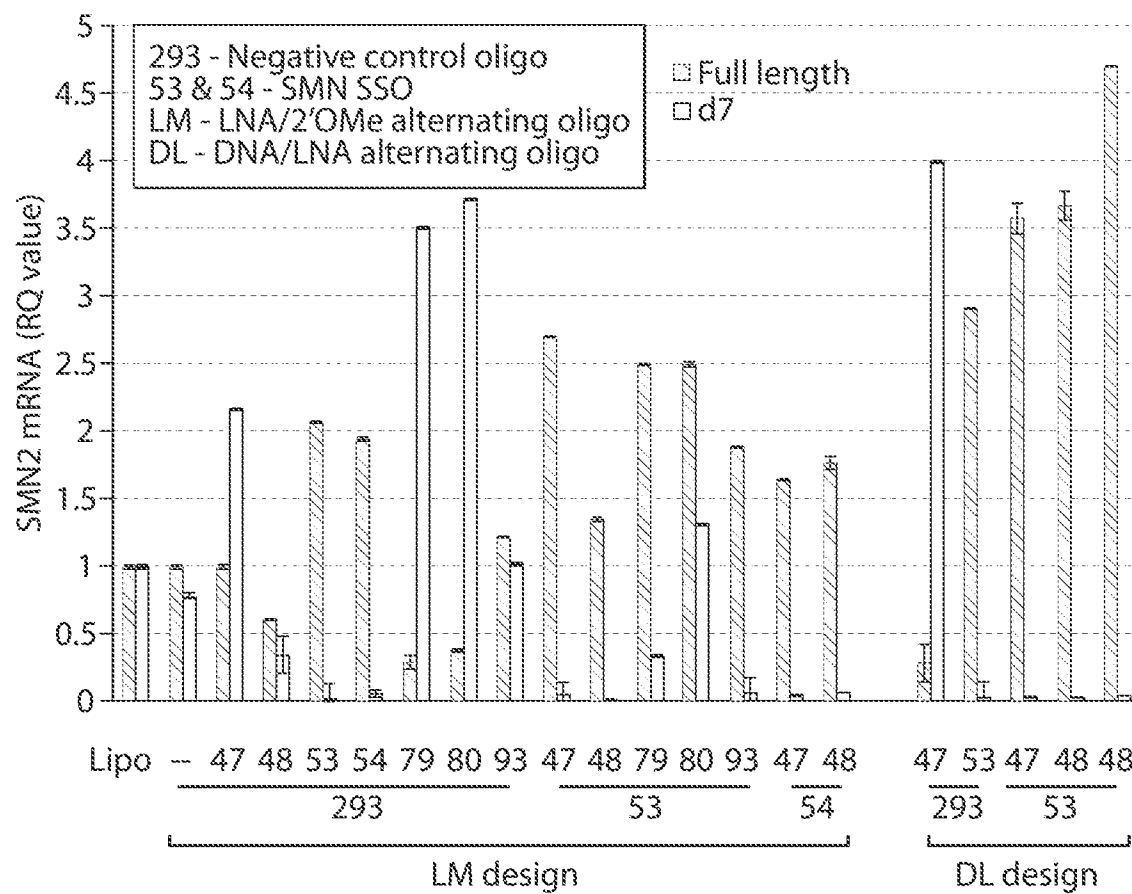


Fig. 9

A. CLASSIFICATION OF SUBJECT MATTER**C12N 15/11(2006.01)i, C07H 21/00(2006.01)i, C12N 15/63(2006.01)i, A61K 31/7088(2006.01)i**

According to International Patent Classification (IPC) or to both national classification and IPC

B. FIELDS SEARCHED

Minimum documentation searched (classification system followed by classification symbols)

C12N 15/11; C07H 21/04; C12N 15/12; A61K 31/423; G01N 33/50; A61K 38/21; C07H 21/00; C12N 15/63; A61K 31/7088

Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched

Korean utility models and applications for utility models

Japanese utility models and applications for utility models

Electronic data base consulted during the international search (name of data base and, where practicable, search terms used)

eKOMPASS(KIPO internal) & Keywords: Survival motor neurone (SMN) activation, PRC2-associated region

C. DOCUMENTS CONSIDERED TO BE RELEVANT

Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
A	US 2011-0086833 A1 (PAUSHKIN, SERGEY V. et al.) 14 April 2011 See abstract; claims 37 and 38.	1-5, 26-27, 40-47 , 81-88, 121-125 , 143-147
A	WO 2011-032109 A1 (SMA FOUNDATION) 17 March 2011 See abstract; claims 13-14 and 24-26.	1-5, 26-27, 40-47 , 81-88, 121-125 , 143-147
A	KANHERE, ADITI et al., `Short RNAs are transcribed from repressed polycomb target genes and interact with polycomb repressive complex-2`, Cell Press, 11 June 2010, Vol. 38, No. 5, pp.675-688, ISSN 1097-2765 See abstract; figures 5 and 6.	1-5, 26-27, 40-47 , 81-88, 121-125 , 143-147
A	WANG, KEVIN C. et al., `Molecular mechanisms of long noncoding RNAs`, Cell Press, 16 September 2011, Vol. 43, No. 6, pp.904-914, ISSN 1097-2765 See abstract.	1-5, 26-27, 40-47 , 81-88, 121-125 , 143-147
A	EP 0999270 A1 (INSTITUT NATIONAL DE LA SANTE ET DE LA RECHERCHE MEDICALE. et al.) 10 May 2000 See abstract; claims 1-5.	1-5, 26-27, 40-47 , 81-88, 121-125 , 143-147



Further documents are listed in the continuation of Box C.



See patent family annex.

* Special categories of cited documents:

"A" document defining the general state of the art which is not considered to be of particular relevance

"E" earlier application or patent but published on or after the international filing date

"L" document which may throw doubts on priority claim(s) or which is cited to establish the publication date of citation or other special reason (as specified)

"O" document referring to an oral disclosure, use, exhibition or other means

"P" document published prior to the international filing date but later than the priority date claimed

"T" later document published after the international filing date or priority date and not in conflict with the application but cited to understand the principle or theory underlying the invention

"X" document of particular relevance; the claimed invention cannot be considered novel or cannot be considered to involve an inventive step when the document is taken alone

"Y" document of particular relevance; the claimed invention cannot be considered to involve an inventive step when the document is combined with one or more other such documents, such combination being obvious to a person skilled in the art

"&" document member of the same patent family

Date of the actual completion of the international search

29 July 2013 (29.07.2013)

Date of mailing of the international search report

29 July 2013 (29.07.2013)

Name and mailing address of the ISA/KR


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INTERNATIONAL SEARCH REPORT

International application No.
PCT/US2013/041440

Box No. II Observations where certain claims were found unsearchable (Continuation of item 2 of first sheet)

This international search report has not been established in respect of certain claims under Article 17(2)(a) for the following reasons:

1. ☒ Claims Nos.: 37-39,80
because they relate to subject matter not required to be searched by this Authority, namely:
Claims 37-39 and 80 pertain to methods for treatment of the human body by therapy, and thus relate to a subject matter which this International Searching Authority is not required, under Article 17(2)(a)(i) of the PCT and Rule 39.1(iv) of the Regulations under the PCT, to search.
2. ☒ Claims Nos.: 7,11,19,22,30-32,36,39,55,61,65,73,76,95,100,102,106,114,130,142,151,161
because they relate to parts of the international application that do not comply with the prescribed requirements to such an extent that no meaningful international search can be carried out, specifically:
Claims 7, 11, 19, 22, 30-32, 36, 39, 55, 61, 65, 73, 76, 95, 100, 102, 106, 114, 130, 142, 151, and 161 are unclear since they refer to claims which are not searchable due to not being drafted in accordance with the second and third sentence of Rule 6.4(a).
3. ☒ Claims Nos.: See Extra Sheet
because they are dependent claims and are not drafted in accordance with the second and third sentences of Rule 6.4(a).

Box No. III Observations where unity of invention is lacking (Continuation of item 3 of first sheet)

This International Searching Authority found multiple inventions in this international application, as follows:

1. ☐ As all required additional search fees were timely paid by the applicant, this international search report covers all searchable claims.
2. ☐ As all searchable claims could be searched without effort justifying an additional fee, this Authority did not invite payment of any additional fee.
3. ☐ As only some of the required additional search fees were timely paid by the applicant, this international search report covers only those claims for which fees were paid, specifically claims Nos.:
4. ☐ No required additional search fees were timely paid by the applicant. Consequently, this international search report is restricted to the invention first mentioned in the claims; it is covered by claims Nos.:

Remark on Protest

- ☐ The additional search fees were accompanied by the applicant's protest and, where applicable, the payment of a protest fee.
- ☐ The additional search fees were accompanied by the applicant's protest but the applicable protest fee was not paid within the time limit specified in the invitation.
- ☐ No protest accompanied the payment of additional search fees.

Continuation of Box No. II:

Claims Nos.: 6, 8-10, 12-18, 20-21, 23-25, 28-29, 33-35, 37-38, 48-54, 56-60, 62-64, 66-72, 74-75, 77-79, 89-94, 96-99, 101, 103-105, 107-113, 115-120, 126-129, 131-141, 148-150, 152-160.

INTERNATIONAL SEARCH REPORT

Information on patent family members

International application No.

PCT/US2013/041440

Patent document cited in search report	Publication date	Patent family member(s)	Publication date
WO 2011-032109 A1	17/03/2011	None	
US 2011-0086833 A1	14/04/2011	WO 2009-151546 A2 WO 2009-151546 A3	17/12/2009 18/03/2010
EP 0999270 A1	10/05/2000	AU 1999-64696 A1 WO 00-21553 A2 WO 00-21553 A3	01/05/2000 20/04/2000 27/07/2000