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(54) Title: IMPROVED COMPOSITIONS FOR TREATING MUSCULAR DYSTROPHY

(57) Abstract: Improved compositions and methods for treating muscular dystrophy by administering eteplirsen, an antisense molecule capable of binding to a selected target site in the human dystrophin gene to induce exon skipping, are described.

INTERNATIONAL SEARCH REPORT

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C. DOCUMENTS CONSIDERED TO BE RELEVANT		
Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
X	"ETEPLIRSEN - Inhibitor of Dystrophin Expression - Treatment of Duchenne Muscular Dystrophy", Drugs of the Future 38(1), 1 January 2013 (2013-01-01), pages 13-17, XP055135338, DOI: 10.1358/dof.2013.38.1.1924780 Retrieved from the Internet: URL:https://journals.prous.com/journals/se rvlet/xmlxsl/dof/20133801/pdf/df380013.pdf ?p_JournalId=2&p_refId=1924780&p_IsPs=N [retrieved on 2014-08-19] page 15, column 2, paragraph 6 - page 16, column 1, paragraph 2 ----- -/--	1-26
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X	<p>Ed Kaye: "Results of the Eteplirsen Phase 2b and Phase 2b Extension Study in Duchenne Muscular Dystrophy", Abstracts 8th Annual Meeting of the Oligonucleotide Therapeutics Society Boston, Massachusetts October 28-31, 2012 Nucleic Acid Therapeutics, 1 December 2012 (2012-12-01), page A13, XP055135355, New Rochelle DOI: 10.1089/nat.2012.1501 Retrieved from the Internet: URL:http://search.proquest.com/docview/1366359015 [retrieved on 2014-08-19] abstract</p>	1-26
X	<p align="center">-----</p> <p>MICHELE A SCULLY ET AL: "Review of Phase II and Phase III clinical trials for Duchenne muscular dystrophy", EXPERT OPINION ON ORPHAN DRUGS, vol. 1, no. 1, 1 January 2013 (2013-01-01), pages 33-46, XP055135324, DOI: 10.1517/21678707.2013.746939 page 38, column 1, paragraph 2; table 1</p>	1-26
A	<p align="center">-----</p> <p>Anonymous: "Efficacy Study of AVI-4658 to Induce Dystrophin Expression in Selected Duchenne Muscular Dystrophy Patients - Full Text View - ClinicalTrials.gov", 22 January 2013 (2013-01-22), XP055135303, Retrieved from the Internet: URL:https://clinicaltrials.gov/ct2/show/NC01396239?term=eteplirsen&rank=1 [retrieved on 2014-08-19] the whole document</p> <p align="center">-----</p> <p align="center">-/--</p>	1-26

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A	<p>Dubowitz Neuromuscular ET AL: "Exon skipping and dystrophin restoration in patients with Duchenne muscular dystrophy after systemic phosphorodiamidate morpholino oligomer treatment: an open-label, phase 2, dose-escalation study", Lancet Published Online July, 1 January 2011 (2011-01-01), pages 595-605, XP055135286, DOI: 10.1016/S0140- Retrieved from the Internet: URL:http://ac.els-cdn.com/S0140673611607563/1-s2.0-S0140673611607563-main.pdf?_tid=656f1116-2792-11e4-858f-00000aab0f6b&acdnat=1408447224_8a7cd06e1ac8e0be5f8ad14e5fc097fe [retrieved on 2014-08-19] the whole document</p> <p align="center">-----</p>	1-26
X,P	<p>Jerry R Mendell ET AL: "Eteplirsen for the treatment of Duchenne muscular dystrophy", Annals of neurology, 1 November 2013 (2013-11-01), pages 637-647, XP055135300, United States DOI: 10.1002/ana.23982 Retrieved from the Internet: URL:http://onlinelibrary.wiley.com/doi/10.1002/ana.23982/abstract the whole document</p> <p align="center">-----</p>	1-26