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(71) Applicant(s):

Ascidian Therapeutics, Inc.

80 Guest Street, Boston 02135, Massachusetts,

United States of America

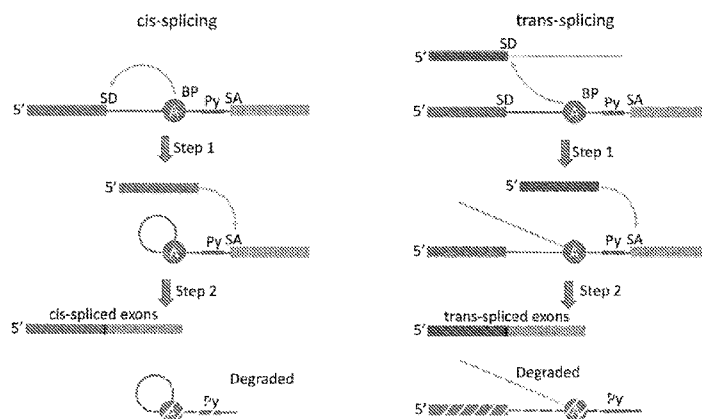
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(54) Title of the Invention: ABCA4 trans-splicing molecules

Abstract Title: ABCA4 trans-splicing molecules

(57) Provided herein are nucleic acid trans-splicing molecules (e.g., pre-mRNA trans-splicing molecules (RTMs); RNA exon editing molecules) capable of correcting mutations in the ABCA4 gene. Such molecules are useful in the treatment of disorders such as ABCA4-associated retinal dystrophies (e.g., Stargardt Disease or cone-rod dystrophy). Also described herein are methods of using the nucleic acid trans-splicing molecules described herein to correct mutations in ABCA4, thereby treating disorders associated with mutations in ABCA4 and use of the nucleic acid trans-splicing molecules described herein for treating disorders associated with mutations in ABCA4 and in the preparation of medicaments for the treatment of disorders associated with mutations in ABCA4.

FIG. 14B



GB 2634434 A continuation

(72) Inventor(s):

Rebekka Krumbach
Scott Dooley
Akiko Doi
Kirk Burkhart
Jesse Gray
Lingtao Peng
Dennis Wu
Akiko Noma
Kirk Gosik
Shimyn Slomovic
Adam Clemens
Robert Bell

(74) Agent and/or Address for Service:

Sagittarius IP
Marlow International Parkway, Marlow, Buckinghamshire,
SL7 1YL, United Kingdom