



- (51) **International Patent Classification:**
C12N 15/90 (2006.01) C12N 9/22 (2006.01)
- (21) **International Application Number:**
PCT/US2023/066969
- (22) **International Filing Date:**
12 May 2023 (12.05.2023)
- (25) **Filing Language:** English
- (26) **Publication Language:** English
- (30) **Priority Data:**
63/341,665 13 May 2022 (13.05.2022) US
63/478,472 04 January 2023 (04.01.2023) US
- (71) **Applicant:** ASCIDIAN THERAPEUTICS, INC.
[US/US]; 80 Guest Street, Boston, Massachusetts 02135 (US).
- (72) **Inventors:** KRUMBACH, Rebekka; 80 Guest Street, Boston, Massachusetts 02135 (US). DOOLEY, Scott; 80 Guest Street, Boston, Massachusetts 02135 (US). DOI, Akiko; 80 Guest Street, Boston, Massachusetts 02135 (US). BURKHART, Kirk; 80 Guest Street, Boston, Massachusetts 02135 (US). GRAY, Jesse; 80 Guest Street, Boston, Massachusetts 02135 (US). PENG, Lingtao; 80 Guest Street, Boston, Massachusetts 02135 (US). WU, Dennis;

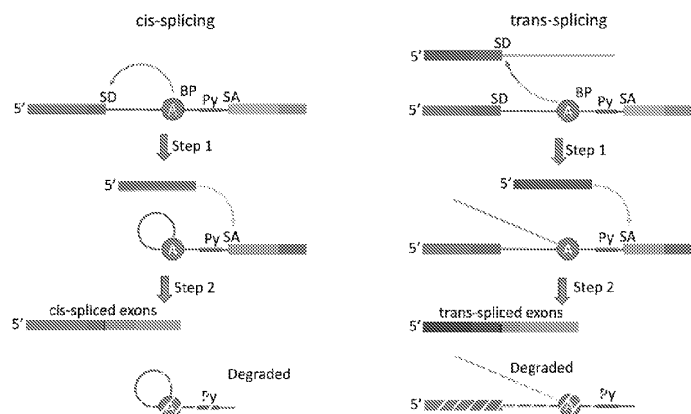
80 Guest Street, Boston, Massachusetts 02135 (US). **NO-MA, Akiko**; 80 Guest Street, Boston, Massachusetts 02135 (US). **GOSIK, Kirk**; 80 Guest Street, Boston, Massachusetts 02135 (US). **SLOMOVIC, Shimyn**; 80 Guest Street, Boston, Massachusetts 02135 (US). **CLEMENS, Adam**; 80 Guest Street, Boston, Massachusetts 02135 (US). **BELL, Robert**; 80 Guest Street, Boston, Massachusetts 02135 (US).

(74) **Agent:** OWENS, Thomas; Wilson Sonsini Goodrich & Rosati, 650 Page Mill Road, Palo Alto, California 94304 (US).

(81) **Designated States** (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CV, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IQ, IR, IS, IT, JM, JO, JP, KE, KG, KH, KN, KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD, MG, MK, MN, MU, MW, MX, MY, MZ, NA, NG, NI, NO, NZ, OM, PA, PE, PG, PH, PL, PT, QA, RO, RS, RU, RW, SA, SC, SD, SE, SG, SK, SL, ST, SV, SY, TH, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, WS, ZA, ZM, ZW.

(54) **Title:** ABCA4 TRANS-SPLICING MOLECULES

FIG. 14B



(57) **Abstract:** 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.

[Continued on next page]

(84) Designated States (*unless otherwise indicated, for every kind of regional protection available*): ARIPO (BW, CV, GH, GM, KE, LR, LS, MW, MZ, NA, RW, SC, SD, SL, ST, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, RU, TJ, TM), European (AL, AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HR, HU, IE, IS, IT, LT, LU, LV, MC, ME, MK, MT, NL, NO, PL, PT, RO, RS, SE, SI, SK, SM, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, KM, ML, MR, NE, SN, TD, TG).

Published:

- with international search report (Art. 21(3))
- with sequence listing part of description (Rule 5.2(a))

(88) Date of publication of the international search report:

28 March 2024 (28.03.2024)