



(51) International Patent Classification:

A61K 31/4178 (2006.01) C07D 401/14 (2006.01)

A61K 47/59 (2017.01) C07D 403/14 (2006.01)

A61K 47/60 (2017.01) C07D 487/04 (2006.01)

(21) International Application Number:

PCT/US2023/010331

(22) International Filing Date:

06 January 2023 (06.01.2023)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/297,090 06 January 2022 (06.01.2022) US

63/382,854 08 November 2022 (08.11.2022) US

(71) Applicant: DESIGN THERAPEUTICS, INC. [US/US];

6005 Hidden Valley Road, Suite 110, Carlsbad, California 92011 (US).

(72) Inventors; and

(71) Applicants: ANSARI, Aseem [IN/US]; 6005 Hidden Valley Road, Suite 110, Carlsbad, California 92011 (US).

BHAT, Abhijit [US/US]; 6005 Hidden Valley Road, Suite 110, Carlsbad, California 92011 (US). JEFFRIES, Sean [US/US]; 6005 Hidden Valley Road, Suite 110, Carlsbad, California 92011 (US).

SHAH, Pratik [US/US]; 6005 Hidden Valley Road, Suite 110, Carlsbad, California 92011 (US).

ZHANG, Chengzhi [US/US]; 6005 Hidden Valley Road, Suite 110, Carlsbad, California 92011 (US).

(74) Agent: OTRUBOVA, Katerina; 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, 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.

(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, 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))
- before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments (Rule 48.2(h))

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

24 August 2023 (24.08.2023)

(54) Title: COMPOUNDS AND METHODS FOR TREATING FRIEDREICH'S ATAXIA

(57) Abstract: The present disclosure relates to compounds and methods for modulating the expression of *fxn*, and treating diseases and conditions in which *fxn* plays an active role. The compound can be a transcription modulator molecule having a first terminus, a second terminus, and oligomeric backbone, wherein: a) the first terminus comprises a DNA-binding moiety capable of noncovalently binding to a trinucleotide repeat sequence GAA; b) the second terminus comprises a protein-binding moiety binding to a regulatory molecule that modulates an expression of a gene comprising the nucleotide repeat sequence GAA; and c) the oligomeric backbone comprising a linker between the first terminus and the second terminus.

