

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization

International Bureau

(43) International Publication Date
13 July 2023 (13.07.2023)



(10) International Publication Number
WO 2023/133284 A2

(51) International Patent Classification:

A61K 47/60 (2017.01) C07K 7/08 (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:

— without international search report and to be republished upon receipt of that report (Rule 48.2(g))



WO 2023/133284 A2

(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.