

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
10 April 2025 (10.04.2025)



(10) International Publication Number
WO 2025/076236 A1

(51) International Patent Classification:

C07D 207/416 (2006.01) A61K 47/59 (2017.01)
C07D 233/66 (2006.01) A61P 25/28 (2006.01)

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).

(21) International Application Number:

PCT/US2024/049814

(22) International Filing Date:

03 October 2024 (03.10.2024)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/587,625 03 October 2023 (03.10.2023) US

(71) Applicant: **DESIGN THERAPEUTICS, INC.** [US/US];
6005 Hidden Valley Road, Suite 110, Carlsbad, California
92011 (US).

(72) Inventors: **NOURIE, Hannah**; 6005 Hidden Valley Road,
Suite 110, Carlsbad, California 92011 (US). **SINHA, San-
tosh**; 6005 Hidden Valley Road, Suite 110, Carlsbad, Cal-
ifornia 92011 (US). **WAYBRIGHT, Jarod**; 6005 Hid-
den Valley Road, Suite 110, Carlsbad, California 92011
(US). **YANG, Fei**; 6005 Hidden Valley Road, Suite 110,
Carlsbad, California 92011 (US). **ZHANG, Chengzhi**;
6005 Hidden Valley Road, Suite 110, Carlsbad, California
92011 (US). **CONVERTINO, Marino**; 6005 Hidden Val-
ley Road, Suite 110, Carlsbad, California 92011 (US). **YEH,
Chien-Hung**; 6005 Hidden Valley Road, Suite 110, Carls-
bad, California 92011 (US).

(74) Agent: **OTRUBOVA, Katerina** et al.; Wilson Sonsini
Goodrich & Rosati, 650 Page Mill Road, Palo Alto, Cali-
fornia 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.

(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,

(54) Title: METHODS AND COMPOUNDS FOR MODULATING HUNTINGTON'S DISEASE

(57) Abstract: The present disclosure relates to transcription modulator molecules having a first terminus, a second terminus, and an oligomeric backbone and methods for treating Huntington's disease (HD).



WO 2025/076236 A1