

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
20 April 2023 (20.04.2023)



(10) International Publication Number
WO 2023/064880 A1

(51) International Patent Classification:

C07D 401/14 (2006.01) A61P 25/00 (2006.01)
C07D 403/04 (2006.01) A61P 31/00 (2006.01)
C07D 403/14 (2006.01) A61P 35/00 (2006.01)
C07D 413/14 (2006.01) A61P 37/00 (2006.01)
C07D 417/14 (2006.01) A61P 39/00 (2006.01)
C07D 471/04 (2006.01) A61K 31/4725 (2006.01)
C07D 487/04 (2006.01) A61K 31/4985 (2006.01)
C07D 519/00 (2006.01) A61K 31/502 (2006.01)
A61P 3/00 (2006.01) A61K 31/5025 (2006.01)
A61P 9/00 (2006.01) A61K 31/517 (2006.01)
A61P 11/00 (2006.01) A61K 31/519 (2006.01)

(21) International Application Number:

PCT/US2022/078081

(22) International Filing Date:

13 October 2022 (13.10.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/255,348	13 October 2021 (13.10.2021)	US
63/255,079	13 October 2021 (13.10.2021)	US
63/255,178	13 October 2021 (13.10.2021)	US
63/393,210	28 July 2022 (28.07.2022)	US
63/393,208	28 July 2022 (28.07.2022)	US

(71) Applicant: REMIX THERAPEUTICS INC. [US/US];
One Kendall Square, Building 600, 4th Floor, Cambridge,
MA 02139 (US).

(72) Inventors: REYNOLDS, Dominic; One Kendall Square,
Building 600, 4th Floor, Cambridge, MA 02139 (US).
SEILER, Michael, W.; One Kendall Square, Building
600, 4th Floor, Cambridge, MA 02139 (US). AGRAW-
AL, Anant, A.; One Kendall Square, Building 600, 4th
Floor, Cambridge, MA 02139 (US). VAILLANCOURT,
Frederic; One Kendall Square, Building 600, 4th Floor,
Cambridge, MA 02139 (US). SMITH, Peter; One Kendall
Square, Building 600, 4th Floor, Cambridge, MA 02139
(US). PRAJAPATI, Sudeep; 4 Partridge Ave., Somerville,
MA 02145 (US). HOPPER, Allen, T.; 75 Adams Street,
Lexington, MA 02420 (US). VYSKOCIL, Stepan; Ke Sta-
dionu 627, 256 01 Benesov (CZ).

(74) Agent: LARKIN, Angelyn; Lando & Anastasi, LLP, 60
State Street, 23rd Floor, Boston, Massachusetts 02109 (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, 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).

Declarations under Rule 4.17:

— as to the applicant's entitlement to claim the priority of the
earlier application (Rule 4.17(iii))

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))
— with sequence listing part of description (Rule 5.2(a))

(54) Title: COMPOUNDS AND METHODS FOR MODULATING NUCLEIC ACID SPLICING

(57) Abstract: The present disclosure features compounds and related compositions that, *inter alia*, modulate nucleic acid splicing, e.g., splicing of a pre-mRNA, as well as methods of use thereof.

WO 2023/064880 A1