

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
14 April 2022 (14.04.2022)



(10) International Publication Number
WO 2022/076648 A1

(51) International Patent Classification:
C12N 15/86 (2006.01)

(21) International Application Number:
PCT/US2021/053908

(22) International Filing Date:
07 October 2021 (07.10.2021)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/089,951 09 October 2020 (09.10.2020) US
63/172,053 07 April 2021 (07.04.2021) US
63/216,322 29 June 2021 (29.06.2021) US
63/227,801 30 July 2021 (30.07.2021) US

(71) Applicant: TENAYA THERAPEUTICS, INC. [US/US];
171 Oyster Point Boulevard, Suite 500, South San Francisco,
California 94080 (US).

(72) Inventors: YANG, Zhihong Jane; 171 Oyster Point
Boulevard, Suite 500, South San Francisco, California
94080 (US). HO, Jaclyn; 171 Oyster Point Boulevard,
Suite 500, South San Francisco, California 94080 (US).
REID, Chris; 171 Oyster Point Boulevard, Suite 500, South

San Francisco, California 94080 (US). YANG, Jin; 171
Oyster Point Boulevard, Suite 500, South San Francisco,
California 94080 (US).

(74) Agent: FLOYD, Jennifer; 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, CZ, DE, DJ, DK, DM, DO,
DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN,
HR, HU, ID, IL, IN, IR, IS, IT, JO, JP, KE, KG, KH, KN,
KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD,
ME, 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,

(54) Title: PLAKOPHILLIN-2 GENE THERAPY METHODS AND COMPOSITIONS

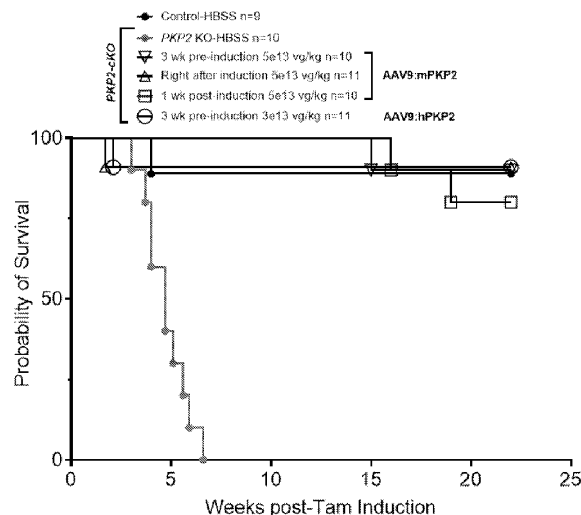


FIG. 19

(57) Abstract: Provided herein are methods and compositions for plakophilin-2 gene therapy for treating heart diseases such as arrhythmogenic right ventricular cardiomyopathy (ARVC) or arrhythmogenic cardiomyopathy (ACM).

[Continued on next page]

MC, 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 applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii))*

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))*