

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
08 June 2023 (08.06.2023)



(10) International Publication Number
WO 2023/102078 A3

(51) International Patent Classification:

C12N 15/86 (2006.01) C07K 14/015 (2006.01)
A61K 48/00 (2006.01)

(21) International Application Number:

PCT/US2022/051451

(22) International Filing Date:

30 November 2022 (30.11.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/284,962 01 December 2021 (01.12.2021) US
63/346,295 26 May 2022 (26.05.2022) US

(71) Applicant: **SHAPE THERAPEUTICS INC.** [US/US];
219 Terry Avenue North, Suite 100, Seattle, WA 98109
(US).

(72) Inventors: **PACKARD, Thomas**; c/o Shape Therapeutics
Inc., 219 Terry Avenue North, Suite 100, Seattle, WA 98109
(US). **BRIGGS, Adrian Wrangham**; c/o Shape Therapeu-
tics Inc., 219 Terry Avenue North, Suite 100, Seattle, WA
98109 (US). **HUSS, David, Jeffrey**; c/o Shape Therapeu-
tics Inc., 219 Terry Avenue North, Suite 100, Seattle, WA
98109 (US). **VIGNEAULT, Francois**; c/o Shape Thera-

peutics Inc., 219 Terry Avenue North, Suite 100, Seattle,
WA 98109 (US). **HAUSE, Ronald, James, Jr.**; c/o Shape
Therapeutics Inc., 219 Terry Avenue North, Suite 100, Seat-
tle, WA 98109 (US). **JIANG, Yue**; c/o Shape Therapeu-
tics Inc., 219 Terry Avenue North, Suite 100, Seattle, WA
98109 (US). **STEIN, Kevin, Christopher**; c/o Shape Ther-
apeutics Inc., 219 Terry Avenue North, Suite 100, Seattle,
WA 98109 (US). **BANJANIN, Bora**; c/o Shape Therapeu-
tics Inc., 219 Terry Avenue North, Suite 100, Seattle, WA
98109 (US).

(74) Agent: **HARWOOD, Melissa, M.** et al.; DLA Piper LLP,
701 Fifth Avenue, Suite 6900, Seattle, WA 98104-7029
(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,

(54) Title: FUNCTIONAL AAV CAPSIDS FOR INTRAVITREAL ADMINISTRATION

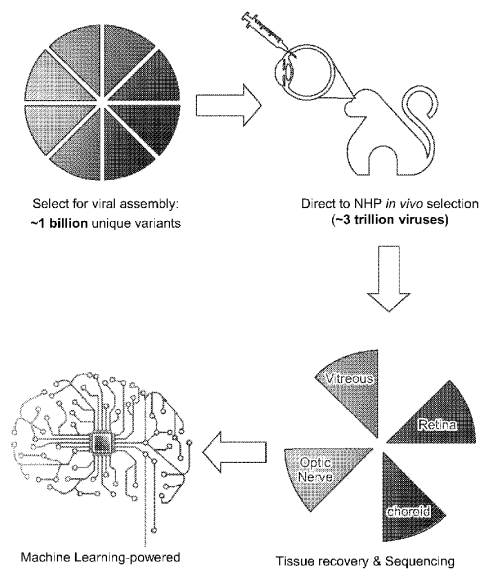


FIG. 1

(57) Abstract: Disclosed herein are engineered AAV VP capsid polypep-
tides with the ability to assemble into virus particles and having improved
tissue tropism to eye tissues, for example, retinal tissues, retinal pigment
epithelial cells, or photoreceptor cells. The capsids are engineered using
the high throughput discovery system described herein. In certain embod-
iments, provided herein are recombinant adeno-associated virus (AAV) VP
capsid polypeptides having at least one mutation in a 581 to 589 region of
the VP capsid polypeptide, corresponding to residues 581 to residue 589
of a VP1 polypeptide of SEQ ID NO: 1.

[Continued on next page]

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).

Declarations under Rule 4.17:

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

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

13 July 2023 (13.07.2023)