

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
19 May 2022 (19.05.2022)



(10) International Publication Number
WO 2022/101363 A1

(51) International Patent Classification:
C07D 225/02 (2006.01)

(21) International Application Number:
PCT/EP2021/081424

(22) International Filing Date:
11 November 2021 (11.11.2021)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/112,457 11 November 2020 (11.11.2020) US

(71) Applicants: **EUROPEAN MOLECULAR BIOLOGY
LABORATORY** [DE/DE]; Meyerhofstrasse 1, 69117 Hei-
delberg (DE). **BOREA THERAPEUTICS S.R.L.** [IT/IT];
via Vincenzo Gioberti 8, 20123 Milan (IT).

(72) Inventor: **HEPPENSTALL, Paul Alexander**; c/o Euro-
pean Molecular Biology Laboratory, Meyerhofstrasse 1,
69117 Heidelberg (DE).

(74) Agent: **LEE, Nicholas**; Lacon London 84 Theobalds Road,
London WC1X 8NL (GB).

(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,
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).

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))
- in black and white; the international application as filed
contained color or greyscale and is available for download
from PATENTSCOPE

(54) Title: MODIFIED VIRAL PARTICLES FOR GENE THERAPY

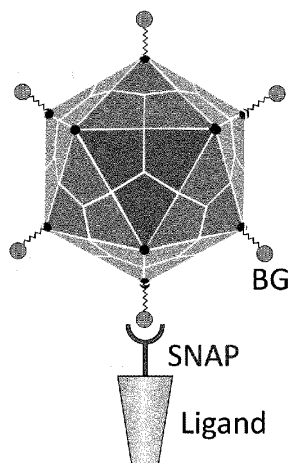


FIG. 1

(57) Abstract: This invention relates to novel surface modified viral capsids and recombinant virions comprising the same. Furthermore, this invention concerns intermediates for the preparation of surface modified viral capsids. The surface modified viral capsids are designed to selectively and/or more efficiently deliver gene therapy. The surface modified viral capsids, when incorporated into a recombinant virion, can be used to treat an illness that is characterized by genetic abnormality.



WO 2022/101363 A1