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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
11 August 2022 (11.08.2022)

WIPO | PCT

(10) International Publication Number
WO 2022/169508 A1

(51) International Patent Classification:
A61K 47/50 (2017.01) *A61K 9/51* (2006.01)
A61K 9/107 (2006.01)

(21) International Application Number:
PCT/US2021/062717

(22) International Filing Date:
09 December 2021 (09.12.2021)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/146,858 08 February 2021 (08.02.2021) US

(71) Applicant: **THE BOARD OF REGENTS OF THE UNIVERSITY OF TEXAS SYSTEM** [US/US]; 210 West 7th Street, Austin, TX 78701 (US).

(72) Inventors: **LEE, Sang, M.**; c/o UT Southwestern Medical Center, 5323 Harry Hines Blvd., Dallas, TX 75390 (US). **SIEGWART, Daniel, J.**; c/o UT Southwestern Medical Center, 5323 Harry Hines Blvd., Dallas, TX 75390 (US).

(74) Agent: **LONG, S., Reid**; Parker Highlander PLLC, 1120 S. Capital of Texas Highway, Bldg. One, Suite 200, Austin, TX 78746 (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, 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))*

WO 2022/169508 A1

(54) Title: UNSATURATED DENDRIMERS COMPOSITIONS,RELATED FORMULATIONS, AND METHODS OF USE THEREOF

(57) Abstract: Described herein are novel lipid compositions comprising unsaturated dendrimers and methods of synthesis of unsaturated dendrimers. The lipid composition can comprise an ionizable cationic lipid, a phospholipid, and a selective organ targeting lipid. Also described herein are pharmaceutical formulations comprising an unsaturated dendrimer, a lipid composition, and a therapeutic agent. Further described in here are methods of mRNA delivery comprising a lipid composition and a therapeutic agent. Further described herein are high-potency dosage forms of a therapeutic formulated with a lipid composition.