

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

(19) World Intellectual Property
Organization

International Bureau

(43) International Publication Date
14 November 2024 (14.11.2024)



(10) International Publication Number
WO 2024/233750 A1

(51) International Patent Classification:

C07C 333/04 (2006.01) C07C 229/16 (2006.01)
A61K 9/127 (2006.01) C07C 237/12 (2006.01)
A61K 39/00 (2006.01) C07C 271/22 (2006.01)
A61K 47/20 (2006.01) C07C 275/16 (2006.01)
A61K 48/00 (2006.01) C07C 327/32 (2006.01)
A61P 7/04 (2006.01) C07D 209/24 (2006.01)
A61P 7/06 (2006.01) C07D 333/60 (2006.01)
C07C 229/12 (2006.01) C12N 15/88 (2006.01)

(21) International Application Number:

PCT/US2024/028521

(22) International Filing Date:

09 May 2024 (09.05.2024)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/501,367 10 May 2023 (10.05.2023) US

(71) Applicant: ARCTURUS THERAPEUTICS, INC.
[US/US]; 10628 Science Center Drive, Suite 250, San
Diego, CA 92121 (US).

(72) Inventors: RAJAPPAN, Kumar; c/o Arcturus Therapeu-
tics, Inc., 10628 Science Center Drive, Suite 250, San
Diego, CA 92121 (US). TANIS, Steven; c/o Arcturus Ther-
apeutics, Inc., 10628 Science Center Drive, Suite 250, San
Diego, CA 92121 (US).

(74) Agent: VARSHNEYA, Pooja et al.; Mintz Levin Cohn
Ferris Glovsky and Popeo, P.C., 555 12th Street NW, Suite
1100, Washington, DC 20004 (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, MU, 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.

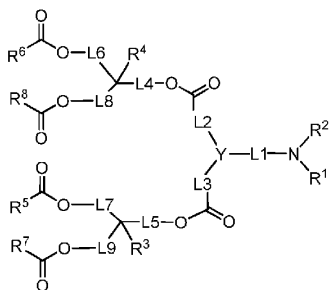
(84) Designated States (unless otherwise indicated, for every
kind of regional protection available): ARIPO (BW, CV,
GH, GM, KE, LR, LS, MW, MZ, NA, RW, SC, SD, SL, ST,
SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ,
RU, TJ, TM), European (AL, AT, BE, BG, CH, CY, CZ,
DE, DK, EE, ES, FI, FR, GB, GR, 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).

Published:

— with international search report (Art. 21(3))

(54) Title: IONIZABLE CATIONIC LIPIDS FOR RNA DELIVERY

(57) Abstract: The present disclosure describes compounds of Formula (I) and
pharmaceutically acceptable salts thereof:



(I)

WO 2024/233750 A1