

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
21 November 2024 (21.11.2024)



(10) International Publication Number
WO 2024/238641 A2

(51) International Patent Classification:
Not classified

(21) International Application Number:
PCT/US2024/029444

(22) International Filing Date:
15 May 2024 (15.05.2024)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/502,219 15 May 2023 (15.05.2023) US

(71) Applicant: **REPLICATE BIOSCIENCE, INC.** [US/US];
11558 Sorrento Valley Road, Suite 6, San Diego, California
92121 (US).

(72) Inventors: **WANG, Nathaniel Stephen**; c/o Replicate
Bioscience, Inc., 11558 Sorrento Valley Road, Suite 6,
San Diego, California 92121 (US). **GEALL, Andrew**; c/

o Replicate Bioscience, Inc., 11558 Sorrento Valley Road,
Suite 6, San Diego, California 92121 (US).

(74) Agent: **GOTTFRIED, Lynn**; Mintz Levin, One Financial
Center, Boston, Massachusetts 02111 (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,

(54) Title: METHODS FOR IDENTIFICATION AND SELECTION OF SELF-REPLICATING RNA MOLECULES FOR BIOMEDI-
CAL APPLICATIONS

Formulate an input srRNA composition with a non-viral delivery vehicle to generate a srRNA delivery
system having a formulated srRNA composition



Determine potency of the formulated srRNA
composition



Select the srRNA delivery system as suitable for a biomedical
application if the formulated srRNA composition retains at least about
25% potency relative to the potency of the unformulated input srRNA
composition

FIG. 1

(57) Abstract: The present disclosure generally relates to methods of identifying and/or selecting self-replicating RNA (srRNA) de-
livery systems suitable for biomedical applications. More particularly, the disclosure relates to methods of identifying and/or selecting
srRNA delivery systems using a combination of critical quality attributes for srRNA vaccines and biotherapeutics. Also provided are
srRNA compositions, formulations obtained by the methods of the disclosure, as well as methods for inducing a pharmacodynamic
effect in a subject in need thereof, as well as methods for preventing and/or treating various health conditions.

[Continued on next page]

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:

- *without international search report and to be republished upon receipt of that report (Rule 48.2(g))*