

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
21 April 2022 (21.04.2022)



(10) International Publication Number
WO 2022/077121 A1

(51) International Patent Classification:

C12N 15/87 (2006.01) C12N 15/10 (2006.01)
A61K 31/7088 (2006.01) C12N 15/113 (2010.01)
A61K 47/42 (2017.01) C12N 15/63 (2006.01)
A61K 9/51 (2006.01) C12N 7/08 (2006.01)

(21) International Application Number:

PCT/CA2021/051458

(22) International Filing Date:

18 October 2021 (18.10.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/093,295 18 October 2020 (18.10.2020) US
63/104,263 22 October 2020 (22.10.2020) US

(71) Applicant: **FELDAN BIO INC.** [CA/CA]; 2666 Boul. du
Parc Technologique, Suite 290, Québec, Québec G1P 4S6
(CA).

(72) Inventors: **LEPETIT-STOFFAES, Jean-Pascal**; 640
rue Saint-Jean, Apt. 1105, Québec, Québec G1R 4W9
(CA). **MESSIER, Nancy**; 3100 rue De La Rivière-Nel-
son, Québec, Québec G2A 1A4 (CA). **GUAY, David**;
2614 rue des Acacias, Québec, Québec G1M 3P5 (CA).
DEL'GUIDICE, Thomas; 251 rue Georges Dor, Québec,
Québec G1C 5S6 (CA). **BARBEAU, Xavier**; 183 rue

du Verger, Lévis, Québec G6W 0A4 (CA). **HALLÉE,
Stéphanie**; 22 René-Collard, Ste-Catherine-de-la-Jacques-
Cartier, Québec G3N 3B1 (CA).

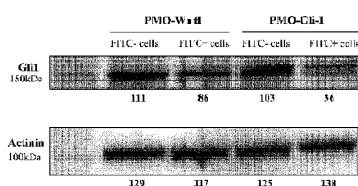
(74) Agent: **ROBIC S.E.N.C.R.L. / LLP**; 630 Boul René-
Levesque W, 20th Floor, Montréal, Québec H3B 1S6 (CA).

(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).

(54) Title: PEPTIDE-BASED TRANSDUCTION OF NON-ANIONIC POLYNUCLEOTIDE ANALOGS FOR GENE EXPRESSION MODULATION

Fig. 3



(57) Abstract: Compositions and methods for delivering non-anionic polynucleotide analog cargoes to the cytosolic/nuclear compart-
ment of eukaryotic cells via a synthetic peptide shuttle agent are described herein. The non-anionic polynucleotide analog cargoes may
be charge-neutral or cationic, and the synthetic peptide shuttle agent is a peptide comprising an amphipathic alpha-helical motif having
both a positively-charged hydrophilic outer face and a hydrophobic outer face, wherein synthetic peptide shuttle agent is not covalently
linked, or linked in a cleavable manner under physiological conditions, to the non-anionic polynucleotide analog cargoes. The non-
anionic polynucleotide analog cargo may be a charge-neutral or cationic antisense synthetic oligonucleotide (ASO) that hybridizes to
an intracellular target RNA for gene expression modification.

[Continued on next page]

WO 2022/077121 A1

Declarations under Rule 4.17:

- *of inventorship (Rule 4.17(iv))*

Published:

- *with international search report (Art. 21(3))*
- *with sequence listing part of description (Rule 5.2(a))*
- *in black and white; the international application as filed contained color or greyscale and is available for download from PATENTSCOPE*