

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
09 July 2020 (09.07.2020)



(10) International Publication Number
WO 2020/142676 A1

(51) International Patent Classification:
A61K 38/16 (2006.01) *C12P 21/06* (2006.01)
C12N 15/85 (2006.01)

TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW,
KM, ML, MR, NE, SN, TD, TG).

(21) International Application Number:
PCT/US2020/012169

Declarations under Rule 4.17:

- as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii))
- as to the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii))

(22) International Filing Date:
03 January 2020 (03.01.2020)

(25) Filing Language: English

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))

(26) Publication Language: English

(30) Priority Data:
62/788,571 04 January 2019 (04.01.2019) US
62/831,342 09 April 2019 (09.04.2019) US
62/903,080 20 September 2019 (20.09.2019) US
62/929,339 01 November 2019 (01.11.2019) US

(71) Applicant: **THE UNIVERSITY OF CHICAGO**
[US/US]; 5801 South Ellis Avenue, Chicago, Illinois 60637
(US).

(72) Inventors: **DICKINSON, Bryan C.**; Polsky Center for Entrepreneurship and Innovation, University of Chicago, 5113 S. Harper Ave., Suite 2C, Chicago, Illinois 60615 (US).
RAUCH, Simone; Polsky Center for Entrepreneurship and Innovation, University of Chicago, 5113 S. Harper Ave., Suite 2C, Chicago, Illinois 60615 (US).

(74) Agent: **STELLMAN, Laurie Friesenhahn**; NORTON ROSE FULBRIGHT US LLP, 98 San Jacinto Blvd., Suite 1100, Austin, Texas 78701 (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, 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,

(54) Title: SYSTEMS AND METHODS FOR MODULATING RNA

(57) Abstract: Aspects of the disclosure relate to a RNA regulatory system comprising at least one of each: i) a RNA hairpin binding domain; ii) a RNA targeting molecule comprising a RNA targeting region and at least one hairpin structure, wherein the hairpin structure of the RNA targeting molecule specifically binds to i; and iii) a RNA regulatory domain.

WO 2020/142676 A1