

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
24 October 2024 (24.10.2024)



(10) International Publication Number
WO 2024/220449 A9

(51) International Patent Classification:

C12N 15/85 (2006.01) A61K 31/352 (2006.01)
A61K 48/00 (2006.01) C07D 493/18 (2006.01)

(21) International Application Number:

PCT/US2024/024837

(22) International Filing Date:

17 April 2024 (17.04.2024)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/459,850 17 April 2023 (17.04.2023) US
63/459,853 17 April 2023 (17.04.2023) US

(71) Applicant: **ASKBIO INC.** [US/US]; 20 TW Alexander Drive, Suite 110, Research Triangle Park, North Carolina 27709 (US).

(72) Inventors: **WHYTESIDE, Graham**; c/o Synpromics Ltd, Roslin Innovation Centre, Easter Bush Campus, Midlothian EH25 9RG (GB). **TORRANCE, Victoria Fiona**; c/o Syn-

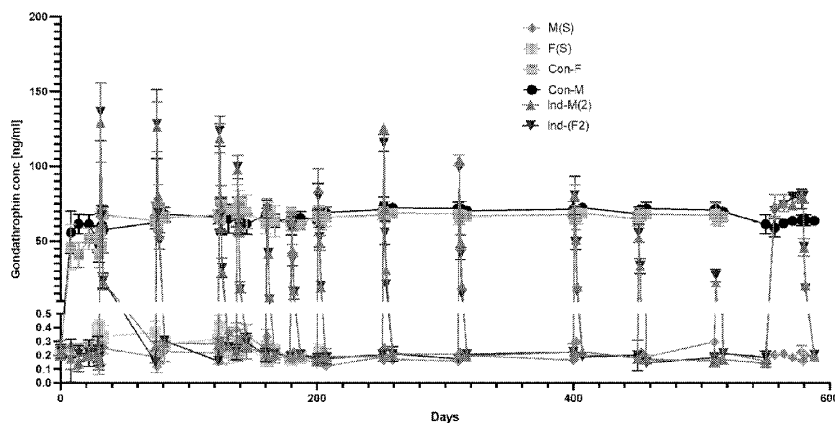
promics Ltd, Roslin Innovation Centre, Easter Bush Campus, Midlothian EH25 9RG (GB).

(74) Agent: **BRAICH, Ravinderjit** et al.; Nixon Peabody LLP, 53 State Street, Exchange Place, Boston, Massachusetts 02109 (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,

(54) Title: REGULATABLE CONTROLLED EXPRESSION OF A TRANSGENE IN PRIMATES



Inductions performed in following order: 80, 80, 40, 20, 10, 1 (IP), 6 (oral), 20, 10, 1, 0.5 and 0.1 (oral), mg/kg. Last induction set was 30-days continuous 1mg/kg oral.

FIG. 15

(57) Abstract: The technology described herein relates generally to methods and compositions for regulatable and controllable expression of a transgene in a subject, e.g., a primate subject.

[Continued on next page]

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).

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))*

Published:

- *with international search report (Art. 21(3))*

(88) Date of publication of the international search report:

16 January 2025 (16.01.2025)

(48) Date of publication of this corrected version:

03 April 2025 (03.04.2025)

(15) Information about Correction:

see Notice of 03 April 2025 (03.04.2025)