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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
20 August 2020 (20.08.2020)

WIPO | PCT

(10) International Publication Number
WO 2020/165318 A1

(51) International Patent Classification:
A61K 45/06 (2006.01) *A61K 31/519* (2006.01)
A61K 31/517 (2006.01) *A61P 3/00* (2006.01)

(21) International Application Number:
PCT/EP2020/053714

(22) International Filing Date:
13 February 2020 (13.02.2020)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
19382102.2 14 February 2019 (14.02.2019) EP

(71) Applicant: **SOM INNOVATION BIOTECH, S.A.** [ES/ES]; Baldiri Reixac 4, Torre I, E-08028 Barcelona (ES).

(72) Inventors: **INSA BORONAT, Raúl INSA BORONAT**; SOM INNOVATION BIOTECH, S.A., Baldiri Reixac 4, Torre I, E-08028 Barcelona (ES). **REIG BOLAÑO, Núria**; SOM INNOVATION BIOTECH, S.A., Baldiri Reixac 4, Torre I, E-08028 Barcelona (ES). **FERRÉ FERRÉ, Aileen**; SOM INNOVATION BIOTECH, S.A., Baldiri Reixac 4, Torre I, E-08028 Barcelona (ES). **HUERTAS GAMBÍN, Óscar**; SOM INNOVATION BIOTECH, S.A., Baldiri Reixac 4, Torre I, E-08028 Barcelona (ES). **ESTEVA GRAS, Santiago**; SOM INNOVATION BIOTECH, S.A., Baldiri Reixac 4, Torre I, E-08028 Barcelona (ES). **SIGNORILE, Luca**; SOM INNOVATION BIOTECH, S.A., Baldiri Reixac 4, Torre I, E-08028 Barcelona (ES). **PERICOT MOHR, Gal.la**; SOM INNOVATION BIOTECH, S.A., Baldiri Reixac 4, Torre I, E-08028 Barcelona (ES).

(74) Agent: **ABG INTELLECTUAL PROPERTY LAW, S.L.**; Avda. de Burgos, 16D , Edificio EUROMOR, E-28036 Madrid (ES).

(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, 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 the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii))*

Published:
— *with international search report (Art. 21(3))*
— *with sequence listing part of description (Rule 5.2(a))*

WO 2020/165318 A1

(54) Title: TRIAMTERENE OR NOLATREXED FOR USE IN THE TREATMENT OF PHENYLKETONURIA

(57) Abstract: The present invention relates to a compound selected from the group consisting of nolatrexed, triamterene, sultopride and hydrastinine or a pharmaceutically acceptable salt thereof, preferably triamterene, nolatrexed or a pharmaceutically acceptable salt thereof, and to combination of said compounds with other active ingredients, for use in the treatment and/or prevention of phenylketonuria, to the use of said compound or its combinations in the manufacture of a medicament for the treatment or prevention of said diseases and to a method of treating and/or preventing by administration of said compound or its combinations.