

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

(19) World Intellectual Property  
Organization

International Bureau

(43) International Publication Date  
17 February 2022 (17.02.2022)



(10) International Publication Number  
**WO 2022/034591 A1**

(51) International Patent Classification:

C12N 15/86 (2006.01) A61P 25/00 (2006.01)

(21) International Application Number:

PCT/IL2021/050982

(22) International Filing Date:

11 August 2021 (11.08.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/064,181 11 August 2020 (11.08.2020) US  
63/167,277 29 March 2021 (29.03.2021) US

(71) Applicant: YISSUM RESEARCH DEVELOPMENT  
COMPANY OF THE HEBREW UNIVERSITY OF  
JERUSALEM LTD. [IL/IL]; Hi-Tech Park, The Ed-  
mond J Safra Campus, Givat-Ram, P.O.B 39135, 9139002  
Jerusalem (IL).

(72) Inventors: AQEILAN, Rami; Salah A Din Street, POB  
54950, 9154901 Jerusalem (IL). REPUDI, Srinivas; 5/52  
Guatemala Street, 9670403 Jerusalem (IL).

(74) Agent: PYERNIK, Moshe et al.; PYERNIK PATENT AT-  
TORNEYS, P.O. Box 10012, 8400101 Beer Sheva (IL).

(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: METHOD FOR THE TREATMENT OF WWOX ASSOCIATED DISEASES

(57) Abstract: The present disclosure provides methods and compositions for the treatment of WW domain-containing oxidoreductase (WWOX)-associated CNS disease. In various embodiments, the present invention involves expressing a heterologous WWOX gene in the brain of the subject, and in various embodiments involves expressing the heterologous WWOX gene in neurons to treat or ameliorate conditions such as WOREE syndrome and SCAR12.



WO 2022/034591 A1