

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
24 June 2021 (24.06.2021)



(10) International Publication Number
WO 2021/127533 A1

(51) International Patent Classification:

C12N 15/864 (2006.01) C12P 19/34 (2006.01)
A61K 35/76 (2015.01) C12N 15/79 (2006.01)

Published:

- with international search report (Art. 21(3))
- with sequence listing part of description (Rule 5.2(a))

(21) International Application Number:

PCT/US2020/066167

(22) International Filing Date:

18 December 2020 (18.12.2020)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

62/950,834 19 December 2019 (19.12.2019) US

(71) Applicant: **THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA** [US/US]; 3600 Civic Center Blvd, 9th Floor, Philadelphia, PA 19104 (US).

(72) Inventors: **WILSON, James, M.**; 1831 Delancey Street, Philadelphia, PA 19103 (US). **HINDERER, Christian**; 4723 Laurel Street, New Orleans, LA 70115 (US). **MILLER, Nimrod**; 271 Irish Road, Berwyn, PA 19312 (US).

(74) Agent: **KODROFF, Cathy, A.** et al.; Howson & Howson LLP, 325 Sentry Parkway East, Five Sentry East, Suite 160, Blue Bell, PA 19422 (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, 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: COMPOSITIONS FOR TREATING FRIEDREICH'S ATAXIA

(57) Abstract: A recombinant adeno-associated virus (rAAV) comprising an AAV capsid and a vector genome comprising a frataxin gene is provided. Also provided is a composition containing an effective amount of rAAV to ameliorate symptoms of Friedreich's ataxia, including, e.g., reduction in progression towards neurocognitive decline and/or cardiomyopathy.

WO 2021/127533 A1