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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
11 September 2025 (11.09.2025)

WIPO | PCT

(10) International Publication Number
WO 2025/188993 A3

(51) International Patent Classification:
C07K 14/47 (2006.01) *C12N 15/86* (2006.01)
A61K 48/00 (2006.01)

(21) International Application Number:
PCT/US2025/018739

(22) International Filing Date:
06 March 2025 (06.03.2025)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/562,412 07 March 2024 (07.03.2024) US

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(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, UY, 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, 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).

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))*
— *with sequence listing part of description (Rule 5.2(a))*

(88) Date of publication of the international search report:
06 November 2025 (06.11.2025)

(15) Information about Correction:
see Notice of 02 October 2025 (02.10.2025)

WO 2025/188993 A3

(54) Title: GENE THERAPY FOR TREATING GNE-RELATED DISORDERS

(57) Abstract: The present invention relates to methods and materials for treating GNE-related disorders such as GNE myopathy, GNE-dependent ALS, thrombocytopenia, sarcopenia and aging using a dual gene recombinant adeno-associated virus comprising the GNE gene and the follistatin gene. This therapy is unique in that it can rebuild lost muscle strength at the same time that it prevents subsequent muscle disease from occurring.