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

CORRECTED VERSION

(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 A8

(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

(71) Applicant: **RESEARCH INSTITUTE AT NATIONWIDE CHILDREN'S HOSPITAL** [US/US]; 700 Children's Drive, Room W172, Columbus, OH 43205 (US).

(72) Inventor: **MARTIN, Paul**; 700 Children's Drive, Columbus, OH 43205 (US).

(74) Agent: **SINTICH, Sharon, M.**; Marshall, Gerstein & Borun LLP, 233 S. Wacker Drive, 6300 Willis Tower, Chicago, IL 60606-6357 (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, 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:
— *without international search report and to be republished upon receipt of that report (Rule 48.2(g))*
— *with sequence listing part of description (Rule 5.2(a))*

(48) Date of publication of this corrected version:
02 October 2025 (02.10.2025)

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

(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.

WO 2025/188993 A8