

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

(19) World Intellectual Property
Organization

International Bureau

(43) International Publication Date
09 November 2023 (09.11.2023)



(10) International Publication Number
WO 2023/215384 A3

(51) International Patent Classification:

A61P 21/00 (2006.01) A61K 31/7125 (2006.01)
C07K 16/22 (2006.01) A61K 39/395 (2006.01)
C12Q 1/34 (2006.01)

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).

(21) International Application Number:

PCT/US2023/020843

(22) International Filing Date:

03 May 2023 (03.05.2023)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/364,188	04 May 2022 (04.05.2022)	US
63/366,447	15 June 2022 (15.06.2022)	US
63/366,662	20 June 2022 (20.06.2022)	US
63/378,987	10 October 2022 (10.10.2022)	US

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))

(88) Date of publication of the international search report:

07 December 2023 (07.12.2023)

(71) Applicant: **SCHOLAR ROCK, INC.** [US/US]; 301 Binney Street, 3rd Floor, Cambridge, Massachusetts 02142 (US).

(72) Inventors; and

(71) Applicants: **NOMIKOS, George** [US/US]; 301 Binney Street, 3rd Floor, Cambridge, Massachusetts 02142 (US). **SONG, Guochen** [US/US]; 301 Binney Street, 3rd Floor, Cambridge, Massachusetts 02142 (US). **CHYUNG, Yung** [US/US]; 301 Binney Street, 3rd Floor, Cambridge, Massachusetts 02142 (US). **WEBSTER, Micah T.** [US/US]; 301 Binney Street, 3rd Floor, Cambridge, Massachusetts 02142 (US). **KERTESZ, Nathalie** [US/US]; 301 Binney Street, 3rd Floor, Cambridge, Massachusetts 02142 (US).

(74) Agent: **JACOBSTEIN, Jeffrey M.**; Finnegan, Henderson, Farabow, Garrett & Dunner, LLP, 901 New York Avenue, NW, Washington, District of Columbia 20001-4413 (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, 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,

(54) Title: USE OF MYOSTATIN INHIBITOR FOR TREATING SPINAL MUSCULAR ATROPHY

(57) Abstract: Administration of apitegromab leads to improvements in motor function and/or quality of life in subjects with spinal muscular atrophy.

WO 2023/215384 A3