

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
17 March 2022 (17.03.2022)



(10) International Publication Number
WO 2022/056428 A1

(51) International Patent Classification:

A61K 31/7064 (2006.01) C07H 1/00 (2006.01)
A61K 31/7068 (2006.01) C07H 3/00 (2006.01)
A61K 31/7072 (2006.01) C07H 3/02 (2006.01)

(21) International Application Number:

PCT/US2021/050149

(22) International Filing Date:

14 September 2021 (14.09.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/077,760 14 September 2020 (14.09.2020) US
63/188,559 14 May 2021 (14.05.2021) US
63/227,611 30 July 2021 (30.07.2021) US
63/231,825 11 August 2021 (11.08.2021) US

(71) Applicant: **WELLSTAT THERAPEUTICS CORPORATION** [US/US]; 14200 Shady Grove Road, Suite 600, Rockville, MD 20850 (US).

(72) Inventors: **VON BORSTEL, Reid, Warren**; 10017 Sorrel Avenue, Potomac, MD 20854 (US). **SIMPSON, David, Michael**; 6611 Sulky Lane, North Bethesda, MD 20852

(US). **GARCIA GARCIA, Rolando, Alejandro**; 2 Martins Landing Court, Gemantown, MD 20874 (US).

(74) Agent: **KREISLER, Lewis, J.**; Wellstat Management Company, LLC, 14200 Shady Grove Road, Suite 600, Rockville, MD 20850 (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,

(54) Title: 2',3'-DIACETYLURIDINE SUBSTITUTED WITH ACETOACETYL AT THE 5' POSITION

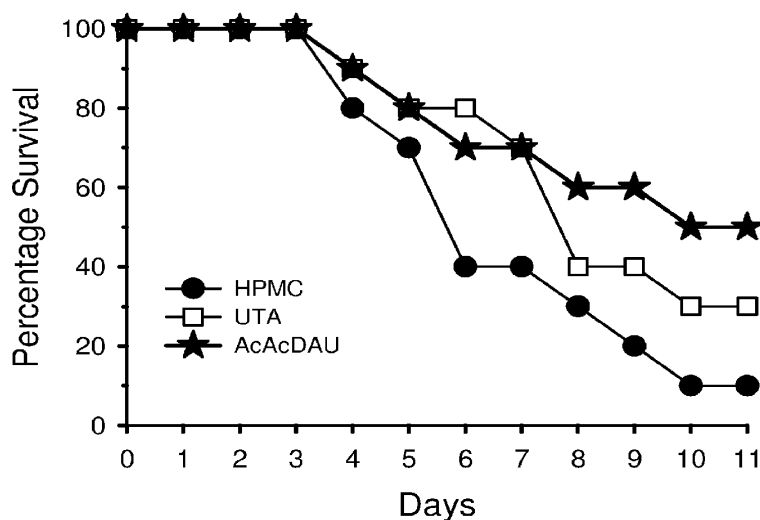


FIG. 8

(57) Abstract: 5'-O-(Acetoacetyl)-2',3'-di-O-acetyluridine is useful in the treatment or prevention of disorders characterized by cerebral metabolic energy failure or diminished mitochondrial energy reserve capacity.

[Continued on next page]

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))*