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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
24 October 2013 (24.10.2013)

WIPO | PCT

(10) International Publication Number
WO 2013/158046 A1

(51) International Patent Classification:
A61K 31/7088 (2006.01) *A61P 43/00* (2006.01)
C12N 15/11 (2006.01)

(21) International Application Number:
PCT/SG2013/000158

(22) International Filing Date:
22 April 2013 (22.04.2013)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
61/687,222 20 April 2012 (20.04.2012) US

(71) Applicant: **AGENCY FOR SCIENCE, TECHNOLOGY AND RESEARCH** [SG/SG]; 1 Fusionpolis Way, #20-10 Connexis, Singapore 138632 (SG).

(72) Inventors: **STEWART, Colin**; c/o Institute of Medical Biology, 8A Biomedical Grove, #06-06 Immunos, Singapore 138648 (SG). **BURKE, Brian**; c/o Institute of Medical Biology, 8A Biomedical Grove, #06-06 Immunos, Singapore 138648 (SG).

(74) Agent: **SPRUSON & FERGUSON (ASIA) PTE LTD**; P.O. Box 1531, Robinson Road Post Office, Singapore 903031 (SG).

(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, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IS, JP, KE, KG, KM, KN, KP, KR, KZ, LA, LC, LK, LR, LS, LT, 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, SC, SD, SE, SG, SK, SL, SM, ST, SV, SY, TH, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, 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, 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, ML, MR, NE, SN, TD, TG).

Declarations under Rule 4.17:

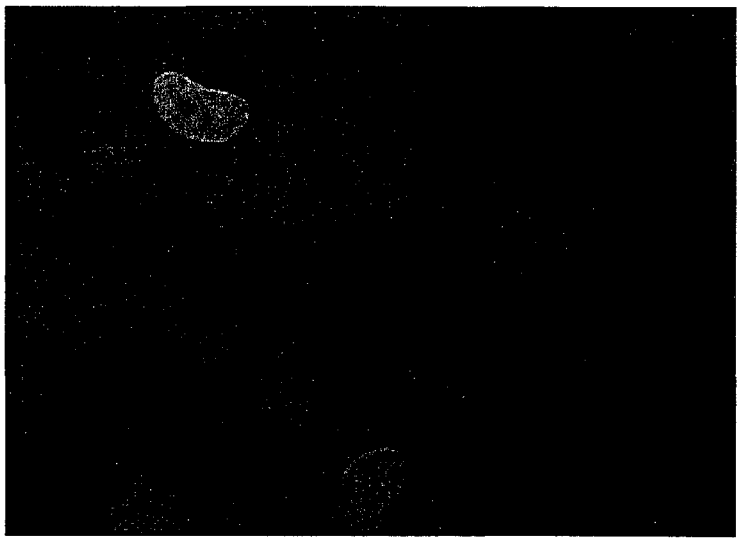
— *of inventorship (Rule 4.17(iv))*

Published:

— *with international search report (Art. 21(3))*

(54) Title: RNAI-BASED THERAPIES FOR CARDIOMYOPATHIES, MUSCULAR DYSTROPHIES AND LAMINOPATHIES

B



(57) Abstract: The present disclosure relates to inhibitor of Sun1 for treatment of laminopathies and to Sun1 as markers indicative of a patient's responsiveness to treatment, enabling improved prediction of a patient's risk, monitoring of laminopathies.

WO 2013/158046 A1