



(51) International Patent Classification:

C12N 15/86 (2006.01) A61K 38/50 (2006.01)

A61K 48/00 (2006.01)

(21) International Application Number:

PCT/US2019/036157

(22) International Filing Date:

07 June 2019 (07.06.2019)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

62/682,772 08 June 2018 (08.06.2018) US

62/822,015 21 March 2019 (21.03.2019) US

(71) Applicant: UNIVERSITY OF FLORIDA RESEARCH FOUNDATION, INCORPORATED [US/US]; 223 Grinter Hall, Gainesville, FL 32611 (US).

(72) Inventors: SWEENEY, Hugh, Lee; 9210 NW 59th Street, Gainesville, FL 32653-7854 (US). SLEEPER, Margaret, Mary; 2015 SW 16th Avenue, Gainesville, FL 32610 (US).

(74) Agent: GREENWALD, Eric, P. et al.; Wolf, Greenfield & Sacks, P.C., 600 Atlantic Avenue, Boston, MA 02210-2206 (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, 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, 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, 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, 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))

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

(54) Title: AAV CARDIAC GENE THERAPY FOR CARDIOMYOPATHY



FIG. 1

(57) Abstract: The present invention is related to compositions and methods useful in treating heart conditions. The disclosed compositions and methods are based on an AAV therapy comprising a recombinant AAV vector for delivering two or more transgenes into the heart of a subject, wherein the transgenes comprise an S100 family protein and an apoptotic inhibitor. In some aspects, targeting multiple sources of one or more heart conditions can provide synergistic benefits during treatment.

