



- (51) **International Patent Classification:**  
C12N 15/864 (2006.01)
- (21) **International Application Number:**  
PCT/EP2015/051081
- (22) **International Filing Date:**  
21 January 2015 (21.01.2015)
- (25) **Filing Language:** English
- (26) **Publication Language:** English
- (30) **Priority Data:**  
14151960.3 21 January 2014 (21.01.2014) EP
- (71) **Applicants:** **VRIJE UNIVERSITEIT BRUSSEL** [BE/BE]; Pleinlaan 2, B-1050 Brussel (BE). **VIB VZW** [BE/BE]; Rijnvisschestraat 120, B-9052 Gent (BE). **UNIVERSITEIT GENT** [BE/BE]; Sint-Pietersnieuwstraat 25, B-9000 Gent (BE).
- (72) **Inventors:** **VANDENDRIESSCHE, Thierry**; Tiensesteenweg 250, B-3360 Bierbeek (BE). **CHUAH, Marinee**; Tiensesteenweg 250, B-3360 Bierbeek (BE). **DE BLESER, Pieter**; Prosper Bosteelstraat 18, B-9255 Bugenhout (BE).
- (74) **Agents:** **VANHALST, Koen Victor Rachel** et al.; De Clercq & Partners, E. Gevaertdreef 10a, B-9830 Sint-Martens-Latem (BE).
- (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, IR, IS, JP, KE, KG, KN, KP, KR, 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).

**Declarations under Rule 4.17:**

— of inventorship (Rule 4.17(iv))

**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))
- with sequence listing part of description (Rule 5.2(a))



WO 2015/110449 A1

(54) **Title:** MUSCLE-SPECIFIC NUCLEIC ACID REGULATORY ELEMENTS AND METHODS AND USE THEREOF

(57) **Abstract:** The present invention relates to nucleic acid regulatory elements that are able to enhance muscle-specific expression of genes, in particular expression in cardiac muscle and/or skeletal muscle, methods employing these regulatory elements and uses of these elements. Expression cassettes and vectors containing these nucleic acid regulatory elements are also disclosed. The present invention is particularly useful for applications using gene therapy, more particularly muscle-directed gene therapy, and for vaccination purposes.