

Monroe Carell Jr. Children's Hospital at Vanderbilt
Duchenne Muscular Dystrophy Research
Prepared for the Fighting Duchenne Foundation
December 23, 2015

- 1) *List of publications that FightDMD gifts/grants have supported:*
 - a. Soslow JH, Damon BM, Saville BR, Lu Z, Burnette WB, Lawson MA, Parra DA, Sawyer DB, Markham LW. Evaluation of Post-Contrast Myocardial T1 in Duchenne Muscular Dystrophy Using Cardiac Magnetic Resonance Imaging. *Pediatr Cardiol.* 2015 Jan;36(1):49-56.
 - b. Galindo CL, Soslow JH, Brinkmeyer-Langford CL, Gupte M, Smith HM, Sengsayadeth S, Sawyer DB, Benson DW, Kornegay JN, Markham LW. Translating golden retriever muscular dystrophy microarray findings to novel biomarkers for cardiac/skeletal muscle function in Duchenne Muscular Dystrophy. *Pediatr Res.* 2015 Dec 16. doi: 10.1038/pr.2015.257. [Epub ahead of print]
 - c. Soslow JH, Damon SM, Crum K, Lawson M, Slaughter JC, Xu M, Arai AE, Sawyer DB, Parra DA, Damon BM, Markham LW Increased myocardial native T1 and extracellular volume in patients with Duchenne muscular dystrophy (Soslow submitted 2015 and accepted pending revisions).
 - d. Posner AD, Soslow JH, Burnette WB, Bian A, Shintani A, Sawyer DB, and Markham LW The correlation of skeletal and cardiac muscle dysfunction in Duchenne muscular dystrophy (Soslow submitted 2015 and accepted pending revisions).
 - e. McKane M, Soslow JH, Xu M, Saville BR, Slaughter JC, Burnette WB, Markham LW Body mass index does not predict premature cardiomyopathy onset for Duchenne muscular dystrophy (McKane submitted 2015)
 - f. Schoenecker J Plasmin activity protects muscle from transformation into bone after injury (Schoenecker submitted 2015)

- 2) *List of grant submissions that FightDMD funds have helped provide the preliminary data:*
 - a. Funded:
 - i. 13CRP14530007 American Heart Association Clinical Research Program Predicting High Risk Cardiomyopathic Disease in Duchenne Muscular Dystrophy Funds: \$153,961 over 2-year period 2013-2014 Soslow Primary Investigator Markham Mentor
 - ii. 1K23HL 123938-02 NIH/NHLBI Quantitative Assessment of Cardiac Disease in Duchenne Muscular Dystrophy Funds: \$762,000 2014-2019 Soslow Primary Investigator Markham Mentor
 - iii. 1 UL1 TR000445-06 NCRR/NIH Vanderbilt CTSA grant Quantitative Assessment of Cardiac Disease in Duchenne Muscular Dystrophy Funds: \$17,500 (matching funds from department of pediatrics and \$7500 from VUIIS) 2014-2019 Soslow Primary investigator for project (Bernard PI for grant)
 - b. Pending (2015):
 - i. Department of Defense Investigator Initiated Research Mechanisms and Assessment of Brain-Derived Neurotrophic Factor (BDNF) as a Treatment for Duchenne Cardiomyopathy (Budget \$575,000 Galindo Primary Investigator
 - ii. American Heart Association, NCRP Innovative Research Grant A three-dimensional , patient-specific culture system for testing current and new therapies for Duchenne cardiomyopathy (Budget \$150,000) Galindo Primary Investigator
 - iii. Parent Project Muscular Dystrophy Exploratory Grant A three-dimensional , patient-specific culture system for testing current and new therapies for Duchenne cardiomyopathy (Budget \$50,000) Galindo Primary Investigator
 - iv. Department of Defense Therapeutic award (Budget \$471,000) Schoenecker Primary Investigator
 - v. American Heart Association (Budget \$900,000) Schoenecker Primary Investigator
 - c. Unfunded
 - i. National Institute of Health Vanderbilt Duchenne Skeletal and Cardiomyopathy Paul D. Wellstone Muscular Dystrophy Cooperative Research Centers RFA AR13-012 1 U54 AR065140-01 (Budget \$5,133,806) 2012 Markham Primary Investigator
 - ii. National Institute of Health Vanderbilt Duchenne Skeletal and Cardiomyopathy Paul D. Wellstone Muscular Dystrophy Cooperative Research Centers RFA AR13-021 1 U54 AR065140-01 (Budget \$4,214,944) 2013 Markham Primary Investigator

- 3) *List of conference presentations/posters/abstracts associated with work funded by FightDMD*
- a. **Soslow JH**, Damon B, Parra DA, Burnette WB, Wang W, Markham LW. Cardiac MRI Derived T1 times in Cardiomyopathy Associated with Duchenne Muscular Dystrophy are Abnormal. Poster presentation, American Heart Association Scientific Sessions, November 2012, Los Angeles, CA.
 - b. **Soslow JH**, Damon BM, Burnette WB, Parra DP and Markham LW. T1 mapping is abnormal before decline in EF in Patients with Becker and Duchenne Muscular Dystrophy. Poster presentation, Society of Cardiovascular Magnetic Resonance Scientific Sessions, February 2013, San Francisco, CA
 - c. **McKane M**, Soslow JH, Posner A, Burnette WB, Markham LW. Duchenne Cardiomyopathy: Updated Natural History and Predicting Heart Failure. Poster and oral presentation, Southeast Pediatric Cardiology Society Conference, September 2013, Jackson, MS.
 - d. **Soslow JH**, Markham LW, Xu M, Saville B, Damon B, Parra DA. Left Ventricular Function by Echocardiography Correlates Poorly with Cardiac MRI Measures in Duchenne Muscular Dystrophy. Poster presentation, Society of Cardiovascular Magnetic Resonance Scientific Sessions, January 2014, New Orleans, LA.
 - e. **Soslow JH**, Damon SM, Damon BM, Parra DA, Burnette WB, Sawyer DB, Stein CM, Markham LW. Myocardial Extracellular Volume is Elevated in Human Duchenne Muscular Dystrophy. Poster presentation, American Heart Association Scientific Sessions, November 2014, Chicago, IL.
 - f. **Galindo C**, Soslow JH, Brinkmeyer-Langford C, Gupte M, Smith H, Sawyer DB, Kornegay J, Markham LW. GRMD gene expression in skeletal and cardiac muscle: Insights into molecular pathways of progression and therapeutic implications for DMD cardiomyopathy. Poster presentation, Experimental Biology, March, 2015, Boston, MA.
 - g. **Soslow JH**, Damon SM, Crum K, Lawson, MA, Slaughter JC, Xu M, Arai AE, Sawyer DB, Parra DA, Damon BM, Markham LW. Myocardial Extracellular Matrix Expansion Detected with Cardiac MRI: A Biomarker of Cardiomyopathic Disease in Duchenne Muscular Dystrophy. Oral e-abstract presentation, American Heart Association Scientific Sessions, November, 2015. Orlando, FL.
 - h. **Soslow JH**, Damon SM, Crum K, Parra DA, Arai AE, Damon BM, Markham LW. Myocardial T1 and T2 Mapping in Duchenne Muscular Dystrophy: Characterization of Late Gadolinium Enhancement. Accepted for poster presentation, Society of Cardiovascular Magnetic Resonance Scientific Sessions, January 2016, Los Angeles, CA.