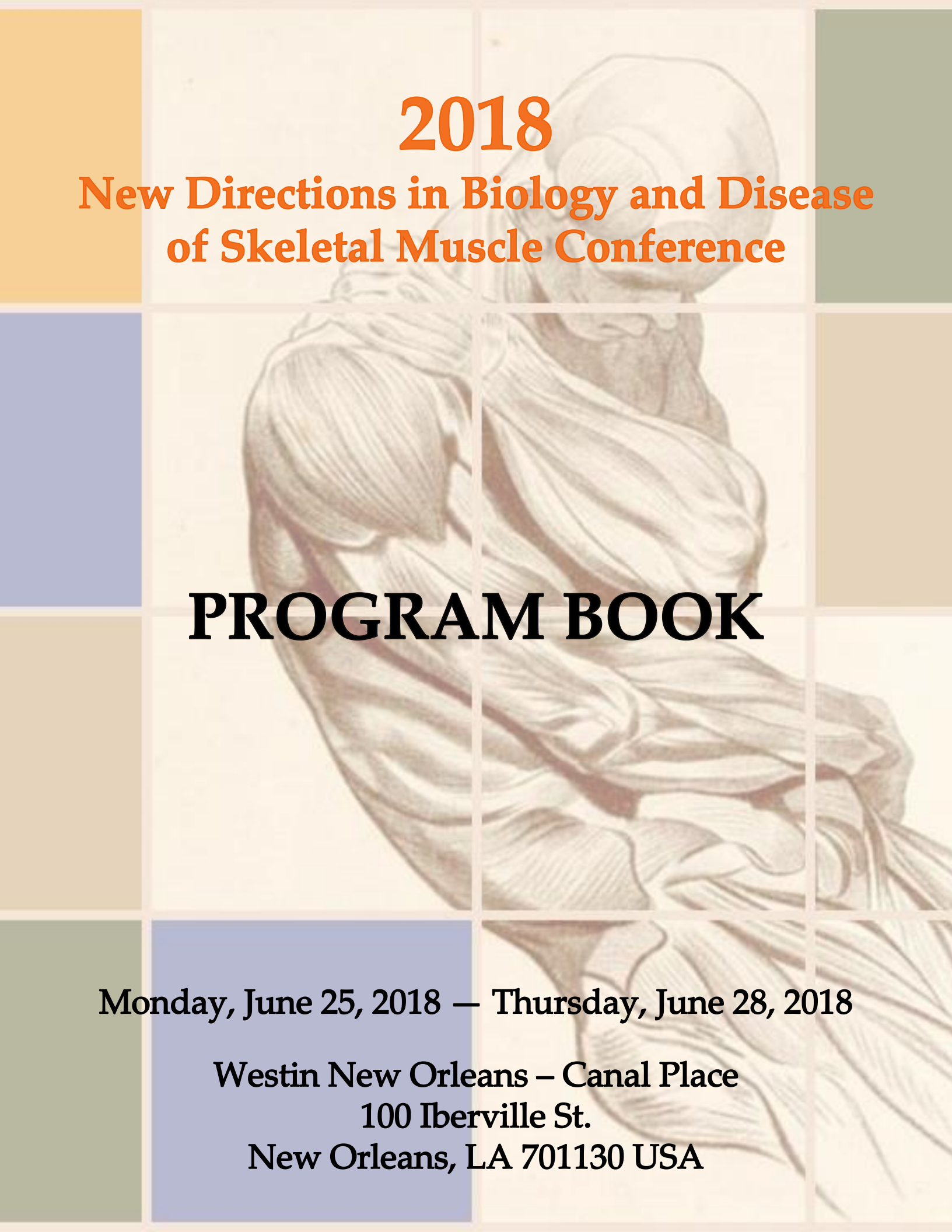
2018

New Directions in Biology and Disease of Skeletal Muscle Conference



PROGRAM BOOK

Monday, June 25, 2018 — Thursday, June 28, 2018

Westin New Orleans – Canal Place
100 Iberville St.
New Orleans, LA 701130 USA

2018 New Directions in Biology and Disease of Skeletal Muscle Conference

We would like to thank all of our sponsors for their contributions to our conference:

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Objectives

The New Directions in Biology and Disease of Skeletal Muscle Conference is being held in New Orleans, Louisiana June 25 – June 28, 2018. This meeting brings together scientists working to understand mechanisms and develop new therapies for muscle disease, especially the muscular dystrophies. The "New Directions" meeting differs from other topically related meetings because of its focus on bringing together industry and academic attendees with a focus on evaluating laboratory based observations and assessing or testing suitability for therapy in the preclinical and clinical setting. This meeting was developed in response to MD Care Act and the recognition that devising and testing therapy for rare neuromuscular disorders requires organization and coordinated efforts among all stakeholders. In addition to the focus on identifying and testing therapeutic pathways, the New Directions meeting places a high emphasis on inclusion of trainees and young investigators, as it is recognized that the challenges of these medical problems will require a diverse and prolonged effort to realize cures for these devastating disorders.

Objective 1: The presentation and sharing of unpublished data. This meeting emphasizes the presentation of unpublished work. Early access to information allows for new collaborations to form moving scientific discovery forward faster into translation.

Objective 2: Promotion of collaboration between industry and academic investigators. As targets are increasingly moving towards development, preclinical and clinical testing, the interaction and partnership between industry and academia is increasingly important. The first session of this meeting is designed to promote industry and advocacy group participation.

Objective 3: Clinical trial planning and outcome. We will devote a specific session to outcomes and endpoints for clinical trials for neuromuscular disease especially the muscular dystrophies and hope to contribute to improved consensus and understanding of appropriate expectations for clinical trials.

Objective 4: Identify both common and unique targets for each muscle disease. This meeting provides a format where multiple different mechanisms of muscle disease are covered providing a backdrop to identify common elements that can be manipulated therapeutically.

Objective 5: Provide trainees and young investigators a forum in which to present data and to encourage trainees to remain studying neuromuscular disease. Trainees are expected to present posters, and senior and junior investigators are engaged by evaluating these presentations.

Conference Organizers

Elizabeth McNally, Northwestern University
H. Lee Sweeney, University of Florida

Coordinators

Christa Stout, University of Florida

Program Committee

Annemieke Aartsma-Rus, Leiden University Medical Center
Dean Burkin, University of Nevada Reno
Dongsheng Duan, University of Missouri
Emanuela Gussoni, Boston Children's Hospital
Mani Mahadevan, University of Virginia
Kathy Mathews, University of Iowa
Dan Michele, University of Michigan
Louise Rodino-Klapac, Nationwide Children's Hospital

KEYNOTE SPEAKER

Eric Olson, Ph.D.

University of Texas Southwestern Medical Center

Correction of Muscle Disease by Genome Editing

Eric Olson, Ph.D.

Annie and Willie Nelson Professorship in Stem Cell Research
Pogue Distinguished Chair in Research on Cardiac Birth Defects
The Robert A. Welch Distinguished Chair in Science
University of Texas Southwestern Medical Center

Dr. Eric Olson is the founding chair of the Department of Molecular Biology at UT Southwestern. He also directs the Hamon Center for Regenerative Science and Medicine and the Wellstone Center for Muscular Dystrophy Research. He holds the Robert A. Welch Distinguished Chair and the Annie and Willie Nelson Professorship.

Eric Olson and his trainees discovered many of the genes that control heart and muscle development and disease. Among their discoveries were myogenin and MEF2, master transcriptional regulators of myogenesis, and Myomaker and Myomixer, the long-sought membrane proteins that control myoblast fusion. Olson's team also discovered the Hand transcription factors, which regulate ventricular growth, and myocardin, the activator of cardiovascular differentiation. Other discoveries include the stress-response pathways that underlie pathological cardiac remodeling and numerous microRNAs that modulate muscle development and disease. His most recent work has provided a new strategy for correction of Duchenne muscular dystrophy using CRISPR gene editing. Olson's discoveries at the interface of developmental biology and medicine have illuminated the fundamental principles of tissue formation and have provided new concepts in the quest for muscle and cardiovascular therapeutics.

Olson has published over 600 scientific articles that have been cited over 90,000 times in the scientific literature. He is a member of the U.S. National Academy of Sciences, the National Academy of Medicine, and the American Academy of Arts and Sciences. His awards include the Research Achievement Award and Eugene Braunwald Mentorship Award from the American Heart Association, the Pasarow Medical Research Award, the Pollin Prize, the Passano Award, and the March of Dimes Prize in Developmental Biology. In 2009, the French Academy of Science awarded Dr. Olson the Fondation Lefoulon-Delalande Grand Prize for Science.

Olson has co-founded multiple biotechnology companies to develop therapies for heart and muscle disease. In his spare time, he plays guitar and harmonica with The Transactivators, a rock band inspired by Willie Nelson, the Texas troubadour who created the Professorship that supports his research.

2018 New Directions in Biology and Disease of Skeletal Muscle Conference

June 25-June 28, 2018; New Orleans, LA

Monday, June 25, 2018

Industry Workshop, *Grand Ballroom, 12th floor*

(all registered conference attendees are welcomed and encouraged to attend)

12:00 - 12:10PM	**Welcome and Introductions**
12:10 – 12:40	**Irina Antonijevic,** Wave Life Sciences *Stereopure Exon Skipping Approach for the Potential Treatment of DMD*
12:40 - 1:10	**Jon Tinsley,** Summit Therapeutics *Development of ezutromid, a first in class utrophin modulator for the treatment of Duchenne muscular dystrophy*
1:10 - 1:40	**Carl Morris,** Solid Biosciences *Developing therapies for Duchenne Muscular Dystrophy*
1:40 – 2:10	**Jane Owens,** Pfizer Inc. *Drug Development for Rare Neuromuscular Disease at Pfizer*
2:10 – 2:40PM	**COFFEE BREAK**
2:40 – 3:10	**Karin Lucas,** Sarepta Therapeutics *Development Programs for Muscular Dystrophy*
3:10 – 3:40	**Andrew Nichols,** Catabasis Pharmaceuticals *Edasalonexent, an NF-kB Inhibitor In Development as a Potential Disease-Modifying Therapy for Duchenne Muscular Dystrophy*
3:40 – 4:10	**Jenny Tobin,** Ironwood Pharmaceutical *Opportunities for sGC stimulators in muscular dystrophy*
4:10 – 4:40	**Angela Cacace,** Fulcrum Therapeutics *Modulating Gene Expression in Neuromuscular Disorders in an Era of Precision Medicine: FSHD and DMD*
5:00 - 6:00PM	**Keynote Speaker**: **Eric Olson,** University of Texas Southwestern Medical Center *Correction of Muscle Disease by Genome Editing*
6:00 - 9:00PM	**Evening Welcome Reception;** *Riverbend Terrace, 11th floor* *Sponsored by Sarepta Therapeutics*

Tuesday, June 26, 2018

7:15AM **Breakfast will be served in Riverbend Terrace, 11th floor**

Session I: Gene Therapy and Gene Editing, *Grand Ballroom, 12th floor*

Chair: Dongsheng Duan

8:00 – 8:20AM **Dongsheng Duan**, University of Missouri
Systemic AAV micro-dystrophin gene therapy for Duchenne muscular dystrophy

8:20 – 8:40 **Casey Childers**, University of Washington
Myotubular myopathy - A Dogs Story

8:40 – 9:00 **Marco Passini,** Sarepta Therapeutics, Inc.
Systemic Delivery of PPMO Results in Widespread Muscle Delivery and Efficacy in Mice and Non-Human Primates for the Treatment of Duchenne Muscular Dystrophy

9:00 – 9:20 **Leonela Amoasii,** University of Texas Southwestern Medical Center
CRISPR/Cas9-mediated genome editing restores dystrophin expression in canine model of muscular dystrophy

9:20 – 9:50AM **COFFEE BREAK**

Session II: Biomarkers

Chair: Annemieke Aartsma-Rus

9:50 – 10:10AM **Annemieke Aartsma-Rus**, Leiden University Medical Center
Introduction to the use of biomarkers in the NMD space

10:10 – 10:30 **Elena Pegoraro**, University of Padova
Genetic modifiers and outcome measures in Duchenne Muscular Dystrophy

10:30 – 10:50 **Pietro Spitali**, Leiden University Medical Center
Non invasive biomarkers in Duchenne Muscular Dystrophy

10:50 – 11:10 **Charles Thornton**, University of Rochester Medical Center
Biomarkers for myotonic dystrophy

11:10 – 11:30 **Belinda Cowling,** IGBMC
Myostatin as a novel blood based biomarker for antisense oligonucleotide-mediated Dnm2 knockdown to treat myotubular myopathy in mice

11:30AM - 1:30PM **LUNCH BREAK (on your own)**

1:30 – 3:30PM **Poster Session I,** *Azalea and Magnolia Ballrooms, 3rd floor*

Sponsored by Pliant Therapeutics

Session III: Clinical Approaches, Gene Therapy, *Grand Ballroom, 12th floor*

Chair: Louise Rodino-Klapac

3:30 – 3:50PM **Louise Rodino-Klapac**, Nationwide Children's Hospital
Gene Therapy for Limb Girdle Muscular Dystrophies

3:50 – 4:10 **Barry Byrne**, University of Florida
Gene Therapy Strategies for Neuromuscular Disease: Clinical Considerations

4:10 – 4:30 **Carsten Bonnemann,** NINDS NIH
First-in-human intrathecal gene transfer study for Giant Axonal Neuropathy: review of safety, immunologic responses and interim analysis of efficacy

4:30 – 4:50PM **Guy Odom,** University of Washington
Nucleotide-based therapy improves cardiac contractile kinetics in a cardiomyopathy model of Duchenne Muscular Dystrophy

Wednesday, June 27, 2018

7:15AM **Breakfast will be served in Riverbend Terrace, 11th floor**

Session IV: Stem Cells and Exosomes, *Grand Ballroom, 12th floor*

Chair: Emanuela Gussoni

8:00 – 8:20AM **Emanuela Gussoni**, Boston Children's Hospital
Satellite cell markers

8:20 – 8:40 **Kathryn Wagner**, Kennedy Krieger Institute
Modeling DMD in hISPCs

8:40 – 9:00 **Charlotte Peterson**, University of Kentucky
Evolving roles for satellite cells in muscle adaptation and aging

9:00 – 9:20 **Premi Haynes,** University of Washington
Sporadic DUX4 expression in FSHD myoblasts is associated with incomplete repression by the PRC2 complex and gain of H3K9 acetylation on the contracted D4Z4 allele

9:20 – 9:50AM **COFFEE BREAK**

Session V: Small Molecule Screens

Chair: Dean Burkin

9:50 – 10:10AM **Dean Burkin**, University of Nevada Reno
Integrin targeted therapies for the treatment of muscular dystrophy

10:10 – 10:30 **Rachelle Crosbie-Watson**, University of California Los Angeles
Development of small molecule modulators of sarcospan for Duchenne muscular dystrophy

10:30 – 10:50 **Barrington Burnett**, Uniformed Services University of the Health Sciences
Developing Small Molecules to Treat Spinal Muscular Atrophy

10:50 – 11:10 **Joanne Young,** CYTOO SA
Application of MyoScreen, a New High-Throughput Phenotypic Screening Platform to Increase the Success Rate of Early Muscle Drug Discovery

11:10AM - 1:30PM **LUNCH BREAK (on your own)**

1:30 – 3:30PM **Poster Session II,** *Azalea and Magnolia Ballrooms, 3rd floor*
Sponsored by Indiana Center for Musculoskeletal Health

Session VI: Clinical Trials in NMD, *Grand Ballroom, 12th floor*

Chair: Kathy Mathews

3:30 – 3:50PM **Kathy Mathews**, University of Iowa
Therapy for neuromuscular disease; Current status and future challenges

3:50 – 4:10 **Richard Finkel**, Nemours Children's Hospital
Spinal Muscular Atrophy: modulation of splicing and gene replacement strategies for treatment of a neurodegenerative disease

4:10 – 4:30 **Julie Parsons**, University of Colorado
Translating Clinical Trials to Clinical Care

4:30 – 4:50 **Anne Connolly**, Washington University
New (and Old) Corticosteroid News

4:50 – 5:10 **Paula Clemens,** University of Pittsburgh
A Phase II, Dose Finding Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of NS-065/NCNP-01 in Boys with Duchenne Muscular Dystrophy (DMD)

5:10 – 5:30 **Hirofumi Komaki,** National Center of Neurology and Psychiatry
A Japanese Phase I/II study of NS-065/NCNP-01, Exon53 skipping drug, in patients with Duchenne muscular dystrophy -A dose-finding study-

5:30 – 6:00 **Francesco Muntoni,** University College London
Golodirsen Induces Exon Skipping Leading to Sarcolemmal Dystrophin Expression in Patients with Genetic Mutations Amenable to Exon 53 Skipping

7:00pm **Boarding for Natchez Steamboat, set sail at 7:30pm sharp!**
Toulouse St. Wharf

7:30 - 9:30PM **Banquet Dinner: Natchez Steamboat Cruise**
Sponsored by Cure Duchenne

Thursday, June 28, 2018

7:15AM **Breakfast will be served in Riverbend Terrace, 11th floor**

Session VII: Myotonic Dystrophy, *Grand Ballroom, 12th floor*

Chair: Mani Mahadevan

8:00 – 8:20AM **Mani Mahadevan**, University of Virginia
Modifiers of Myotonic Dystrophy

8:20 – 8:40 **Ruben Artero**, University of Valencia
Derepressing MBNL protein expression in Myotonic Dystrophy with candidate oligonucleotide therapeutics

8:40 – 9:00 **Tom Cooper,** Baylor College of Medicine
Modeling DM1 in heart and skeletal muscle to determine mechanisms and test therapeutic approaches

9:00 – 9:30AM **COFFEE BREAK**

Session VIII: Muscle Injury and Repair

Chair: Dan Michele

9:30 – 9:50AM **Dan Michele**, University of Michigan
Mechanisms of muscle membrane stability and repair

9:50 – 10:10 **Jyoti Jaiswal**, Children's National Medical Center
Mitochondria mediated myofiber repair and its role in muscle diseases

10:10 – 10:30 **Alexis Demonbreun**, Northwestern University
The annexin repair complex promotes Ca2+ efflux at the site of sarcolemmal damage through vesicle release

10:30 – 10:50 **Jane Seto,** Murdoch Children's Research Institute
ACTN3 genotype influences muscle mass regulation and glucocorticoid-induced muscle wasting

10:50 – 11:10 **Justin Boyer,** Cincinnati Children's Hospital Medical Center
Erk1/2-dependent survival of satellite cells underlies dystrophic skeletal muscle remodeling in the mouse

11:10 – 11:30 **Closing Remarks**

11:30AM **Adjourn**

We thank you all for your participation in the 2018 New Directions in Biology and Disease of Skeletal Muscle Conference! Please mark your calendars for our next New Directions conference July 6-9, 2020 at the New York Marriott Marquis in New York, NY!

Abstracts & Posters

Abstracts are listed in alphabetical order by submitting author's last name (**bold**) within each category. Abstract numbers, below, correspond to board numbers where the posters will be displayed.

Gene Therapy and Gene Editing

1. CRISPR/Cas9-mediated genome editing restores dystrophin expression in canine model of muscular dystrophy
Amoasii, Leonela; Hildyard, John; Li, Hui; Shelton, John M; Sanchez-Ortiz, Efrain; Bassel-Duby, Rhonda; Piercy, Richard; Olson; Eric N

2. RyR pathway modulators dantrolene and RyCal ARM210 boost antisense mediated exon skipping in human DMD patient derived culture models
Barthelemy, Florian; Wang, Richard T; Hsu, Christopher; Douine, Emilie D; Pyle, April; Nelson, Stanley F; Miceli, Carrie M

3. Therapeutic approaches for heart damages in muscular dystrophies
Biquand, Ariane; Suel, Laurence; Charton, Karine; Lostal, William; Richard, Isabelle

4. A novel target for splice-modulating therapy: Skipping of a common pseudoexon-inducing mutation that causes severe collagen VI-related muscular dystrophy
Bolduc, Véronique; Foley, Reghan A; Degefa, Herimela Solomon; Sarathy, Apurva; Zhou, Haiyan; Donkervoort, Sandra; Hu, Ying; Cummings, Beryl B; Lek, Monkol; Li, Yan; Gartioux, Corine; Allamand, Valérie; Wilton, Steve D; Hanssen, Eric; Lamandé, Shireen R; MacArthur, Daniel; Wagener, Raimund; Muntoni, Francesco; **Bönnemann, Carsten G**

5. Does Neuroguidin play a role in skeletal muscle fiber type remodeling?
Callier, Mathew C; Resnick, Jessica D; Gilbert, Carolyn A; Pandorf, Clay E

6. Antibody-Oligonucleotide Conjugates (AOCs) Represent a Significant Advance Over Existing Technology for Exon Skipping in Duchenne Muscular Dystrophy (DMD)
Darimont, Beatrice; Huang, Hanhua; Burke, Rob; Doppalapudi, Ramana; Johns, Rachel; Balu, Palani; Cochran, Michael; Shahmoradgoli, Maria; Chu, David; Erdogan, Gulin; Chen, Yanling; Kwon, Hae Won; Shi, Yunyu; Hood, Michael; Moon, Michael; Cortes, Allan; Arias, Joel; Raney, Anneke; Geall, Andrew; Levin, Arthur

7. An Intronic Mutation in Dysferlin Leads to Expression of a Pathogenic Pseudoexon That Can Be By-Passed Using Antisense Oligonucleotides to Restore the Normal Reading Frame
Dominov, Janice A; Uyan, Özgün; McKenna-Yasek, Diane; Nallamilli, Babi Ramesh Reddy; Kergourlay, Virginie; Bartoli, Marc; Levy, Nicolas; Hudson, Judith; Evangelista, Teresinha; Lochmuller, Hanns; Krahn, Martin; Rufibach, Laura; Hegde, Madhuri; Brown, Robert H

8. Development of Nanoparticles to Deliver a CRISPR/Cas9 Therapy for Duchenne Muscular Dystrophy
Emami, Michael; Young, Courtney; Ji, Ying; Pyle, April; Meng, Huan; Spencer, Melissa

9. Effects of exercise on the efficacy of microdystrophin gene therapy
Fathalikhani, Daniel; Bukovec, Katherine; Hamm, Shelby; Addington, Adele; Zhang, Haiyan; Perry, Justin; Mansueto, Alex; McMillan, Ryan; Lawlor, Michael; Coleman, Kirsten; Brown, David; Morris, Carl; Gonzalez, Pat; Grange, Robert

10. **Calcium responsiveness in myotubes derived from muscle cells of myotubularin-deficient dogs treated with a single infusion of rAAV8-cMTM1**
    Dupont, Jean Baptiste; Bukovec, Katherine; Mack, David; Parker, Maura; Hamm, Shelby; Childers, Martin; **Grange, Robert**

11. **Allele-Specific knockdown of a dominant, heterozygous COL12A1 mutation as a proof of concept for precision medicine**
    Mohassel, Payam; **Hu, Ying**; Donkervoort, Sandra; Ezzo, Daniel; Bolduc, Véronique; Foley, Reghan A; Bönnemann, Carsten G

12. **A mutation-independent approach via transcriptional upregulation of a disease modifier gene rescues muscular dystrophy in vivo**
    Kemaladewi, Dwi; Bassi, Prabhpreet; Lindsay, Kyle; Hyatt, Ella; Ivakine, Zhenya; Cohn, Ronald

13. **CRISPR/Cas9 correction of a dystrophin actin binding domain mutation in mice**
    Kyrychenko, V; Mireault, A; Min, Y-L; Caballero, D; Amoasii, L; Shelton, J; McAnally, J; Rodriguez, C; Bassel-Duby, R; Olson, E

14. **Applying genome-wide CRISPR screens for therapeutic discovery in FSHD**
    Lek, Angela; Zhang, Tracy; Woodman, Keryn; Kodani, Andrew; Pakula, Anna; Spinazzola, Janelle; Criscione, June; Sanjana, Neville; Lek, Monkol; Jones, Peter; Kunkel, Louis

15. **Modulation of BIN1 expression rescues X-linked centronuclear myopathy in mice**
    Lionello, Valentina M; Nicot, Anne-Sophie; Djerroud, Sarah; Kretz, Christine; Kessler, Pascal; Buono, Suzie; Messaddeq, Nadia; Koebel, Pascale; Prokic, Ivana; Hérault, Yann; Laporte, Jocelyn; Cowling, Belinda

16. **Biodistribution and safety study of AAV9 in non-human primates for CAPN3 gene transfer**
    Lostal, William; Roudaut, Carinne; Deschamps, Jack-Yves; Richard, Isabelle

17. **Correction of Duchenne Muscular Dystrophy Exon 44 Deletion Mutations in Mice and Human Cells by CRISPR/Cas9**
    Min, Yi-Li; Rodriguez, Cristina; Li, Hui; Mireault; Alex A; Shelton, John M; McAnally, John R; Amoasii, Leonela; Kyrychenko, Viktoriia; Malladi, Venkat; Long, Chengzu; Bassel-Duby, Rhonda; Olson, Eric N

18. **Novel interactions of cardiac dystrophin with caveolar proteins: implications for gene therapy**
    Wang, Hong; Johnson, Eric; Scott, Charlotte; Hau, Kwan; Marrosu, Elena; Zhang, Liwen; Duan, Dongsheng; **Montanaro, Federica**

19. **Macrophages as a Trojan horse for antisense oligonucleotide delivery to muscle**
    Novak, James; Davi, Mazala; Hogarth, Marshall; Boehler, Jessica; Hoffman, Eric; Nagaraju, Kanneboyina; Jaiswal, Jyoti; Partridge, Terence

20. **Nucleotide-based therapy improves cardiac contractile kinetics in a cardiomyopathy model of Duchenne Muscular Dystrophy**
    Kolwicz, SC Jr; Hall, JK; Moussavi-Harami, F; Chen, X; Hauschka, SD; Chamberlain, JS; Regnier, M; **Odom, GL**

21. **Sarcospan Rescues Cardiac Function in Duchenne Muscular Dystrophy Mouse Models in the Absence of Abundant Utrophin Upregulation**
Parvatiyar, Michelle S; Kanashiro-Takeuchi, Rosemeire M; Dieseldorff Jones, Karissa; Brownstein, Alexandra J; Collado, Judd R; Gopal, Jay; Hammond, Katherine G; Beedle, Aaron M; Pinto, Jose Renato; Crosbie-Watson, Rachelle H

22. **Systemic Delivery of PPMO Results in Widespread Muscle Delivery and Efficacy in Mice and Non-Human Primates for the Treatment of Duchenne Muscular Dystrophy**
Gan, Li; Wood, Jenna; Yao, Monica; Wu, Leslie; Treleaven, Chris; Estrella, Nelsa; Wentworth, Bruce; Charleston, Jay; Rutkowski, Joseph; Hanson, Gunnar; **Passini, Marco**

23. **First-in-human intrathecal gene transfer study for Giant Axonal Neuropathy: review of safety, immunologic responses and interim analysis of efficacy**
Saade, Dimah; Bharucha-Goebel, Diana; Jain, Mina; Waite, Melissa; Norato, Gina; Cheung, Ken; Foley, Reghan A; Soldatos, Ariane; Rybin, Denis; Lehky, Tanya; Hu, Ying; Whitehead, Matthew; Calcedo Del Hoyo, Roberto; Jacobson, Steven; Nath, Avindra; Grieger, Josh; Samulski, Jude R; Gray, Steven J; Bönnemann, Carsten G

24. **Characterization and Functional Rescue of a Mouse Model of Congenital Muscular Dystrophy with Megaconial Myopathy**
Sayed, Ambreen A; Vary, Calvin P; Sher, Roger B; Cox, Gregory A

25. **CRISPR/Cas9 genome editing of Utrophin miRNA-binding sites in DMD patient hiPSCs**
Sengupta, Kasturi; Mishra, Manoj K; Pyle, April D; Spencer, Melissa J; Khurana, Tejvir S

26. **High throughput screening reveals small molecule enhancers of sarcospan for the treatment of Duchenne muscular dystrophy**
Shu, C; Damoiseaux, R; Alam, M.P; John, V; and Crosbie-Watson, R.H.

27. **Loss of miR-133b accelerates Duchenne muscular dystrophy pathogenesis in a mouse model for the disease**
Taetzsch, Thomas; Shapiro, Dillon; Valdez, Gregorio

28. **Clinical phenotypes as predictors of DMD exon 51 skipping therapy: a systematic review**
Waldrop, Megan A; Ben Yaou, Rabah; Lucas, Karin K; Martin, Ann; O'Rourke, Erin; Delalande, Olivier; Hubert, Jean-Francois; FILNEMUS8; Ferlini, Alessandra; Muntoni, Francesco; Leturcq, France; Tuffery-Giraud, Sylvie; Weiss, Robert B; Flanigan, Kevin M

29. **Long-Term AAV-9 SERCA2a Gene Therapy Ameliorated Dilated Cardiomyopathy in an Aged Mouse Model of Duchenne Muscular Dystrophy**
Wasala, Nalinda; Yue, Yongping; Lostal, William; Wasala, Lakmini; Niranjan, Nandita; Hajjar, Roger; Babu, Gopal; Duan, Dongsheng

30. **CRISPR-Cpf1 correction of muscular dystrophy mutations in human cardiomyocytes and mice**
Zhang, Y; Long, C; Li, H; McAnally, JR; Baskin, KK; Shelton, JM; Bassel-Duby, R; Olson, EN

Biomarkers

31. Serum Protein Biomarkers for Dysferlinopathy
Hollander, Zsuzsanna; Dai, Darlene LY; Chen, Virginia; Singh, Amrit; **Albrecht, Douglas E**; Lee, Elaine; Rufibach, Laura; Williams, Bradley; Mittal, Plavi; Windish, Hillarie P; Mayhew, Anna; Jacobs, Marni; Day, John W; Jones, Kristi J; Bharucha-Goebel, Diana X; Harms, Matt; Pestronk, Alan; Walter, Maggie C; Stojkovic, Tanya; Sparks, Susan; Bravver, Elena; Diaz Manera, Jordi; Pegoraro, Elena; Paradas, Carmen; Mendell, Jerry R; Lochmuller, Hanns; Bushby, Kate; Straub, Volker; Assadian, Sara; Wilson-McManus, Janet E; Smith, Derek S; Borchers, Christoph H; McManus, Bruce; Ng, Raymond

32. Exosome mRNA splice-variant biomarkers of muscular dystrophies in human urine
Antoury, Layal; Hu, Ningyan; Balaj, Leonora; Das, Sudeshna; Georghiou, Sofia; Darras, Basil; Clark, Tim; Breakefield, Xandra; Wheeler, Thurman

33. An Imaging Study of the Diaphragm and Accessory Respiratory Muscles in Duchenne Muscular Dystrophy
Barnard, Alison; Lott, Donovan; Batra, Abhinandan; Triplett, William; Forbes, Sean; Riehl, Samuel; Willcocks, Rebecca; Smith, Barbara; Vandenborne, Krista; Walter, Glenn

34. Mass Spectrometry based Quantification of Microdystrophin and Dystrophin
Brown, Kristy J; Boehler, Jessica F; Claeys, Annie; Gonzalez, Patrick; Ricotti, Valeria; Morris, Carl A

35. Myostatin as a novel blood based biomarker for antisense oligonucleotide-mediated Dnm2 knockdown to treat myotubular myopathy in mice
Buono, Suzie; Koch, Catherine; Robé, Anne; Kretz, Christine; Gomez Oca, Raquel; Guo, Shuling; Depla, Marion; Monia, Brett P; Laporte, Jocelyn; Thielemans, Leen; **Cowling, Belinda**

36. Dynamic Breathing MRI: a promising biomarker of diaphragmatic function in COL6-related dystrophy patients and LAMA2-related dystrophy patients
Yun, Pomi; Norato, Gina; Hsieh, Nathan; Zhu, Robert; Dastgir, Jahannaz; Leach, Meganne; Donkervoort, Sandra; Yao, Jianhua; Arai, Andrew E; Bönnemann, Carsten G; **Foley, A Reghan**

37. Identification of developmental myosin positive fibres acts both as a clinical biomarker for muscle disease and an important component of the process to confirm ezutromid target engagement
Tinsley, J; Muntoni, F; Layton, G; Faelan, C; Patterson-Kane, J; Heatherington, A; Davies K; PhaseOut DMD study team

38. mitoRACE: In Vivo Assessment of Mitochondrial Function using Multiphoton NADH Fluorescence
Willingham, Thomas; Zhang, Yingfan; Glancy, Brian

Stem Cells and Regeneration

39. JARID2 Regulates Skeletal Muscle Differentiation through Regulation of Canonical Wnt Signaling Pathway
Adhikari, Abhinav; Davie, Judy

40. **Laminin enhancing small molecule therapy improves preclinical outcome measures in a murine model of Laminin-α2 Congenital Muscular Dystrophy (LAMA2-CMD)**
Barraza-Flores, Pamela; Burkin, Dean

41. **Erk1/2-dependent survival of satellite cells underlies dystrophic skeletal muscle remodeling in the mouse**
Boyer, Justin G; Molkentin, Jeffery D

42. **Modulation of the IL-6 trans-signalling pathway with engineered extracellular vesicles as a novel therapeutic approach for Duchenne Muscular Dystrophy**
Conceicao, Mariana; Wiklander, Oscar; Gupta, Dhanu; McClorey, Graham; Lundin, Per; EL Andaloussi, Samir; Wood, Matthew

43. **Sunitinib promotes myogenic regeneration and prevents Duchenne Muscular Dystrophy disease progression via transient STAT3 activation**
Fontelonga, Tatiana M; Jordan, Brennan; Nunes, Andreia M; Bolden, Nicholas; Barraza-Flores, Pamela; Burkin, Dean J

44. **Directed differentiation of iPSCs to skeletal muscle progenitors: a rescue strategy for a mouse model of LGMD2i**
Hamm, Shelby; Hollinger, Katrin; Chamberlain, Jeffrey; Mack, David; Grange, Robert

45. **Myosin X drives filopodia of mammalian myoblasts to promote cell fusion**
Hammers, David W; Coker, Cora C; Matheny, Michael K; Merscham-Banda, Melissa; Wakefield, Zachary R; Heimsath, Ernest G; Kang, Hansol; Hammer, John A; Cheney, Richard E; Sweeney, H. Lee

46. **A role for group 2 innate lymphoid cells in muscular dystrophy**
Kastenschmidt, Jenna; Avetyan, Ileen; Ayer, Rachel; Yahia, Rayan; Pham, Phillip; Rios, Rodolfo; Mannaa, Ali; Villalta, Armando S

47. **Twist2+ cells contribute to type IIb myofibers via Semaphorin3a-Nrp1 signaling**
Li, Stephen; Jainchander, Priscilla; Sanchez-Ortiz, Efrain; Bassel-Duby, Rhonda; Olson, Eric N; Liu, Ning

48. **In vivo investigation of skeletal muscle regeneration in DBA/2J-mdx mice**
Mázala, Davi; Novak, James; Nearing, Marie; Chen, Yi-Wen; Jaiswal, Jyoti; Partridge, Terry

49. **Possible involvement of early growth response 3 in age-related reduction of muscle satellite cells**
Ogura, Yuji; Kurosaka, Mitsutoshi; Sato, Shuichi; Kotani, Takashi; Fujiya, Hiroto; Funabashi, Toshiya

50. **The cellular basis of muscle growth, regeneration and hypertrophy varies with age and disease**
Partridge, Terence; Nearing, Marie; Mazala, Davi; Novak, James

51. **MuRF1 and MuRF2 modulate myogenic-adipogenic differentiation cellular fate in skeletal muscle**
Silvestre, João G; Baptista, Igor L; Labeit, Siegfried; Moriscot, Anselmo S

52. **Constricted migration of myoblasts induces nuclear damage and impairs differentiation**
Smith, Lucas R; Irianto, Jerome; Discher, Dennis E

53. **Extracelluar Matrix Remodeling in Dystrophic Skeletal Muscle Influences Stem Cell Function**
Stearns-Reider, Kristen; Hammond, Katherine; Hicks, Michael; Chin, Jesse; McCarthy, Siobhan; Maity, Alok; Wollman, Roy; Hansen, Kirk; Pyle, April; Crosbie-Watson, Rachelle

54. **Lysophosphatidic acid acyltransferase 3 incorporates DHA into phospholipids of satellite cells and is upregulated by PPAR-delta**
Valentine, William; Tokuoka, Suzumi; Hishikawa, Daisuke; Kita, Yoshihiro; Shindou, Hideo; and Shimizu, Takao

Clinical Trials in NMD

55. **A Phase II, Dose Finding Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of NS-065/NCNP-01 in Boys with Duchenne Muscular Dystrophy (DMD)**
Clemens, Paula; Rao, Vamshi; Connolly, Anne; Harper, Amy; Mah, Jean; Smith, Edward; McDonald, Craig; Morgenroth, Lauren; Osaki, Hironori; Hoffman, Eric

56. **CALLISTO: A Phase I Open-Label, Sequential Group, Cohort Study of Pharmacokinetics and Safety of Omigapil in LAMA2 and COL6-Related Dystrophy Patients**
Foley, A Reghan; Leach, Meganne; Averion, Gilberto; Hu, Ying; Yun, Pomi; Neuhaus, Sarah; Saade, Dimah; Arevalo, Cynthia; Fink, Margaret; DeCoster, Jameice; Mendoza, Christopher; Mayer, Oscar; Hausmann, Rudolf; Petraki, Diana; Cheung, Ken; Bönnemann, Carsten G

57. **Combining Prednisolone with Myostatin Inhibition in a Mouse Model of Duchenne Muscular Dystrophy**
Hart, Cora; Hammers, David; Matheny, Michael; Wakefield, Zachary; Kang, Hansol; Sweeney, Lee

58. **A Japanese Phase I/II study of NS-065/NCNP-01, Exon53 skipping drug, in patients with Duchenne muscular dystrophy -A dose-finding study-**
Komaki, Hirofumi; Takeshima, Yasuhiro; Matsumura, Tsuyoshi; Ozasa, Shiro; Funato, Michinori; Egawa, Yoichi; Takeda, Shin'ichi

59. **Meta-analyses of deflazacort vs prednisone/prednisolone in patients with nonsense mutation Duchenne muscular dystrophy**
Shieh, Perry B; **Moon, Jay**; O'Mara, Edward; Elfring, Gary; Trifillis, Panayiota; McIntosh, Joseph; Santos, Claudio; Parsons, Julie; Apkon, Susan; Darras, Basil; Campbell, Craig; McDonald, Craig

60. **Slope analysis of 6-minute walk distance as an alternative method to determine treatment effect in trials in Duchenne muscular dystrophy**
Moon, Jay; Souza, Marcio; Elfring, Gary L; McIntosh, Joseph; Peltz, Stuart W

61. **Golodirsen Induces Exon Skipping Leading to Sarcolemmal Dystrophin Expression in Patients With Genetic Mutations Amenable to Exon 53 Skipping**
Frank, Diane E; Mercuri, Eugenio; Servais, Laurent; Straub, Volker; Morgan, Jennifer; Domingos, Joana; Schnell, Frederick; Dickson, George; Popplewell, Linda; Seferian, Andreea; Monforte, Mauro; Guglieri, Michela; **Muntoni, Francesco**; and the SKIP-NMD Study Group

Muscle Injury and Repair

62. Eccentric Contraction-Induced Strength Loss and Recovery in Dystrophic Skeletal Muscle
Baumann, Cory; Warren, Gordon; Lowe, Dawn

63. A novel protocol to mimic human walking gait in mdx dystrophic muscle in vitro
Bukovec, K; Hu, X; Virgilio, K; Jones, B; Peirce-Cottler, S; Blemker, S; Grange, RW

64. Novel approaches to automated utrophin imaging in cells and tissue for Duchenne muscular dystrophy drug discovery
Elsey, David; Guiraud, Simon; Monecke, Sebastian; Geese, Marcus; Edwards, Ben; Squire, Sarah; Guhl, Anna; Lomow, Alexander; Tinsley, Jonathon; Harriman, Shawn; Davies, Kay; Powell, David; **Burch, Peter**

65. Identification of a muscle enriched RNA binding protein localized to Cajal Bodies-like nuclear structures
Cannavino, Jessica; Morales, M Gabriela; Bassel-Duby, Rhonda; Olson, Eric N

66. Satellite cell migration is regulated by MMP-13 and required for skeletal muscle growth and repair
Kok, Hui Jean; Smith, Lucas; Barton, Elisabeth

67. Rhabdomyolysis phenotype in a family with RYR1-related myopathy
Lawal, TA; Todd, JJ; Chrismer IC; Witherspoon JW; Cosgrove MM; Allen C; Jain MS; Waite M; Emile-Backer M; Bönnemann CG; Meilleur KG

68. Inverse correlation between utrophin expression and sensitivity to eccentric contraction in dystrophin-deficient mdx skeletal muscle
Lindsay, Angus; McCourt, Preston M; Lowe, Dawn A; Ervasti, James M

69. Krüppel like factor 2 (KLF2) deficient myeloid cells promote muscle repair by enhancing the immune response to injury
Manoharan, Palanikumar; Song, Taejong; Radzyukevich, Tatiana L; Sadayappan, Sakthivel; Lingrel, Jerry B; Heiny, Judith A

70. Myofiber development and regeneration in a mouse model of muscular dystrophy
Massopust, Ryan; Thompson, Wesley

71. Dynamic lipid mediator changes support infiltrating macrophage subtype transitions during skeletal muscle injury and regeneration
Giannakis, N; Sansbury, B.E; Patsalos, A; Hays, T; Han, X; Spite, M; **Nagy, L**

72. Doxorubicin exposure causes subacute cardiac atrophy dependent upon the striated muscle-specific ubiquitin ligase Muscle Ring Finger-1
Parry, Traci L; Huang, Wei; Beak, Ju Youn; Jensen, Brian C; Willis, Monte S

73. Macrophage BACH1, a heme regulated transcriptional repressor, controls HMOX1 and skeletal muscle regeneration
Patsalos A; Tzerpos P; Varga T; Pap A; Horvath A; Giannakis N; Lyroni K; Koliaraki V; Daniel B; Pintye E; Dezso B; Kollias G; Spilianakis C.G; Nagy L

74. **Is Regional Fat Distribution a Determinant of Myosteatosis Estimates in Older Men?**
Harris-Love, Michael; **Pennington, Donte**; Avila, Nilo; Adams, Bernadette; Ismail, Catheeja; Zaidi, Syed; Kassner, Courtney; Liu, Frank; Kondapaneni, Saritha; Conner, Anthony; Blackman, Marc

75. **Search for microRNAs that regulate the skeletal muscle regenerative process through the inhibition of MuRF1 and MuRF2**
Silva, William J; Graça, Flavia A; Cruz, A; Silvestre, João G; Labeit, Siegfried; Miyabara, Elen H; Yan, Chao YI; Wang, Da Zhi; Moriscot, Anselmo S

76. **Recombinant human laminin-111 and 211 protein therapy prevents disease progression in an immune deficient mouse model of LAMA2-CMD**
Smith, Christina R; Barraza-Flores, Pamela; Hermann, Hailey; Burkin, Dean J

77. **Long-term quercetin and Lisinopril supplementation provides limited protection to dystrophic skeletal muscle from D2-mdx mice**
Spaulding, Hannah R; Quindry, Tiffany; Quindry, John C; Selsby, Joshua T

78. **The murine anti-myostatin antibody mRK35 induces skeletal muscle hypertrophy in a mouse model of milder spinal muscular atrophy (SMA)**
St. Andre, Michael; Khairallah, Ramzi J; O'Shea, Karen M; Yen, Pei-Fen; Lam, Steven, Rybin, Denis; Howell, Kelly; Chen, Karen S; Ko, Chien-Ping; Owens, Jane

79. **A murine anti-myostatin antibody induces skeletal muscle hypertrophy in B10-mdx mice treated daily with a corticosteroid**
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80. **DMD patient-derived skin fibroblasts are more sensitive to Transforming growth factor-β stimulation compared to healthy donor-derived fibroblast**
Sun, Yumei L; Song, Xiaomin; Li, Xianfeng; Owens, Jane; Girgenrath, Mahasweta

81. **Computational model predicts the role of the microenvironment in muscle regeneration from injury**
Virgilio, Kelley; Martin, Kyle; Peirce, Shayn; Blemker, Silvia

82. **Application of MyoScreen, a New High-Throughput Phenotypic Screening Platform to Increase the Success Rate of Early Muscle Drug Discovery**
Young, Joanne; Duchemin-Pelletier, Eve; Flaender, Mélanie; Lorintiu, Oana; Poydenot, Pauline

Neuromuscular Biology

83. **Syntrophin binds directly to multiple spectrin-like repeats in dystrophin and mediates binding of nNOS to repeats 16-17**
Adams, Marvin; Odom, Guy; Kim, Min Jeong; Chamberlain, Jeffrey; Froehner, Stanley

84. **Cullin-3 is required for normal skeletal muscle development**
Blondelle, Jordan; Tallapaka, Kavya; Shapiro, Paige; Ghassemian, Majid; Singer, Jeffrey D; Lange, Stephen

85. **Soluble Heparin Binding EGF-like Growth Factor regulates skeletal muscle expression of GALGT2 (B4GALNT2) and GALGT2-inducible therapeutic muscle genes**
Cramer, Megan L; Martin, Paul T

86. **Investigation of utrophin and developmental myosin expression in the mdx mouse: Correlation with patient data from a Phase 2 clinical trial of ezutromid**
Guiraud, S; Edwards, B; Squire, S; Moir, L; Berg, A; Babbs, A; Ramadan, N; Elsey, D; Tinsley, J; Harriman, S; Powell, D; **Davies K.E.**

87. **Characterizing the spatial distribution of transcripts in skeletal muscle**
Denes, Lance; Wang, Eric

88. **Selective ribosome biogenesis regulates muscle growth and diseases by modulating protein synthesis**
Bennett, Alexisl; Marchetti, Michael; Spinazzola, Janelle; Kunkel, Louis; **Gupta, Vandana**

89. **Cycles of myofiber degeneration and regeneration lead to remodeling of the neuromuscular junction in two models of Duchenne muscular dystrophy**
Haddix, Seth; Lee, Young il; Kornegay, Joe N; Thompson, Wesley J

90. **Vital Imaging of Myonuclei**
Hastings, Robert Louis; Thompson, Wesley J

91. **Clinicopathologic and genetic characterization of a founder mutation in FKRP**
Jones, Karra A; Lee, Angela J; Butterfield, Russell J; Cox, Mary O; Konersman, Chamindra G; Grosmann, Carla; Abdenur, Jose E; Boyer, Monica; Beson, Brent; Wang, Ching; Dowling, James; Gibbons, Melissa A; Ballard, Alison; Janas, Joanne S; Leshner, Robert T; Bonnemann, Carsten G; Malicki, Denise M; Weiss, Robert B; Moore, Steven A; Mathews, Katherine D

92. **Characterization of the D2/mdx mouse as a clinically relevant model of Duchenne Muscular Dystrophy and demonstration that treatment with mRK35, a murine anti-myostatin antibody, induces functional improvements in D2/mdx and aged Bl10/mdx mice**
St. Andre, Michael; Thottam, John Paul; Beermann, Mary Lou; **Kumar, Ajay**; Girgenrath, Mahasweta; Owens, Jane

93. **RACE-2494, a systemically-acting 'myostatin+' ligand trap, attenuates muscle weakness and alleviates respiratory dysfunction in neuromuscular disorders**
Li, Jia; Cannell, Marishka; Suragani, Rajasekhar NVS; Pearsall, R Scott; Kumar, Ravindra

94. **ACTN3 genotype influences muscle mass regulation and glucocorticoid-induced muscle wasting**
Seto, Jane; Shah, Manan; Meehan, Lyra; Bek, Lucinda; Tiong, Chrystal; Roeszler, Kelly; Quinlan, Kate; Houweling, Peter; North, Kathryn

95. **A Role for Terminal Schwann Cells in Neuromuscular Synaptic Competition and Maturation**
Smith, Ian W; Thompson, Wesley J

96. **The E3 ligase Fbxw7 regulates postnatal skeletal muscle growth and metabolism**
Stec, Michael; Gromova, Anastasia; Richards, Alicia; Jimenez-Morales, David; Krogan, Nevan; Sacco, Alessandra

97. 8 and 16 Weeks of Chronic Tobacco Smoke Exposure Negatively Impacts Morphological Characteristics of Motor Axons and Neuromuscular Junctions in the Diaphragm of Mice
Willms, Alexander; Miguez, Kayla; Leduc-Gaudet, Jean-Phillipe; Baglole, Carolyn; Hussain, Sabah; Hepple, Russell

98. Using CRISPR gene editing coupled to a high-throughput functional assay to improve diagnosis of the collagen-VI related myopathies
Woodman, Keryn G; Lek Angela; Hu, Ying; Donkervoort, Sandra; Bönnemann, Carsten G; Lek, Monkol

Disease Mechanisms

99. Small molecule inhibitors of DUX4 expression rescue FSHD pathophysiology in vitro
Accorsi, Anthony; Rojas, Alejandro; Maglio, Joe; Shen, Ning; Robertson, Alan; Chang, Aaron; Graef, John; Barnes, Ricky; Eyerman, Dave; Rahl, Pete; Ronco, Lucienne; Wallace, Owen; Cacace, Angela

100. High potassium diet reduces skeletal muscle fibrosis and improves strength in dystrophic mdx mice
Acuña, Maria; Salas, Daniela; Diaz, Jessica; Vio, Carlos; Brandan, Enrique

101. KPT-350 ameliorates Duchenne muscular dystrophy symptoms in dystrophic zebrafish and mice
Hightower, Rylie M; Gibbs, Devin E; Lee, Christopher S; Spinazzola, Janelle M; Widrick, Jeffrey J; Tamir, Sharon; Cochran, Shelton; Chang, Hua; Landesman, Yosef; Kunkel, Louis M; **Alexander, Matthew S**

102. A Drosophila model for LGMD1F
Fernandez-Costa, Juan M; Paredes-Martinez, Francisco; **Artero, Ruben**

103. Leucine activates the GSK-3β/β-CATENIN pathway in skeletal muscle under disuse
Bento, Mirella R; Silva, William J; Graca, Flávia A; Ferian, Andrea; Baptista, Igor L; Labeit, Siegfried; Moriscot, Anselmo S

104. CTGF/CCN2 inhibition improves muscle and locomotor function in an ALS mouse model
González, David; Rebolledo, Daniela; van Zundert, Brigitte; **Brandan, Enrique**

105. The Three Little Pigs Regulate Organelle Positioning at the Sarcomere in Skeletal Muscle
Carroll KJ; Anderson DM; Sanchez-Ortiz E; Nelson BR; Makarewich CA; McAnally J; Bassel-Duby R; and Olson EN

106. Localization of the FSHD protein DUX4 and DUX4 mRNA in vivo
Hall, John K; Zavaljevski, Maja; Bisset, Darren R; Tawil, Rabi; Wagner, Kathryn R; Wang, Leo; **Chamberlain, Joel R**

107. The importance of archvillin and gamma-sarcoglycan interaction for normal mechanical signal transduction
Chou, Chih-Hsuan; Vassilakos, Georgios; Puglise, Jason; Matheny, Michael; Luna, Elizabeth; Barton, Elisabeth

108. A low molecular weight compound screen in FSHD patient myotubes identifies modulators of Dux4 activity and novel mechanisms of action
Hupper, Nicole; Wilson, Liz S; Beckwith, Rohan EJ; Glass, David J; Trendelenburg, Anne-Ulrike; **Clarke, Brian A**

109. Protein kinase A activation inhibits DUX4 gene expression in myotubes from patients with facioscapulohumeral muscular dystrophy
Cruz, Joseph M; Hupper, Nicole; Wilson, Liz S; Trendelenburg, Anne-Ulrike; **Clarke, Brian A**

110. Targeting dynamin 2 rescues the three main forms of centronuclear myopathies
Tasfaout, Hichem; Buono, Suzie; Prokic, Ivana; Ross, Jacob; Kretz, Christine; Guo, Shuling; Koebel, Pascal; Monia, Brett; Bitoun, Marc; Ochala, Julien; Laporte, Jocelyn; **Cowling, Belinda**

111. Sialic Acid Therapy and Gene Therapy in a GNE Myopathy Model – Visualizing the Endpoints
Crowe, Kelly E; Martin, Paul T

112. Interaction between myostatin and mTORC1 pathways in skeletal muscle: Implications for thyroid hormone action
Cruz, André; Moriscot, Anselmo Sigari

113. Large in-frame 5' deletions in DMD associated with mild Duchenne muscular dystrophy: two case reports and a novel patient-derived cell model
Gibbs, Elizabeth M; Barthélémy, Florian; Douine, Emilie; Shieh, Perry B; Khanlou, Negar; Crosbie-Watson, Rachelle H; Nelson, Stanley F; Miceli, M Carrie

114. Role of sarcospan in utrophin-based treatment of dystrophic pathology
Gibbs, Elizabeth; Lam, Jessica; Tharani, Tanya; Crosbie-Watson, Rachelle

115. Myofiber mineralocorticoid receptor in acute and chronic muscle injury
Hauck, J. Spencer; Lowe, Jeovanna; Rastogi, Neha; Swager, Sarah; Rafael-Fortney, Jill A

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Haynes, Premi; Bomsztyk, Karol; Miller Daniel G

117. Vamorolone targets dual nuclear receptors to simultaneously treat cardiomyopathy and inflammation in mdx mouse models
Heier, Christopher R; Fiorillo, Alyson A; Tully, Christopher B; Mazala, Davi A; Uaesoontrachoon, Kitipong; Hoffman, Eric P; Nagaraju, Kanneboyina; Spurney, Christopher F

118. MiR-486 is an Epigenetic Regulator of Pathological Progression of Duchenne Muscular Dystrophy
Hightower, Rylie M; Alexander, Matthew S

119. Antibody-enzyme fusions degrade pathogenic glycogen in Lafora disease and demonstrate a novel cell-penetrating platform for treating neurological disorders
Brewer, Kathryn M; Uittenbogaard, Annette; Austin, Grant; McCarthy, John; DePaoli-Roach, Anna; Roach, Peter J; Pauly, James R; McKnight, Tracy; Armstrong, Dustin D; **Hodges, Bradley L**; Gentry, Matthew S

120. Fibroadipogenic progenitors control the onset and severity of Limb Girdle Muscular Dystrophy 2B
Hogarth, Marshall; Defour, Aurelia; Lazarski, Christopher; Gallardo, Eduard; Diaz Manera, Jordi; Partridge, Terence A; Jaiswal, Jyoti K

121. The FLExDUX4 transgenic mouse can be used to develop FSHD-like mouse models with pathophysiology ranging in severity
Jones, Takako; Chew, Guo-Liang; Barraza-Flores, Pamela; Schreier, Spencer; Dagda, Marisela; Wuebbles, Ryan D; Burkin, Dean J; Bradley, Robert K; **Jones, Peter L**

122. Regulation of myoblast fusion by cIAP1: Therapeutic Perspectives
Lala-Tabbert, Neena; Fukano, Marina; Holbrook, Janelle; LaCasse, Eric; Korneluk, Robert

123. Modulation of genetic modifiers in dystrophin-deficient zebrafish as a means of future therapeutics
Lambert, Matthias R; Spinazzola, Janelle M; Gibbs, Devin E; Conner, James R; Kunkel, Louis M

124. Nebulin's C-Terminus is Necessary for Proper Sarcomeric Structure and Function
Li, Frank; Kolb, Justin; Barton, Elisabeth; Granzier, Hendrikus

125. Identification of a novel target for transcriptional modulation of utrophin
Lin, Ling; Sun, Chicheng; Moore, Natalie; Accorsi, Anthony; Qadir, Deena; Fridman, Leticia; Robertson, Alan; Ronco, Lucienne; Barnes, Ricky; Eyerman, David; Rahl, Peter; Wallace, Owen; Cacace, Angela

126. Mineralocorticoid receptor antagonist finerenone improves function and pathology of dystrophic skeletal and cardiac muscles in mice
Lowe, Jeovanna; Haupt, Michael; Peczkowski, Kyra K; Rastogi, Neha; Kadakia, Feni K; Zins, Jonathan G; Smart, Suzanne; Sander, Peter; Kolkhof, Peter; Raman, Subha V; Janssen, Paul M.L; Rafael-Fortney, Jill A

127. Tamoxifen therapy in a murine model of myotubular myopathy (TAM4MTM)
Maani, Nika; Sabha, Nesrin; Rezai Kamran; Ramani, Arun; Groom, Linda; Eltayeb, Nadine; Mavandadnejad, Faranak; Pang, Andrea; Russo, Giulia; Brudno, Michael; Haucke, Volker; Dirksen, Robert; Dowling, James

128. Are there healthy individuals lacking dystrophin? An analysis of over 75,000 sequenced males
Marshall, Jamie L; Wang, Qingbo; Yang, Leon; Cummings, Beryl B; Chiumiento, Marco; Laricchia, Kristen; Armean, Irina M; Weisburd, Ben; Francioli, Laurent C; Karczewski, Konrad J; Zappala, Zachary; and MacArthur, Daniel G

129. Skeletal myosin binding protein-C paralogs are necessary for skeletal muscle function
McNamara, James; Song, Taejong; Sadayappan, Sakthivel

130. Dysregulation of the TGF-beta pathway in collagen VI-related muscular dystrophy predisposes myofibers to injury and interferes with muscle regeneration
Mohassel, Payam; Rooney, Jachinta; Yun, Pomi; Zou, Yaqun; Bonnemann, Carsten G

131. Discovery of MENR, a novel RNA binding protein involved in muscle wasting
Morales, M Gabriela; Cannavino, Jessica; Bassel-Duby, Rhonda; Olson, Eric N

132. Mechanisms of skeletal muscle wasting in a mouse model for myotonic dystrophy
Morriss, Ginny R; Cooper, Thomas A

133. Assessing the pathologic role of DUX4 in a humanized mouse model of FSHD
Mueller, Amber; O Neill, Andea; Lach-Martinez, Anna; Sakellariou, Elvina; Jones, Takako; Jones, Peter; Bloch, Robert

134. Examining Autophagy Dysregulation in Duchenne Muscular Dystrophy Human Stem Cell Derived Skeletal Muscle
Muliono, Alvin; Fong, Lauren K; Bennett, Monica Hayhurst; Schmidt, Uli

135. Oral administration of erythromycin decreases RNA toxicity in myotonic dystrophy type 1
Nakamori, M; Taylor, K; Sobczak, K

136. Eccentric contraction causes loss of microtubule lattice organization in mdx skeletal muscle expressing mini- or micro-dystrophin
Nelson, D'anna M; Lindsay, Angus; Lowe, Dawn A; Ervasti, James M

137. Loss of the α7 integrin causes cardiac arrhythmia in patients and a mouse model of α7 integrin-related congenital muscular dystrophy
Nunes, Andreia M; Fontelonga, Tatiana M; Bugiardini, Enrico; Phadke, Rahul; Pittman, Alan M; Morrow, Jasper; Parton, Matt; Houlden, Henry, G; Hanna, Michael; Maas, Roderick; Raaphorst, Joost; Küsters, Beeno; Matthews, Emma; Burkin, Dean J

138. Mapping D4Z4-driven expression profile and dynamics in FSHD
Panamarova, Maryna; Tassin, Alexandra; Belayew, Alexandra; Zammit, Peter S

139. Intermittent prednisone promotes favorable metabolic reprogramming of dystrophic muscle and limits steroid-mediated side effects
Quattrocelli, Mattia; Zelikovich, Aaron S; Bien Peek, Clara; Salamone Isabella M; Demonbreun, Alexis R; Kuntz, Nancy L; Barish, Grant; Bass, Joseph; McNally, Elizabeth M

140. Early denervation-induced fibrosis is modulated by CTGF but not for TGF-β : role of HIF-1α and LPA
Rebolledo, Daniela; Córdova, Adriana; Valle, Roger; Contreras, Osvaldo; Murphy-Ullrich, Joanne; Walkinshaw, Gail; Lipson, Ken; Brandan, Enrique

141. Effect of serotonin modulation on dystrophin-deficient zebrafish
Spinazzola, Janelle M; Lambert, Matthias R; Gibbs, Devin E; Conner, James R; Krikorian, Georgia L; Pareek, Prithu; Rago, Carlo; Kunkel, Louis M

142. Large-scale testing of effective chemical therapies to treat nemaline myopathy
Sztal, Tamar E; McKaige, Emily A; Briers, Bronwyn; Williams, Caitlin; Oorschot, Viola; Ramm, Georg; Bryson-Richardson, Robert J

143. Thrombospondin-1-Mediated TGFβ Signaling In Myofibers Causes Muscle Atrophy Via The PERK-eIF2α-ATF4 ER-Stress Pathway
Vanhoutte, Davy; Schips, Tobias; Brody, Matthew J; Sargent, Michelle A; Molkentin, Jeffrey D

144. Haploinsufficiency of Dock3 improves Duchenne muscular dystrophy (DMD) pathologies
Wang, Yimin; Hightower, Rylie; Millican, Reid; Ballestas, Mary; Spinazolla, Janelle; Widrick, Jeffrey; Alexander, Matthew

145. Skeletal Muscle Contraction Alters the Microtubule Network Which Impacts Function
Ward, Christopher; Venegas, Camilo; Khairallah, Ramzi; Shi, Guoli; Williams, Katrina; Joca, Humberto; Stains, Joseph

146. Auto-antibodies targeting components of sarcolemma repair represent a pathogenic mechanism in idiopathic immune myopathies
McElhanon, Kevin E; Capati, Ana; Young, Nicholas; Hampton, Jeffrey; Beck, Eric X; Sahenk, Zarife; Aggarwal, Rohit; Oddis, Chester V; Jarjour, Wael N; **Weisleder, Noah**

147. Simvastatin provides long-term enhancement of dystrophic diaphragm and cardiac function as evaluated by in vivo ultrasonography
Whitehead, Nicholas; Bible, Kenneth; Kim, Min Jeong; Froehner, Stanley

148. The muscle-specific ubiquitin ligase MuRF1 regulates autophagy in vivo via FOXO1/3 ubiquitination to inhibit NF-kB signaling and protect against cardiac inflammation in vivo
Parry, Traci L; **Willis, Monte S**

149. Characterization and Potential Signaling of Dystrophic Muscle Released Exosomes
Wilson, Brittany E; Munoz, Eric R; Pautz, Carina M; Selsby, Joshua T; Hudson, Matthew B

150. AAV1 mediated expression of the Striated Muscle Activator of Rho Signalling (STARS) increases muscle force production in the muscle of the mdx mouse model for Duchenne Muscular Dystrophy
Sadler, Kate; Della Gatta, Paul; Russell, Aaron

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The Natchez, a sternwheel steamboat, is the iconic symbol of the city of New Orleans. With her water moving paddlewheel, 32 note steam calliope playing music from the gilded era, 1925-built steam engines, and the whistle blowing sound of the 1860's, passengers aboard the Natchez experience a journey to the past.

Duchenne muscular dystrophy is a progressive, degenerative muscle-wasting condition that affects 1: 300,000 boys worldwide. Often, families have never heard the word Duchenne prior to a diagnosis. Duchenne is not represented by an icon.

Did you know?
The Natchez sits at 265 feet long x 46 feet wide and weighs 1,384 tons.

Did you know?
The red paddlewheel is 25 feet in diameter and weighs over 26 tons.

Did you know?
The Natchez was built by William T. Bergeron in 1975.

Did you know?
William Cook, only grandson of William T. Bergeron, was an honorary captain of the vessel in 2004.

Did you know?
William Cook, currently 18 years old, was diagnosed with Duchenne muscular dystrophy in 2007.

No trip to New Orleans is complete without a cruise on the iconic Natchez along the mighty Mississippi.

In honor of her builder and his grandson, allow the Natchez and all her power to serve as an icon of Duchenne muscular dystrophy. Allow her majestic beauty to represent *hope*.

Together, we will CURE DUCHENNE!

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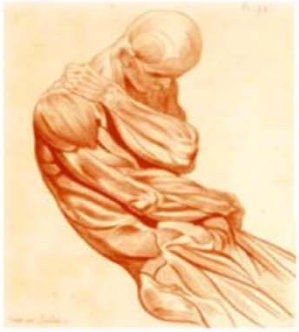

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