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:
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Objective 1: The presentation and sharing of unpublished data. This meeting emphasizes the presentation of unpublished work.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

Program Committee
Anнемieke 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 Micheie, University of Michigan
Louise Rodino-Klapac, Nationwide Children’s Hospital

Coordinators
Christa Stout, University of Florida
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.
# Monday, June 25, 2018

## Industry Workshop, Grand Ballroom, 12th floor
(all registered conference attendees are welcomed and encouraged to attend)

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
</tr>
</thead>
<tbody>
<tr>
<td>12:00 – 12:10</td>
<td><strong>Welcome and Introductions</strong></td>
</tr>
</tbody>
</table>
| 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*

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**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

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**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; Hs, 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; Mansuetu, 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
  Kyrchenko, 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; Kyrchenko, Viktoria; 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, Jayoti; 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 Megacnial 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; Tuffer-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; Claeyts, 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

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   Silvestre, João G; Baptista, Igor L; Labeit, Siegfried; Moriscot, Anselmo S
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   Smith, Lucas R; Irianto, Jerome; Discher, Dennis E

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   Stearns-Reider, Kristen; Hammond, Katherine; Hicks, Michael; Chin, Jesse; McCarthy, Siobhan;
   Maity, Alok; Wollman, Roy; Hansen, Kirk; Pyle, April; Crosbie-Watson, Rachelle

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cells and is upregulated by PPAR-delta
   Valentine, William; Tokuoka, Suzumi; Hishikawa, Daisuke; Kita, Yoshihiro; Shindou, Hideo; and
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**Clinical Trials in NMD**

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    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,
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    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

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    Dystrophy
    Hart, Cora; Hammers, David; Matheny, Michael; Wakefield, Zachary; Kang, Hansol; Sweeney, Lee

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    Komaki, Hirofumi; Takeshima, Yasuhiro; Matsumura, Tsuyoshi; Ozasa, Shiro; Funato, Michinori;
    Egawa, Yoichi; Takeda, Shin’ichi

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    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

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    effect in trials in Duchenne muscular dystrophy
    Moon, Jay; Souza, Marcio; Elfring, Gary L; McIntosh, Joseph; Peltz, Stuart W

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    Frank, Diane E; Mercuri, Eugenio; Servais, Laurent; Straub, Volker; Morgan, Jennifer; Domingos,
    Joana; Schnell, Frederick; Dickson, George; Popplewell, Linda; Seferian, Andreea; Monforte, Mauro;
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Baumann, Cory; Warren, Gordon; Lowe, Dawn

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Bukovec, K; Hu, X; Virgilio, K; Jones, B; Peirce-Cottler, S; Blemker, S; Grange, RW

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Cannavino, Jessica; Morales, M Gabriela; Bassel-Duby, Rhonda; Olson, Eric N

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Kok, Hui Jean; Smith, Lucas; Barton, Elisabeth

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Lawal, TA; Todd, JJ; Chrismer IC; Witherspoon JW; Cosgrove MM; Allen C; Jain MS; Waite M; Emile-Backer M; Bönnemann CG; Meilleur KG

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Lindsay, Angus; McCourt, Preston M; Lowe, Dawn A; Ervasti, James M

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Manoharan, Palanikumar; Song, Taejong; Radzyukevich, Tatiana L; Sadayappan, Sakthivel; Lingrel, Jerry B; Heiny, Judith A

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Massopust, Ryan; Thompson, Wesley

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Giannakis, N; Sansbury, B.E; Patsalos, A; Hays, T; Han, X; Spite, M; Nagy, L

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Parry, Traci L; Huang, Wei; Beak, Ju Youn; Jensen, Brian C; Willis, Monte S

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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
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   Harris-Love, Michael; Pennington, Donte; Avila, Nilo; Adams, Bernadette; Ismail, Catheeka; Zaidi, Syed; Kassner, Courtney; Liu, Frank; Kondapaneni, Saritha; Conner, Anthony; Blackman, Marc

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   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

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   Smith, Christina R; Barraza-Flores, Pamela; Herrmann, Hailey; Burkin, Dean J

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   Spaulding, Hannah R; Quindry, Tiffany; Quindry, John C; Selsby, Joshua T

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   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

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   Sun, Yumei L; Song, Xiaomin; Li, Xianfeng; Owens, Jane; Girgenrath, Mahasweta

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   Virgilio, Kelley; Martin, Kyle; Peirce, Shayn; Blemker, Silvia

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   Young, Joanne; Duchemin-Pelletier, Eve; Flaender, Mélanie; Lorintiu, Oana; Poydenot, Pauline

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   Adams, Marvin; Odom, Guy; Kim, Min Jeong; Chamberlain, Jeffrey; Froehner, Stanley

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   Blondelle, Jordan; Tallapaka, Kavya; Shapiro, Paige; Ghasseman, Majid; Singer, Jeffrey D; Lange, Stephen

**Neuromuscular Biology**
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Cramer, Megan L; Martin, Paul T

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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.

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Denes, Lance; Wang, Eric

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Bennett, Alexisl; Marchetti, Michael; Spinazzola, Janelle; Kunkel, Louis; Gupta, Vandana

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Haddix, Seth; Lee, Young il; Kornegay, Joe N; Thompson, Wesley J

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Hastings, Robert Louis; Thompson, Wesley J

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Jones, Karra A; Lee, Angela J; Butterfield, Russell J; Cox, Mary O; Konersman, Chamintra 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

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Li, Jia; Cannell, Marishka; Suragani, Rajasekhar NVS; Pearsall, R Scott; Kumar, Ravindra

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Seto, Jane; Shah, Manan; Meehan, Lyra; Bek, Lucinda; Tiong, Chrystal; Roeszler, Kelly; Quinlan, Kate; Houweling, Peter; North, Kathryn

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Smith, Ian W; Thompson, Wesley J

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Stec, Michael; Gromova, Anastasia; Richards, Alicia; Jimenez-Morales, David; Krogan, Nevan; Sacco, Alessandra
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Willms, Alexander; Miguez, Kayla; Leduc-Gaudet, Jean-Phillipe; Baglole, Carolyn; Hussain, Sabah; Hepple, Russell

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Woodman, Keryn G; Lek Angela; Hu, Ying; Donkervoort, Sandra; Bönnemann, Carsten G; Lek, Monkol

**Disease Mechanisms**

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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

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Acuña, Maria; Salas, Daniela; Diaz, Jessica; Vio, Carlos; Brandan, Enrique

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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

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Fernandez-Costa, Juan M; Paredes-Martinez, Francisco; Artero, Ruben

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Bento, Mirella R; Silva, William J; Graca, Flávia A; Ferian, Andrea; Baptista, Igor L; Labeit, Siegfried; Moriscot, Anselmo S

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González, David; Rebolledo, Daniela; van Zundert, Brigitte; Brandan, Enrique

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Carroll KJ; Anderson DM; Sanchez-Ortiz E; Nelson BR; Makarewich CA; McAnally J; Bassel-Duby R; and Olson EN

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Hall, John K; Zavaljevski, Maja; Bisset, Darren R; Tawil, Rabi; Wagner, Kathryn R; Wang, Leo; Chamberlain, Joel R

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Chou, Chih-Hsuan; Vassilakos, Georgios; Puglise, Jason; Matheny, Michael; Luna, Elizabeth; Barton, Elisabeth
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

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Tasfaout, Hichem; Buono, Suzie; Prokic, Ivana; Ross, Jacob; Kretz, Christine; Guo, Shuling; Koebel, Pascal; Monia, Brett; Bitoun, Marc; Ochala, Julien; Laporte, Jocelyn; Cowling, Belinda

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Crowe, Kelly E; Martin, Paul T

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Cruz, André; Moriscot, Anselmo Sigari

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Gibbs, Elizabeth M; Barthélémy, Florian; Douine, Emilie; Shieh, Perry B; Khanlou, Negar; Crosbie-Watson, Rachelle H; Nelson, Stanley F; Miceli, M Carrie

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Gibbs, Elizabeth; Lam, Jessica; Tharani, Tanya; Crosbie-Watson, Rachelle

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Hauck, J. Spencer; Lowe, Jeovanna; Rastogi, Neha; Swager, Sarah; Rafael-Fortney, Jill A

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Haynes, Premi; Bomsztyk, Karol; Miller Daniel G

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Heier, Christopher R; Fiorillo, Alyson A; Tully, Christopher B; Mazala, Davi A; Uaesoontrachoon, Kitipong; Hoffman, Eric P; Nagaraju, Kanneboyina; Spurney, Christopher F

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Hightower, Rylie M; Alexander, Matthew S

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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
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Hogarth, Marshall; Defour, Aurelia; Lazarski, Christopher; Gallardo, Eduard; Diaz Manera, Jordi; Partridge, Terence A; Jaiswal, Jyoti K

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Jones, Takako; Chew, Guo-Liang; Barraza-Flores, Pamela; Schreier, Spencer; Dagda, Marisela; Wuebbles, Ryan D; Burkin, Dean J; Bradley, Robert K; Jones, Peter L

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Lala-Tabbert, Neena; Fukano, Marina; Holbrook, Janelle; LaCasse, Eric; Korneluk, Robert

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Lambert, Matthias R; Spinazzola, Janelle M; Gibbs, Devin E; Conner, James R; Kunkel, Louis M

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Li, Frank; Kolb, Justin; Barton, Elisabeth; Granzier, Hendrikus

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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

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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

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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

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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

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McNamara, James; Song, Taejong; Sadayappan, Sakthivel

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Mohassel, Payam; Rooney, Jachinta; Yun, Pomi; Zou, Yaqun; Bonnemann, Carsten G

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Morales, M Gabriela; Cannavino, Jessica; Bassel-Duby, Rhonda; Olson, Eric N
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   Morriss, Ginny R; Cooper, Thomas A

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   Mueller, Amber; O Neill, Andea; Lach-Martinez, Anna; Sakellariou, Elvina; Jones, Takako; Jones, Peter; Bloch, Robert

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   Muliono, Alvin; Fong, Lauren K; Bennett, Monica Hayhurst; Schmidt, Uli

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   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

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   Panamarova, Maryna; Tassin, Alexandra; Belayew, Alexandra; Zammit, Peter S

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   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; Brandon, 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

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   Vanhoutte, Davy; Schips, Tobias; Brody, Matthew J; Sargent, Michelle A; Molkentin, Jeffrey D
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Wang, Yimin; Hightower, Rylie; Millican, Reid; Ballestas, Mary; Spinazolla, Janelle; Widrick, Jeffrey; Alexander, Matthew

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Ward, Christopher; Venegas, Camilo; Khairallah, Ramzi; Shi, Guoli; Williams, Katrina; Joca, Humberto; Stains, Joseph

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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

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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
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!
Please join us for the 2019 Advances in Skeletal Muscle Biology in Health and Disease Conference. This conference is well suited for informal interactions and the presentation of data that might be too preliminary for a larger audience. Overall, the goal is to facilitate advances in skeletal muscle biology through discussions that promote new ideas, research lines, and collaborations.

Join us March 6-8, 2019 at the University of Florida in Gainesville, FL

Grant Writing Workshop and Poster Sessions

Grant Writing Workshop: The meeting will include a grant writing workshop oriented toward trainees and new investigators with focus on NIH grants.
Posters: There will be two poster sessions and posters will be on display during the entire conference.

To learn more about the Myology Institute and the conference, please visit our website, http://myology.institute.ufl.edu/