



# 2022 New Directions in Biology and Disease of Skeletal Muscle Conference

June 20-23, 2022; Ft. Lauderdale, FL

**Monday, June 20, 2022;** Las Olas Ballroom

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

- 11:30 – 11:40AM**      **Welcome and Introductions**
- 11:40 – 12:10**      **David Brown**, Stealth BioTherapeutics  
*Targeting mitochondria in DMD: insights from the mitochondria-targeting peptide elamipretide*
- 12:10 – 12:40**      **Joanne Donovan**, Edgewise Therapeutics  
*Taking a New Approach to Protect Muscle in Duchenne and Becker muscular dystrophy: EDG-5506*
- 12:40 – 1:10**      **Anthony Accorsi**, Fulcrum Therapeutics  
*Rebalancing Repair and Replacement in Dystrophic Muscle: a New Therapeutic Horizon*
- 1:10 – 1:40**      **Scott Turner**, Pliant Therapeutics  
*Increased Laminin Binding Through Integrin Activation Protects Dystrophic Muscle*
- 1:40 – 2:10**      **Hanhua Huang**, Avidity Biosciences  
*Engineering Antibody Oligonucleotide Conjugates (AOCs): Taking Receptor-Mediated Uptake One Step Further*
- 2:10 – 2:30PM**      **COFFEE BREAK**
- 2:30 – 3:00**      **Stefano Zanotti**, Dyne Therapeutics  
*FORCE™ platform for targeted delivery of oligonucleotide therapeutics for muscle diseases*
- 3:00 – 3:30**      **Olivier Danos**, RegenxBio  
*RGX-202, an AAV-microdystrophin gene therapy for Duchenne Muscular Dystrophy*
- 3:30 – 4:00**      **Pat Gonzalez**, Solid Biosciences  
*Microdystrophin Gene Therapy for the Treatment of Duchenne Muscular Dystrophy*
- 4:00 – 4:30**      **Angela Kodsi**, Pfizer  
*Program update on Pfizer's fordadistrogene movaparvovec, an investigational therapy for DMD*
- 4:30 – 5:00**      **Teji Singh**, Sarepta Therapeutics  
*Exploring New Horizons in the Care of Individuals with Duchenne Muscular Dystrophy: Clinical Update on an Investigational rAAVrh74-based Gene Transfer Therapy*
- 5:15 – 6:15PM**      **Keynote Speaker- Jared Rutter, University of Utah**  
*Mitochondria, metabolism and cellular decisions*
- 6:30 – 8:30PM**      **Evening Welcome Reception; Sky Terrace (outdoors)**

# Tuesday, June 21, 2022

7:30AM Breakfast; Las Olas Foyer

## Session I: Extracellular Matrix Receptors and Skeletal Muscle Function

Chair: Rachelle Crosbie

8:00 – 8:20AM **Soumya Joseph**, University of Iowa  
*Cryo-EM structure of LARGE1 and implications for matriglycan polymerization*

8:20 – 8:40 **Wendy Gordon**, University of Minnesota  
*Effects of breaking the DGC on tension sensing and mechanotransduction*

8:40 – 9:00 **Erika Geisbrecht**, Kansas State University  
*Fine-tuning of adhesive functionality at the muscle-ECM interface*

9:00 – 9:20 **Judith R. Reinhard**, University of Basel  
*Therapeutic effect of linker protein-mediated gene therapy in a mouse model for LAMA2-related muscular dystrophy*

9:20 – 9:40 **Joseph C. Reynolds**, University of California, Los Angeles  
*The extracellular matrix environment from dystrophin-deficient muscle contributes to cell membrane instability in Duchenne muscular dystrophy*

9:40 – 10:10AM COFFEE BREAK

## Session II: Exploring Dystrophin-associated Signaling Networks

Chair: Rachelle Crosbie

10:10 – 10:30AM **Shenhav Cohen**, Israel Institute of Technology  
*Insulin receptor turnover in fasting is dependent on NAGLU-mediated  $\beta$ -dystroglycan deglycosylation*

10:30 – 10:50 **Marco Mangone**, Arizona State University  
*Transcriptome changes during the initiation and progression of Duchenne Muscular Dystrophy in *C. elegans**

10:50 – 11:10 **Matthew Wood**, Oxford University  
*Dystrophin restoration therapies and restoration of dystrophin function*

11:10 – 11:30 **Mourkioti Foteini**, University of Pennsylvania  
*Mechanosensing regulation of dystrophic muscle stem cells*

11:30 – 11:50 **Swathy Krishna**, Iowa State University  
*Phosphoproteomics and reconstructed kinase signaling networks reveal unique consequences of diet-induced insulin resistance in dystrophic muscles*

11:50AM – 2:00PM LUNCH BREAK (on your own)

2:00PM

## Abstract Presentation Session I-Data Blitz; Las Olas Ballroom

Pre and Post-Doctoral Trainees; 5 minute presentations

Chair: Rachelle Crosbie

2:00 – 2:05

**Michael Anderson**, University of Minnesota

*Dystroglycan proteolysis is conformationally-regulated and disrupted by mutations in musculoskeletal diseases*

2:06 – 2:11

**Jean Kok**, University of Florida

*Satellite cells provide a critical source of IGF-I during skeletal muscle growth and regeneration*

2:12 – 2:17

**Emanuela Pannia**, The Hospital for Sick Children

*Skeletal muscle ablation of *Pik3c2b* improves body weight regulation and enhances muscle oxidative capacity*

2:18 – 2:23

**Phillip Witcher**, Cincinnati Children's Hospital Medical Center

*Investigating the role of the muscle fusogens in normal and dystrophic myofibers*

2:24 – 2:29

**Brian Lin**, Johns Hopkins University

*TRPC6 inhibition normalizes inflammatory spatial transcriptomic signatures and eccentric remodeling in dystrophic cardiomyopathy*

2:30 – 2:35

**Andrea Armani**, Whitehead Institute for Biomedical Research

*Novel insights into lysosomal signaling in muscle wasting*

2:36 – 2:41

**Taryn Loomis**, University of California, Davis

*Extracellular Matrix Stiffness and Architecture Drive Fibro-Adipogenic Progenitors' Activation into Myofibroblasts*

2:42 – 2:47

**Danesh Sopariwala**, University of Texas Health Science Center at Houston

*Estrogen-related receptors alpha and gamma are indispensable for murine skeletal muscle contractile function and exercise tolerance*

2:48 – 2:53

**Curtis Nutter**, University of Florida

*Humanized DMPK CTG expansion knockin models for myotonic dystrophy type 1*

2:54 – 2:59

**Eshwar R. Tammineni**, Rush University Medical Center

*A junctophilin1 fragment cleaved by calpain counters the hyperglycemia and 2 diabetes - promoting effects of calcium stress in muscle*

3:00 – 3:05

**Maria Paz Ramirez**, University of Minnesota

*Phosphorylation Modulates the Mechanical Properties of a Utrophin N-terminal Fragment*

3:06 – 3:30PM

**BREAK**

## Session III: **Cardiomyopathies Associated with Dystrophy**

Chair: Beth McNally

3:30 – 3:50PM

**Beth McNally**, Northwestern University  
*Models for evaluating dystrophic cardiomyopathy*

3:50 – 4:10

**Jill Rafael-Fortney**, Ohio State University  
*Mineralocorticoid Receptor Signaling in DMD*

4:10 – 4:30

**Jim Martin**, Baylor College of Medicine  
*Hippo signaling in heart regeneration*

4:30 – 4:50

**Tara Tassin**, UT Southwestern Medical Center  
*Altered cardiac metabolism in Duchenne muscular dystrophy-associated cardiomyopathy*

4:50 – 5:10PM

**Christopher Heier**, Children's National Hospital  
*The glucocorticoid receptor acts locally in heart and skeletal muscle to protect against dystrophy and drive drug efficacy*

## Wednesday, June 22, 2022

7:30AM

Breakfast; Las Olas Foyer

## Session IV: **Circadian Rhythms and Muscle Function**

Chair: Mattia Quattrocchi

8:00 – 8:20AM

**Karyn Esser**, University of Florida  
*Circadian clocks, MyoD1 and sarcomere homeostasis*

8:20 – 8:40

**Katja Lamia**, Scripps  
*Daily running enhances molecular and physiological circadian rhythms in skeletal muscle*

8:40 – 9:00

**Ke Ma**, City of Hope  
*Circadian clock as a therapeutic target for muscle diseases*

9:00 – 9:20

**Mattia Quattrocchi**, Cincinnati Children's  
*Chronopharmacology of muscle metabolism*

9:20 – 9:40

**Matthew Alexander**, University of Alabama at Birmingham  
*Chronodisruption in Duchenne muscular dystrophy mice and patients*

9:40 – 10:10AM

COFFEE BREAK

## Session V: **Mitochondria and Muscle Disease: Targets and Therapies**

Chair: Anne Murphy

10:10 – 10:30AM

**Anu Suomalainen**, University of Helsinki  
*Niacin cures NAD-deficiency in mitochondrial myopathy*

- 10:30 – 10:50**      **Hakan Westerblad**, Karolinska Institutet  
*Mitochondrial Ca<sup>2+</sup> uptake and muscle weakness in mitochondrial myopathies*
- 10:50 – 11:10**      **Rosario Rizzuto**, University of Padua  
*The Mitochondrial Calcium Uniporter in muscle trophism and regeneration*
- 11:10 – 11:25**      **Joan Serrano**, The Ohio State University  
*Muscle-expressed sweet taste receptors regulate mitochondrial function and muscle fitness in mice and humans*
- 11:25 – 11:40**      **Fabio Arturo Iannotti**, Institute of Biomolecular Chemistry  
*Gut microbiota dysbiosis participates in the pathogenesis of Duchenne muscular dystrophy through dysregulation of the endocannabinoid system*
- 11:40 – 11:55**      **Tanja Taivassalo**, University of Florida  
*Impact and interplay of corticosteroid regimen and exercise training in boys with DMD: preliminary findings*
- 11:55AM – 2:00PM**      **LUNCH BREAK (on your own)**
- 2:00 – 3:15PM**      **Poster Session I; Atlantic Ballroom**
- 3:15 – 3:30PM**      **BREAK; Transition back to Las Olas Ballroom**
- Session VI: Imaging Muscle Diseases: Structure, Function, and Metabolism; Las Olas Ballroom**  
Chair: Glenn Walter
- 3:30 – 3:50PM**      **Glenn Walter**, University of Florida  
*Quantitative magnetic resonance in the age of therapeutic development for muscle diseases*
- 3:50 – 4:10**      **Carsten Bönnemann**, NIH  
*Diagnostic muscle imaging in the context of next generation genetics*
- 4:10 – 4:30**      **Silvia Blemker**, University of Virginia  
*Integration of imaging and multi-scale computer modeling to explore muscle structure, function, and disease.*
- 4:30 – 4:50**      **Jeanine Prompers**, UMC Utrecht  
*Metabolic imaging using magnetic resonance spectroscopy – Applications in heart and skeletal muscle*
- 4:50 – 5:05**      **Sean Forbes**, University of Florida  
*MRI/MRS assessment of micro-dystrophin gene transfer in skeletal muscle of mdx mice*
- 5:05 – 5:15**      **Jeroen Jeneson**, University Medical Center Utrecht  
*Magnetic Resonance reveals mitochondrial dysfunction and muscle remodeling in Spinal Muscular Atrophy*

# Thursday, June 23, 2022

7:30 – 9:45AM **Poster Session II; Atlantic Ballroom (Breakfast Included)**

9:45 – 10:00AM **BREAK; Transition back to Las Olas Ballroom**

## **Session VII: Gene Therapy for the Muscular Dystrophies; Las Olas Ballroom**

Chair: Lee Sweeney

- 10:00 – 10:20AM **Lee Sweeney**, University of Florida  
*Potential benefits and limitations associated with high doses of AAV.micro-dystrophin gene therapy for Duchenne muscular dystrophy*
- 10:20 – 10:40 **Barry Byrne**, University of Florida  
*Immune Response to Systemic Use of AAV Gene Therapy*
- 10:40 – 10:55 **Dan Levy**, Pfizer Inc.  
*Cardiac safety assessments in the Phase 1b trial of fordadistrogene movaparvovec (PF-06939926) for Duchenne muscular dystrophy (DMD)*
- 10:55 – 11:10 **Quentin Giraud**, Université de Strasbourg  
*MTM1 overexpression efficiently rescues BIN1-related centronuclear myopathy*
- 11:10 – 11:25 **Liubov Gushchina**, Nationwide Children's Hospital  
*rAAV-mediated TCAP Gene Replacement Therapy: A Promising Therapeutic Approach for LGMDR7*
- 11:25 – 11:40 **Ricardo Galli**, Amsterdam University Medical Center  
*Nemaline myopathy type 6: from pathology to therapeutics.*
- 11:40 – 11:50 **Closing Remarks**
- 12:00PM **Adjourn**

**Thank you for your participation in our program, we look forward to seeing you at New Directions 2024!**

