



# 2024 New Directions in Biology and Disease of Skeletal Muscle Conference

June 23-26, 2024; Ft. Lauderdale, FL

**Sunday, June 23, 2024;** Las Olas Ballroom

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

Moderator: Carl Morris, Somite Therapeutics / PHDL Consulting

- 12:00 – 12:10PM**      **Welcome and Introductions**
- 12:10 – 12:40**      **Husam Younis, Avidity Bioscience**  
*Therapeutic Promise of Antibody-Oligonucleotide Conjugates for Rare Neuromuscular Disease*
- 12:40 – 1:10**      **Stefano Zanotti, Dyne Therapeutics**  
*FORCE™ platform for the development of targeted therapeutics for rare muscle diseases*
- 1:10 – 1:40**      **Jeff Jacobs, Fulcrum Therapeutics**  
*Losmapimod, a p38 Small Molecule Inhibitor, Selectively Inhibits the DUX4 Program Without Negatively Impacting Myogenesis in FSHD*
- 1:40 – 2:10**      **David A. Brown, Stealth BioTherapeutics**  
*Targeting mitochondria in myopathies: translational insights and emerging approaches*
- 2:10 – 2:40PM**      **COFFEE BREAK**
- 2:40 – 3:10**      **Andre Mueller-York, Sarepta Therapeutics**  
*Precision Genetic Medicines for LGMDs: Targeting the Root Cause of Disease*
- 3:10 – 3:40**      **Joanne Donovan, Edgewise Therapeutics**  
*A New Approach to Protecting Muscle: Targeting Fast Myosin in Duchenne and Becker Muscular Dystrophy*
- 3:40 – 4:10**      **Ye Liu, RegenxBio**  
*Nonclinical and clinical updates on RGX-202, an AAV8 vector encoding a novel microdystrophin for the treatment of Duchenne Muscular Dystrophy*
- 4:10 – 4:40**      **Jessica Boehler, Solid Biosciences**  
*SGT-003, a Next Generation Microdystrophin Gene Therapy for Treatment of Duchenne Muscular Dystrophy*
- 4:50 – 5:50PM**      **Kay Davies, University of Oxford**  
*The Role of Utrophin in the Therapy of Duchenne Muscular Dystrophy*
- 6:15 – 8:15PM**      **Evening Welcome Reception; Sky Terrace (outdoors)**

# Monday, June 24, 2024

7:30AM Breakfast; Las Olas Foyer

## **Session I: Gene Therapy for Muscle Disease- Immune Response**

Chair: Melissa Spencer, University of California, Los Angeles

8:00 – 8:20AM **Melissa Spencer, University of California, Los Angeles**  
*Mechanisms and impact of immune responses to AAV-based therapies*

8:20 – 8:40 **Juliette Hordeaux, University of Pennsylvania**  
*Cardiovascular toxicities of rAAV in nonhuman primate models*

8:40 – 9:00 **Carmen Leon Astudillo, University of Florida**  
*Thrombotic microangiopathy and systemic AAV: The role of the complement system*

9:00 – 9:20 **Alyson Fiorillo, Virginia Commonwealth University**  
*Reduction of dystrophin-targeting microRNAs increases expression of clinically relevant dystrophin isoforms*

9:20 – 9:40 **Isabelle Marty, University Grenoble Alpes – Inserm**  
*Functional benefit of CRISPR/Cas9-induced deletion for RYR1 dominant mutation*

9:40 – 10:10AM COFFEE BREAK

## **Session II: Muscle Repair, Recovery, and Regeneration— a view from the muscle**

Chair: Matthew Alexander, University of Alabama at Birmingham

10:10 – 10:30AM **Matthew Alexander, University of Alabama at Birmingham**  
*Exploring new roles for dystrophin using a novel conditional knockout mouse*

10:30 – 10:50 **Jason Doles, Indiana University**  
*Impaired myogenesis in GNE myopathy*

10:50 – 11:10 **Radbod Darabi, University of Houston**  
*Patient-Derived Human iPSCs: Advancing Research and Treatments for Skeletal Muscle Disorders*

11:10 – 11:30 **Olivier Pourquie, Harvard University**  
*Making human skeletal muscle in vitro for cell therapy of muscular dystrophies*

11:30 – 11:50 **Elisabeth Barton, University of Florida**  
*Cell-cell communication in the face of muscle damage*

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

1:00-1:45PM

## Career Opportunities in Industry; Las Olas Ballroom

Pre and Post-Doctoral Trainees Only

Moderators: Rachelle Crosbie, University of California, Los Angeles and  
Andrew Judge, University of Florida

Panelists:

**Carl Morris**, Somite Therapeutics; PHDL Consulting

**Ben Barthel**, Edgewise Therapeutics

**Stephen Fasul**, Sarepta Therapeutics

2:00PM

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

Pre- and Post-Doctoral Trainees; 5-minute presentations

Chair: Andrew Judge, University of Florida

2:00 – 2:05

**Alberto Benetollo**, University of Padova

*The CFTR Corrector C17 improved skeletal muscle function in a LGMDR3 model: pharmacological profile and efficacy studies in mouse*

2:06 – 2:11

**Benjamin Clock**, University of California Irvine

*MEGF10 Supports Muscle Stem Cell Retention and Repopulation*

2:12 – 2:17

**Jeremy Ducharme**, University of Florida

*Pancreatic cancer disrupts the skeletal muscle molecular clock in a FoxP1-dependent manner*

2:18 – 2:23

**Helen Rueckert**, Duke University

*Heterogeneity of non-myogenic mesenchymal cells (NMMCs) in rotator cuff muscles and their roles in the pathology of massive rotator cuff tears*

2:24 – 2:29

**Christopher Gilbert**, The Ohio State University

*YTHDF2 governs muscle size through a targeted modulation of proteostasis*

2:30 – 2:35

**Yangyi Luo**, University of Florida

*Contribution of Insulin-Like Growth Factor-I secreted from Fibro-Adipogenic Progenitors to Skeletal Muscle Regeneration and Cellular Dynamics*

*FEBS Certificate Presentation, Laszlo Nagy*

## Session III: New Delivery Vectors and Cargo Modalities for Muscle Gene Therapy

Chair: Douglas Millay, Cincinnati Children's Hospital

2:40 – 3:00PM

**Douglas Millay**, Cincinnati Children's Hospital

*Hijacking the muscle fusogens on therapeutic delivery vehicles*

3:00 – 3:20

**Akitsu Hotta**, Kyoto University

*LNP-based delivery for repeatable and sustainable genome editing therapy*

- 3:20 – 3:40**      **Rita Perlingeiro, University of Minnesota**  
*Path from bench-to bedside: iPS cell-based therapy for muscular dystrophies*
- 3:40 – 4:00**      **Mark St. Pierre, Keros Therapeutics**  
*RKER-065 enhanced the therapeutic effect of phosphorodiamidate morpholino oligomer in a Duchenne muscular dystrophy mouse model.*
- 4:00 – 4:20**      **Brian Lin, Medical College of Wisconsin**  
*TRPC6 inhibition in DMD attenuates the inflammatory immune response elicited by lipid nanoparticle delivery system*
- 4:30 – 6:00PM**      **Poster Session I; Atlantic Ballroom**

## Tuesday, June 25, 2024

**7:30AM**      **Breakfast; Las Olas Foyer**

### **Session IV: Circulating and Tissue Resident Mononuclear Cells in Muscle disease, Injury, and Repair**

Chair: Laszlo Nagy, Johns Hopkins All Children's Hospital

- 8:00 – 8:20AM**      **Laszlo Nagy, Johns Hopkins All Children's Hospital**  
*Macrophages always maintain, sometimes harm, and often heal skeletal muscle*
- 8:20 – 8:40**      **Elizabeth Wohlfert, University of Buffalo**  
*Investigating the impact of Chronic Toxoplasma Infection on Muscle*
- 8:40 – 9:00**      **Carlos Aguilar, University of Michigan**  
*Understanding & Re-Writing Cellular & Molecular Programs After Skeletal Muscle Trauma*
- 9:00 – 9:20**      **Michael Hicks, University of California, Irvine**  
*Spatial Transcriptomics combined with CRISPR-Cas9 to interrogate cell-niche interactions*
- 9:20 – 9:40**      **Andreas Patsalos, Johns Hopkins University School of Medicine**  
*Spatiotemporal multi-omic mapping of regenerative inflammation in skeletal muscle reveals a dynamic multilayered tissue architecture and macrophage subtypes-dominated zones*
- 9:40 – 10:10AM**      **COFFEE BREAK**

### **Session V: Mechanisms of Muscle Disease and Injury**

Chair: Elizabeth McNally, Northwestern University

- 10:10 – 10:30AM**      **Elizabeth McNally, Northwestern University**  
*Extracellular matrix and its role in muscular dystrophy pathogenesis*
- 10:30 – 10:50**      **Gretchen Meyer, Washington University School of Medicine St. Louis**  
*Regulation of Muscle Mass and Strength by Adipose Tissue*

- 10:50 – 11:10**      **Da-Zhi Wang, University of South Florida**  
*CIP/MLIP in muscular dystrophy of cardiac and skeletal muscles*
- 11:10 – 11:30**      **Chi Bun Chan, The University of Hong Kong**  
*Deficiency of muscle-generated brain-derived neurotrophic factor (BDNF) causes inflammatory myopathy through reactive oxygen species-mediated necroptosis and pyroptosis*
- 11:30 – 11:50**      **Elizabeth Pruzinsky, University of Pennsylvania**  
*The Role of the Nuclear Receptor Co-regulator RIP140 (Nrip1) in the Control of Muscle Endurance Fitness*
- 11:50AM – 2:00PM**      **LUNCH BREAK (on your own)**
- 1:00 – 1:45PM**      **Elements of a Successful Academic Career; Las Olas Ballroom**  
**Pre and Post-Doctoral Trainees Only**  
**Moderators: Rachelle Crosbie, University of California, Los Angeles and Andrew Judge, University of Florida**
- Panelists:  
**Elizabeth McNally**, Editor-in-Chief JCI; Northwestern University  
**Armando Villalta**, University of California, Irvine  
**Lucas Smith**, University of California, Davis  
**Laszlo Nagy**, Commissioning Editor & Editor, FEBS Letters; Founding Editor, FEBS Open Bio; Johns Hopkins University School of Medicine
- 2:00PM**              **Abstract Presentation Session II-Data Blitz; Las Olas Ballroom**  
**Pre- and Post-Doctoral Trainees; 5-minute presentations**  
Chair: Rachelle Crosbie, University of California, Los Angeles
- 2:00 – 2:05**              **Hsin-Yu Chen, University of Michigan Medical School**  
*Syntaxin 4-enhanced plasma membrane repair is independent of dysferlin in skeletal muscles*
- 2:06 – 2:11**              **Ashlee Long, Northwestern University**  
*The extracellular matrix differentially directs myoblast motility and differentiation in distinct forms of muscular dystrophy*
- 2:12 – 2:17**              **Joseph Reynolds, University of California, Los Angeles**  
*Enhancing sarcolemmal stability: sarcospan overexpression alters the matrisome in a severe model of muscular dystrophy*
- 2:18 – 2:23**              **Martin Schonk, University of Florida**  
*Myofiber-specific FoxP1 deletion protects against pancreatic cancer-induced muscle atrophy and weakness.*
- 2:24 – 2:29**              **Astrid Brull, National Institutes of Health**  
*Use of Circular RNA Guides to Correct a Hotspot of Dominant-Negative Glycine Substitutions Causing Collagen VI-Related Dystrophies*

2:30 – 2:35

**Nika Maani, The University of Toronto and The Hospital for Sick Children**  
*microRNA-133a as an indicator of disease progression and treatment response in a mouse model of X-linked Myotubular Myopathy*

*Communications Award Presentation, Andrew Judge and Rachelle Crosbie*

## **Session VI: Beyond Skeletal Muscle: Lessons from the Heart**

Chair: Jeff Molkentin, Cincinnati Children's Hospital & University of Cincinnati

2:40 – 3:00PM

**Jeff Molkentin, Cincinnati Children's Hospital & University of Cincinnati**  
*Engineering a novel universal gene therapy strategy for dystrophy/myopathy treatment*

3:00 – 3:20

**Jennifer Davis, University of Washington**  
*Unlocking the Secrets of Fibroblast Biology*

3:20 – 3:40

**Basil Petrof, McGill University**  
*Innate immune memory in DMD: A different kind of muscle training*

3:40 – 4:00

**Fabio Rossi, The University of British Columbia**  
*FAP heterogeneity*

4:00 – 4:20

**Arden Piepho, The Ohio State University**  
*Cardiac inflammation in a severe model of Duchenne muscular dystrophy is characterized by T-cells that exacerbate cardiomyopathy pathology.*

4:30 – 6:00PM

**Poster Session II; Atlantic Ballroom**

## **Wednesday, June 26, 2024**

7:30AM

**Breakfast; Las Olas Foyer**

## **Session VII: AAV Gene Therapy Approaches for Skeletal and Cardiac Muscle**

Chair: H. Lee Sweeney, University of Florida

8:00 – 8:20AM

**H. Lee Sweeney, University of Florida**  
*Approaches to AAV gene therapy for DMD and hurdles to be overcome*

8:20 – 8:40

**Jeff Chamberlin, University of Washington**  
*Partial reversal of fibrosis in old mdx mice*

8:40 – 9:00

**Karen Chen, SMA Foundation**  
*Reflections on AAV Gene Therapy for Spinal Muscular Atrophy*

- 9:00 – 9:20 **Tsz Ling (Elaine) Leung, University of Nevada, Reno**  
*Role of alpha7 integrin phosphorylation and signaling on muscle disease progression in Duchenne Muscular Dystrophy*
- 9:20 – 9:40 **Robert Jimenez, University of California, Los Angeles**  
*Elucidating the impact of AAV immune complexes on macrophage uptake and chemokine induction*
- 9:40 – 10:10AM **COFFEE BREAK**

## **Session VIII: Therapeutic Targets in the Inflammation-Fibrosis Axis**

Chair: Jill Rafael-Fortney, The Ohio State University

- 10:10 – 10:30AM **Jill Rafael-Fortney, The Ohio State University**  
*Mineralocorticoid receptor signaling in the muscle microenvironment*
- 10:30 – 10:50 **Armando Villalta, University of California, Irvine**  
*Treg and macrophage interactions regulate muscle fibrosis*
- 10:50 – 11:10 **Noah Weisleder, The Ohio State University**  
*Antibodies targeting proteins involved in sarcolemma repair exacerbate inflammatory and inherited myopathies*
- 11:10 – 11:30 **Davy Vanhoutte, Cincinnati Children's - University of Cincinnati**  
*Thbs1 regulates skeletal muscle mass in a TGF $\beta$ -Smad2/3-ATF4-dependent manner*
- 11:30 – 11:50 **Mattia Quattrocelli, Cincinnati Children's - University of Cincinnati**  
*cAMPing is not a date: the non-genomic action of pro-atrophic glucocorticoids breaks the muscle clock through the Activating Transcription Factor 4*
- 11:50 – 12:00PM **Closing Remarks**
- 12:00PM **Adjourn**

**Thank you for your participation in our program, we look forward to seeing you at New Directions 2026!**

