



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

- 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** **Robyn van Poelgeest, 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
- 4:15 – 5:15PM** **Kay Davies, University of Oxford**
The Role of Utrophin in the Therapy of Duchenne Muscular Dystrophy
- 6:00 – 8:30PM** **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 **Abstract Selected Speaker #1**

9:20 – 9:40 **Abstract Selected Speaker #2**

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**
Title TBD

11:30 – 11:50 **Abstract Selected Speaker #1**

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

12:15-1:30PM 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

2:00PM **Abstract Presentation Session I-Data Blitz;** Las Olas Ballroom
Pre- and Post-Doctoral Trainees; 5-minute presentations
Chair: TBD

2:00 – 2:05 Trainee #1

2:06 – 2:11 Trainee #2

2:12 – 2:17 Trainee #3

2:18 – 2:23 Trainee #4

2:24 – 2:29 Trainee #5

2:30 – 2:35 Trainee #6

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 **Abstract Selected Speaker #1**

4:00 – 4:20 **Abstract Selected Speaker #2**

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**
Title TBD
- 9:00 – 9:20 **Abstract Selected Speaker #1**
- 9:20 – 9:40 **Abstract Selected Speaker #2**
- 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 **Abstract Selected Speaker #1**
- 11:30 – 11:50 **Abstract Selected Speaker #2**
- 11:50AM – 2:00PM **LUNCH BREAK (on your own)**

- 12:15 – 1:30PM **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

- 2:00PM **Abstract Presentation Session II-Data Blitz; Las Olas Ballroom**
Pre- and Post-Doctoral Trainees; 5-minute presentations
Chair: TBD

- 2:00 – 2:05 **Trainee #1**
- 2:06 – 2:11 **Trainee #2**
- 2:12 – 2:17 **Trainee #3**
- 2:18 – 2:23 **Trainee #4**

2:24 – 2:29 Trainee #5

2:30 – 2:35 Trainee #6

Session VI: Beyond Skeletal Muscle: Lessons from the Heart

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

2:40 – 3:00PM **Jeff Molquentin, 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 **Abstract Selected Speaker #1**

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 **Abstract Selected Speaker #1**

9:20 – 9:40 **Abstract Selected Speaker #2**

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

Title TBD

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

Abstract Selected Speaker #1

11:30 – 11:50

Abstract Selected Speaker #2

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!

