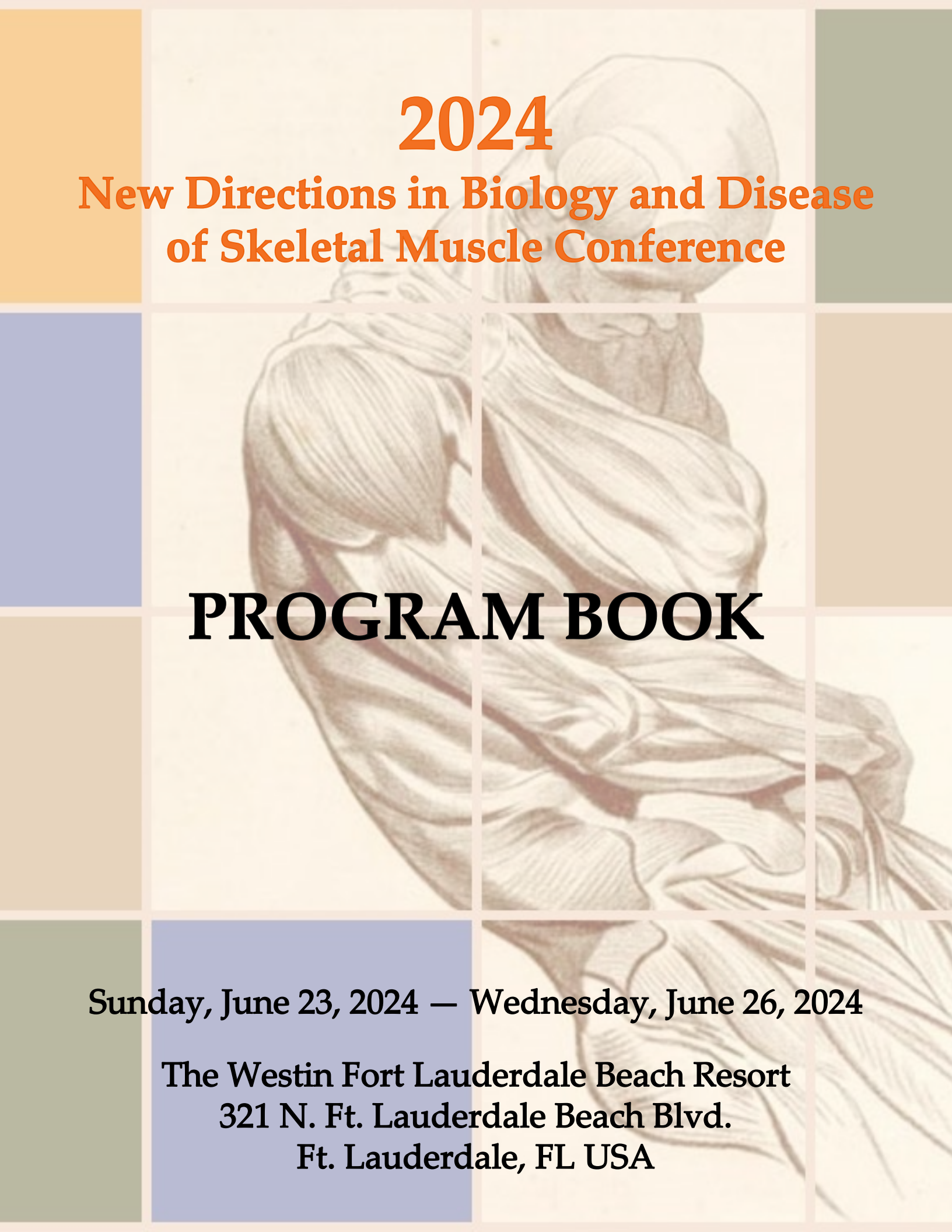
2024

New Directions in Biology and Disease of Skeletal Muscle Conference



PROGRAM BOOK

Sunday, June 23, 2024 — Wednesday, June 26, 2024

The Westin Fort Lauderdale Beach Resort
321 N. Ft. Lauderdale Beach Blvd.
Ft. Lauderdale, FL USA

2024 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 will be held in Ft. Lauderdale, FL on June 23-26, 2024. 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 cutting edge research. This meeting specifically emphasizes the presentation of unpublished work through oral and poster presentations. 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. With rigorous mechanistic understanding of the disease processes and moving targets 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: Identify both common and unique targets for among muscle diseases. 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 4: 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

Matthew Alexander, University of Alabama at Birmingham
Douglas Millay, Cincinnati Children's Hospital
Jeff Molkentin, Cincinnati Children's Hospital & University of Cincinnati
Laszlo Nagy, Johns Hopkins All Children's Hospital
Jill Rafael-Fortney, The Ohio State University
Melissa Spencer, University of California, Los Angeles

KEYNOTE SPEAKER

Professor Dame Kay Davies

University of Oxford

The Role of Utrophin in the Therapy of Duchenne Muscular Dystrophy

Kay Davies, Ph.D., DBE FMedSci FRS

Dr Lee's Professor of Anatomy Emerita

Co-Director of the MDUK Oxford Neuromuscular Centre in the Department of Physiology, Anatomy and Genetics at the University of Oxford

Professor Dame Kay Davies, Ph.D., DBE FMedSci FRS is the Dr Lee's Professor of Anatomy Emerita and co-Director of the MDUK Oxford Neuromuscular Centre in the Department of Physiology, Anatomy and Genetics at the University of Oxford. She received a BA degree in Chemistry and a graduate PhD degree in Biochemistry from the University of Oxford. She was appointed Professor of Genetics in 1996 and then appointed Dr Lee's Professor of Anatomy at the University of Oxford in 1998. She was founding Director of the MRC Functional Genomics Unit 1999-2017 and co-founded the Oxford Centre of Gene Function in 2000 with Professors Ashcroft and Donnelly, a new institute going from genetic association in human disease to function in the whole organism. Kay's research interests lie in the molecular analysis and development of treatments for genetic diseases, particularly Duchenne muscular dystrophy (DMD). She has been involved in exon skipping, gene therapy and utrophin up-regulation for the therapy of DMD. She has published more than 400 papers and won numerous awards for her work and co-founded companies to translate her work to the clinic. Kay is a founding Fellow of the Academy of Medical Sciences and a Fellow of the Royal Society. She was appointed Governor of the Wellcome Trust in 2008 and became Deputy Chair 2013-17. She was made Dame Commander of the British Empire for services to science in 2008.

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 CRIPSR-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 TGFβ-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!

ABSTRACTS

Abstracts are organized by session then alphabetically by the **submitting author's last name**. Abstract numbers correspond to the poster/board numbers for the poster sessions. Even numbered posters will present in our first poster session on Monday, June 24th. Odd numbered posters will present in our second poster session on Tuesday, June 25th.

Gene Therapy for Muscle Disease- Immune Response

1. **Evaluating Investigational PN-Containing Oligonucleotide WVE-N531 for Patients with Duchenne Muscular Dystrophy Amenable to Exon 53 Skipping: A Phase 1b/2 Open-Label Study**
   Angelov, Angel; Servais, Laurent; Campbell, Craig; Malhi, Suki; Hu, Xiao Shelley; Narayanan, Padma; Hart, Andrew; Haegele, Joseph A.; Singh, Kuldeep; Rheinhardt, Jeanette; Ghosh, Anamitra; Saint, Sue; Bhatia, Siddharth; Casey, Chelley; Li-Kwai-Cheung, Anne-Marie

2. **Immune responses associated with rAAV-mediated gene therapy in muscle diseases**
   Hobson, Orinda; Prima, Victor; Russ, Jenny; Sweeney, H. Lee; Hammers, David W.

3. **Elucidating the impact of AAV immune complexes on macrophage uptake and chemokine induction**
   Jimenez, Robert; Williams, Blake; Emami, Michael; Spencer, Melissa

4. **TRPC6 inhibition in DMD attenuates the inflammatory immune response elicited by lipid nanoparticle delivery system**
   Guru, Sameer; Brusacoram, Annabelle; **Lin, Brian**

5. **Functional benefit of CRISPR/Cas9-induced deletion for RYR1 dominant mutation**
   Beaufils M.; Melka M.; Brocard J.; Benoit C.; Debbah N.; Mamchaoui K.; Romero N.B.; Dalmas-Laurent A.F.; Quijano-Roy S.; Fauré J.; Rendu J.; **Marty I.**

6. **Gut-thymus-muscle axis in DMD pathogenesis**
   Farini, Andrea; Villa, Chiara; Strati, Francesco; Perillo, Federica; Molinaro, Monica; Vadalà, Rebecca; Cirrincione, Elisa; Santarelli, Federica; Quattrocelli, Mattia; Marasca, Federica; Caprioli, Flavio; Bodega, Beatrice; Cassani, Barbara; Facciotti, Federica; **Torrente, Yvan**

Muscle Repair, Recovery, and Regeneration— a view from the muscle

7. **Cell-cell communication in the face of muscle damage**
   Barton, Elisabeth; Luo, Yangyi; Kopinke, Daniel; Feng, Yue

8. **The CFTR Corrector C17 improved skeletal muscle function in a LGMDR3 model: pharmacological profile and efficacy studies in mouse**
   Benetollo, Alberto; Scano, Martina; Parrasia, Sofia; Biasutto; Lucia; Nogara, Leonardo; Blaauw, Bert; Sandonà, Dorianna

9. **Utility of a 3D Engineered Skeletal Muscle Organoid System to Assess Exon Skipping and Dystrophin Protein Restoration in a Human DMD Cell Model**
   Estrella, Nelsa L.; **Blake, Sara L.;** Fredericks, Maureen; Bonilla, Arianna M.; Brennan, Christopher M.; Streeter, Matthew; Hicks, Amy N.; Kamer, Kimberly J.; Kheirabadi, Mahboubeh; Dhanabal, Mohanraj; Qian, Ziqing Leo; Girgenrath, Mahasweta

10. **Early adolescent genotoxic stress leads to a reduction in skeletal muscle stem cells and persistent damage of the niche**
    Yang, C; Puviindran, V; Bachman, J; Panken, D; Torres, Ponce E; **Chakkalakal, JV**

11. **Strategic Interventions for Volumetric Muscle Loss: Two-Stage Treatment Paradigm Utilizing Provisional Biomaterial-Mediated Stabilization Followed by a Definitive Treatment**
    Clark, Andrew R.; Kulwatno, Jonathan; Kanovka, Sergey S.; Klarmann, George J.; Natoli, Roman M.; McKinley, Todd O.; Potter, Benjamin K.; Dearth, Christopher L.; Goldman, Stephen M.

12. **MEGF10 Supports Muscle Stem Cell Retention and Repopulation**
    Clock, Benjamin; Colobong, Sebastien; Pavar, Nikitha; Pavani, Shreya; Hicks, Michael

13. **Development of an In Vitro Niche System to Monitor the Effects of Muscle Stem Cell Receptors on Niche Formation**
    Colobong, Sebastien; Clock, Benajmin; Pavar, Nikitha; Hicks, Michael

14. **Human iPSC-derived iPAX7 myogenic progenitors are transcriptionally mature after incorporation into murine myofiber in vivo**
    Crist, Sarah; Azzag, Karim; Kiley, James; Coleman, Ilsa; Magli, Alessandro; Perlingeiro, Rita

15. **The endocannabinoid system: a novel target for muscle degeneration and regeneration.**
    Dalle, Sebastiaan; Costamagna, Domiziana; Schouten, Moniek; Koppo, Katrien

16. **Determining the limits of skeletal muscle regeneration in the highly regenerative rodent model, Acomys cahirinus**
    Davenport, Mackenzie L; Fong, Amaya; Albury, Kaela N; Henley-Beasley, C Spencer; Barton, Elisabeth R; Maden, Malcolm; Swanson, Maurice S

17. **A novel CRISPR-Cas9 strategy to target DYSTROPHIN mutations downstream of exon 44 in patient-specific DMD iPSCs**
    Dhoke, Neha; Kim, Hyunkee; Azzag, Karim; Crist, Sarah; Kiley, James; Perlingeiro, Rita

18. **Comparative Analysis of Forelimb Grip Strength Across Age Ranges and Strains in Murine Models of Duchenne Muscular Dystrophy.**
    Elustondo, Pia; Srinivassane, Sadish; McDonald, Katherine; McKenna, Meagan; Hoffman, Eric; Nagaraju, Kanneboyina

19. **Studying Intramuscular Fat Deposition and Muscle Regeneration: Insights from a Comparative Analysis of Mouse Strains, Injury Models, and Sex Differences**
    Norris, Alessandra M.; Fierman, Kiara E.; Campbell, Jillian; Pitale, Rhea; Shahraj, Muhammad; Kopinke, Daniel

20. **Characterization of Three-Dimensional Engineered Muscle Tissue (EMT) System for Skeletal Muscle Disorder Research**
    Finnemore, Eric; Cao, Tianxin; Harris, Thomas; Daniela Santos

21. **Exploring the Secretory Product of Neural Stem Cells in an ALS Mouse Model**
    Gao, Jinghui; Hankin, Rachel; Zheng, Yaochao; Sikal, Aria Mathi; Yao, Yao

22. **Spatial Transcriptomics combined with CRIPSR-Cas9 to interrogate cell-niche interactions**
    Hicks, Michael; Clock, Ben

23. **Characterization of a rat Duchenne muscular dystrophy model**
Lee, Young il; Hart, Cora; Herr, Jeffrey; Henley-Beasley, Spencer; Gloger, Grace; Zerpa, Eli; Hammers, David; Russell, Alan; Barton, Elisabeth; Sweeney, H. Lee

24. **The TAS1R2 sweet taste receptor regulates skeletal muscle mass recovery following hindlimb immobilization**
Link, Grace; Kondo, Saki; King, Kendall; Serrano, Joan; Kyriazis, George A.

25. **RKER-065, a novel ActRII ligand trap, counteracted the adverse musculoskeletal effects of glucocorticoid treatment in a murine model of Duchenne muscular dystrophy**
Linzey, Michael; Zhen, Gehua; St. Pierre, Mark; Hack, Faith; Nathan, Remya; Cahill, Morgan; Daman, Tyler; Macaluso, Savanna; Todorova, Radina; Grenha, Rosa; Lerner, Lorena; Lachey, Jenn; Fisher, Ffolliot; Seehra, Jasbir

26. **Hedgehog Signaling Controls Cellular Crosstalk During Muscle Regeneration**
Liu, Xinyue; Kopinke, Daniel

27. **Contribution of Insulin-Like Growth Factor-I secreted from Fibro-Adipogenic Progenitors to Skeletal Muscle Regeneration and Cellular Dynamics**
Luo, Yangyi Edward; Abe-The, Zoe; Fatima, Naureen; Appu, Ambili B; Kuo, Li-Ying; Kopinke, Daniel; Barton, Elisabeth R.

28. **An Imminent Registry to Study Long-Term Safety and Quality of Life in Patients With Duchenne Muscular Dystrophy (DMD) Treated With Vamorolone**
Kuntz, Nancy L.; Iyadurai, Stanley; Ingenito, Gary; Khaliq, Nadeem; **Madpak, Anthony**

29. **Rescuing glucotoxicity-induced skeletal muscle atrophy and triggering muscle regeneration by activation of satellite cells – an in vitro and in vivo study**
Mitra, Akash; Shanavas, Shanooja; Chaudhury, Debajit; Bose, Bipasha; Das N, Undurti; Shenoy P, Sudheer

30. **Withdrawn- No Abstract or Poster**

31. **Electrical Pulse Stimulation Protects C2C12 Myotubes against Hydrogen Peroxide-induced Cytotoxicity via Nrf2/antioxidant Pathway**
Pribil Pardun, Sarah L.; Bhat, Anjali; Anderson, Cody; Allen, Michael F.; Bruening, Will; Joacob, Joel; Pendyala, Ved Vasishtha; Yu, Li; Bruett, Taylor; Zimmerman, Matthew C.; Park, Song-young; Zucker, Irving H.; Gao, Lie

32. **Inactivation of CRTCs in muscle stem cells disrupts cell fate homeostasis and impairs muscle regeneration**
Ren, Junxiao; Li, Yufen; Chu, Leah; Wang, Yubo; Zhou, Yumei; Yue, Feng

33. **RKER-065 enhanced the therapeutic effect of phosphorodiamidate morpholino oligomer in a Duchenne muscular dystrophy mouse model.**
St. Pierre, M.; Zhen, G.; Linzey, M.; Hack, F.; Nathan, R.; Cahill, M.; Daman, T.; Macaluso, S.; Todorova, R.; Grenha, R.; Lachey, J.; Lerner, L.; Seehra, J.; Fisher, F.M.

34. **Estradiol deficiency as a consequence of aging contributes to the loss of satellite cell number and function in female mice**
Sullivan, **Brian P.;** Lee, Alexie A.; Shams, Ahmed S.; McMillin, Shawna L.; Ebeling, Mara C.; Kyba, Michael; Lowe, Dawn A.

35. **Do aging-related post-transcriptional myosin modifications contribute to sarcopenia?**
Brown, Alon T.; Loya, Amy K.; Kronert, William A.; Bernstein, Sanford I.; **Swank, Douglas M.**

36. **Do the Enhanced Regeneration Capabilities of p21 Knock-out Mice Slow Sarcopenia?**
Zilberman, Aleeza; Glasheen, Bernadette; Blaber, Elizabeth; **Swank, Douglas**

37. **The role of Notch1 in Pax3-mediated myogenesis from pluripotent stem cells**
Yamashita, A.M.S., Garay, B.I., Kim, H., Bosnakovski, D., Abrahante, J.E., Abreu, P., Ahlquist, A.and Perlingeiro, R.C.R.

38. **Transforming Muscle Regeneration in Aging: Targeting the Integrated Stress Response Pathway**
Zhang, Hanzhi; Brown, Alexander; Scaramozza, Annarita; Brack, Andrew; Liu, Xuhui; Feeley, Brian

39. **Engineered Supportive Holder for Advancing Mouse Hindlimb Grip Strength Assessment**
Zheng, Yaochao; Davarzani, Amin; Gao, Jinghui; Karumbaiah, Lohitash; Yao, Yao

New Delivery Vectors and Cargo Modalities for Muscle Gene Therapy

40. **In Vitro Efficacy of an Endosomal Escape Vehicle (EEV™) – DMD Exon 45 Skipping Oligonucleotide Conjugate in DMD Patient-Derived Skeletal and Cardiac Muscle Cells**
Brennan, Christopher M.; McKee, Holly; Liu, Zekun; Wang, Cindy; Streeter, Matthew; Kheirabadi, Mahboubeh; Qian, Ziqing Leo; Girgenrath, Mahasweta; Estrella, Nelsa L.

41. **Nonclinical Data for PGN-EDODM1 Demonstrated Nuclear Delivery, Mechanistic and Meaningful Activity for the Potential Treatment of DM1**
Gilbert, J.; Klein, A.; Lonkar, P.; Foy, J.; Yu, S.; Reid, T.; Sarkar, K.; Cleary, J.; Berglund, J.A.; Furling, D.; **Holland, A.**

42. **Single- and Repeat-Dose Nonclinical Data for PGN-EDO51 Demonstrated Favorable Pharmacology and Safety Profiles for the Treatment of DMD**
Sweeney, C.; Gilbert, J.; Lamore, S.; Lonkar, P.; Foy, J.; **Holland, A.**

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44. **Heterogeneity of non-myogenic mesenchymal cells (NMMCs) in rotator cuff muscles and their roles in the pathology of massive rotator cuff tears**
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45. **Aerobic Physical exercise protects mice from skeletal muscle atrophy in a model of HIV infection**
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47. **Integrated multi-omics approach reveals the role of striated preferentially expressed gene (SPEG) in skeletal muscle including its relationship with myospryn complex**
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48. **Sigmar1-CPT1b signaling upregulation in skeletal muscle of G93A*SOD1 overexpressing mouse model of ALS**
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50. **Leucine supplementation prevents the development of skeletal muscle dysfunction in a rat model of HFpEF by modulating mitochondria**
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51. **Dissecting lysosomal signals to fight Pompe disease.**
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52. **Deficiency of muscle-generated brain-derived neurotrophic factor (BDNF) causes inflammatory myopathy through reactive oxygen species-mediated necroptosis and pyroptosis**
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53. **Syntaxin 4-enhanced plasma membrane repair is independent of dysferlin in skeletal muscles**
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59. **Direct effects of mineralocorticoid receptor signaling on dystrophic fibroblasts**
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63. **Defective collagen biosynthesis as an early initiating factor of pathological fibrosis in dystrophic muscle**
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65. **Durable Exon Skipping and Dystrophin Production with Endosomal Escape Vehicle (EEV™) - Oligonucleotide Conjugates in Preclinical Models of DMD**
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67. **CDP-ribitol prodrug treatment ameliorates ISPD-deficient muscular dystrophy**
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70. **Dysferlin null skeletal muscle displays sex-specific differences in pathology and extracellular matrix composition**
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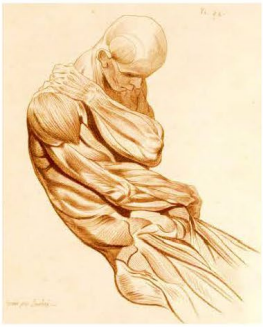

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