



2020

NOW VIRTUAL

27TH ANNUAL FSHD SOCIETY

International Research Congress

Thursday, June 25th

10:00 AM - Welcoming Remarks & Meeting Overview

10:05 AM - Keynote: The Past/Present/Future of FSHD - George Padberg MD, PhD

Session 1: Discovery Research & Models, moderated by Robert Bloch (Chair) & Silvere van der Maarel (Co-Chair)

10:35 AM - Peter Zammit "Active skeletal muscle regeneration in Facioscapulohumeral muscular dystrophy"

10:50 AM - Katherine Williams "Single-nucleus RNA-seq identifies divergent populations of FSHD2 myotube nuclei"

11:05 AM - Maria Traficante "SLC34A2 as a Potential Biomarker for FSHD"

11:17 AM - Yuanfan Zhang "FSHD Zebrafish Models for Drug Discovery"

11:30 AM - Break

Session 2: Genetics & Epigenetics, moderated by Silvere van der Maarel (Chair) & Michael Kyba (Co-Chair)

11:45 AM - Sven Bocklandt "High throughput analysis of tandem repeat contraction associated with Facioscapulohumeral Muscular Dystrophy (FSHD) by optical mapping"

12:00 PM - Alexander Liu "A long-read sequencing approach for investigating repeat number and DNA methylation of the D4Z4 region"

12:14 PM - Richard J. L. F. Lemmers "Two families with chromosome 10q-linked FSHD identify DUX4 as principal disease gene"

12:30 PM - Break

Session 3: Pathology and disease mechanisms, moderated by Michael Kyba (Chair) & Kathryn Wagner (Co-Chair)

12:45 PM - Angela Lek "Hypoxia-signaling is a key driver of DUX4-induced pathology"

12:55 PM - Amy Campbell "Role for aberrant protein synthesis in facioscapulohumeral muscular dystrophy"

1:10 PM - Julie Dumonceaux "Regulated necrosis is involved in DUX4-mediated toxicity"

1:25 PM - Joel Chamberlain "An AAV-DUX4 Mouse Model of FSHD Reflects Disease Pathology Based on the Level of DUX4 Protein Expression"

1:43 PM - Michael Kyba "Mechanisms of Pathology in FSHD"

1:59 PM - Day 1 Closing Remarks



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Friday, June 26

10:00 AM - Welcoming Remarks

Session 4: Interventional Strategies, Moderated by Julie Dumonceaux (Chair) & Scott Harper (Co-Chair)

10:03 AM - Yi-Wen Chen "LNA and 2'MOE gapmers for treating facioscapulohumeral muscular dystrophy"

10:19 AM - Afrooz Rashnonejad "DUX4 inhibition by AAV.CRISPR-Cas13b in FSHD mouse models"

10:33 AM - Rika Maruyama "DUX4 transcript knockdown with antisense gapmers for the treatment of facioscapulohumeral muscular dystrophy"

10:47 AM - Katelyn Daman "A combined ex vivo and xenograft pipeline for FSHD drug development"

11:02 AM - Lindsay Wallace "DUX4-targeted RNAi-based Gene Therapy for FSHD"

11:18 AM - Break

Session 5: Clinical Studies & Outcome Measures, Moderated by Kathryn Wagner (Chair) & Baziel von Engelen (Co-Chair)

11:31 AM - Christopher R.S. Banerji "Facioscapulohumeral muscular dystrophy 1 patients participating in the UK FSHD registry can be subdivided into 4 patterns of self-reported symptoms"

11:50 AM - Peter Lunt "Interaction of factors in (epi)genotype-phenotype relationship in FSHD1 explored in scatter plots created from our own and published data"

12:15 PM - Rabi Tawil "Design and baseline characteristics of a Phase 2, Randomized, Placebo-Controlled, 24-Week Study of the Efficacy and Safety of Losmapimod in Treating Subjects with FSHD: ReDUX4"

12:35 PM - Michelle Mellion "ReDUX4: Addressing the Challenges of Clinical Trials During the COVID-19 Pandemic"

12:57 PM - Break

1:07 PM - Lucienne Ronco "A biomarker of DUX4 activity to evaluate losmapimod treatment effect in FSHD Phase 2 trials"

1:29 PM - Dr. Jeffrey Statland "ACE-083 trial outcomes and lessons learned"

1:44 PM - Industry Panel: Romesh Subramanian, Michelle Mellion, Anthony Saleh, and Jane Owens. Moderated by: Fran Sverdrup

2:00 PM - Best Poster Prize & Young Scientist Award

2:08 PM: 2021 IRC Announcement & closing comments

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