



FARA 2020 Biomarker & Clinical Endpoint Meeting

Thursday September 24th and Friday September 25th

Thursday September 24th - Clinical Outcome Assessments

8 - 10:00 am **EDT** - Chair: Jane Larkindale

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| 8:00am | Opening remarks and welcome | | |
| 8:05-8:45am | FDA on clinical outcome measures | Michelle Campbell | FDA |
| 8:45-9:10am | Identification of the Most Important Friedreich's Ataxia Symptoms: The First Step in Developing the Friedreich's Ataxia Health Index (FA-HI) | Chad Heatwole | University of Rochester |
| 9:10-9:35am | Applications of the Friedreich's Ataxia integrated Clinical Database (FA-ICD): Test-retest reliability of the FARS scales in 6 Clinical Trials of Friedreich's Ataxia. | Christian Rummey | Clinical Data Science GmbH |
| 9:35-10:00am | Upper-limb dexterity and pyramidal dysfunction in Friedreich Ataxia, an integrated rapid alternating movement study. | Massimo Pandolfo | Hôpital Erasme, Université libre de Bruxelles |

12 – 12:50pm am **EDT** - Chair: Jane Larkindale

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| 12:00-12:25pm | A translational medicine approach for clinical trial readiness of MIN-102 (leriglitzzone) in Friedreich's ataxia | Uwe Meya | Minoryx Therapeutics |
| 12:25 – 12:50pm | Efficacy, safety, and pharmacodynamic results of the double-blind, placebo-controlled, pivotal phase 2b study of omeveloxolone in Friedreich's ataxia | David Lynch | Children's Hospital of Philadelphia |

Thursday September 24th - Clinical Outcome Assessments

4:30 - 7:05 pm **EDT** - Chair: Sub Subramony

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| 4:30-5:10pm | Digital technologies, bright shiny objects and the future of clinical trials | Jesse Cedarbaum | Coeruleus Clinical Sciences |
| 5:10-5:35pm | Digital endpoints for home-based assessment of pediatric Friedreich's ataxia patients | Arne Mueller | Novartis Institutes for BioMedical Research |
| 5:35-6pm | Gait as a Predictor of Disease Progression in Friedreich's Ataxia. | Arnaud Gouelle | Gait and Balance Academy, ProtoKinetics |
| 6-6:10pm | FA-CHILD A 6 Month-Interval Natural History Study in Children with Friedreich's ataxia | David Lynch | Children's Hospital of Philadelphia |
| 6:10-6:35pm | Measurement of upper limb function in Friedreich ataxia via instrumented measures of daily activity | Louise Corben | Murdoch Children's Research Institute |
| 6:35-6:45pm | Determining the validity of conducting the modified Friedreich Ataxia Rating Scale through video | Martin Delatycki | Murdoch Children's Research Institute |
| 6:45-7:05pm | Summary talk | Jane Larkindale | Clinical Path Institute |

Friday September 25th - Diagnostic, Monitoring and Pharmacodynamic biomarkers

8 - 9:45 am **EDT** - Chair: Ian Blair

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| 8:00-8:05am | Welcome | | |
| 8:05-8:30am | Brain MRI Biomarkers for Friedreich Ataxia: A Focus on the Superior Cerebellar Peduncles using the IMAGE-FRDA and ENIGMA-Ataxia datasets | Ian Harding | Monash University |
| 8:30-8:55am | Lipid metabolism disruption in Friedreich's Ataxia | Clementina Mesaros | University of Pennsylvania |
| 8:55-9:20am | Neurofilaments on their way to clinical use | Andreas Jeromin | Quanterix Corp, Cohen Veterans Biosciences |
| 9:20-9:45am | Dynamic characteristics of DMN and sensory-motor states highly correlates with age of symptoms onset in Friedreich Ataxia, a hidden Markov modeling of MEG power envelopes study. | Gilles Naeije | Hôpital Erasme, Université libre de Bruxelles |

4 - 5:50 pm **EDT** - Chair: Steve Hersch

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| 4-4:25pm | Biomarkers of Friedreich's ataxia Confirmed in humans and mice | Gino Cortopassi | University of California, Davis |
| 4:25-4:50pm | Frataxin detection in cerebrospinal fluid and serum of individuals with Friedreich's ataxia | Barbara Grubinska | Voyager Therapeutics |
| 4:50-5pm | Preclinical biodistribution and toxicology of AAV9-based gene replacement therapy for Friedreich's ataxia | Barbara Perez | University of Florida |
| 5-5:10pm | Nrf2 and frataxin interplay and consequences for FRDA | Eskenazi Nicolas | University of Pennsylvania |
| 5:10-5:20pm | Quantification of frataxin isoform E in whole blood | Leah Quinton | University of Pennsylvania |
| 5:20-5:50pm | Summary talk | Steve Hersch | Voyager Therapeutics |