Monday Afternoon September 4, 2017
Session 3: Clinical session
14:00-14:30 ... Nutritional Ketosis
Kieran Clark, Oxford, UK
14:30-15:00 ... Ketone Body Treatment in FADDs
Gepe Vissers, Utrecht/Amsterdam, Netherlands
15:00-15:15 ... 3-hydroxybutyrate (3-HB) treatment in multiple acyl-CoA dehydrogenase deficiency: a systematic literature review and international retrospective cohort study
Willeijn J. van Rijt, Groningen, the Netherlands
15:15-15:30 ... Results from a 78-week Single-arm, Open-label Phase 2 Study to Evaluate UX007 in Pediatric and Adult Patients with Moderate to Severe Long-Chain Fatty Acid Oxidation Disorders (LC-FADD)
Jerry Vockley, Pittsburgh, PA, USA
15:30-15:45 ... Break
15:45-16:15 ... An overview of FADDs and unmet needs in Latin America
Ida Schwartz, Porto Alegre, Brazil
16:15-16:30 ... Health-related Quality of Life in Patients with long-chain Fatty Acid Oxidation Disorders
Suzan JG Knottnerus, Amsterdam, The Netherlands
16:30-16:45 ... Intravenous Sources of Medium Chain Triglycerides for Critically Ill Patients with Fatty Acid Oxidation Disorders
Heather Bausell, Chicago, IL, USA
16:45-17:15 ... Panel Discussion
17:15-17:30 ... Summary / Closing remarks
17:30-18:45 ... Cocktail Reception & Poster Session with Oral Presentations for Jr. Investigators interested in presenting
19:00 ... INFORM Advisory Committee Meeting

INFORM SYMPOSIUM at ICIEM / September 7, 2017
FATTY ACID OXIDATION DISORDERS
Chair: Mike Bennett (Philadelphia, PA, USA) / Co-Chair: Eric Goetzman (Pittsburgh, PA, USA)
Anti-oxidant therapy as an adjunct for treatment of long chain fatty acid oxidation disorders
Guilhian Leipnitz, Brazil
Novel mechanisms of pathogenesis in mitochondrial trifunctional protein deficiency: implications for clinical outcome and treatment
Areeg El-Gharbawy, USA
Rhabdomyolysis and inflammation in metabolic muscle disease
Yamina Hamel, France

Sunday September 3rd & Monday September 4th preceding the 13th ICIEM in Rio de Janeiro, Brazil

Sunday September 3, 2017
Session 1: Welcome and Keynote Speaker
15:30-16:30 ... Registration
16:30-16:40 ... Welcome and Overview to INFORM 2017
& Keynote Speaker Introduction
Jer Ryska Vockley, Pittsburgh PA, USA
16:45-17:45 ... Keynote, The Impact of NBS on FADDs
Nicola Langa, Salt Lake City, Utah, USA
17:45-18:15 ... Discussion
19:00-21:30 ... Networking Reception and Opening Dinner

Monday Morning September 4, 2017
Session 2: Basic Science
7:30-8:30 ... Registration / Breakfast
8:30-9:00 ... Myotubes from CPT2 Deficient Patients as a Disease Model
Fatima Djouadi, Paris, France
9:00-9:30 ... Free Fatty Acid Receptors and the Gut Microbiome: Questions in relation to FAO Defects
Niels Gregersen, Aarhus Denmark
9:30-9:45 ... Endoplasmic reticulum-mitochondria crosstalk and redox homeostasis disruption in very long-chain acyl-CoA dehydrogenase deficient fibroblasts
Bianca Seminotti, Pittsburgh, PA, USA
9:45-10:00 ... Development and characterization of patient-specific iPSC-derived retinal pigmentary epithelia (RPE)-like cells as a model of LCHAD-associated retinopathy
Tiffany DeVine, Portland, Oregon, USA
10:00-10:30 ... Break
10:30-11:00 ... NAD+ Therapy Improves Cardiac Function and Bioenergetics in a Mouse Model of Heart Failure
Matt Hirschey, Duke University, USA
11:00-11:30 ... Lessons Learned from Animal Models of VLCAD Deficiency
Ute Spiekerkoeter, Freiburg, Germany
11:30-11:45 ... VLCAD deficiency related chronic inflammation pattern is suggestive of systemic mediators
Megan Beck, Memphis, TN, USA
11:45-12:00 ... Experimental evidence that fatty acids accumulating in VLCAD deficiency disrupt mitochondrial respiration in heart, liver and brain of young rats
Cristiane Cecatto, Porto Alegre, RS, Brazil
12:00-12:30 ... Panel Discussion
12:30-14:00 ... Lunch