United Leukodystrophy Foundation/ALD-Connect Scientific Meeting
July 31, 2014
Embassy Suites Hotel, 222 St. Paul Place, Baltimore, MD 21202

8:00 AM   Introductions and Welcomes
William Kintner, D.Min, Th.M., President of ULF
William Rizzo, M.D., Chairman, Medical & Scientific Advisory Board, ULF
Ann Moser, B.A., Vice-Chair, Medical & Scientific Advisory Board, ULF
Florian Eichler, M.D., ALD Connect and Medical & Scientific Advisory Board, ULF

**Session 1: Focus on ALD - Moderated by Florian Eichler, M.D.

8:15 AM   Michele Caggana, Sc.D., FACMG – Newborn Screening Program, New York State Department of Health, Wadsworth Center, Albany, NY
Newborn screening for X-ALD: the initial New York experience

8:45 AM   Troy Lund, M.D., Ph.D. – University of Minnesota, Minneapolis, MN
Biomarkers in X-ALD

9:15 AM   John Fink, M.D. – University of Michigan, Ann Arbor, MI
Insight into the pathophysiology of the axonopathy in AMN

9:45 AM   Kathy Zackowski, Ph.D., O.T. - Kennedy Krieger Institute, Baltimore, MD
Gait abnormalities in adrenomyeloneuropathy

10:15 AM  BREAK

10:30 AM  Peter Barker, Ph.D. – Johns Hopkins University, Baltimore, MD
Magnetic resonance spectroscopy in leukodystrophies

11:00 AM  Johannes Berger, Ph.D. – Medical University of Vienna, Vienna, Austria
Pharmacologic induction of ABCD2 as a possible therapeutic approach in X-linked ALD

11:30 AM  Florian Eichler, M.D. – Massachusetts General Hospital, Harvard Medical School, Boston, MA
Disease Modification and Gene Correction in X-ALD

12:00 PM  LUNCH

**Session 2: Focus on other leukodystrophies – Moderated by William Rizzo, M.D.

1:00 PM   Marjo van der Knaap, M.D., Ph.D. – VU University Medical Center, Amsterdam, The Netherlands
A new class of leukodystrophies: defects in mitochondrial and cytoplasmic tRNA synthetases

1:30 PM   Ian Duncan, BVMS, Ph.D. – University of Wisconsin, Madison, WI
Modeling the natural history of Pelizaeus Merzbacher disease
2:00 PM  Mel Feany, M.D., Ph.D. – Harvard Medical School, Boston, MA
Drosophila model of Alexander disease

2:30 PM  Gustavo Maegawa, M.D., Ph.D. – Johns Hopkins University, Baltimore, MD
Development of high-throughput screening assays for therapeutic small molecules for Krabbe Disease

3:00 PM  BREAK

3:15 PM  Nancy Braverman, M.S., M.D. – McGill University, Montreal, Canada
Peroxisomal biogenesis disorders: high throughput screening for drugs that correct peroxisome import and clinical trial of Betaine therapy

3:35 PM  Genevieve Bernard, M.D., MSc, FRCPC – McGill University, Montreal, Canada
PolIII-related leukodystrophies: research update

3:55 PM  Adeline Vanderver, M.D. – Children’s Hospital National Medical Center, Washington DC
Using whole exome DNA sequencing to search for undiagnosed leukodystrophies

4:15 PM  Matthias Eckhardt, Ph.D. – Rheinische Friedrich-Wilhelms University, Bonn, Germany
A new mouse model to study the molecular pathology of Canavan disease

4:35 PM  Reuben Matalon, M.D., Ph.D. – University of Texas Medical School, Galveston, TX and Guangping Gao, Ph.D. – University of Massachusetts Medical School, Worcester, MA
Gene therapy for Canavan disease