Monday, September 27

7:30-8:00  Registration and Continental Breakfast

8:00-8:15  Introduction
Anne Parise (FDA)
John Porter (NINDS/NIH)

8:15-8:45  Keynote Speaker
Kurt Fischbeck (NINDS/NIH)

8:45-10:30  AON Development in Neuromuscular Diseases: Background and State of the Art

DM: Charles Thornton (Univ. of Rochester)
SMA: Adrian Krainer (Cold Spring Harbor Labs)
ALS: Timothy Miller (Washington Univ.)
DMD: Francesco Muntoni (University Col. London)

10:30-10:45  Break

Session 1: Preclinical Studies and Toxicology

Working Group:
Frank Bennett Isis Pharmaceuticals
Don Cleveland Univ. of California San Diego
Scott Henry Isis Pharmaceuticals
David Jacobsen-Kram FDA
Art Levin Santaris Pharma
Judith van Deutekom Prosensa
Dominic Wells Royal Veterinary College
Bruce Wentworth Genzyme

10:45-11:45  Preclinical Experience with AON Drugs in Neuromuscular Disease—ALS
Don Cleveland
Frank Bennett

11:45-12:30  Panel Discussion for Session 1: “Where are the Opportunities and Gaps in Preclinical Development of AON Therapeutics for Neuromuscular Disease?”
Preclinical Studies and Toxicology Working Group Members (plus Lois Freed (FDA))
12:30-1:30 Lunch

1:30 – 2:30 Advanced Case Study: Three Perspectives on Preclinical Considerations for AONs in DMD

DMD Parent: Chuck Riesbeck
Academic Investigator: Annemieke Aartsma-Rus
Corporate AON Developer: Judith van Deutekom

2:30-2:50 Break

Session 2: Biomarkers

Working Group:
Karla Blonsky Biobasix
Tom Crawford Johns Hopkins Univ.
Mike Howard Univ. of Utah
Krista Vandenborne Univ. of Florida
Marc Walton FDA

2:50-3:30 Biomarker Development and Implementation

FDA Perspective on Biomarker Development
Marc Walton

Realities of International Multi-Center Biomarker Development
Karla Blonsky

3:30-4:50 State of the Art in Biomarker Development in DMD, SMA, ALS, and DM

DMD: Eric Hoffman and Krista Vandenborne
SMA: Tom Crawford
ALS: Timothy Miller
DM: Charles Thornton

4:50-5:30 Panel Discussion for Session 2: Where are the Gaps?
Biomarkers Working Group Members

5:30 Adjourn
Tuesday, September 28

7:30-8:00  Continental Breakfast

8:00-8:15  Introduction
          John Porter (NIH)
          Anne Pariser (FDA)

Session 3:  Clinical Trial Design/Endpoints

Working Group:
Kathie Bishop         Isis Pharmaceuticals
Laurie Burke         FDA
Kate Bushby          Newcastle, TREAT-NMD
Giles Campion        Prosensa
Richard Finkel       Children’s Hospital of PA
Jerry Mendell        Nationwide Children’s
Dick Moxley          Univ. of Rochester
Stephen Shrewsbury   AVI Biopharma

8:15-9:15  Clinical Trial Designs for Small Populations
          June Cai (FDA)
          Anne Pariser
          Ed Connor

9:15-10:15 Advanced Case Study: Designing Clinical Trials for Rare Diseases
           Petra Kaufmann (NINDS/NIH)

10:15-10:35 Break

10:35-11:35 Panel Discussion for Session 3
                   Clinical Trial Design/Endpoints Working Group Members (plus Ron Farkas (FDA))

11:35-12:35 Lunch

Session 4:  Patient Registries and Assessing Long-Term Outcomes

Working Group:
John Day             Univ. of Minnesota
Kevin Flanigan       Nationwide Children’s
Berch Griggs         Univ. of Rochester
Ed Kaye              Genzyme
Hanns Lochmuller     Newcastle TREAT-NMD
Anne Pariser         FDA
Vanessa Rangel-Miller Emory University
12:35-12:55  Registries Overview and Partnering Models
            Hanns Lochmuller

12:55-1:15   Case Study on Use of Registries in Long-Term Surveillance
            Ed Kaye

1:15-1:35    Registries for AON Follow-Up in Neuromuscular Disease
            Kevin Flanigan

1:35-1:55    Patient Perspectives on Registries in Neuromuscular Disease
            Vanessa Rangel-Miller

1:55-2:40    Panel Discussion for Session 4
            Patient Registries and Assessing Long-Term Outcomes Working Group Members

2:40-3:00    Wrap-Up: Lessons on Development of AON Drugs for Neuromuscular Disease
            Francesco Muntoni

3:00         Close