TREAT-NMD Conference 2011

— Geneva, Switzerland, 8–11 November 2011 —

Tuesday 8th November

14:00 – 14:30 Opening of conference and introductory keynote
Kate Bushby – Newcastle University, UK

Session one: Patient and industry forums

14:30 – 16:00 Patient forum
Chaired by:
Pat Furlong – PPMD, USA

16:00 – 16:30 Coffee

16:30 – 18:00 Industry forum
Chaired by:
Ed Connor – Children's National Medical Center, Washington DC, USA
Joe Irwin – Lakeside Regulatory Consulting Services Ltd, UK

18:30 – 20:00 Drinks reception with poster viewing

Wednesday 9th November

Session two: Translational research in adult NMDs

Chaired by:
Michael Rose – Kings College London, UK
Benedikt Schoser – Ludwig-Maximilians University in Munich, Germany

08:30 – 10:00 Keynote presentations
Michael Rose – Kings College London, UK
Translational research beyond the laboratory

Michael Shy – Wayne State University, USA
A translational approach to therapeutic development in the inherited neuropathies

Ichizo Nishino – National Center of Neurology and Psychiatry, Tokyo, Japan
Development of therapy for DMRV/hIBM

Charles Thornton – University of Rochester School of Medicine, USA
The current state of myotonic dystrophy translational research

10:00 – 10:30 Panel discussion: Challenges of translational research in NMDs
Moderators : Michael Rose, Benedikt Schoser
Panel members : Mike Shy, Ichizo Nishino, Charles Thornton plus additional panellists

10:30 – 11:00 Coffee

Session three: Natural history, clinical outcome measures and standards of care in paediatric diseases

Chaired by:
Janbernd Kirschner – University Medical Centre Freiburg, Germany
Kathryn North – University of Sydney, Australia

11:00 – 12:30 **Keynote presentations**

Eugenio Mercuri – Università Cattolica del Sacro Cuore, Rome, Italy
Outcome measures in paediatric neuromuscular disorders: old measures and new concepts
Jan Kirschner – University Medical Centre Freiburg, Germany
Registries and care sites in action for Duchenne muscular dystrophy: the CARE-NMD project

Anne Rutkowski – Cure CMD, USA
Congenital muscular dystrophy - clinical trial readiness takes a global village

12:30 – 13:00 **Panel discussion: Natural history, care standards and outcome measures considerations in paediatric diseases**

Moderators: Jan Kirschner, Kathy North
Panel members: Eugenio Mercuri, Anne Rutkowski plus additional panellists

13:00 – 14:00 **Lunch**

14:00 – 16:00 **Poster session (coffee served from 15:30)**

Session four: Gene replacement: production and delivery of therapeutics

Chaired by:
Jerry Mendell – Nationwide Children's Hospital, Columbus, USA
Serge Braun – AFM, France

16:00 – 17:30 **Keynote presentations**

Chris Henderson (invited) – Columbia University, New York, USA

Jerry Mendell – Nationwide Children's Hospital, Columbus, USA
Gene Therapy: Myth, Milestones and the Way Forward

Thomas Voit – Institut de Myologie, Paris, France
Progress towards systemic delivery of AAV-based dystrophin gene correction: The Paris-Evry-Nantes connection

Janice McLaughlin – Newcastle University, UK
Potential familial and patient responses to gene therapy: lessons from current genetics provision in healthcare

17:30 – 18:00 **Panel discussion: The future of gene therapy**

Moderators: Jerry Mendell, Serge Braun
Panel members: Thomas Voit, Janice McLaughlin plus additional panellists

Thursday 10th November

Session five: Emerging technologies and SOPs in diagnostics and biomarkers

Chaired by:
Nicolas Lévy – Faculté de Medecine de la Timone, Marseille, France
Alessandra Ferlini – University of Ferrara, Italy

08:40 – 10:00 **Keynote presentations**

Nicolas Lévy – Faculté de Medecine de la Timone, Marseille, France
Exploring the genome in Neuromuscular disorders : challenges, perspectives, and benefits

Alessandra Ferlini – University of Ferrara, Italy
Biomarkers discovery in neuromuscular diseases by omic procedures

Eric Hoffman – Children's National Medical Center, Washington DC, USA
New approaches to diagnostics and biomarkers

Giuseppe Novelli – University of Rome Tor Vergata, Italy
Clinical Utility and Validity of Genomic Biomarkers

10:00 – 10:30 **Panel discussion: sharing resources**
Moderators: Nicolas Lévy, Alessandra Ferlini
Panel members: Eric Hoffman, Giuseppe Novelli plus additional panellists

10:30 – 11:00 **Coffee**

Session six: Antisense technologies – strategies and successes

Chaired by:
Steve Wilton – University of Western Australia, Australia
Valerie Cwik (invited) – MDA, USA

11:00 – 12:10 **Keynote presentations**
Francesco Muntoni – Institute of Child Health, University College London, UK
Lessons on Development of AON Drugs for Duchenne Muscular Dystrophy

Nathalie Goemans – University Hospitals, Leuven, Belgium
Exon skipping with 2OMePS antisense oligonucleotides in DMD : current clinical trials and future perspectives

Kathie Bishop – Isis Pharmaceuticals, USA
Development of Antisense Oligonucleotide Therapeutics for the Treatment of SMA and DM1

Aurelie Goyenvalle – Oxford University, UK
AAV-U7snRNA mediated Exon-skipping Approach for Duchenne Muscular Dystrophy Therapy

12:10 – 12:40 **Panel discussion: Expectations versus reality**
Moderators: Steve Wilton, Valerie Cwik
Panel members: Francesco Muntoni, Nathalie Goemans, Kathie Bishop,
Aurelie Goyenvalle plus additional panellists

12:40 – 13:30 **Lunch**

13:30 – 15:30 **Poster session**

15:30 – 16:00 **Coffee**

16:00 – 16:40 **Keynote presentation**
Ed Connor – Children's National Medical Center, Washington DC, USA
Muscular Dystrophy and drug development- challenges and opportunities

Session seven: Life quality versus quality of life

Chaired by:
Pauline McCormack – Newcastle University, UK
Thomas Sejersen – Karolinska Institute, Stockholm, Sweden

16:40 – 17:00 **Q&A session: life quality versus quality of life**
Introduced and facilitated by Tom Shakespeare, WHO

17:00 – 17:45 **Keynote presentations**
Tom Shakespeare – World Health Organisation
Disabling barriers: break to include

Gail Geller – Johns Hopkins University, Baltimore, USA
The Paradox of Promise and the Many Faces of Hope in Duchenne Muscular Dystrophy

17:45 – 18:30 **Panel discussion: The realities of participation in clinical studies**
Pauline McCormack, Thomas Sejersen, Tom Shakespeare,
Gail Geller, Robert Palm, Pat Moeschen plus additional panellists

20:00 – 23:00 **Conference Gala Dinner**
for all delegates

Friday 11th November

Session eight: Stem cell therapies – towards clinical trials

Chaired by:
Hanns Lochmüller – Newcastle University, UK
Francesca Pasinelli – Fondazione Telethon, Italy

08:40 – 10:00 **Keynote presentations**

Edward Wirth – Geron Corporation, USA
Human Embryonic Stem Cell-Derived Oligodendrocyte Precursors for the Treatment of Spinal Cord Injury and Neurodegenerative Diseases

Johnny Huard – University of Pittsburgh, USA
Stem Cell Therapies for Duchenne Muscular Dystrophy: Where we have been and where we are going

Chris Denning – Nottingham University, UK
Modelling genetic heart disease using pluripotent stem cells

10:00 – 10:30 **Panel discussion: stem cell therapies**

Moderators : Hanns Lochmüller, Francesca Pasinelli
Panel members : Edward Wirth, Johnny Huard, Chris Denning plus additional panellists

10:30 – 11:00 **Coffee**

Session nine: What is the future for translational research in rare diseases?

Chaired by:
Ségolène Aymé – INSERM, France
Steve Groft (invited) – Office of Rare Diseases, USA

11:00 – 12:00 **Keynote speeches**

Gert-Jan van Ommen – Leiden University Medical Center, Netherlands
Translational Genomics: integrating rare and common diseases

Clemens Müller-Reible – University of Würzburg, Germany
Advances in genome analysis: is more always better?

12:00 – 12:30 **Panel discussion: access to new therapies and technologies**

Moderators: Ségolène Aymé, Steve Groft
Panel members: Gert-Jan van Ommen, Clemens Müller-Reible plus additional panellists

12:30 – 13:00 **Closing keynote and final remarks**

Volker Straub – Newcastle University, UK