

TREAT-NMD Conference 2011

— Geneva, Switzerland, 8–11 November 2011 —

Tuesday 8th November

- 09:00 Registration desk open and setting up of posters
- 14:00 – 14:30 Opening of conference and introductory keynote
Kate Bushby – Newcastle University, UK
with Marianne Wilde

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
Joseph 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
Therapeutic development for the Inherited Neuropathies; a Multidisciplinary, Collaborative Approach
- 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
- 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

Stuart Elborn – European Cystic Fibrosis Society

How care site accreditations can improve outcomes – experience from Cystic Fibrosis

Janbernd 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

CMD: It Takes a Global Village and Perspective

12:30 – 13:00

Panel discussion: Natural history, care standards and outcome measures considerations in paediatric diseases

Moderators: Janbernd Kirschner, Kathryn North

Panel members: Eugenio Mercuri, Stuart Elborn, Anne Rutkowski, Thomas Sejersen (Karolinska Institute)

13:00 – 14:00

Lunch

14:00 – 16:00

Poster session 1 (coffee served from 15:30)

Posters to be presented: CR1 to CR28; DC1 to DC5; OM1 to OM10

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

Jerry Mendell – Nationwide Children's Hospital, Columbus, USA

Gene Therapy: Myth, Milestones and the Way Forward

Markus Rüegg – University of Basel, Switzerland

Therapeutic approaches for myopathies of the extracellular matrix

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, Markus Rüegg, Janice McLaughlin, Kathy Klinger (Genzyme)

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

High Throughput Molecular Screening for Neuromuscular Disorders - the NMD-Chip consortium project

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

10:30 – 11:00

Coffee

Session six: Antisense technologies – strategies and successes

Chaired by:

Steve Wilton – University of Western Australia, Australia

Annemieke Aartsma-Rus – Leiden University Medical Center, The Netherlands

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, Annemieke Aartsma-Rus

Panel members: Francesco Muntoni, Nathalie Goemans, Kathie Bishop,

Aurelie Goyenvalle

12:40 – 13:30

Lunch

13:30 – 15:30

Poster session 2

Posters to be presented: PCR1 to PCR22; REG1 to REG9; SE1 to SE19

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 – PEALS, 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, Geneva, Switzerland
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
- Pat Moeschen – Independent speaker, USA (via video-link)
Not waiting to live, and not living to wait. How I am beating MD
- 17:45 – 18:30 **Panel discussion: The realities of participation in clinical studies**
Pauline McCormack, Thomas Sejersen, Tom Shakespeare,
Gail Geller, Cynda Rushton (Johns Hopkins), Pat Moeschen, Robert Palm (Progena Foundation)
- 20:00 – 23:00 **Conference Gala Dinner**

Friday 11th November

Session eight: Stem cell therapies – towards clinical trials

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

- 09:00 – 10:00 **Keynote presentations**
Johnny Huard – University of Pittsburgh, USA
Exhaustion of Muscle Progenitor Cells in Muscular Dystrophy: Implication for Stem Cell Therapy
- 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 : Johnny Huard, Chris Denning
- 10:30 – 11:00 **Coffee**

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

Chaired by:

Kate Bushby – Newcastle University, UK

Eric Hoffman – Children's National Medical Center, Washington DC, USA

11:00 – 12:00

Keynote speeches

Gert-Jan van Ommen – Leiden University Medical Center, Netherlands

Fundamental, translational and clinical research for NMDs

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: Kate Bushby, Eric Hoffman

Panel members: Gert-Jan van Ommen, Clemens Müller-Reible and Volker Straub

12:30 – 13:00

Closing keynote and final remarks

Volker Straub – Newcastle University, UK