



TREAT-NMD
Neuromuscular Network

International Conference 2015

6-8 December 2015

Cosmos Club
2121 Massachusetts Ave NW
Washington - DC

#TNMD15

Programme overview

Sunday 6th December

2.00pm

Conference opens

- 2.10pm The essential resource in achieving novel therapies for neuromuscular disease: patient-focused drug development
- 4.00pm The current challenges of preclinical research and study design
- 6.00pm Networking reception

Monday 7th December

- 9.00am Innovative trial designs and outcome measures
- 11.00am Biochemical and imaging outcomes
- 2.00pm Early approval and post-marketing, rethinking the registries in orphan disease
- 4.00pm Standards of care

Tuesday 8th December

- 9.00am Registries
- 11.00am International trial readiness and access to emerging therapies

12.30pm

Conference closes



Welcome to Washington

The TREAT-NMD International Conference is again designed to address some of the key challenges facing the neuromuscular community as we move forward with developing new therapies for patients. Each session will be highly interactive with the audience, who will represent all key stakeholder groups: patient organisations, academics, clinicians, industry and regulators. The sessions will briefly address and introduce the state of the art in each area and then interactive discussions with the session participants and audience will address key topics facing the community. As in previous conferences, these discussion sessions ensure there is maximum interaction between the audience and the invited experts.

Sunday - 6th December Conference opens at 2.00pm

*Growing the drug pipeline for neuromuscular diseases:
optimising resources for clinical development of new therapies*

Opening comments

Eric Hoffman

Children's National Medical Centre
USA

Welcome address

Annemieke Aartsma-Rus

Chair of the TREAT-NMD Alliance Executive Committee
Leiden University Medical Centre
The Netherlands



The essential resource in achieving novel therapies for neuromuscular disease: patient-focused drug development

2.10pm - 3.40pm

Session Chair



John Porter

Parent Project Muscular Dystrophy

As we come closer to having therapies for neuromuscular diseases, the issues are becoming increasingly complex. Whether and how patients and their families are engaged in the drug development process, and the level of communication throughout clinical development, will play a pivotal role in success or failure of a therapy development program. Feasibility of clinical trial design hinges on early and frequent patient engagement, while attitudes about acceptable benefits versus risks are becoming an important factor in regulatory approvals.

This session will set the stage for the overall TREAT-NMD conference by providing concrete recommendations for active participation of patients and caregivers in drug development. Three perspectives will be presented. Pat Furlong, from the viewpoint of patient advocacy, will discuss the importance of patient/care provider views and active participation from the early stages of clinical trial design through marketing approval. Marc Boutin, from a regulatory agency and drug developer perspective, will discuss how patients connect to and help drive biomedical breakthroughs through regulatory approval. Finally, Cathy Turner and Olav Velhuizen will discuss outcomes of the TREAT-NMD workshop, "Participants not Subjects: Engaging Patients and Families in Paediatric Clinical Research."



The key message of this session is that success in development of novel therapeutics for neuromuscular disease can happen only if a partnership is created where patients and families have a voice and are involved in decisions and planning rather than simply serving as subjects in clinical trials.

Speakers

Pat Furlong - PPMD, USA

A patient advocacy perspective on engaging patients in drug development

Marc M. Boutin - National Health Council, USA

The role of patients in the regulatory process

Cathy Turner & Olav Veldhuizen - Newcastle University, UK

Participants not subjects: The outcomes of a TREAT-NMD workshop on engaging patients in research

Panel discussion with speakers



Coffee break

3.40pm - 4.00pm



The current challenges of preclinical research and study design

4.00pm - 5.30pm

Session Chairs



Kanneboyina Nagaraju

Children's National Medical Centre, USA



Raffaella Willmann

Swiss Foundation for Research on Muscle Diseases

Panel Discussion Moderator



Markus Rüegg

University of Basel, Switzerland

In the last years, several treatment approaches for neuromuscular diseases entered clinical trials or pilot trials. The preclinical phase often delivers the data that justify the trials and therefore supports patients' hopes and expectations.

Poor experimental design, conduct, analysis and reporting often contribute to the false positive results. Challenges of study design and of animal model limitations will be discussed.



Speakers

Jonathan Kimmelman - McGill University, Canada

Validity threats in pre-clinical research

Annamaria De Luca - University of Bari, Italy

Translational research in DMD: state of art and bottlenecks of the mdx mouse model

Arthur Burghes - Ohio State University, USA

Testing treatments for spinal muscular atrophy in animal models: SMN inducers and other possible treatments

Summary and closing remarks

Richard Harris - NPR (National Public Radio)



Networking reception

6.00pm - 10.00pm
(further details on next page)



Sunday 6th December

Networking reception

6.00pm - 10.00pm

The Council of Insurance Agents & Brokers
701 Pennsylvania Avenue NW, Suite 750
Washington, DC 20004

Food and drinks will be served throughout the evening

We would like to take this opportunity to thank
Joel Wood and the Foundation to Eradicate Duchenne
for making this reception possible.



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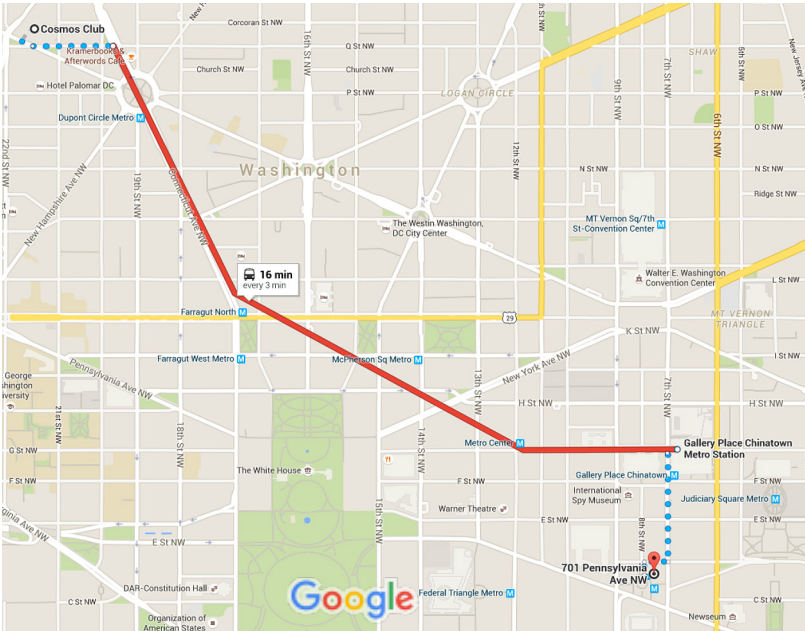


Getting to the reception

- Metro -

Take the Red Line for 3 stops from DuPont Circle to Gallery Place/Chinatown.

The DuPont metro stop is right on DuPont circle, about a block from the Cosmos Club. The CIAB offices are two blocks south of Gallery Place metro stop. The metro journey should only take a few minutes and cost about \$2 or \$3.



- Walk -

A very nice 2 mile walk through the center of DC. that will take about 30-40 minutes.



Monday 7th December

Innovative trial designs and outcome measures

9.00am - 10.30am

Session Chairs



Joel Schneider
Solid Biosciences, USA



Petra Kaufmann
NCATS, USA

The accelerating pace of clinical development for rare neuromuscular diseases is accompanied by unique sets of challenges and opportunities for all stakeholders. As more drugs enter into trials, and both patient and clinical trial center availability become increasingly prevalent issues, streamlining clinical testing will be a necessity.

As we learn more about the clinical progression of neuromuscular diseases, opportunities to refine endpoint selection and trial design can make clinical trials more efficient. This session will address how recent advances in the utilization of natural history, novel biomarkers and statistical methods are evolving clinical trial design.



Speakers

Craig McDonald - University of California, Davis, USA
Prognostic endpoints and innovative DMD trial design
(via online conferencing)

Susan Ward - Independent Advisor
Patient trajectories of disease progression in DMD – overcoming
the challenges of heterogeneity

Chris Coffey - University of Iowa, USA
Innovative trial designs for rare diseases such as DMD

Charles Mohan - The United Mitochondrial Disease Foundation, USA
Trial readiness – patient organization perspective

Joyce Kullman - Vasculitis Foundation, USA
Integration of the researcher, clinician and patient perspective

Panel discussion with speakers



Coffee break

10.30am - 11.00am



Biochemical and imaging outcomes

11.00am - 12.30pm

Session Chairs



Carsten Bonnemann

NINDS, USA



Volker Straub

Newcastle University, UK

The role of biomarkers in rare neuromuscular diseases for clinical diagnosis or treatment response is becoming more important and their use drives research towards personalized medicine. Biomarkers offer an invaluable tool for monitoring disease progression, prognosis and response to drug treatment during clinical trials. They can represent excellent substitutes for clinical endpoints because they can predict a clinical benefit more quickly than other measures and in a less invasive way than muscle biopsies.

In addition, diagnosis can happen earlier because testing for biomarkers is quicker and easier than genetic testing, disease progression can be accurately measured allowing better clinical management of symptoms and existing treatments (including drug dosage) can be adjusted to precisely meet the needs of individual patients to ensure they get the maximum benefit.



Speakers

Pietro Spitali - Leiden University Medical Centre, The Netherlands
Selecting biomarkers through integrated omics technologies

Yetrib Hathout - Children's National Medical Centre, USA
Serum biomarkers in dystrophinopathies

Lee Sweeney - University of Florida, USA
Muscle MRI as an outcome measure in DMD

Giorgio Tasca - Catholic University School of Medicine, Italy
Understanding FSHD pathology by MRI



Lunch break

12.30pm - 2.00pm



Early approval and post-marketing, rethinking the registries in orphan disease

2.00pm - 3.30pm

Session Chairs



Lauren Morgenroth

CINRG, USA



Filippo Buccella

Parent Project Onlus, Italy

With very few drugs available to treat patients with inherited neuromuscular diseases, the main focus of the field has been the move to trial readiness and the support of clinical and pre-clinical studies. However we are now reaching the point where the next phase of drug delivery becomes highly relevant. This means entering into new partnerships and relationships to understand the mechanisms whereby drugs will be made available in different healthcare systems and how information will continue to be collected on the safety, efficacy and optional use of these drugs.

Post-marketing surveillance traditionally refers to the collection of patient data on the safety of a drug that is marketed. More recently, accelerated approval mechanisms increasingly embraced by both FDA and EMA are moving phase 3 efficacy studies into the post-marketing space. In the orphan disease space, it is increasingly recognized by regulators, patient organizations, and clinical investigators that a shared disease-focused infrastructure for post-marketing of many or all drugs will be necessary. A coordinated approach will provide conservation of patient, family and physician resources.



Monday 7th December

In this session we will address the post-marketing needs for inherited neuromuscular diseases. We will present initiatives being started by the EMA and learn about the experience of another orphan disease: Cystic Fibrosis, and how they have established a disease platform.

Speakers

Stefano Marini - President, European CRO Federation, Italy
Road-Map to a successful disease registry generating linked post-marketing treatment registries

Chris Dowd - Cystic Fibrosis Foundation, USA
Alternative uses for patient registries. A look into the utilization of a chronic disease patient registry in post-marketing, FDA mandated studies



Coffee break

3.30pm - 4.00pm



Standards of care

4.00pm - 5.30pm

Session Chairs



Nathalie Goemans

University Hospitals Leuven, Belgium



Cynthia Gagnon

Université de Sherbrooke, Canada

Therapeutic developments in neuromuscular disorders have highlighted the impact of care and management on the outcomes currently measured in clinical trials. Implementation of standards of care is a prerequisite for any therapeutic trial.

This session gives an update on the standards of care and guidelines for different neuromuscular disorders in the context of clinical trial readiness.



Speakers

Bernard Brais - Université McGill, Canada
Standards of care in OPMD

Nicholas Johnson - University of Utah, USA
Standards of care in pediatric DM1

Cynthia Gagnon - Université de Sherbrooke, Canada
Standards of care in adult DM1

Richard Finkel - Nemours Children's Hospital, USA
Standards of care in SMA and related implications for clinical trial

Michela Guglieri - Newcastle University, UK
Standards of care, their application and the impact for trials in
DMD



Free evening



Tuesday 8th December

Registries

9.00am - 10.30am

Session Chairs



Hugh Dawkins

Department of Health, Australia



Hanns Lochmüller

Newcastle University, UK

Commentator



Jan Verschuuren

Leiden University Medical Centre, The Netherlands

Patient registries have proven to be instrumental for clinical research in neuromuscular disorders, and have proven their utility to assess feasibility and assist recruitment for clinical trials in DMD and SMA. They are also facilitating further research in conditions such as myotonic dystrophy, FSH dystrophy, CMT and other rare NMD. New developments for the TREAT-NMD registries include post-marketing surveillance and integration of next-generation –omics data with clinical phenotypes.



Speakers

Hanns Lochmüller - Newcastle University, UK

The role of neuromuscular registries in -omics research and RD-Connect

Lawrence Korngut - University of Calgary, Canada

The Canadian model of modular NMD registries, supported by the Canadian neuromuscular network

Petra Kaufmann - NCATS, NIH, USA

NCATS perspective on registries



Coffee break

10.30am - 11.00am



International trial readiness and access to emerging therapies

11.00am - 12.30pm

Session Chairs



Eric Hoffman

Children's National Medical Centre, USA



Emil Kakkis

Ultragenyx, USA

Increasing numbers of drug development programs in neuromuscular disease put increasing demands on patients, families and the clinical teams, as well as companies working to meet demands for post-marketing programs and early/expanded access (compassionate use). It is important to ensure that these resources are efficiently utilized to bring the most drugs to market to the most patients in the fastest time. This session discusses emerging innovations in clinical trial design and conduct, in both pre-marketing and post-marketing spaces, as well as expanded access programs.



Speakers

Hiroki Morizono - Children's National Medical Centre, USA
A mobile health toolbox for outcomes in the community setting

Emil Kakkis - Ultragenyx, USA
Early access programs for orphan drugs

Lori Reilly - Executive Vice President, PhRMA, USA
Economics and policies surrounding pricing and access to orphan drugs

Nabarun Dasgupta - Harvard Medical School, USA
Epidemico; social media reporting of adverse events

Michelle Eagle - Newcastle University, UK
Tina Duong - Stanford University, USA
International clinical evaluator certification to improve reliability of clinical trials



Closing remarks

12.30pm - 12.40pm

Kevin Flanigan

Incoming Vice-Chair of the TREAT-NMD
Alliance Executive Committee



We would like to take this opportunity to thank the following sponsors for helping to make this conference possible



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