16TH INTERNATIONAL CONGRESS ON NEUROMUSCULAR DISEASES (ICNMD)

Roche- and Sarepta-sponsored virtual industry forum

GENE TRANSFER THERAPY:

addressing the unmet needs of Duchenne muscular dystrophy (DMD)

Saturday 29 May 2021 | 17:15–17:35 CEST

The aim of gene therapy is to prevent or slow the progression of genetic disorders.

Research is ongoing to address the need for effective therapeutic options for DMD. Gene transfer therapy has the potential to target the genetic root cause of DMD by delivering a shortened version of dystrophin and restoring its function in muscle cells.

Please join us for this virtual industry forum, where we will explore how gene therapy may be applied to address the unmet needs of genetic disorders, such as DMD, and the challenges and outstanding questions on using gene transfer therapy to treat DMD.

We look forward to welcoming you to our virtual Roche- and Sarepta-sponsored virtual industry forum!

FACULTY

Professor Wolfgang Müller-Felber, Dr von Hauner Children's Hospital, Munich, Germany

AGENDA TOPICS

- Key milestones in the history of gene therapy
- An overview of gene therapy
- Gene transfer therapy for Duchenne: opportunities and challenges
- Considerations around the durability of gene transfer therapies
- Summary and close

This forum is intended for healthcare professionals only.