7th CONGRESS OF THE EUROPEAN ACADEMY OF NEUROLOGY

Roche-sponsored virtual industry forum

GENE THERAPY:

addressing the unmet needs of neuromuscular disorders

Sunday 20 June 2021 | 16:15–16:30 CEST

The aim of gene therapy is to treat, prevent or slow the progression of genetic disorders. This complex therapeutic technology is gaining momentum as new evidence of its potential efficacy accumulates from early successes in clinical trials and examples of regulatory approvals increase.

Duchenne muscular dystrophy (DMD) is an X-linked genetic disorder caused by the lack of functional dystrophin, an essential component of a protein complex that strengthens and protects muscle fibres, resulting in muscle weakness and wasting, and premature death. Gene therapy has the potential to target the root cause of DMD by increasing the expression of a shorter version of dystrophin (microdystrophin) and restoring its function in muscle cells.

Please join us for this virtual industry forum, where we will explore how gene therapy works, its essential components and delivery approaches, as well as how gene therapy may be applied to address the unmet needs of genetic disorders, such as DMD.

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

FACULTY

Professor Maggie Walter, Ludwig Maximilian University, Munich, Germany

AGENDA TOPICS

- Experiences from carers and people living with DMD
- An overview of gene therapy
  - Definition of gene therapy, key components and delivery systems
- Key considerations about gene therapy
  - The role of the immune response in treated individuals
- Gene therapies for the treatment of neuromuscular disorders such as DMD