

Tentative Outline

Special/Thematic Issue for the journal "Current Gene Therapy"

Title of the Thematic Issue:

RNA interference and neurodegenerative diseases: time is muscle, time is nerve

Guest Editor: Marco Ceccanti

Scope of the Thematic Issue:

Neuromuscular diseases are intriguing and severe disorders affecting both the nervous system and the muscles. Some of them are considered rare diseases and the develop of effective treatment was kept aloof for many years. In the last decade, the pharmaceutical engineering and the new RNA interference-based therapies, such as small interfering RNA (siRNA) and antisense oligonucleotides (ASO), gave a new hope to researchers and patients. Among all, Duchenne dystrophy, spinal muscular atrophy (SMA), hereditary transthyretin-mediated amyloidosis (hATTR) and, in a short time, SOD1 amyotrophic lateral sclerosis (ALS) are diseases where RNA interference has changed life expectancy and quality of life.

Once the veil of Maya is torn, the new critical question is about the timing for starting these new treatments. "Time is muscle, time is nerve": almost all the available treatments can help to stop or slow down the disease progression, but the muscle (or nerve) lost is lost forever. In this setting, the early diagnosis takes on even greater role than in the past and our positions about genetic tests on the asymptomatic relatives should be discussed again.

Keywords: Neuromuscular diseases, spinal muscular atrophy, hereditary transthyretin-mediated amyloidosis, amyotrophic lateral sclerosis, Duchenne dystrophy, asymptomatic carriers, early diagnosis

Sub-topics:

The sub-topics to be covered within the issue are given below:

- Neuromuscular diseases
- Early diagnosis
- Role of asymptomatic carriers
- Screening programs

Schedule:

- ✧ Thematic issue submission deadline: June 30, 2023
- ✧ Manuscript submission deadline: Dec 31, 2023
- ✧ Peer Review Due: Feb 28, 2024
- ✧ Revision Due: May 31, 2024
- ✧ Final manuscripts due: June 30, 2024

Details of Guest Editor:

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