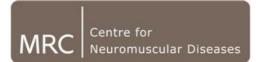


MYOPATHIES AND HEART INVOLVEMENT









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Francesco Muntoni: disclosures

Duchenne

- CI of 4 antisense oligonucleotides clinical trials with Sarepta.
- PI of Sarepta Phase III AAVrh74 micro-dystrophin
- CI of Genethon AAV8 micro-dystrophin

Spinal Muscular Atrophy

- PI of Ionis/ Biogen antisense oligonucleotide nusinersen Phase III study (Shine)
- PI of Avexis AAV9 phase III trial in SMA 1 (STRIVE-EU)-PI of Avexis AAV9 phase III trial in presymptomatic SMA 1-2 (SPRINT)
- PI of Roche risdiplam phase III trial in SMA 2 and 3 (Jewelfish)

Myotubular Myopathy

- PI of upcoming AAV8 MTM1 gene replacement trial
- · Other financial disclosures
- Member of Pfizer rare disease SAB
- SAB participation for Avexis/Novartis, Biogen, Dynacure, Dyne Therapeutics, Roche, Sarepta



Cardiac involvement in myopathic conditions

Dilated cardiomyopathy

Cardiac conduction system defects

Hypertrophic cardiomyopathy

Restrictive cardiomyopathy



Cardiac involvement in myopathic conditions

- Role of different class of proteins on risk to develop specific types of cardiomyopathy
 - Importance of precise diagnosis for individualised monitoring and management plans, and implementation of standards of care
- Multidisciplinary involvement of
 - neurologist / pediatrician
 - cardiologist
 - respiratory physician
 - palliative care physician
- · anaesthetist
- · Role of experimental therapies under development



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