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Stakeholder collaboration for spinal muscular atrophy therapy development

In a 2016 Policy View,[1] we highlighted the importance of collaborative efforts and a constructive dialogue among patient representatives, academics, industry, and regulators for streamlining new therapy development in rare diseases, using the example of Duchenne muscular dystrophy. This approach was applied to the field of spinal muscular atrophy (SMA) in a stakeholder meeting recently co-organized by SMA Europe, the European Medicines Agency, and the TREAT-NMD alliance.

For more on the **stakeholder meeting** see http://www.ema.europa.eu/docs/en_GB/document_library/Agenda/2016/11/WC500215691.pdf

SMA is a motor neuron disorder of severe unmet medical needs, particularly in children. Patients with the most severe type I SMA never achieve the ability to sit independently and, without major supportive care, die before the age of 2 years. Patients with the milder type II or type III disorder survive longer, but have progressive disability. Several topics discussed at the stakeholder meeting were the same as those identified in the Duchenne muscular dystrophy discussion, including variability of standards of care; need for developing outcome measures based on what patients find important; collecting natural history; and identifying and validating appropriate tools for efficacy assessment, reflecting objective and clinically significant outcomes.[1]

Other topics were more specific to SMA, including the choice of appropriate controls in clinical trials, the huge burden for families involved in around the clock care for their child, and how trial participation contributes to this burden. The use of placebo in trials of SMA was extensively discussed, and it was recognised that, for SMA type I patients in homogeneous populations with a short lifespan and a well-characterised disease progression, use of a placebo control might not always be required if a large therapeutic effect is expected, and single arm trials might be acceptable. However, randomised placebo-controlled trials might be necessary for disorders such as SMA type II and type III, due to variability in clinical features and disease progression in those patients. Important new data were also presented at the meeting, including the results of a preference study in patients with type II or type III SMA, and the first work on potential definition of clinical minimally important changes for the most commonly used scale in SMA.[2]

With the potential of having drugs approved for SMA in the near future, the landscape for SMA will change. This event laid the foundations for a continuous dialogue among stakeholders, essential to tackle the outstanding issues in this devastating disease.

AA-R is employed by Leiden University Medical Center, which has patents on exon skipping technology; as co-inventor of some of these patents, AA-R is entitled to a share of royalties. AA-R also reports grants from ZonMw, Duchenne Parent Project Netherlands, Prinses Beatrix Spierfonds, European Union 7th Framework Programme, and Parent Project Muscular Dystrophy. FM is a member of a scientific advisory board for Pfizer; reports grants from Roche, Isis/Biogen, GOSH Charity, Spinal Muscular Atrophy Trust, and Wellcome Trust; and has received personal fees from Roche, Biogen, AveXis, and Pfizer. RF is a member of a scientific advisory board for AveXis and Roche; is a consultant for Biogen, Ionis, AveXis, Roche, and Novartis; and reports grants from Biogen and Ionis. All other authors declare no competing interests.

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