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PRESS RELEASE

TREAT-NMD, EMEA and patient group workshop on Antisense Oligonucleotide therapies in Duchenne muscular dystrophy (DMD) signals collaborative approach to the issues of personalized medicine

On 25 September 2009 a broad group of 98 DMD experts assembled by TREAT-NMD – including clinicians involved in current clinical trials, patient/parent groups and pharmaceutical companies working on Duchenne therapies – met at the London offices of EMEA, the European Medicines Agency, to begin a dialogue on the regulatory issues surrounding the unprecedented level of personalization inherent in antisense oligonucleotide therapies for genetic conditions like DMD. EMEA representatives included the chairs and members of the committees for Human Medicinal Products (CHMP), Paediatrics (PDCO), Advanced Therapies (CAT), Orphan Drugs (COMP) as well as members of the Scientific Advice Working Party (SAWP) and senior members of the EMEA secretariat. Furthermore, representatives from the Standing Committee for European Doctors (CPME) and EMEA eligible patient organizations such as the European Genetic Alliances' Network (EGAN) and the International Alliance of Patients' Organizations (IAPO) participated in the workshop. Input from Duchenne patient groups and experts worldwide ensured that there was global representation at the meeting, which also included a representative of the US Food and Drug Administration (FDA).

Recent promising preclinical and clinical trial results of antisense oligonucleotides (AOs) as a therapy for boys affected by DMD suggest that this novel approach could provide a therapeutic option for the majority of affected individuals. Studies that will hopefully lead to the registration of the first of these compounds, which are designed to skip exon 51 of the DMD gene, will start in the near future. Although more than 80% of Duchenne boys could potentially benefit from the “exon skipping” approach, each exon targeted requires a unique AO, and thus treating all the different mutations known to cause DMD requires the development of large numbers of AOs, each treating only a small subset of the patient population. This represents a personalized approach to therapy that is currently

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without precedent for a genetic disease. It is the concern of advocacy groups and scientists in the field that if the standard regulatory and product development path of taking new drugs into the clinic has to be followed for each individual AO, this will threaten the viability of this promising approach.

The meeting with EMEA therefore sought to take steps to identify a pathway that will allow the safe and efficient progress of these drugs through the approval process. Extensive preparatory work prior to the meeting ensured that the DMD expert scientist and advocacy groups were able to present a united voice to the regulatory authorities, clearly identifying the areas of concern whilst seeking to partner with EMEA to guide future developments. This is the second time that TREAT-NMD has convened a broad, strategic meeting with the regulatory authorities, and the approach has been welcomed as a unique forum for discussion that benefits both sides.

“The usual procedure is for pharmaceutical companies to apply for marketing authorization for each drug individually, but it was clear that for some of these new therapies a far broader discussion was needed to establish the overarching strategies and find out how the regulatory authorities would view applications for these highly similar compounds,” said TREAT-NMD partner and workshop chair Francesco Muntoni. “We are delighted that EMEA have been so willing to interact with the Duchenne community and are confident that by dealing with these issues openly and constructively now we can help smooth the pathway of all these therapies towards approval without compromising on safety or efficacy.”

Following the meeting Pat Furlong, Founding president & CEO of Parent Project Muscular Dystrophy noted the landmark status of this meeting for the field. “It was brilliant. To be honest, as we crossed the Thames in the ferry, I realized it was quite symbolic. Thank you for including me in this historic meeting”.

The regulatory experts also underlined the success of the workshop and Dr. Agnes Saint Raymond, Head of Sector for Scientific Advice, Orphan Drugs and Paediatric Medicinal Products at the EMEA, added "the EMEA and its Committees welcome the opportunity to continue the dialogue with Treat-NMD as part of their exchanges with Patients' organisations and Health professionals. These are promising times for children and adults with Duchenne and Regulatory authorities strongly encourage early collaboration in drug development to maximise the chances of success at approval time."

Meeting Outcomes

During the workshop, EMEA representatives indicated that they would be willing to be flexible; with the help of the tools and procedures they have in place, and are prepared to be engaged in more detailed discussion regarding the development of a regulatory pathway for approval of future exons. The regulatory experts stressed that they are willing to discuss alternative ways forward for very small populations. Furthermore it was noted that even though each exon may have to be approved individually it may not be necessary to do separate studies for each of them as many data can be shared and/or data can be extrapolated. However it was pointed out that regulators will always need sufficient data to evaluate the medicinal product and conclude on the benefit-risk balance. To ensure that adequate data for approval of the medicinal products are collected the regulatory experts strongly recommended that sponsors discuss early with the SAWP and the PDCO and agree on the trials before they are conducted. Moreover there are fast regulatory procedures of approval for medicinal products especially if they are life saving and there is an unmet medical need. The EMEA invited the community to approach them early in the drug development and emphasised that collaboration of the community is key, complimenting the community on its harmonisation.

Following the meeting TREAT-NMD will produce a workshop report for publication and maintain dialogue with EMEA to help respond to the needs of the community. Further information is available to interested parties via the TREAT-NMD coordination office.

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Notes to Editors

About TREAT-NMD

TREAT-NMD is a Network of Excellence, coordinated from Newcastle University UK, facilitating collaborative research in neuromuscular disease that aims to create the infrastructure to ensure that the most promising new therapies reach patients as quickly as possible. The network brings together the key players in the neuromuscular field and is a natural partner for biotech and pharmaceutical companies developing new therapeutics for neuromuscular conditions. Its tools and services and access to an unparalleled depth of expertise can support, simplify and accelerate the trial and approval process. Its suite of services includes defined and recruitable patient cohorts obtained through its global patient registries, experienced trial sites accessed through its care and trial sites registry, outcome measures validated for NMDs, regulatory advice, GCP training, clinical evaluator training, care standards generation, and advisory board setup.

For more information, please visit: www.treat-nmd.eu

About Patient Organisations involved in the meeting

United Parent Projects Muscular Dystrophy (UPPMD) is owned and managed by parent project organisations set up by parents of children with Duchenne Muscular Dystrophy in many different countries all over the world. These national projects are run by parents for parents. UPPMD is managed by parents you have chosen to lead your national parent projects. The organisation came together after we realised that there was no sense in working in isolation from each other.

We all share the same aims and dreams and they can be realised more efficiently using collective experiences and resources. UPPMD is made up of patient organisations whom attended the meeting including Action Duchenne UK, Parent Project Muscular Dystrophy US, Dutch Duchenne Parent Project, Parent Project Onlus Italy, Duchenne Ireland and Parent Project Australia. In addition to UPPMD, patient organisations including Muscular Dystrophy Campaign UK, Charley's Fund USA, Cure Duchenne USA and Muscular Dystrophy Association attended and contributed to the workshop.

For more information, please visit: www.uppmd.org

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