TREAT-NMD AND THE ICC: A FRUITFUL COLLABORATION TO FOSTER CLINICAL TRIALS AND RESEARCH IN SMA

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One of the objectives of TREAT-NMD is to focus and reach consensus on the organization of clinical trials in SMA. This goal is pursued through the strong and continuous interaction with the US colleagues working under the auspices of the International Coordinating Committee (ICC) group.

Since TREAT-NMD start in 2007, a few milestones in SMA field have been reached. 1) a Consensus Statement on Standards of Care for SMA generated by an ICC working group has been disseminated internationally via ICC and TREAT-NMD. 2) a general consensus on Outcome measures (OMs) for evaluating patients with SMA has been developed. The consensus-building process went through the analysis of several factors, including available data and literature on natural history, validation processes, use of the scale worldwide (info collected by ICC). A TREAT-NMD-led workshop was hosted by EMEA (London, 10/13/2008) to set a collaborative agenda for future trials in SMA involving 50 representatives (healthcare professionals, scientists, patients and pharmaceutical industry) and EMEA Committees' representatives. Participation by the ICC ensured global representation at the meeting, the outcomes of which will also be shared with the FDA. This meeting was a key milestone in the process of developing consensus on OMs and endpoints and can be seen as the start of a longer dialogue on regulatory issues on rare neuromuscular disorders. The joint work on OMs continued in 2009, with a meeting held in Rome in April: the opportunity provided by SMA Europe to further validate (and cross validate with QoL questionnaires) the existing measures is an important chance to put our best foot forward toward these goals. Agreement was reached on next steps for the motor function scales and neuromuscular modules of the PedsQL for SMA type II. A final protocol for European studies should be available in the next month and there are plans for training sessions in a short period.

New initiatives are under development that will strengthen more the interactions between TREAT-NMD and the ICC group, dealing with validation of genetic biomarkers, identification of topics for training and education of professionals and of potential for multinational clinical trials for SMA.