

TREAT-NMD DATABASE CURATORS MEETING, 11 JUNE 2007

Meeting report by Hanns Lochmüller

On June 11 2007, the first TREAT-NMD workpackage 04.2 curator meeting took place at the Eurordis headquarters in Paris. The meeting was convened by Christophe Beroud (work package leader) and Hanns Lochmüller (activity leader), and attended by representatives of participating members (UNEW, MD-NET, FTELE, AFM, INSERM, NIEH) as well as guests from Switzerland and the US.

The aim of WPO4.2 is to develop and manage supranational patient registries/databases for neuromuscular disorders, in particular Duchenne Muscular Dystrophy (DMD) and Spinal Muscular Atrophy (SMA). The curator meeting reviewed and compared already existing registries, agreed on the mandatory core set of data, discussed the legal and ethical framework, and developed a plan for implementation of the European registries on DMD and SMA.

The primary objective of the European TREAT-NMD registries on DMD and SMA is to assess feasibility and to aid planning and recruitment of clinical trials. Other secondary objectives may include studies of epidemiology, genotype-phenotype correlations, natural history, standards of care, etc. For SMA, national registries do not exist in Europe. For DMD, national registries exist in France, the UK, and the Czech Republic. These registries are not currently harmonized. They use different modes of data collection and updating, different levels of data protection and informed consent, and collect different sets of data (for an overview see www.treat-nmd.eu/biobanks). National curators for the TREAT-NMD registries on DMD and SMA will be located in Newcastle, Montpellier, Ferrara, Munich and Budapest. Already existing and new registries will be integrated and feed into the European meta-database. Collaborations have already been set up between TREAT-NMD, PPUK, the United Dystrophinopathy Project run from the University of Utah, and the Czech Parent Project.

A simple mandatory set of data was discussed and agreed on for SMA and DMD. This was aided by previous discussions on the TREAT-NMD website forum and by the 152nd ENMC workshop in Naarden. The mandatory data items that are to be uniformly collected by national registries for each DMD and SMA patient are as follows: personal and contact data of the patient, mutation, ambulation, steroid use and respiratory status. National registries are encouraged to run optional modules such as assessment of standards of care and to coordinate this action with other TREAT-NMD activities.

The legal and ethical framework was explained by S Geismann (lawyer, Germany) and C Roy-Toole (barrister, UK). Patient registries are regulated by EU directive 95/46/EG on data protection, which has been implemented by national legislation in all member states and secures a very similar level of data protection in all countries within the EU. In general, personal data is protected, and there are fewer requirements for irreversibly anonymised data. The general principles are accuracy of data and minimisation of data. However, if the research (database) is expected to have a direct impact

on patients, irreversible anonymisation is not appropriate, since it is necessary to retain some way of linking the data back to the individual patient (for example to notify them that they may be a suitable candidate for an upcoming clinical trial). This is in keeping with intended TREAT-NMD operations and demanded by participating patients and patient organizations. Accordingly, it was agreed on to use pseudonymised (encrypted) data and to implement informed consent procedures. Feedback to patients could include the dissemination for example of information on standards of care or the opportunity to take part in clinical trials. The EuroBioBank informed consent may serve as a template.

The following steps and timelines for integrating existing and building up new national registries were set out: before the end of July, each of the new national registries will decide on the most appropriate "mode of operation". Existing registries will develop a plan for implementing necessary modifications. A plan for technical implementation on the national level will be developed by national registries and supported by the work package leader in Montpellier. Further actions include information and involvement of national patient organizations and clinicians, seek IRB (ethics board) approval for new registries or amending IRB approval for existing registries, translation and adaptation of informed consent, patient information, questionnaire and other documents, hiring of curator personnel, and curator training (November 2007). Furthermore, the group suggested developing a charter for the TREAT-NMD databases that will be presented to the Governing Board. The charter shall set out guidelines and principles including access rights and intellectual property rights and act as a "best practice" model for the future.

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