



Meeting Report: Second annual curators' training course and inaugural meeting of the Global Database Oversight Committee, 5–6 September 2008



Sixty participants from twenty different countries gathered in the Mediterranean resort of La Grande Motte near Montpellier for the second annual TREAT-NMD registry curators' training meeting, followed by the inaugural meeting of the Global Database Oversight Committee. The meeting was well organized and hosted by Professor Christophe Bérout and his Montpellier team. The TREAT-NMD global database for DMD and SMA receives patient details from individual national registries across the world, and this meeting provided an opportunity for the individuals involved in setting up the registries and ensuring the quality of the data they contain (the "database curators") to come together to share experiences and learn new techniques.

Highlights of the first training day included a presentation by Stefanie Possekel of Santhera Pharmaceuticals, who provided a valuable industry perspective on the registries initiative. Stefanie described the pharmaceutical research and development process and showed how a smoothly running global database with accurate, up-to-date information on patients' genetic mutations and clinical condition could greatly facilitate trial feasibility studies for pharmaceutical companies, providing information on the numbers and locations of suitable patients in a matter of days or weeks. Once a trial is ready to be launched, the individual national registries can then be used to disseminate information on the trial to suitable patients, which again saves valuable time in the recruitment process. Earlier in the summer, Santhera had already provided an opportunity for the global database to be piloted when they requested information for a feasibility study for a trial they are planning.

Case studies showing how different countries have tackled setting up a national registry in very different ways revealed the benefits of choosing the method that is most appropriate for a particular national situation. In Germany, an online system where patients can self-register has proved very successful, while in Bulgaria, clinicians at the Alexandrovska University Hospital have taken on the challenge of setting up a registry themselves as part of a broader initiative to create a national "Neuromuscular Centre", for which they were successful in obtaining funding from the Bulgarian government. The Bulgarian example also shows how the registries initiative is able to catalyse other efforts at a national level to improve diagnosis and care standards and how this type of approach can be successful in securing government funding. Further case studies highlighted the development of a national DMD patient registry in Japan and the re-launch of the International SMA registry hosted by the University of Indiana. Representatives of Duchenne Connect and the United Dystrophinopathy Project (both U.S.A.) reported on the outcome of a recent meeting including additional North-American partners and TREAT-NMD. This meeting identified important areas of further development, in particular strategies to address data duplicates and different levels of genetic testing.

Participants were split into “beginners” and “advanced” groups to receive training in the UMD genetic database system (see www.umd.be for further information). This system will be used to run the TREAT-NMD global database, although national registries are free to use any system they choose. Other sessions focussed on the technical requirements for uploading pseudonymised data from the individual national registries to the global database, and on the publicity support available from the TREAT-NMD coordination office to anyone in the process of setting up a registry.

Any initiative dealing with sensitive patient information has to have a robust system to ensure patients’ personal and medical data is protected. This is dealt with on a national level by the individual registries, who have obtained all necessary ethical approvals, and by the establishment of an Oversight Committee for the global database. Each national registry sending data to the global database is entitled to send a delegate to the Oversight Committee, which also includes patient organisation representatives and ethics experts. The committee is responsible for ensuring the registry is run to the required standard and also reviews all applications for information from the database from researchers and pharmaceutical companies. This is of course an essential step to protect sensitive data, but since it is in patients’ interests as well as the interests of trial planners to ensure the process is not hindered by excessive bureaucracy, the committee has agreed to respond to all enquiries within two weeks. Most decisions will therefore be made “electronically”, by email, web or teleconference. It will, however, convene in person once a year, and this meeting will normally coincide with the annual curators’ training meeting.

With this inaugural meeting having established the terms of reference for the functioning of the global database and its oversight committee, the database is now officially able to accept enquiries from companies and researchers. A website dedicated to the global database will come online in the near future, but anyone interested in the interim should contact Hanns Lochmüller (hanns.lochmuller@ncl.ac.uk) in the first instance.

For further information on the TREAT-NMD registries initiative, visit <http://www.treat-nmd.eu/patientregistries/>

If you are interested in setting up a registry in your own country, much useful information can be found in the online “registries toolkit” here: <http://www.treat-nmd.eu/registriestoolkit>, and we also encourage you to contact us at info@treat-nmd.eu so we can provide you with support.