

Building an International Registry of MLD Patients

Description of the objectives and schedule of the task force

Aims

The aim of the registry is to collect information on the natural course of MLD. From this information secondary endpoints should be defined to measure the effect of interventions, and to find candidates for experimental therapies.

Proposed procedure

An MLD Registry Task Force is to be created.

The registry is developed in a two-step fashion as outlined below.

1st step of development

A 1st step aims at answering basic questions that will be needed as guidelines for later work:

- How many MLD patients exist?
- In which countries?
- What type of MLD? (Late infantile, juvenile, adult)

This step does not require complicated procedures with local authorities, ethical committees etc. as the data are collected in a completely anonymous way. The procedure would be similar to that used in the German Leukodystrophy Network. Work on this part of the International MLD Registry can be started right away. Initially, a relatively small group of persons are required.

Persons who can get the initial phase started include:

Alfried **Kohlschuetter**, Hamburg, Germany

Timothy **Cox**, Cambridge, U.K.

J.E. **Wraith**, Manchester, England

Charles **Peters**, Kansas City, USA

Patrick **Aubourg**, Paris, France

This group will also give an estimation about the financial means which are necessary to support such a registry.

A list of national representatives in participating countries has to be set up.

Country	Representative	Remarks
UK	Ed Wraith	Manchester
France	Odile Boespflug-Tanguy	Clermont-Ferrand
France	Patrick Aubourg	Paris
Italy	Maria Sessa	Milano
Japan	T. Ohashi	Tokyo
USA	Florian Eichler	Boston, Massachusetts
USA	Charles Peters	Kansas City, Missouri
USA	Sakkubai Naidu	Baltimore, Maryland
Germany	Ingeborg Krägeloh Mann	Tuebingen
Germany	Alfried Kohlschütter	Hamburg

2nd step of development

A 2nd step consists in developing a complex database which contains information on the

- clinical and cognitive status
- neuroimaging status
- electrophysiological findings

at variable moments in time.

This step will require intensive preparation and the evaluation of existing databases (such as those for cerebral palsy and Fabry disease) for the purpose of an MLD registry.

Persons to be involved in this step should include:

Alfried **Kohlschuetter**, Hamburg, Germany
Ingeborg **Kraegeloh-Mann**, Tuebingen, Germany (clinical aspects)
Sakkubai **Naidu**, Baltimore, USA (neuroimaging)
Maria **Sessa**, Milan, Italy (electrophysiology)
Patrick **Aubourg**, Paris, Italy

Dr. Kohlschuetter presently intends to meet Drs. Wraith and Cox in the U.K., and perhaps Dr. Aubourg in France before March 31, 2006. Dr. Kohlschuetter can meet Dr. Peters in July 2006 during a trip to the U.S. Funding provided, other meetings of the MLD registry task force and at earlier points in time are desirable.

The task force will report to all participants of the Soho Hotel meeting in April and August 2006.

Organizations which can be addressed for financial support:

The Myelin Project
The United Leukodystrophy Foundation
European Leukodystrophy Association

Hamburg, 27 January 2006, Alfried Kohlschuetter