



## Ethics and Patient's Rights

The ethical objective to achieve is to protect patients and their families' rights while making data available for research purpose. A team is currently conducting reflection on the ethical issues related to the LeukoDataBase, with support from the Ethics Committee of the project.

### What are the research team missions?

- Harmonising individuals protection rules between participating countries
- Informing and taking into consideration the expectations of patients and their families

What has already been done? (See [www.leukotreat.eu](http://www.leukotreat.eu))

### LeukoDataBase Charter

The LeukoDB Charter sets out the principles agreed upon by the Members of the LeukoDataBase network. The LeukoDB Charter aims at: informing participants about the data used through the LeukoDataBase, and informing members, partners and users of the LeukoDB Network about their commitment regarding the LeukoDataBase functioning. This Charter is based upon international reference documents (international treaties and European directives) on personal data protection and right of persons regarding research.

### Informed consent and agreement of the patient

An information and consent document has been elaborated and will be communicated by all LeukoDataBase users. It explains what involves participation to the LeukoDataBase in order to allow informed consent.

### Ethics questionnaire about "patients and their families' expectations"

This questionnaire aims at evaluating expectations of patients and their families regarding research, and thereby improves the information level and provides a better support. It should create a momentum to reinforce the participation of the patients and their families, and also encourage dialogue among all research members.



# The LeukoDataBase

The European database on  
leukodystrophies



## The LeukoTreat Project

LeukoTreat is a 3-year research project funded by the European Commission up to March 1, 2013. The LeukoTreat project aims at promoting the research and development of therapeutic strategies for the largest number of Leukodystrophies (LDs) affected patients.

The project involves the following actions:

- Creating a European database on LDs (LeukoDataBase)
- Identifying biomarkers
- Developing innovative strategies (medicinal products, gene therapy and cell therapy)
- Implementing an ethical reflection between research teams and LD patients and their families



> For more information on the LeukoTreat Project: <http://leukotreat.eu>

## Leukodystrophies

Leukodystrophies (LDs) are rare neurodegenerative diseases affecting the myelin, the white substance coating the nerves like an insulating sheath. When leukodystrophy occurs, myelin can no longer conduct messages properly along the nerves. Leukodystrophies destroy the central nervous system (brain and spinal cord) in children and adults. Leukodystrophies gradually paralyses all their vital body functions (paralysis, blindness, deafness, brain damage, heart problems, blindness, speech and feeding problems).

The overall incidence of LDs is approximately 1 in 10 000 of the population (affecting children and adults) and there is approximately 1000 new cases per year in Europe. Up to now, more than 20 diseases have been identified as leukodystrophies. However, in spite of the remarkable progress made during the last ten years, treatments are still experimental.

Several associations support affected patients and their families, and encourage medical research, in particular the European Leukodystrophy Association (ELA) which is partner of this project.

> For more information on leukodystrophies and ELA: [www.ela-asso.com](http://www.ela-asso.com)



## The LeukoDataBase

The LeukoDataBase (or LeukoDB) is being developed within the LeukoTreat project, with the aim to be the reference European database on leukodystrophies.

The central objective of this unique database at the European scale is to collect and centralise as much information as possible.

The LeukoDataBase centralises three categories of information, for each patient affected with leukodystrophy: Clinical data, Biological Samples (existing biopsies from patients), Mutation data (for each gene known to be implicated in leukodystrophy).



The LeukoDataBase will allow a better understanding of:

- Natural history of these diseases
- Their evolution (number of patients, distribution, follow-up)
- Their genetic nature (mutation, how the disease is transmitted)

In addition, it will be much easier to recruit volunteer patients for future therapeutic trials.

The LeukoDataBase also aims to disseminate new knowledge and knowhow to all European countries.

