

NCL: THE DISEASE

In an affected cell...

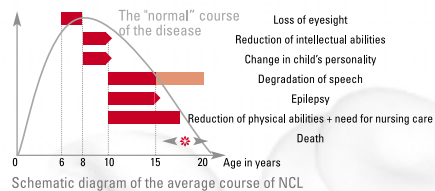


... the cell becomes poisoned and dies.
... the CLN3 protein responsible for the removal of harmful substances is missing.
... nutrients continue to be supplied to it.

Schematic diagram of a body cell suffering from NCL.

NCL stands for Neuronal Ceroid Lipofuscinosis ("Batten Disease"). This is a hereditary metabolic disease, the symptoms of which first appear between the age of 2 and 6 years. Children with NCL lack the protein that is responsible for the removal of endogenous toxic substances from the body's daily energy production. It is as if the body's "refuse collection" fails to arrive.

There are different types of NCL. The difference is the age at which children start to show the first symptoms. The NCL-Foundation concentrates on one of the most frequently diagnosed types, namely juvenile NCL. The initial symptoms appear relatively late at the age of 6 years. Today, one in 200 of all adults carries the hereditary factor required to pass on the disease. In Germany alone there are about 150 children who suffer from juvenile NCL.



All of the types of NCL have one thing in common: the course of the disease is

brutal, inevitable, and fatal. First of all each child loses its eyesight. There then follow various degenerative changes in intellect, speech, and the rest of the body. The child is mostly fully aware of these developments and suffers changes in personality because she/he is a prisoner in her/his own body. The disabilities slowly increase over the years. The need for a wheelchair is followed by bed confinement, and subsequently death. In this situation death (at about 20 years of age) often comes as a relief for all persons affected, finally there is an end to the many years of suffering.

THE NCL-FOUNDATION

The NCL-Foundation was established in August 2002 by Dr. Frank Husemann, following the diagnosis with NCL of his son, Tim. The foundation's objective is to do as much as possible to defeat this disease. It is a "National Contest for Life".

The basic concept of the foundation calls for sharp focusing of aims and activities.

There are currently three main areas of focus:

- the type of disease focused on is juvenile NCL
- activities are concentrated on setting up a contact network and securing donations and financing
- activities are carried out with minimum staffing levels; at present there is only one employee.

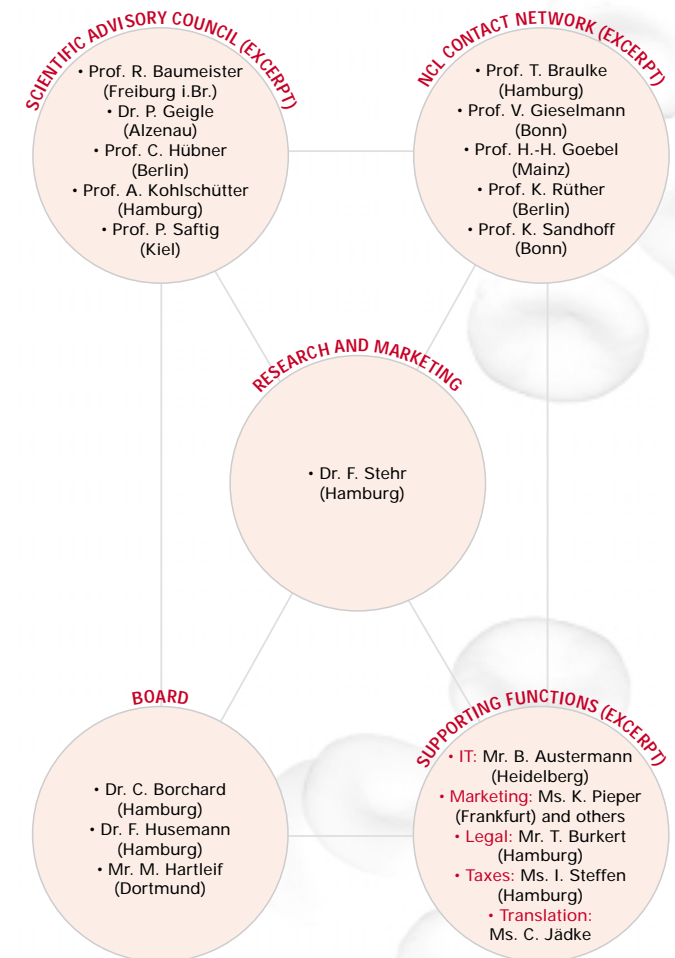
The Board and the Scientific Advisory Council work on an honorary basis. The Council gives advice on medical and scientific questions. The NCL contact network comprises various NCL experts and will be extended over time. Advice on legal questions and tax matters is also given free of charge from sponsors. In the long term, the work of the foundation will have to be financed through private donations and with the help of sponsors.

NCL RESEARCH

At present there is no cure for NCL, and the symptoms can only be relieved. Medical science has yet to understand this disease. Why do the cells of NCL patients die? Why are the eyes and brain affected so badly?

Basic research can provide the answers. Different cell cultures and model systems can be used to simulate the course of the disease. At the same time substances that contribute to the production of a cure may be found. Knowledge already gained from related fields and from comparable diseases can be used. However, these steps do need to be taken and the NCL-Foundation will help to pave the way. Preliminary results from a research group at the University of Leipzig are expected next year.

ORGANIZATION OF THE NCL-FOUNDATION



FINANCES

The NCL-Foundation's current medium-term financial planning is based on a finance volume of € 150,000 per year. In 2003 revenues exceeded this goal. This can mainly be attributed to the high level of engagement of many private sponsors.

It was possible to keep the expenses below the envisaged expenditure level. This would not have been possible without the support of many sponsors, especially in the area of public relations. In this way the NCL-Foundation will have a "healthy" start to 2004.



We should particularly like to express our thanks to the "Round Table Germany". This charitable organization has about 3,200 members, and in 2003 it has given its support to the NCL-Foundation at 220 German member organizations within the framework of the "National Charity Project 2003". Numerous member groups will continue their honorary charity work for the NCL-Foundation over the years to come.

Companies from all over Germany from various business sectors have also made considerable contributions to the Foundation's work through their donations. Amongst these companies were: Accenture (Kronberg), Für Sie (Hamburg), Lucia (Lüneburg), Mark-E (Hagen), Schwarzkopf (Hamburg). All donating companies have one thing in common: the desire to help children at risk from this disease as rapidly as possible.



Some individual member groups of the charitable organization Lions have also started to support the battle against NCL. We should like to highlight the activities of the Hamburg District here, as the "Leos", the Lions' youth organization, have already promised their help in 2004 through events held in connection with "National Charity Day" (8 May 2004). **Special thanks to all donors and sponsors!**



NCL ACCOUNT FOR DONATIONS

Account number: 1059 22 30 30

Bank code: 200 505 50

Bank: Hamburger Sparkasse

Purpose: Name and address of the donor; donor reference if required.

For all donations exceeding € 100 you will receive a receipt for tax purposes. We therefore ask you to state your address in the purpose field.

For donations of less than € 100 the bank deposit or transfer slip is valid as a donation receipt for tax purposes.

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ANNUAL REPORT 2003

The first year following the setting-up of the NCL-Foundation in August 2002 was very promising. Several important milestones were reached on the way to achieving our ambitious targets:

- On 1 November 2003 Dr. Frank Stehr, a biochemist, took up a full-time post with the foundation. The focus of his work will be on establishing a network of contacts and initiating research.
- In September 2003 an interdisciplinary research group at the University of Leipzig started work on a project to combat NCL. This project was initiated by the NCL-Foundation.
- The NCL-network underwent further expansion. In June 2003 the second German NCL Congress was chaired by the NCL-Foundation.
- The information campaign via the press and television was intensified. Since the summer of 2003 airtime has been dedicated for the first time to NCL on the TV channels NTV and CNN.

However, there is still very much more left to do because the battle against this rare metabolic disease cannot, unfortunately, be won within a matter of a few months.

