

INFORMATION

Focus of educational work in 2007:

01/07: At the **New Year's colloquium** in Jena the NCL-Foundation informed 110 pediatricians about NCL.

02/07: The **NCL-Leaflet**, that informs physicians about the disease, is being dispersed to approx. 250 participants of medical symposia in Dortmund and Fulda. "Adult-onset neuronal ceroid lipofuscinosis" by Prof. Ludolph (neurologist) is published in the „Nervenarzt“. At the **Minicircle-Workshop** (Bielefeld) 30 researchers discuss gene-therapeutic remedy possibilities for NCL.

03/07: At the **Pro Retina-Symposium** eye specialists and researchers are being informed about NCL; as well as at the conference of the Society for Children and Youth Medicine and Pediatric Surgery in Halle.



04/07: Developed in 2005, the **NCL-school lesson „Biology up-to-date“** is now available as a download on the Hamburg Educational Server. At the yearly conference of the Northern German Society for Children and Youth Medicine 400 pediatricians could be reached.

05/07: **Ophthalmic Medical Training** at the Schlosspark-Klinik (Berlin). The magazine "ProPraxis Pädiatrie" reports on the speech computer project of the Foundation.



07/07: At the **international NCL-Congress** in the USA the NCL-Foundation arranges for a separate workshop that aims at encouraging collaboration between research-supporting non-profit organizations.

09/07: Initiated by the NCL-Foundation, a **written NCL-Training** is published in the medical magazine „Kinder- und Jugendarzt“. Together with this issue, the leaflet is being dispersed nationwide to **10.600 pediatricians**. At the professional conference SSIEM the NCL-Foundation is present with an **information booth**.



10/07: In order to integrate „NCL“ in the classroom, the NCL-Foundation together with the Regional Teacher Training and School Development Institute arrange for the **Teacher Training „DNA-mutations in humans: diagnosis and therapy through genetic treatments“**.

RESEARCH STEPS

Therapy	Healing	>2025 The process of preclinical and clinical stages must be passed through several times until the healing of the disease.
	Palliation / Delay	2018 Relief and delay respectively of NCL-symptoms.
Clinic	Clinical examination (Stages I-IV)	2016 Test on NCL-patients: safety, efficiency, effectiveness.
	Functional- / toxicity test in large animal model	2012 Tests in larger animal models, GMP-active pharmaceutical drug production.
Preclinical stages	Toxicity tests on rat	2011 Test of a therapy approach on healthy rats concerning side effects.
	Therapy approach in cell culture and mouse model	2008 Test of a therapy approach in disease models.
		2007
Basic Research	Functional analysis / clarification of mechanisms	2003 CLN3 takes part in transport processes; function has not been conclusively clarified yet.
	Localization	2003 Lysosomal membrane
	Generating cell and animal models	2002 CLN3-Knock in-mouse 1999 CLN3-Knock out-mouse 1998 Yeast-model
Diagnostic	Cause	1995 the disease-causing mutation in the CLN3-gene was discovered on chromosome 16, which is molecular-biologically verifiable.
	Phenomenological disease description	Physiological parameters, anamnesis, lymphocyte-vacuoles.
Description	Description of individual forms	1903-09 Batten, Spielmeyer and Vogt report on the juvenile NCL-form.
	First description of disease	1826 The Norwegian Stengel describes for the first time an NCL-case.

RESEARCH & FUNDING

RESEARCH RESULTS 2007

- Identification of **new NCL-genes**, which encode certain chloride-ion-channels (Thomas Jentsch, Berlin).
- **CLN3-reporter-mouse-model**: high CLN3-expression verified inter alia in the kidney (Beverly Davidson, Iowa City).
- **CLN3-splice variants** could have residual function - therefore **folding aids** would be useful (Sara Mole, London).
- Use of **immunosuppressant** improves motor abilities of the CLN3-mouse; results of **immunodeficient CLN3-mutants** show effects of the immune system on the course of the disease (David Pearce, Rochester).
- **Presymptomatic CLN2-gene-therapy** on mice leads to 75% survival rate (Marco Passini, Genzyme).
- **CNS-enzyme-replacement-therapy** for late-infantile CLN2-Knock out-mouse (Beverly Davidson, Iowa City).
- First **stem cell based therapy** for infantile and late-infantile NCL-children (Robert Steiner, Portland).

- With the support of **McDonald's Kinderhilfe** the NCL-Foundation was able to acquire 20 speech computers to be distributed nationwide to schools for the blind. This project aims at reducing the psychological problems caused by loss of speech.



- Procurement to fund an NCL research project through the **NTSAD** at Sue Cotman. Active pharmaceutical small molecules are to be tested in the NCL-cell-culture-system.

- The publicly funded research institution „**German Mouse Clinic**“ (Munich) is going to study the CLN3-mouse-model of Sue Cotman in detail.

- Continuation of the co-operation with the **Griebel-Foundation**: support of another project with Prof. Klaus Rüther (Charité, Berlin) in order to further study the retinal degeneration process on the CLN3-mouse-model. This is going to be the basis for judging the effectiveness of a **gene therapy of the eye**.

- **BILD hilft e.V. „Ein Herz für Kinder“** is going to finance the first 3 years of research to develop a gene therapy for juvenile NCL.

- Using the **6th national NCL-Congress**, neuro-immunologists were linked together with NCL-experts. That is how, for example, Jon Cooper (London) and Rudolf Martini (Würzburg) are going to start research collaboration.



FUNDRAISING

12/06: Hamburger des Jahres 2006

The NCL-Foundation is being honoured as „Hamburger des Jahres“. Frank Husemann accepts the honour awarded by the „Welt“ and „HamburgTV“, as well as the Haspa check for 12,000 €.

02/07: Art auction "LebensKünstler"

Auctioneer Joseph Weber and Birgit Saatrübe auction off 30 donated pieces of art of selected German artists.

04/07: Restaurant event "3 Sterne & Co. für NCL"

12 restaurants (i. a. Poletto, Sgroi, Landhaus Scherrer, Randel, Schaueremann and Insel am Alsterufer) cook for NCL.

04/07: NCL-Foundation at "beckmann" (ARD)

Reinhold Beckmann speaks with Frank Husemann about the NCL-Foundation and the fate of his son.

05/07: American BBQ at Hamburg Marriott Hotel

The Marriott Hotel celebrates 80 years Marriott with a barbecue in favour of the NCL-Foundation.



06/07 NCL Golf Trophy (GC Adendorf, Lüneburg)

Golf club Adendorf invites for the second time to play golf against NCL.

08/07: BILD-metropress-Golf-Cup, Friedberg

Members of the Eagles Charity Golf Club, among others Jan Josef Liefers and Jörg Wontorra, play golf for the NCL-Foundation.



08/07: Auto Charity Challenge 2007

Taxi drives in exclusive sport cars, organized by Michael Fromm, Kay Andresen and the ADAC-center for safe driving / Lüneburg / Embsen.



09/07: 4th Charity-Dinner "Cooking for Kids"

Stranded at the Elbe waterside with Tim Mälzer: 120 guests meet at the "Alter Hamburger Jachtclub".



10/07: Seed financing of an NCL gene therapy secured

Bild hilft e.V. (non profit organization) „Ein Herz für Kinder“ sponsors the development of a gene therapy of the eye with 275,000 €.

11/07: 5th Wiesbadener Galaball der Immobilien

Anja Strumpf Events, actress Nina Bott, tenor Björn Casapietra and 1.000 guests support the NCL-Foundation.

12/07: René Lezard Christmas Angels

The René Lezard shops sell nationwide Christmas angels to support the research concerning NCL.

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NCL-ACCOUNT FOR DONATIONS

NCL-Stiftung, Account number 10 59 22 30 30
Bank Hamburger Sparkasse, Bank code 200 505 50


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

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Since 2006 the NCL-Foundation has been a member of the ACHSE, an association of patient groups that support under the patronage of Eva Luise Köhler the research concerning orphan diseases.



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Annual Report 2007

of the charitable NCL-Foundation



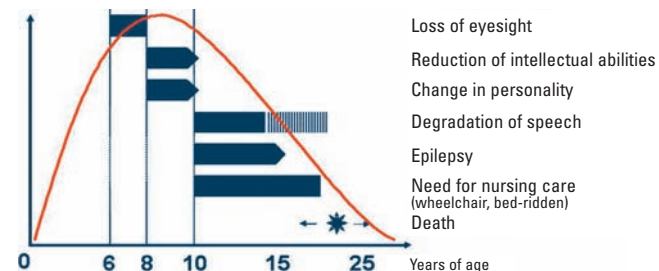
The NCL-Foundation was founded in 2002 and aims at developing a therapy of the **deadly childhood disease** NCL (Neuronal Ceroid Lipofuscinosis). The Foundation focuses on four areas:

Information · Research · Funding · Fundraising

In 2007 the NCL-Foundation put their main emphasis on the following:

- Focussing on the **gene therapy of the eye**
- Initiating **NCL-congresses and NCL-symposia** (Munich, New York)
- **Co-financing** of an NCL research project with the Berlin-based Charité
- **Awareness training** through biology classes, teacher training and publications
- Well-directed **PR-work** ("beckmann", ARD)

NCL is a genetic metabolic disease which breaks out when the children are at preschool age. After an agonizing degenerative process death usually occurs at about 25 years of age. The children affected lose their eye-sight (after an initially normal development) at the age of about 6-9 years and consequently also lose the ability to talk, to think and to act.



Presentation of the course of the disease in juvenile NCL.

The disease is rare and is being neglected by the pharmaceutical industry. Private involvement is needed.

Please help us, too, to heal NCL.

