

9th National NCL-Congress

Date of congress: Monday, August 23, 2010

Congress-location: Hamburg Marriott Hotel
ABC-Straße 52, 20354 Hamburg, Germany

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Venue

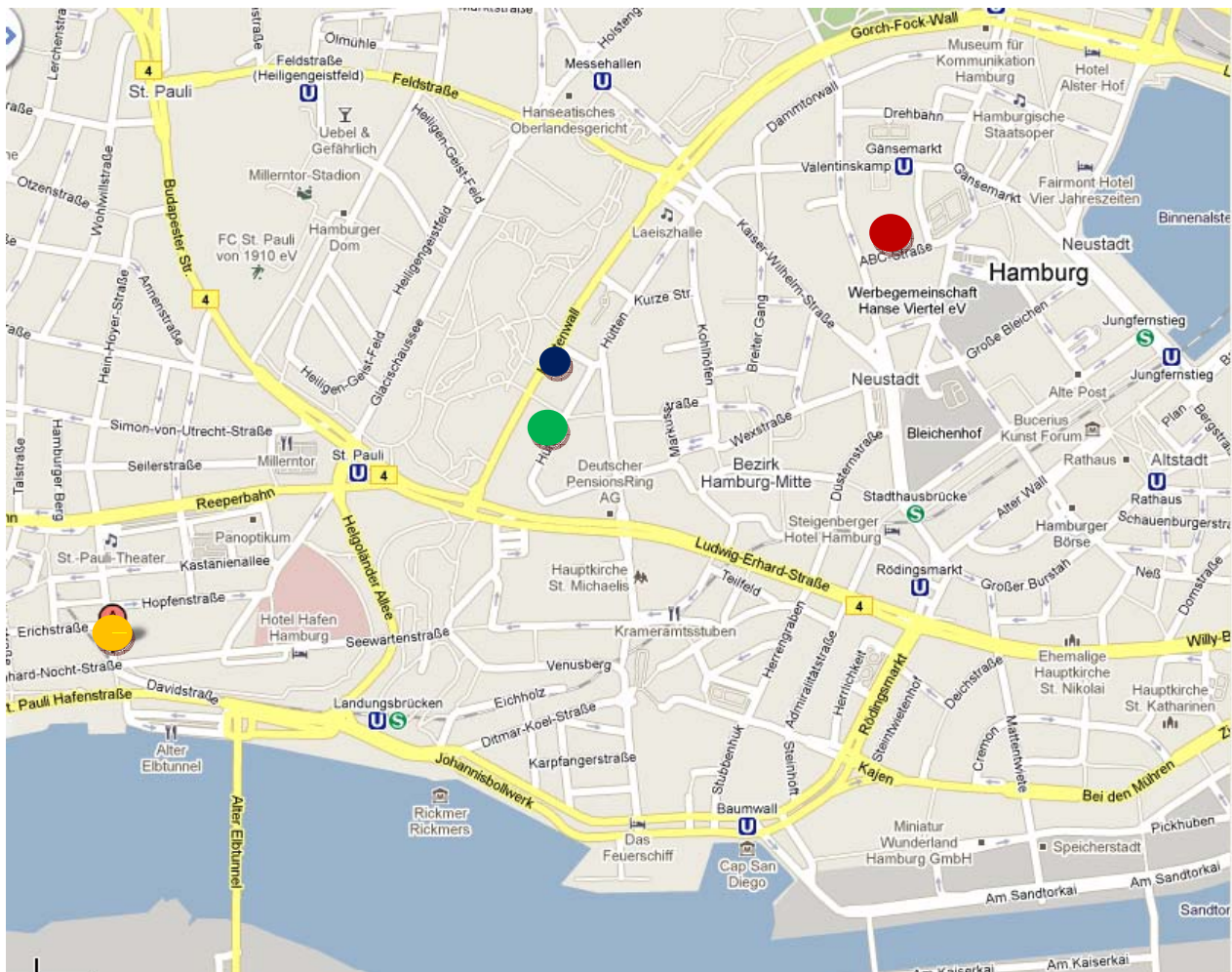
Lectures and registration will take place at:

Hamburg Marriott Hotel

ABC-Straße 52

20354 Hamburg

Germany



Legend

- Marriott Hotel
- Lindner Hotel
- NCL-Stiftung
- Dinner location (Aug 23) Empire Riverside Hotel: Restaurant "waterkant"



Registration

Registration will take place from 09:00 to 10:00 on Monday August 23 at the reception desk. Upon registration, all participants will receive further meeting information and a name badge.

Social Programme

Sunday 22 August

Informal get-together in a nice brewery
Gröninger Privatbrauerei
Willy-Brandt-Straße 47
20457 Hamburg
Tel.: +49 (0)40 - 33 13 81
www.groeninger-hamburg.de

We will have a table there starting from 19:00. Please, mention "Frank Stehr" at the reception desk.

It closes at 22:00.

Monday 23 August

Dinner will start at 18:30. There will be a taxi service at the Hamburg Marriott Hotel.

Dinner location:

Restaurant "waterkant"
located in the "Empire Riverside Hotel"
Bernhard-Nocht-Straße 97
20359 Hamburg
Tel. +49 (0)40 31 11 9 - 70 480
www.restaurant-waterkant.de

Further Information

Photography

Please note that photographs taken at this event may be used for promotional purposes by the NCL-Foundation, by inclusion on the NCL-Foundation Website and/or marketing materials. If you have any concerns or queries regarding this, please contact us.

Useful contacts

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Scientific Programme

Registration: 09:00-10:00

Speaker session time: 10:00-18:00

- 10:00 – 10:15 Welcome (Frank Stehr, NCL-Foundation)
- 10:15 – 10:45 Clinical picture and molecular basis of NCL (Robert Steinfeld)
- 10:45 – 11:15 The blood-brain barrier: a barrier to the treatment of neuronal ceroid lipofuscinoses? (David J. Begley)

Gene delivery – Part I

- 11:15 – 11:45 Delivery of Drugs and Macromolecules Across the Blood-Brain Barrier Using Nanoparticles (Jörg Kreuter)
- 11:45 – 12:15 Protein nanoparticles: A new kind of drug delivery vehicle (Heiko Manninga)
- 12:15 – 12:45 A brief history of CAV-2 vector gene transfer to the CNS and the interesting clinical characteristics for these tools (Eric J. Kremer)

12:45 – 13:30 Lunch break

Gene delivery – Part II

- 13:30 – 14:00 Neuronal Gene Tay-Sachs and Related Diseases (Timothy M. Cox)
- 14:00 – 14:30 Biology of the Cell in the Absence of CLN3 (Beverly Davidson)
- 14:30 – 15:00 Gene Therapy for the CNS and Eye Manifestations of Neuronal Ceroid Lipofuscinoses (Ronald Crystal)
- 15:00 – 15:30 Genetic Reactivation of Cone Photoreceptors Restores Visual Responses in Retinitis Pigmentosa (Jens Duebel)

15:30 – 16:00 Coffee break

- 16:00 – 17:30 Podiums discussion
- 17:30 – 18:00 NCL research prize award ceremony: Autophagy in NCL Disease Pathogenesis (Matthew Micsenyi)

18:30 Dinner

The blood-brain barrier: a barrier to the treatment of neuronal ceroid lipofuscinoses?

David J. Begley Kings College London, Pharmaceutical Sciences, Blood-Brain Barrier Group, Pharmaceutical Sciences, Hodgkin Building, Guys Campus, London SE1 1UL,UK

A primary function of the blood-brain barrier (BBB) is to create a regulatory interface between the systemic extracellular fluid and the extracellular fluid of the brain, so that a very stable environment can be maintained within the cerebral compartment. The central nervous system (CNS) requires a very constant chemical extracellular fluid environment so that normal neuronal function and synaptic neurotransmission can be maintained efficiently. The endothelial cells that form the capillaries of the brain, and thus constitute the BBB, create tight junctions between each individual endothelial cell and these junctions effectively abolish any aqueous paracellular diffusional pathway in these capillaries. In consequence, blood gases, glucose, amino acids, other nutrients and many waste products all have to be exchanged by a transcellular route across the endothelial cells of the BBB. Specialised and polarised transport mechanisms are present in the BBB to ensure that the needs of the brain and the maintenance of its special environment are assured.

The BBB is also a protective barrier which shields the CNS from many neurotoxic substances which circulate in the blood. These neurotoxins may be naturally occurring metabolites, or xenobiotics ingested in the diet or otherwise acquired from the environment. The cells of the mature CNS are unable to divide or replace themselves rapidly, if at all, in many areas of the adult brain, and in addition there is a steady and continuous rate of neuronal cell death throughout life in the CNS. An acceleration of this rate of neuronal and glial attrition resulting, for example, from an increased entry of neurotoxins into the CNS will produce a premature neurological decline and functional debilitation. The BBB contains a number of active ATP-dependent efflux transporters - (the ABC cassette) - which remove neurotoxins, xenobiotics (and frustratingly many potentially useful therapeutic drugs) from the CNS.

The BBB, as a result of its regulatory and protective functions, thus presents a formidable obstacle to brain entry for many therapeutic agents intended to treat CNS diseases, whether administered intravenously or orally, and it provides a great challenge to the pharmaceutical industry in the design of new and effective CNS therapeutic agents.

The BBB presents a particularly difficult hurdle for the delivery of the high molecular weight enzyme replacement therapies (ERT) designed to treat lysosomal storage disorders, and also any possible gene vectors, intended to restore normal lysosomal enzyme and transport activity. In addition, some current small molecule substrate reduction therapies (SRT) and chaperones do appear to cross the BBB but the mechanisms by which they do so are unknown. There is currently little information regarding the passage and exchange of storage products between the blood and brain.

There is also evidence that in mouse models of a number of lysosomal storage disorders, for example, in Sandhoff disease, GM1 gangliosidosis and Batten disease (CLN3), that the BBB itself may be compromised and damaged and that the leakage of blood components through the barrier may contribute to the disease process and enhance and accelerate CNS cell damage.

Therefore, in the context of treating lysosomal storage disorders with neurological involvement the BBB is a major hurdle which needs to be understood and overcome before therapy directed at the CNS can be planned in a rational manner.

Delivery of Drugs and Macromolecules Across the Blood-Brain Barrier Using Nanoparticles

Jörg Kreuter, Institut für Pharmazeutische Technologie, Goethe-Universität, Frankfurt, Germany

The blood-brain barrier (BBB) represents an insurmountable obstacle for the delivery of a large number of drugs to the central nervous system (CNS). One of the possibilities to overcome this barrier is drug delivery to the brain using nanoparticles. Drugs that have been transported into the brain and led to a pharmacological effect after intravenous injection using this carrier include the hexapeptide dalargin, the dipeptide kyotorphin, loperamide, tubocurarine, doxorubicin, and the NMDA receptor antagonists MRZ 2/576 and MRZ 2/596. To achieve a significant transport across the blood-brain barrier the coating of the nanoparticles with polysorbate 80 (Tween[®] 80) was a key factor.

Experiments with the extremely aggressive glioblastoma 101/8 transplanted intracranially showed a long term survival for 6 months of up to 40 % of the rats after intravenous injection of the polysorbate 80-coated nanoparticle preparation. The surviving animals showed a total remission by histological investigation. Untreated controls died within 10 - 20 days, the animals in the doxorubicin control and uncoated doxorubicin nanoparticle groups died between 10 – 50 days.

Besides small molecules large molecules such as nerve growth factor NGF also can be transported across the BBB with these nanoparticles and yielded significantly higher brain concentrations as well as very considerable pharmacological effects in the passive avoidance reaction test (PAR test) and in a number of Parkinson models.

The mechanism of the drug transport across the blood-brain barrier with the nanoparticles is endocytotic uptake by the brain capillary endothelial cells followed either by release of the drugs in these cells and diffusion into the brain or by transcytosis. After intravenous injection of the nanoparticles, apolipoproteins A-I or E adsorb on the particles surface which in turn promotes the interaction with receptors on the endothelial cells followed by endocytosis, thus mimicing the uptake of naturally occurring lipoprotein particles. This hypothesis was supported by electron microscopy as well as by the achievement of antinociceptive effects with loperamide-loaded albumin nanoparticles with covalently bound apo E and A-I. Apart from these apolipoproteins, covalent attachment of transferrin or insulin or of antibodies against their respective receptors yielded similar antinociceptive effects.

Protein nanoparticles: A new kind of drug delivery vehicle

Heiko Manninga, Life Science Inkubator Betriebs GmbH & Co. KG, Ludwig-Erhard-Allee 2, 53175 Bonn, Germany

A new kind of delivery vehicle, based on virus like particles (VLPs), will be presented. These new approach for the specific delivery of therapeutic relevant substances shares the advantages of classical nanoparticles and virus based delivery systems but excludes the disadvantages of complete viral system like adenoviral or retroviral systems. Our VLPs, named as “protein nanoparticles” have the capability of transporting different kind of potential drugs into a target cell. They consist of one recombinant produced protein and are easy to purify. With a dissociation-reassociation procedure, one can fill these particles with the compound of interest.

We have tested so far packaging small molecules as well as DNA and siRNA and could show delivery of the effecting molecule to their point of action. Also small proteins should be possible and will be tested in the near future.

Like the natural virus from which the VLPs are designed, the particles have a defined cell tropism. In our case the tropism is restricted to neuronal and kidney cells. We are exploring the capability of the VLPs as delivery system in different kind of diseases, to define the possible uses for this new system.

A brief history of CAV-2 vector gene transfer to the CNS and the interesting clinical characteristics for these tools

EJ Kremer, Institut de Génétique Moléculaire de Montpellier CNRS 5535, France

During the maturation of the gene therapy field in the early '90s, we hypothesized that vector derived from common human pathogens would have inherent clinical disadvantages - in contrast to success in animal models. We therefore initiated development of vectors derived from the best understood nonhuman adenovirus at that time.

I'll present the evolution of our canine type 2 (CAV-2) vector story. In particular, one of the initial "raison d'être" for the development of CAV-2 vector was its potential to evade the memory immune response to common human pathogens. This is particularly important in the context of the ubiquitous anti-adenovirus response. I'll show our encouraging data where we characterised the cross-reaction to anti-Ad memory T cells and antibodies, and the potential to activate dendritic cells.

Then, I'll highlight CAV-2's unexpected tropism for neurons when injected the brain parenchyma and muscle, and its surprising level of retrograde transport. With respect to these characteristics I'll also detail the molecular basis for each. I'll also show data that demonstrates that this tropism and retrograde transport is likely species-independent. More specifically, CAV-2's tropism and retrograde transport in human brain tissue and nonhuman primates.

Finally, I'll give an update on our use of helper-dependent CAV-2 vectors expressing β -glucuronidase for preclinical therapy in MPS VII dog CNS.

Neuronal Gene Tay-Sachs and Related Diseases

Timothy M. Cox, Department of Medicine, University of Cambridge, UK

After 1881 when Dr. Waren Tay at The London Hospital in Whitechapel described in what turned out to be the first lysosomal disease and, like Bernard Sachs in New York, noted its familial nature and tragic consequences, heroic efforts have been made by investigators who have ventured down many convoluted paths to treat this disorder. Classical Tay-Sachs disease causes rapidly progressive visual, motor and cognitive impairment in young infants: it is incurable and thus remains an iconic neurodegenerative condition which, with justification, can be viewed as emblematic of our commune within the clinical and laboratory sciences.

Tay-Sachs and Sandhoff diseases are caused by defects in the α - and s-subunits of β -Nacetyl-hexosaminidases (Hex), respectively; deficiency of the cognate activator protein also causes GM2 gangliosidosis. Since their identification 20 years ago, mutations in any of these genes induce neurodegenerative disease with lysosomal accumulation of glycosphingolipids, principally GM2 ganglioside. Injury to neurones and glia is associated with neuroinflammation, cellular malfunction and neuronal injury. Not only have the molecular structures of the s-hexosaminidases been solved, but genetically modified mice and cats with hex deficiency have been characterized. Animals harbouring mutations in the common s-subunit of Hex, die prematurely with severe neurological disease: as authentic experimental models of human GM2 gangliosidoses, they provide invaluable resources for therapeutic research.

As a result of its high gene frequency in the Ashkenazim and the demand for prevention, Tay-Sachs disease is the paradigm of successful genetic and biochemical screening – involving as it has, diverse interventions favoured by each of the various communities within this high-risk ethnic group.

Thanks to screening in enlightened countries, Tay-Sachs disease is now very rare in the Ashkenazim; but patients with Tay-Sachs and Sandhoff diseases remain a formidable challenge and continue to be diagnosed (often after unconscionable delays) principally in non-Jews.

Over many years, diverse stratagems for treating Tay-Sachs and related diseases (GM2 gangliosidoses) have been explored – these include disappointing studies of systemic, as well as intracranial delivery of various macromolecular hexosaminidase formulations to overcome the blood-brain barrier. The remarkable effect of enteral N-butyldeoxynojirimycin, which enters brain tissue and prolongs the lifespan of Sandhoff mice, led to human trials of this iminosugar as a



substrate inhibitor in human GM2 gangliosidosis; unfortunately it appears not to be sufficiently active to justify further clinical testing. The identification of pyrimethamine, a licensed anti-protozoal agent for long-term use which enters the brain, as a weak inhibitor with in vitro activity as a pharmacological chaperone of s-hexosaminidases, has been a signal discovery -and one underpinned by molecular modelling and structural studies. Trials with this agent, designed to improve folding and enhance intracellular delivery of nascent variant hexosaminidases to the lysosome, are underway in attenuated forms of GM2 gangliosidosis. Although the stratagem holds promise for some late-onset patients, it has no place in infants and others with mutant enzymes that do not interact with the chaperone molecule and which remain inactive in situ.

We have obtained salutary effects by gene transfer: this addresses the cellular pathology and strikingly improves neurological outcome and survival in animal models. An international consortium to produce and systematically test rAAV vector systems for clinical use has been established. Delivery of therapeutic vector and the design of ethical and informative clinical trials are the principal obstacles. Wide support will be critical in this devastating disease - hence participation by committed patient advocates and European experts will be needed, as well as greatly welcomed.

Biology of the Cell in the Absence of CLN3

Colleen S. Stein, Luis Tecedor, Mark Schultz, and **Beverly L. Davidson**, University of Iowa, Iowa City, USA

The Juvenile form of Neuronal Ceroid Lipofuscinosis (JNCL) is a neurodegenerative disease caused by mutations in the gene that codes for the protein called CLN3. While the exact molecular function of CLN3 is not known, various defects have been described in cells that lack CLN3. One defect that has been consistently observed is impairment in endocytosis. Cells use endocytosis to engulf fluid and proteins from the extracellular environment. Our lab is interested in 1) understanding the basis for this defect, and 2) finding small drugs able to correct this defect in CLN3-deficient cells. Some types of endocytosis occur at areas on the cell membrane that are rich in particular lipids such as cholesterol and sphingolipids. These areas are sometimes referred to as lipid rafts. A number of proteins are known to integrate or associate with lipid rafts. Our laboratory has assayed several of these proteins and found that they are mislocalized in CLN3-deficient cells. We are currently examining whether raft lipids are altered in CLN3-deficient cells. In parallel, we are collaborating with the NIH to establish an assay that is amenable to high throughput screening of small molecules. In this way we hope to identify drugs that restore function to CLN3 deficient cells, and have potential as therapeutics for JNCL.



Gene Therapy for the CNS and Eye Manifestations of Neuronal Ceroid Lipofuscinoses

Ronald Crystal, Department of Genetic Medicine, Weill Cornell Medical College, New York, NY, USA

Our group has a highly focused program for developing treatment strategies for two of the neuronal ceroid lipofuscinoses (NCL): the late infantile (LINCL) and the juvenile (JNCL) forms. To circumvent the challenge of the blood brain barrier, we have developed a gene transfer strategy using adeno-associated virus to deliver the normal gene directly to the CNS. Our initial work focused on late infantile neuronal ceroid lipofuscinosis, a NCL caused by mutations in the CLN2 gene. We have successfully translated our work to the human setting by completing one clinical trial with 10 children with LINCL using AAV serotype 2, demonstrating stabilization of the CNS disease in the majority of the children. We have all the necessary approvals for initiating a 2nd clinical trial for 16 LINCL subjects who will receive CNS administration of AAVrh.10 coding for CLN2, a non-human primate-derived AAV serotype that has markedly better efficacy than other AAV serotypes for treating CNS

disorders. In addition we have also initiated studies to develop gene therapeutic strategies for the treatment of the CNS manifestations of JNCL as well as the retinal manifestations of both LINCL and JNCL. Our newest studies include: (1) detailed, state-of-the-art neuro-imaging and retinal assessment in subjects with LINCL, correlating these with disease severity and phenotype, tools critical for the assessment of the impact of therapy; and (2) using AAVrh.10 to address the CNS and retinal manifestations of LINCL and JNCL.

Genetic Reactivation of Cone Photoreceptors Restores Visual Responses in Retinitis Pigmentosa

Jens Duebel, Friedrich Miescher Institute for Biomedical Research, Basel, Swiss

Retinitis pigmentosa refers to a diverse group of hereditary diseases affecting two million people worldwide that lead to incurable blindness. As a common pathology, rod photoreceptors die early whereas light insensitive, morphologically altered cone photoreceptors persist longer. It is unknown if these cones are accessible for therapeutic intervention. We show that expression of archaeobacterial halorhodopsin in light-insensitive cones can substitute for the native phototransduction cascade and restore their light sensitivity in mouse models of retinitis pigmentosa. Resensitized photoreceptors activate all retinal cone pathways, drive sophisticated



retinal circuit functions including directional selectivity, activate cortical circuits, and mediate visually guided behaviors. Using human ex vivo retinas we show that halorhodopsin can reactivate light-insensitive human photoreceptors. Finally, we identified blind patients with persisting, light-insensitive cones for potential halorhodopsin-based therapy.

Autophagy in NCL Disease Pathogenesis

Matthew C. Micsenyi, Nafeeza Ali, Gloria Stephney, Kostantin Dobrenis, Steven U. Walkley
Dominick P. Purpura Department of Neuroscience, Albert Einstein College of Medicine, Bronx, NY.

We are currently evaluating the role of macroautophagy in the CNS of classic late infantile NCL (LINCL) and juvenile NCL (JNCL). Our preliminary findings show that macroautophagy is upregulated in primary cortical neuronal cultures generated from the *Cln2*^{-/-} mouse model, while our *in vivo* studies suggest this mechanism becomes progressively inefficient through the course of disease. Most notably, in the CNS of *Cln2*^{-/-} mice we have identified the intraneuronal

accumulation of ubiquitin-positive insoluble protein aggregates containing the autophagy adapter proteins p62 and NBR1. The presence of such protein aggregates has been extensively linked to autophagy dysfunction, and has been reported in several other lysosomal diseases, as well as a number of later onset neurodegenerative disorders. Our future focus will be to elucidate how inefficient autophagy and protein aggregate accumulation relate to neurodegeneration in LINCL, and to translate these studies to JNCL. Additionally, we are investigating the function of the ubiquitin-proteasome system (UPS). Given the critical role autophagy and the UPS play in coordinating metabolic homeostasis and proteolytic quality control in neurons, our interest lies in how alterations in these mechanisms may contribute to disease pathogenesis. We believe a better understanding of these systems in NCL disease may identify potential targets for therapeutic intervention.

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