

9th JNCL Young Investigator Symposium

Date of Young Investigator Symposium: Sunday, 24th September & Monday, 25st September 2023

Date of International NCL Congress: Tuesday-Saturday, 26-30th September 2023

YI Meeting location: Hamburg Marriott Hotel, ABC-Straße 52, 20354 Hamburg

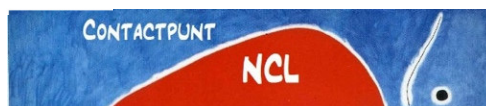
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Registration:

9th JNCL Young Investigator Symposium: at the Hamburg Marriott Hotel, ABC-Straße 52, 20354 Hamburg

from **09:00 to 9:45 on Sunday, Sept 24th**. Name badge and further information will be provided.

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Hamburger Sparkasse

Information:

Addresses

For those that accepted to stay at the Superbude (Breakfast included), the address is Spaldingstrasse 152, 20097 Hamburg. For those that made their own bookings, we refer to the arrangements you have made yourself.

Lunch is provided at the Hamburg Marriott Hotel during the two JNCL Young Investigator Symposium days.

Further Information:

The content in this documentation is solely the responsibility of the authors and does not necessarily represent the official views of the NCL Foundation.

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

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1st Day of 9th JNCL Young Investigator Symposium

Sunday, 24th September 2023

10:00 – 10:20 Welcome: **NCL Foundation** (GER)

10:20 – 10:50 **Dominic Winter - Characterization of the lysosomal proteome by mass-spectrometry-based proteomics**
(University of Bonn, Bonn, GER)

10:50 – 11:10 Coffee Break

Chair: Julia Heiby, University of Jena

11:10 – 11:40 **Prerana Gogoi - Toward structure determination of ceroid-lipofuscinosis, neuronal 3 protein using cryo-electron microscopy**
(Perelman School of Medicine, University of Pennsylvania, USA)

11:40 – 12:10 **Tobias Raisch - Structural studies of CLN3**
(Max Planck Institute of Molecular Physiology, Dortmund, GER)

12:10 – 12:40 **Hannah Leppert - Dissecting the function of CLN3 through isoforms and interactomes**
(Sanford Health, Sioux Falls, USA)

12:40 – 14:00 Lunch

Chair: Tobias Raisch, MPI Dortmund

14:00 – 14:30 **Julia Heiby - Characterization of lysosomal proteome in Batten Disease: Insights from mouse models and cell culture**
(Leibniz Institute on Aging, Jena, GER)

14:30 – 15:00 **Kwamina Nyame - The Batten disease glycerophosphodiester storage inhibits lysosomal phospholipid catabolism**
(Stanford University, Stanford, USA)

15:00 – 15:30 **Hannah Best - CLN3 is required for the efflux of lysosomal K⁺**
(Cardiff University, Cardiff, UK)

15:30 – 16:00 Coffee Break

Chair: Hannah Best, Cardiff University

16:00 – 16:30 **Mitchell Rechtzigel - Deep, untargeted biomarker discovery in CLN3^{Δex7/8} minipig serum**
(Sanford Research, Sanford, USA)

16:30 – 17:00 **Matthew Stratton - Antisense oligonucleotides to treat vision loss in a porcine model of CLN3 Batten disease**
(Rosalind Franklin University of Medicine and Science, Chicago, USA)

17:00 – 17:30 **Seda Yasa - Mapping the molecular and functional alterations in CLN3 disease microglia**
(Massachusetts General Hospital, Boston, USA)

17:30 – 18:00 **Neuza Domingues - Unveiling the molecular mechanisms of Batten Disease in the absence of CLN3**
(University of Coimbra, Coimbra, Portugal)

18:30 – 21:30 Social Event for participants of the 9th JNCL Young Investigator Symposium,
La Cocina Cooking School, Ludwig-Erhard-Straße 37, 20459 Hamburg

2nd Day of 9th JNCL Young Investigator Symposium

Monday, 25st September 2023

Chair: Herman van der Putten, NCL Foundation

09:30 – 09:50 **Karen Kahn - Living with Batten**
(ForeBatten Foundation, Chapel Hill, USA)

09:50 – 10:20 **Rebecca Ahrens-Nicklas - Therapy development for CLN3 Disease: Challenges and opportunities**
(CHOP, University of Pennsylvania, Philadelphia, USA)

10:20 – 10:50 **Masood Ahmad Wani - Synaptic and morphological abnormalities in CLN3-deficient neurons: Implications for the pathogenesis of juvenile Neuronal Ceroid Lipofuscinosis**
(Johannes Gutenberg University Mainz, Mainz, GER)

10:50 – 11:10 Coffee Break

Chair: Robert Steinfeld, University Medial Center Göttingen

11:10 – 11:40 **Chris Minnis - CLN3 behind the looking glass - implications to disease mechanism using long-read RNA sequencing**
(UCL, London, UK)

11:40 – 12:10 **Haoyu Zhang - CLN3 transcript complexity revealed by public data mining**
(UCL, London, UK)

12:10 – 12:40 **Ursula Heins Marroquin - Neurometabolic zebrafish model for CLN3-deficiencies**
(University of Luxembourg, Luxembourg)

12:40 – 13:10 **Valentina Marchica - Development of genetically accurate zebrafish models of CLN3 disease**
(RVC, London, UK)

13:10 – 14:30 Lunch

Chair: Rebecca Ahrens-Nicklas, CHOP

14:30 – 15:00 **Miriam Aurilia - High-content imaging approaches to identify new druggable targets to tackle Batten disease**
(TIGEM, Pozzuoli, Napels, IT)

15:00 – 15:30 **Claudia La Vecchia - Combining lysosomal isolation with multi-omics approaches to study and treat Batten disease**
(TIGEM, Pozzuoli, Napels, IT)

15:30 – 16:00	Ewa Ziolkowska - Identifying and treating Cln3 disease outside the central nervous system (Washington University, St. Louis, USA)
16:00 – 16:30	Coffee Break
Chair: Rebecca Ahrens-Nicklas, CHOP	
16:30 – 17:00	Melissa Pratt - Small molecule compound AF38469 stimulates lysosomal biogenesis and rescues lysosomal function in <i>in-vitro</i> models of Batten Disease through inhibition of SORT1 (Sanford Health, Sioux Falls, USA)
17:00 – 17:30	Clarissa Booth - <i>In-vivo</i> short-term screening with small molecule compound AF38469 results in rescue of brain lysosomal storage and neuro-inflammation through SORT1 inhibition in mouse models of Batten disease (Sanford Health, Sioux Falls, USA)
17:30 – 18:00	Llinos Honeybun - Development of a phenotypic drug screen for CLN3 disease (Cardiff University, Cardiff, UK)
18:00 – 18:30	Summary
19:00 – 21:30	Social Event for participants of the 9 th JNCL Young Investigator Symposium