



8th International Symposium on NBIA

October 13-15, 2022

Website: [>> more information <<](#)

Lausanne, Switzerland
Mövenpick Hotel Lausanne

Program committee:

Susan Hayflick, Oregon Health & Science University, Portland (US) | Thomas Klopstock, LMU Klinikum, Munich (DE) | Markus Nielbock, Hoffnungsbaum e.V., Würselen (DE)
Agnès Rötig, Institut Imagine, Paris (FR) | Valeria Tiranti, IRCCS Istituto Neurologico Carlo Besta, Milan (IT)

Local organizing committee:

Patrick Herren, Fatemeh Mollet, Esther Schärer, Olav Zilian, NBIA Suisse (patient advocacy association), Lausanne (CH)

SCHEDULE

THURSDAY, October 13, 2022

13:00 Registration

14:00 Welcome Address

Fatemeh Mollet, NBIA Suisse, Lausanne, Switzerland
Patricia Wood, NBIA Disorders Association, El Cajon, USA
Thomas Klopstock, LMU Klinikum, Munich, Germany

SESSION 1 NBIA general

Chair: Thomas Klopstock

14:15 **Key note lecture: molecular mechanisms of iron-sulfur protein biogenesis and its link to iron metabolism**
Roland Lill, Marburg, Germany

15:00 **Epidemiology of NBIA disorders**
Hana Kolářová, Prague, Czech Republic

15:20 **Unreported NBIA in known disease genes**
Agnès Rötig, Paris, France

15:40 **Iron chelation in neurodegenerative disorders – state of the art and what it means for NBIA**
David Devos, Lille, France

16:00 Break

| SESSION 2 | PKAN & CoPAN | Chair: Valeria Tiranti |
|-----------|--|------------------------|
| 16:30 | Massive iron accumulation in PKAN-derived neurons and astrocytes: light on the human pathological phenotype Sonia Levi, Milan, Italy | |
| 16:50 | Zebrafish models with inborn errors of CoA biosynthesis Dario Finazzi, Brescia, Italy | |
| 17:10 | Mitochondrial and Mitophagy in PKAN: insights from a <i>Drosophila</i> model Zhihao Wu, Dallas, USA | |
| 17:30 | Yeast as a model for functional analysis of mutations associated with PKAN disorder and for the identification of therapeutic molecules Paola Goffrini, Parma, Italy | |
| 17:50 | Modelling CoPAN in mice Ivano Di Meo, Milan, Italy | |
| 18:10 | Combination of a specific diet and the microbiome compensates lethality of a PKAN animal model Hein Schepers, Groningen, the Netherlands | |
| 18:30 | End of the day | |

FRIDAY, October 14, 2022

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| 08:30 | Update on the CoA-Z clinical trial Penelope Hogarth, Portland, USA |
| 08:50 | Optimization and efficacy of the clinical compound BBP-671 in a PKAN mouse model Suzanne Jackowski, Memphis, USA |
| 09:10 | The PanK Activator BBP-671 for PKAN: results from a phase 1 trial in healthy volunteers, and update on pivotal trial plans Susanne A. Schneider, Munich, Germany |
| 09:30 | Roundtable discussion on urgent needs and next steps in PKAN/CoPAN |
| 10:30 | Break |

| SESSION 3 | MPAN | Chair: Agnès Rötig |
|-----------|--|--------------------|
| 11:00 | Expanding our understanding of <i>C19orf12</i> function to develop therapeutic approaches for MPAN Arcangela Iuso, Munich, Germany | |
| 11:20 | Modelling <i>c19orf12</i> deficiency in zebrafish Barbara Gnutti, Brescia, Italy | |
| 11:40 | Roundtable discussion on urgent needs and next steps in MPAN | |
| 12:30 | Break | |

| SESSION 4 | BPAN | Chair: Susan Hayflick |
|-----------|--|-----------------------|
| 13:30 | Is it all about autophagy? - WDR45's role in autophagy and beyond Mario Mauthe, Groningen, the Netherlands | |
| 13:50 | Towards an understanding of the molecular function of WDR45/WIP14 in autophagy and BPAN Tassula Proikas-Cezanne, Tübingen, Germany | |
| 14:10 | Vulnerability of midbrain dopaminergic neurons in BPAN Lena Burbulla, Munich, Germany | |
| 14:30 | Characterization of a whole-body <i>Wdr45</i> knock-out, a mouse model for BPAN Arcangela Iuso, Munich, Germany | |
| 14:50 | Roundtable discussion on urgent needs and next steps in BPAN | |
| 15:40 | Flash talks - selected poster presentations | |
| 15:50 | Poster Session & Coffee Break | |
| 17:15 | End of the day | |

| SESSION 5 | PLAN/INAD & FAHN | Chair: Ody Sibon |
|-----------|--|------------------|
| 09:00 | Towards precision therapies for NBIA Manju Kurian, London, United Kingdom | |
| 09:20 | PLA2G6-associated neurodegeneration – update Manju Kurian, London, United Kingdom | |
| 09:40 | Developing gene therapy for PLAN and moving towards clinical trials Ahad Rahim, London, United Kingdom | |
| 10:00 | Moving forward with FAHN research Sunita Venkateswaran, Ottawa, Canada | |
| 10:20 | Roundtable discussion on urgent needs and next steps in PLAN/INAD & FAHN | |
| 11:00 | Closing remarks Thomas Klopstock, LMU Klinikum, Munich, Germany | |
| 11:15 | End of the conference | |

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