



# 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) | Patricia Wood, NBIA Disorders Association, El Cajon (US)

#### Local organizing committee:

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

# SCHEDULE

#### THURSDAY, October 13, 2022

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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 1NBIA generalChair: Thomas Klopstock14:15Key note lecture: molecular mechanisms of iron-sulfur protein biogenesis and its link to iron metabolism<br/>Roland Lill, Marburg, Germany15:00Epidemiology of NBIA disorders<br/>Hana Kolářová, Prague, Czech Republic15:20Unreported NBIA in known disease genes<br/>Agnès Rötig, Paris, France15:40Iron chelation in neurodegenerative disorders - state of the art and what it means for NBIA<br/>David Devos, Lille, France

16:00 Coffee break



SESSION 2	PKAN & CoPAN	Chair: Valeria Tiranti
16:30	Massive iron accumulation in PKAN-derived neurons and astrocytes: light or Sonia Levi, Milan, Italy	n the human pathological phenotype
16:50	Zebrafish models with inborn errors of CoA biosynthesis Dario Finazzi, Brescia, Italy	
17:10	Mitochondrial and mitophagy in PKAN: insights from a Drosophila mode Zhihao Wu, Dallas, USA	el
17:30	Yeast as a model for functional analysis of mutations associated with PKA on of therapeutic molecules Paola Goffrini, Parma, Italy	N disorder and for the identificati
17:50	Modelling CoPAN in mice Ivano Di Meo, Milan, Italy	
18:10	<b>Combination of a specific diet and the microbiome compensates lethality</b> <b>Hein Schepers,</b> Groningen, the Netherlands	of a PKAN animal model
18:30	End of the day	
19:30	Dinner	

#### FRIDAY, October 14, 2022

08:30	Update on the CoA-Z clinical trial Penelope Hogarth, Portland, USA
08:50	<b>Optimization and efficacy of the clinical compound BBP-671 in a PKAN mouse model</b> <b>Suzanne Jackowski,</b> 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 / Thomas Klopstock, Munich, Germany
09:30	Roundtable discussion on urgent needs and next steps in PKAN/CoPAN
10:30	Coffee break

SESSION 3	MPAN	Chair: Agnès Rötig
11:00	Expanding our understanding of C19orf12 function to develop theraped Arcangela Iuso, Munich, Germany	itic approaches for MPAN
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 Lunch 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/WIPI4 in autophagy and BPAN Tassula Proikas-Cezanne, Tübingen, Germany
14:10	<b>Vulnerability of midbrain dopaminergic neurons in BPAN</b> <b>Lena Burbulla,</b> Munich, Germany
14:30	Characterization of a whole-body Wdr45 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
16:00	Poster session & Coffee break
17:30	End of the day
19:30	Dinner

## SATURDAY, October 15, 2022

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

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