

Preliminary Program (As of March 26, 2024)

Day 1

9:00-9:15 **Welcome and opening remarks**
Ziv Gan-Or, Guy Rouleau

9:15-10:30 **The role of *GBA1* and the lysosomal glycosphingolipid metabolism pathway in LSDs**
Chair: Ellen Sidransky

- The normal structure and function of Glucocerebrosidase – Speaker: **Gregory Grabowski**
- The lysosomal glycosphingolipid metabolism pathway: an overview – Speaker: **Peter Lansbury**
- Gaucher disease and related lysosomal storage disorders – Speaker: **TBD**

10:30-11:00 **Coffee break**

11:00-12:30 **Mechanisms in *GBA1*-associated neurodegeneration 1**
Chair: Edward Fon

- Lysosomal dysfunction and alpha-synuclein accumulation – Speaker: **Joe Mazzuli**
- *GBA1* and the mitochondria – Speaker: **TBD**
- *GBA1* and the immune system – Speaker: **Malu Tansey**

12:30-13:30 **Lunch**

13:30-15:00 **Mechanisms in *GBA1*-associated neurodegeneration 2**
Chair: Thomas Durcan

- Is there a role for ER stress in *GBA1*-associated neurodegeneration? – Speaker: **Mia Horowitz**

- Other mechanisms potentially involved in *GBA1*-associated neurodegeneration – Speaker: **Stacy Henry**
- Loss- vs. Gain-of-function mechanisms – a discussion / panel – Participants: **Mia Horwitz, Ziv Gan-Or**, others...

15:00-15:30 **Break**

15:30-17:00 **Data Blitz 1 – Genetics and basic science studies on *GBA1* and the lysosomal glycosphingolipid metabolism pathway**

Chair: Ziv Gan-Or

- 8-12 presentations of new data on *GBA1* and the glycosphingolipid metabolism pathway in PD. – Speakers: **TBD**

Day 2

9:00-10:30 **Genetics of *GBA1*-associated neurodegeneration**

Chair: Christine Klein

- *GBA1* frequencies in PD and dementia across different populations – Speaker: **Sonja Scholz**
- The importance of the intronic *GBA1* variant identified in people of African origin – Speaker: **Oluwadamilola Ojo**
- Classification and penetrance of *GBA1* variants, and the potential role of modifiers – Speaker: **Ziv Gan-Or**

10:30-11:00 **Coffee break**

11:00-12:30 **The role of other players in the lysosomal glycosphingolipid metabolism pathway in neurodegeneration**

Chair: Peter Lansbury

- Is SMPD1 a PD gene? – Speaker: **Jia Nee Foo**
- The role of other genes in the lysosomal glycosphingolipid metabolism pathway in PD – Speaker: **Konstantin Senkevich**
- What we know and what we don't know about the role of lipids in the lysosomal glycosphingolipid metabolism pathway in PD – Speaker: **Irina Alecu**

12:30-13:30 **Lunch**

13:30-15:00 **Characteristics of individuals with *GBA1*-associated neurodegeneration**

Chair: Roy Alcalay

- The phenotype of *GBA1*-associated PD – Speaker: **Kathrin Brockmann**
- *GBA1* and REM-sleep behavior disorder – Speaker: **Ronald Postuma**
- Pathology in *GBA1*-associated PD/dementia – Speaker: **Lorraine Kalia**

15:00-15:30 **Break**

15:30-17:00 **Collaborative efforts to advance *GBA1* research and treatment**

Chairs: TBD

- G-CAN: an overview – Speaker: **Lorna Chebon-Bore**
- Panel Discussion: Accelerating *GBA1* discoveries and treatment through collaboration – panelists: **TBD**

Day 3

9:00-10:30 **Data Blitz 2 – Clinical studies on *GBA1* and the lysosomal glycosphingolipid metabolism pathway**

Chair: Michael Schlossmacher

- 8-12 presentations of new data on *GBA1* in the clinic – Speakers: **TBD**

10:30-11:00 **Coffee break**

11:00-13:00 **Current status of *GBA1* clinical trials**

Chair: Ron Postuma

- The landscape of the current pre-clinical and clinical trials on *GBA1* – Speaker: **Simon Stott**
- What can we learn from the MOVES-PD trial? – Speaker: **Pablo Sardi**
- Overview of completed and ongoing Ambroxol trials – Speaker: **Stephen Mullin**
- Gene therapy for *GBA1*-associated neurodegeneration – Speaker: **TBD**

13:00-14:00 **Lunch**

14:00-15:00 **Consideration in clinical trial design for *GBA1*-associated neurodegeneration**

Chairs: TBD

- Mutation classification and genetic stratification in *GBA1* trials – Speaker: **Ziv Gan-Or**
- The current status of biomarkers for *GBA1* PD – Speaker: **Roy Alcalay**
- Other consideration for inclusion / exclusion and endpoints in *GBA1* trials – Speaker: **Joana Almeida**

15:00-16:00 **Break**

16:00-17:00 **Concluding Session: Moving Forward**

Chair: TBD

- Trial ready populations and the future of clinical genetic screening – Speaker: **TBD**
- Creation of task forces: Basic science, translational studies, clinical studies – panel **TBD**
- Concluding remarks: **Ziv Gan-Or**