

Thursday, June 5th

8:00 - 9:00 Registration & Breakfast
9:00 - 9:05 Welcome and opening remarks – Ziv Gan-Or
9:05 - 9:45 Dimitri Krainc – Keynote presentation: GBA1 modifiers and the current state of GBA1 targeted therapies

9:45 - 10:30 Small molecules targeting GBA1 Session 1
Chair: Peter Lansbury

9:45 – 10:05 Marco Toffoli – Overview of Ambroxol trials

10:05 – 10:20 Mark Henderson – Update on small molecule screens

10:20 – 10:30 Q&A
10:30 - 11:00 Coffee break
11:00 - 12:30 Small molecules targeting GBA1 session 2
Chair: Ellen Sidransky

11:00 – 11:20 Daniel Ysselstein – Update from Vanqua Therapeutics

11:20 – 11:40 Jonas Hannestad – Update from Gain Therapeutics

11:40 – 12:00 Indranath Ghosh – Update from Congruence Therapeutics

12:00 – 12:20 Raquel Costa – Update from Bial

12:20 – 12:30 Q&A
12:30 - 14:00 Lunch + Posters session (Posters 1-14, A-C)
14:00 - 14:30 Pola and Rich Sussman – A Patient and Care Partner perspective on GBA1 PD

14:30 - 15:30 Data Blitz 1
Chair: Thomas Durcan

14:30 – 14:40 Oriol Narcis – An Integrated Understanding of Immunity in Idiopathic, Prodromal and GBA1 Parkinson's Disease

14:40 – 14:50 Claire Leckey – Development of an ultra-sensitive LC-MS/MS method for lyso-Gb1 and Gb1 quantitation in mouse CSF: enabling pre-clinical and clinical applications for monitoring Gaucher and Parkinson's disease

14:50 – 15:00 Daan van Kruining – Disrupted Glycosphingolipid Homeostasis in GBA-Associated Parkinson's Disease Across Human and Model Organisms

15:00 – 15:10 Oliver Davis – Combining stabilized glucocerebrosidase with a blood-brain barrier TransportVehicle™ enhances brain delivery of active enzyme in preclinical models of Gaucher and Parkinson's diseases

15:10 – 15:20 Jonggeol Jeffrey Kim – Multi-ancestry Exome-wide Analysis Identifies Novel Genetic Modifiers of GBA1 Penetrance in Parkinson's disease

15:20 – 15:30 Jillian Kluss – Glucosylsphingosine levels and GCase activity in central and peripheral biofluids in a large cohort of monogenic and sporadic Parkinson's disease

15:30 - 16:00 Coffee Break

DAY 1

Thursday,
June 5th

GBA1
Canada
MEETING 2025

16:00 - 17:00 Interactions between progranulin, LRRK2 and glucocerebrosidase activity

Chair: Michael Schlossmacher

16:00 – 16:25 Hideyuki Takahashi – The interplay between progranulin and glucocerebrosidase and its therapeutic implications

16:25 – 16:50 Rachel Saunders-Pullman – The interplay between LRRK2 and glucocerebrosidase; conflicting evidence and therapeutic implication

16:50 – 17:00 Q&A

18:00 Cocktail Reception sponsored by Prevail Therapeutics
(Open to ALL attendees)

Prevail
THERAPEUTICS

A Wholly Owned Subsidiary
of Eli Lilly and Company

DAY 2

Friday,
June 6th

7:30 - 9:00 Registration & Breakfast

9:00 - 10:30 How are population-based studies informing GBA1 targeting strategies

Chair: Shalini Padmanabhan

9:00 – 9:25 Andrew Singleton – Genetic modifiers of GBA1 PD risk and progression

9:25 – 9:50 Roy Alcalay – Parkinson's disease in Gaucher's disease

9:50 – 10:15 Kathrin Brockmann – GBA1 Consortium: Joint Analysis to Inform Clinical Trials

10:15 – 10:30 Q&A

10:30 - 11:00 Coffee break

11:00 - 12:00 Data Blitz 2

Chair: Joe Mazzuli

11:00 – 11:10 Soyoung Lee – A Potent GCase Activator Demonstrating Therapeutic Potential in Preclinical Models of Parkinson's Disease with GBA Mutations

11:10 – 11:20 Leon Murphy – CSM-101, a small molecule agonist of TRPML1 for Parkinson's-related disorders

11:20 – 11:30 Michael Schlossmacher – Gba1 p.D409V Mutant Alleles Confer Protection During Acute Infection by a Neurotropic RNA Virus Following Nasal Inoculation

11:30 – 11:40 Samantha Howard – Optimized liver targeted GBA expression via AAV3b for Type 1 Gaucher Disease treatment

11:40 – 11:50 Joshua Shulman – Mapping the causal chain from genetic risk variants to lipid dysmetabolism in Parkinson's disease

11:50 – 12:00 Raquel Nunez-Toldra – SPR301 - Harnessing GCase85 for Enhanced -Synuclein Clearance in GBA1-Linked Parkinson's Disease

DAY 2

Friday,
June 6th

GBA1
Canada
MEETING 2025

12:00 - 14:00 **Lunch + Poster session (Posters 15-27, D-F)**

14:00 - 15:30 **Clinical considerations in GBA1-associated neurodegeneration**

Chair: Sonja Scholz

14:00 - 14:20 **Lola Cook** – Genetic counseling for GBA1 carriers

14:20 - 14:40 **Ari Zimran** – Is there a room for pre-implantation genetic diagnosis in the context of GBA1-associated neurodegeneration?

14:40 - 15:00 **Tanya Simuni** – Lessons from GBA1-associated PD in PPM1

15:00 - 15:20 **Micol Avenali** – Evidence for and against DBS in GBA1-PD

15:20 - 15:30 **Q&A**

15:30 - 16:00 **Coffee break**

16:00 - 17:00 **Gain vs. loss of function mechanisms in GBA1**

Chair: Rejko Krüger

16:00 - 16:25 **Mia Horowitz** – ER stress and the evidence for gain of function role for GBA1 mutations in PD

16:25 - 16:50 **Anastasia Henry** – The common E326K variant in GBA1-PD; evidence for loss of function

16:50 - 17:00 **Q&A**

18:00 **Cocktail Reception (By Invitation ONLY)**

DAY 3

Saturday,
June 7th

7:30 - 9:00 **Registration & Breakfast**

9:00 - 10:30 **Gene and cell therapy for GBA1-associated disease**

Chair: Ziv Gan-Or

9:00 - 9:20 **Swathi Ayloo** – Update from Sanofi

9:20 - 9:40 **Kim McDowell** – Update from Capsida Biotherapeutics

9:40 - 10:00 **Sarah Neuhaus** – Update from Prevail Therapeutics

10:00 - 10:20 **Avram Frint** – Update from Aspen Neuroscience

10:20 - 10:30 **Q&A**

10:30 - 11:00 Coffee break

11:00 - 12:15 Targeting other lysosomal genes for PD

Chair: Peter McPherson

11:00 – 11:15 Ricardo Feldman – ASAH1
 11:15 – 11:30 Edward Fon – CTSB
 11:30 – 11:45 Richard Hite – TMEM175
 11:45 – 12:00 Friederike Zunke – SCARB2
 12:00 – 12:15 Q&A

12:15 – 14:15 Lunch + Poster session (Posters 28-40, G-H)

14:15 – 14:55 Data Blitz 3

Chair: Edward Fon

14:15 – 14:25 Thao Nguyen-Tran – Increased activation in pathway mediated by GBA1 novel transglycosidase function in GBA1-PD patients
 14:25 – 14:35 Yu Chen – Analysis of GBA1 isogenic dopaminergic neurons reveals bidirectional regulation of glycoprotein nonmetastatic melanoma protein B (GPNMB) by α -glucocerebrosidase deficiency
 14:35 – 14:45 Donovan Worrall – CLCN3 May Limit Alpha-Synuclein Accumulation by Promoting Lysosomal Acidification and Function
 14:45 – 14:55 Manoj Kumar – Mutant GBA1 microglia induce α -synuclein aggregation in dopamine neurons from GBA1/PD patients

14:55 - 15:50 Role of glucocerebrosidase and lipid / mitochondria dysregulation in neurodegeneration

Chair: Michela Deleidi

14:55 – 15:10 Michela Deleidi – The role of GBA1 in mitochondrial dysfunction
 15:10 – 15:25 Marta Martinez-Vicente – Glucocerebrosidase and lipid dysregulation in neurons
 15:25 – 15:40 Robert Krencik – Glucocerebrosidase and lipid dysregulation in astrocytes
 15:40 – 15:50 Q&A

15:50 - 16:10 Coffee break

16:10 - 16:40 Resources available for GBA1 research

Chair: Ziv Gan-Or

16:10 – 16:25 Tatiana Foroud – New glycosphingolipid biomarkers in PPMI
 16:25 – 16:40 Alastair Noyce – GP2 prodromal project

16:40 - 17:00 Ziv Gan-Or - Awards for best trainee abstracts, G-Can and concluding remarks