

3rd International GBA1 Meeting 2026

May 22-23, Phoenix, Arizona, US

Satellite meeting of World Parkinson's Congress

Preliminary Conference Program

Day 1: May 22		
Time	Session	Speaker
7:30 - 9:00	Registration & Breakfast	-
9:00 – 9:10	Welcome and opening remarks	Ziv Gan-Or
9:10 – 9:40	Keynote presentation: <i>Recent developments in GBA1 research and future perspectives</i>	Tony Futerman, The Weizmann Institute of Science
9:40-10:30	GBA1 therapeutics	Chair: Ari Zimran, Shaare Zedek Medical Center
9:40 – 10:00	Overview of GBA1-targeting trials	Simon Stott, Cure Parkinson's
10:00 – 10:20	Report on the ACTIVATE trial	Tiago Fonseca, Bial
10:20 – 10:30	Q&A	-
10:30 – 11:00	Coffee break	
11:00 – 12:30	Advances in GBA1 mechanisms	Chair: Peter Bauer, Centogene
11:00 – 11:20	The cholinergic system in GBA1-PD	Teus van Laar, University of Groningen
11:20 – 11:40	GBA1 and Lysosomal Oligogenic Risk for Parkinson's Disease	Joshua Shulman, Baylor College of Medicine
11:40 – 12:00	Targeting Lysosomal pH Restores Mitochondrial Quality Control in GBA1-Mutant Parkinson's Disease	Preethi Sheshadri, UK Dementia Research Institute

12:00 – 12:20	Lysosomal glucocerebrosidase is needed for ciliary Hedgehog signaling	Suzanne Pfeffer, Stanford University
12:20 – 12:30	Q&A	-
12:30 – 14:30	Lunch + Poster session (Posters 2201-2215)	
14:30 – 15:30	Data Blitz 1	Chair: Jodi Maple Grødem
14:30 – 14:40	Liver-direct gene therapy enables brain delivery of GCase and extends survival in neuronopathic Gaucher disease mice	Maria Laura Allende, National Institutes of Health
14:40– 14:50	Longitudinal IQ and olfactory assessments in GBA1 variant carriers with and without Parkinson disease	Ellen Sidransky, National Institutes of Health
14:50 – 15:00	Dual targeting of GCase trafficking and activity to rescue Parkinson's disease-associated defects in GBA1 mutant dopaminergic neurons	Camille Lacarrière-Keïta, McGill University
15:00 – 15:10	Development of a small molecule TRPML1 agonist for Parkinson's disease	Valerie Cullen, Lysoway Therapeutics Inc
15:10 – 15:20	SIM0817: Small Molecule GCase Activator for the Treatment of Parkinson's Disease: Improved Potency and Favorable Druggability	Xiaoyuan Wei, Simcere Pharmaceutical
15:20 – 15:30	Genetic screening in people with Parkinson's disease: challenges and solutions in identification of gba1 mutation carriers for future clinical trials	Jurrian van der Valk, Centre for Human Drug Research
15:30 – 16:00	Coffee break	
16:00 – 17:00	In the pipeline: updates from pharma on ongoing preclinical studies and clinical trials	Chair: Omer Siddiqui, NeuroVC
16:00 – 16:15	Venglustat in GD – implications for GD and PD	Pramod Mistry, Yale School of Medicine
16:15 – 16:30	An update on the development of the GCase modulator GT-02287 for Parkinson's disease	Jonas Hannestad, Gain Therapeutics

16:30 – 16:45	Vq-101, an allosteric activator of lysosomal glucocerebrosidase, shows sustained target and pathway engagement in patients with Parkinson's disease	Daniel Ysselstein, Vanqua Bio
16:45 – 17:00	Q&A	-
18:00-21:00	Cocktail Reception + meet the experts	

Day 2: May 23		
Time	Session	Speaker
8:00 - 9:00	Registration & Breakfast	-
9:00 – 9:10	Announcement of trainee awards for best abstracts Announcement of <i>GBA1</i> funding	Ziv Gan-Or, Shalini Padmanabhan
9:10 - 10:00	Novel therapeutic approaches for <i>GBA1</i>-associated diseases	Chair: Shalini Padmanabhan, The Michael J. Fox Foundation
9:10 – 9:40	Keynote presentation – Base editing for GD and <i>GBA1</i> -associated PD: lessons from Tay Sachs	Richard Proia, National Institutes of Health
9:40 – 10:00	<i>GBA1</i> in neurons vs astrocytes	Marie Davis, University of Washington
10:00 – 10:10	Q&A	
10:10 – 10:40	Coffee break	
10:40 – 12:00	Data Blitz 2	Chair: Konstantin Senkevich, McGill University

10:40 – 10:50	Development of an efficacious systemic AAV gene therapy for neuronopathic Gaucher disease with applicability to GBA1-associated PD	Ellen Sidransky, National Institutes of Health
10:50 – 11:00	GBA2 as a regulator of glycosphingolipid synthesis and Gaucher disease pathogenesis	Hannah Duffy, National Institutes of Health
11:00 – 11:10	Glycosphingolipid dysregulation in <i>GBA1</i> -PD: combined plasma and brain analysis	Linda Montavoci, Oxford University
11:10 – 11:20	Neurotinib, a brain penetrant c-abl inhibitor, prevents activation and phosphorylation of c-abl kinase and α -synuclein, in in vivo and in vitro models	Nahid Tayebi, National Institutes of Health
11:20 – 11:30	A Novel Regulator of GCase: CLCN3 Regulates Lysosomal Function in Parkinson's Disease	Donovan Worrall, McGill University
11:30 – 11:40	Single-cell RNA sequencing analysis of the peripheral immune system in idiopathic, <i>LRRK2</i> and <i>GBA1</i> Parkinson's disease	Mikaela Rosen Perez, Icahn School of Medicine
11:40 – 11:50	Interim Results from the AGP1 Trial: High-Dosage-Ambroxol in GBA1-Associated Parkinson's Disease (Sidransky syndrome)	Ari Zimran, Shaare Zedek Medical Center
11:50 – 12:00	Linking PD GENERation Genetic Testing with Continuous Digital Phenotyping to Characterize GBA1 Variant-Associated Progression in Parkinson Disease	Ro'ee Gilron, Rune Labs
12:00 – 14:00	Lunch + Poster session (Posters 2301-2316)	
14:00-15:30	Clinical considerations in <i>GBA1</i>-PD	Chair: Simon Stott, Cure Parkinson's
14:00 – 14:20	The global burden of <i>GBA1</i> -PD – clinical implications	Ziv Gan-Or, McGill University
14:20 – 14:40	Treatment of non-motor symptoms in <i>GBA1</i> -PD	Avner Thaler, Tel Aviv University
14:40 – 15:00	Biomarkers for <i>GBA1</i> -PD	Fernanda Cerqueira,

		The Michael J. Fox Foundation
15:00 – 15:20	Challenges in genetic counseling for <i>GBA1</i> in PD	Valentina Caceres, Indiana University School of Medicine
15:20 – 15:30	Q&A	-
15:30 – 16:00	Coffee break	
16:00 – 17:00	<i>GBA1</i> modifiers	Chair: Friederike Zunke, University Hospital Erlangen
16:00 – 16:20	Bidirectional regulation of GPNMB by β -glucocerebrosidase deficiency	Yu Chen, National Institutes of Health
16:20 – 16:40	CRISPR screening for modifiers of GCase activity	Melissa Roberts, Denali Therapeutics
16:40 – 16:50	Q&A	-
16:50 – 17:00	Concluding remarks	Ziv Gan-Or
18:00 – 20:00	Goodbye cocktail	