

3rd IWGDD Symposium, Trieste, Italy (03-06 May 2026)

Bridging the gap: from basic research to patient care

Meeting Agenda	
Sunday 03/May/2026	
15:00 - 16:30	<i>Simultaneous IWGDD Working Groups - Open to Members Only</i>
16:30 - 18:00	<i>Educational Session - Open to Members & Eligible Sponsors Only</i>
19:00 - 22:00	Opening Lecture & Dinner How it All Began: The Story of Gaucher Disease and the IWGDD Keynote: Bruno Bembi (Italy)
Monday 04/May/2026	
09:00 - 10:45	Session 1: Patients' perspective (Part 1) Moderators: Maurizio Scarpa, Derralynn Hughes, Tanya Collin-Histed
09:00 - 09:45	Plenary Lecture An Overview of the Current Situation of Rare Diseases in Africa Chris Hendriksz (United Kingdom)
09:45 - 10:45	Case Study Presentations African physicians share experiences and challenges in diagnosing and managing GD Pheobe Wamalwa (Kenya), Kandi-Catherine Muze (Tanzania), Magy Abdelwahab (Egypt)
10:45 - 11:15	Coffee Break
11:15 - 12:30	Session 1: Patients' perspective (Part 2) Moderators: Maurizio Scarpa, Derralynn Hughes, Tanya Collin-Histed
11:15 - 11:45	IGA in Africa: Highlights of ongoing initiatives Presented by Roselyn Karungari Kanja (Kenya)

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11:45 - 12:30	Selected Oral Presentations
11:45 - 12:00	Expanding Global Reach: The Impact and Evolution of the International Gaucher Alliance's Global Gaucher Connect Programme (GGCP) Vesna Aleksovska (North Macedonia)
12:00 - 12:15	Different diseases, different needs: Patient preferences for gene therapy and beliefs about prescribed medications vary in lysosomal storage diseases Eleonore Corazolla (The Netherlands)
12:15 - 12:30	Guidelines on Home Therapy in Gaucher Disease Carolina Toneloto (Brazil)
12:30 - 13:30	Lunch
13:30 - 15:30	Session 2: Laboratory – Genetics & Biochemistry Moderators: Paula Rozenfeld, Andrea Dardis
13:30 - 14:15	Plenary Lecture GCase genomic modifiers: an opportunity for designing therapeutics Andres Klein (Chile)
14:15 - 15:30	Selected Oral Presentations
14:15 - 14:30	Beyond Sequence Homology: Dissecting Complex Alleles and Epigenetic Landscapes at the GBA1–GBA1LP Locus via Native DNA Nanopore Long-Read Sequencing Natascha Bergamin (Italy)
14:30 - 14:45	Elevated soluble ACE2 in Gaucher patients - an evolutionary advantage? Tova Hershkovitz (Israel)
14:45 - 15:00	Glucosylated phytosterols: a new player in Gaucher disease? Johannes Aerts (The Netherlands)
15:00 - 15:15	Immune and Inflammatory Signatures in Gaucher Disease: Implications for Gene and Precision Therapies. Margarita Ivanova (United States)
15:15 - 15:30	When glucosylsphingosine does not normalize: pharmacogenetic determinants of biochemical–clinical dissociation during eliglustat therapy Sonia Roca-Esteve (Spain)
15:30 - 16:00	Coffee Break

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16:00 - 18:15	Session 3: Gaucher Disease Clinical Spectrum Moderators: Shoshana Revel-Vilk, Francesca Carubbi
16:00 - 16:45	Plenary Lecture Incorporating skin evaluation into the standard care of patients with Gaucher disease Ayelet Ollech (Israel)
16:45 - 18:15	Selected Oral Presentations
16:45 - 17:00	Perinatal-Lethal Gaucher Disease: Clinical, Biochemical and Pathological Insights from 15 French Cases Magali Pettazzoni (France)
17:00 - 17:15	Lyso-Gb1 Dynamics in Untreated Patients with Gaucher Disease Shoshana Revel-Vilk (Israel)
17:15 - 17:30	Gaucher Disease type 1 in Elderly Individuals: A Systematic Review François Maillot (France)
17:30 - 17:45	Development and Validation of an Age-Adapted Disease Severity and Burden Scoring System for Neuronopathic Gaucher Disease (nGD/GD3) Leah Svarney (United States)
17:45 - 18:00	Risk and Prevalence of Overweight and Obesity Among Adults with Gaucher Disease Ari Zimran (Israel)
18:00 - 18:15	An AI-Driven Multilayer Model of Lung Involvement in Neuronopathic Gaucher Disease (nGD): Validation in a 45-Patient Cohort and Implications for Management Sara Mitchell (United States)
Tuesday 05/May/2026	
08:30 - 10:30	Session 4: Laboratory – Basic Research Moderators: Johannes Aerts
08:30 - 09:15	Johannes Aerts Basic Science Lecture 2026 Decoding the Brain's Lysosomes: A Cell-Type-Specific Protein Atlas and the identification of a new Lysosomal Disorder Ali Ghoochani (United States)
09:15 - 10:30	Selected Oral Presentations

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09:15 - 09:30	Evaluation of dopaminergic neurons from isogenic iPSC lines derived from a donor with Gaucher disease and Parkinson disease reveal the role of GPNMB in GBA1-associated parkinsonism Ellen Sidransky (United States)
09:30 - 09:45	Mutant microglia from Parkinson's disease patients with heterozygous GBA1 mutations are key determinants of alpha-synuclein aggregation and dopamine neuron pathology Ricardo A. Feldman (United States)
09:45 - 10:00	Utilising machine learning for Gaucher disease: predicting structural defects due to mutation of beta-glucocerebrosidase Thomas J. McCorvie (United Kingdom)
10:00 - 10:15	Novel High-Resolution Exosomal Transcriptomics for Biomarker Discovery in Gaucher Disease Reena V. Kartha (United States)
10:15 - 10:30	Comparison of human and zebrafish glucocerebrosidase and generation of chimeric forms: importance of non-catalytic loops for catalytic activity and transglucosylation Mats J. Bulterman (The Netherlands)
10:30 - 11:00	Coffee Break
11:00 - 13:00	Session 5: Comorbidities in Gaucher Disease Moderators: Predrag Rodic, Ida Schwartz
11:00 - 11:45	Plenary Lecture Developing therapeutic strategies for GBA1-PD: hints from experimental models Alessio Di Fonzo (Italy)
11:45 - 13:00	Selected Oral Presentations
11:45 - 12:00	Longitudinal follow-up of GBA1-carriers for prodromal features of Parkinson disease, Sidransky syndrome Michal Becker-Cohen (Israel)
12:00 - 12:15	Mechanisms Underlying Mutant LRRK2's Modifying Effect on GBA1-Associated Parkinson Disease Vera Serebryany-Piavsky (Israel)
12:15 - 12:30	Non-Motor and Neuropsychological Differences Across the GBA1 Spectrum Ellen Sidransky (United States)

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12:30 - 12:45	Not All 'Mild' Genotypes Are Equal: Divergent Skeletal Burden in c.[1226A>G] Homozygous versus c.[1226A>G]/Mild Gaucher Disease. Irene Serrano-Gonzalo (Spain)
12:45 - 13:00	The "Perfect Storm" of Wnt Inhibitors Drives Early Osteoporosis in Female Patients with Gaucher Disease Margarita Ivanova (United States)
13:00 - 14:00	Lunch
14:00 - 14:45	Session 6: Rapid Communication Moderators: Marc Berger, Reena Kartha
	Expression of Mild GBA1 Mutations in A Fly Model Aparna Kuppuramalingam (Israel)
	Accelerating GD Diagnosis and Enabling Reliability differential detection with ASMD, thanks to MSMS analysis Magali Pettazzoni (France)
	The platelets lipidomic signature in Gaucher Disease Giuseppe Uras (United Kingdom)
	Cellular context matters: strengths and limitations of a GBA1-knockout model for functional assessment of GBA1 variants Maximiliano E. Ormazabal (Italy)
	Parkinson's disease and dementia with Lewy bodies in Gaucher disease: a multicenter longitudinal study Marco Percetti (Italy)
	Severe Secondary GM1 Ganglioside Accumulation in Gaucher Patients' Cell Lines Rodolfo Tonin (Italy)
14:45 - 16:45	IWGGD Annual General Meeting (AGM) - Open to Members Only
18:00 - 20:00	Walking Tour
20:00	Congress Dinner

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Wednesday 06/May/2026

08:30 - 10:15	Session 7: Therapies in Gaucher Disease (Part 1) Moderators: Ozlem Goker-Alpan, Magy Abdelwahab
08:30 - 09:15	Plenary Lecture Genome Editing landscape: from bench to bedside Andres Muro (Italy)
09:15 - 11:15	Selected Oral Presentations
09:15 - 09:30	Therapeutic efficacy of a novel glucocerebrosidase variant in a new preclinical model of neuronopathic Gaucher disease Allan Feng (United States)
09:30 - 09:45	A Phase 1 First-in-Human, Single- and Multiple- Ascending Dose Study of Glucosylceramide Synthase (GCS) Inhibitor YH35995 in Healthy Adult Male Participants YuKyung Kim (Republic of Korea) - Sponsored Presentation by YUHAN
09:45 - 10:00	Two-year follow up of avigagene parvec (FLT201) investigational AAV gene therapy in adults with Gaucher Disease type 1: Results from GALILEO-1 and GALILEO-2 Ida Schwartz (Brazil) - Sponsored Presentation by Spur Therapeutics
10:00 - 10:15	The PROCEED Study: A Phase 1/2 Dose-Escalation Investigation of Systemic AAV9-Based Gene Therapy for Peripheral Manifestations of Gaucher Disease Ozlem Goker-Alpan (United States) - Sponsored Presentation by Eli Lilly and Company
10:15 - 10:45	Coffee Break
10:45 - 12:00	Session 7: Therapies in Gaucher Disease (Part 2) Moderators: Ozlem Goker-Alpan, Magy Abdelwahab
10:45 - 11:00	Dose Spacing in Enzyme Replacement Therapy for Stable Type 1 Gaucher Disease: A Non-Inferiority Sequential Trial Emulation from the French Gaucher Disease Registry Yann Nguyen (France)
11:00 - 11:15	Safety and efficacy of venglustat versus imiglucerase in patients with Gaucher Disease Type 3 (LEAP2MONO): a phase 3, randomized, double-blind multicenter trial Karl Eugen Mengel (Germany) - Sponsored Presentation by Sanofi
11:15 - 11:30	Chaperone and antioxidant dual action iminosugar hybrids for Gaucher Disease Francesca Clemente (Italy)

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11:30 - 11:45	High-Dose Ambroxol for Gaucher Disease Type 3 in Children: A Prospective Exploratory Study <i>Huma Cheema (Pakistan)</i>
11:45 - 12:00	Development of a novel systemic AAV gene therapy for neuronopathic Gaucher disease <i>Ellen Sidransky (United States)</i>
12:00 - 12:15	<i>Best Oral & Poster Presentation Awards</i>
12:15 - 12:30	<i>Closing Remarks</i>

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