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

Venue: Savoia Excelsior Palace, Riva del Mandracchio 4, 34124 Trieste, Italy

Bridging the gap: from basic research to patient care

Sunday 03/May/2026	
15:00 - 16:30	*Simultaneous IWGGD Working Groups - Open to Members Only*
16:30 - 18:00	*Educational Session - Open to Members & Eligible Sponsors Only*
19:00 - 20:00	***Opening Lecture*** How it All Began: The Story of Gaucher Disease and the IWGGD ***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 (Poster Viewing)***
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)***

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 (Poster Viewing)***
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 (Poster Viewing)***

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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) ***Ozlem Goker-Alpan (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, Kasper Ter-Horst***
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 (Poster Viewing)***
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 (Poster Viewing)***
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*

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 beside ***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 avigbagene 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 (Poster Viewing)***
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 ***Huma Cheema (Pakistan)***
11:45 - 12:00	Development of a novel systemic AAV gene therapy for neuronopathic Gaucher disease ***Ellen Sidransky (United States)***
12:00 - 12:15	***Best Oral & Poster Presentation Awards***
12:15 - 12:30	*Closing Remarks*

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