

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 | <i>Simultaneous IWGGD Working Groups - Open to Members Only</i> |
| 16:30 - 18:00 | <i>Educational Session - Open to Members & Eligible Sponsors Only</i> |
| 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) |
| 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 Aleksovaska (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) |
| 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, Ozlem Goker-Alpan |
| 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 |
| 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) |
| 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 - 10:30 | Session 7: Therapies in Gaucher Disease (Part 1) Moderators: Ozlem Goker-Alpan, Huma Cheema |
| 11:45 - 13:00 | Selected Oral Presentations |
| 08:30 - 08:45 | Therapeutic efficacy of a novel glucocerebrosidase variant in a new preclinical model of neuronopathic Gaucher disease Allan Feng (United States) |
| 08:45 - 09:00 | 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 |
| 09:00 - 09:15 | 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 |
| 09:15 - 09:30 | 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 |
| 09:30 - 09:45 | 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) |
| 09:45 - 10:00 | 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 |
| 10:00 - 10:15 | Chaperone and antioxidant dual action iminosugar hybrids for Gaucher Disease Francesca Clemente (Italy) |
| 10:15 - 10:30 | Development of a novel systemic AAV gene therapy for neuronopathic Gaucher disease Ellen Sidransky (United States) |
| 10:30 - 11:00 | Coffee Break (Poster Viewing) |
| 11:00 - 12:30 | Session 7: Therapies in Gaucher Disease (Part 2) Moderators: Ozlem Goker-Alpan, Huma Cheema |
| 11:00 - 11:45 | Plenary Lecture The landscape of emerging genome editing therapeutics Gregory Newby (United States) |
| 11:45 - 12:00 | Best Oral & Poster Presentation Awards |
| 12:00 - 12:30 | Closing Remarks |

Gold Sponsors:



Silver Sponsors:



Bronze Sponsors:

