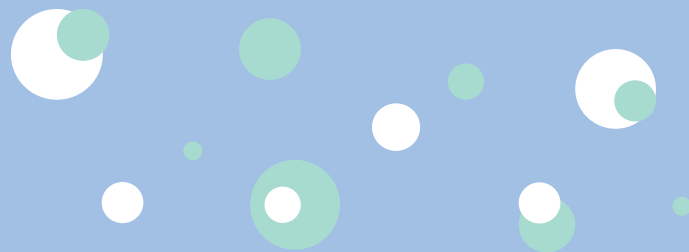


# 2nd IWGGD Symposium, Belgrade, Serbia (19-22 May 2024)



## Meeting Agenda

### Sunday 19 May 2024

15:00 – 16:30	Pre-meeting – <b>Simultaneous IWGGD Working Groups</b> (Additional Morbidities, Diagnostic, Laboratory, Management and Monitoring, Supportive Care) Halls: Baltic, Aegean, Danube, Drina, Executive Boardroom
16:30 – 18:00	Pre-meeting – <b>Educational session</b> ; pre-registration requested Hall: Mediterranean/Adriatic
18:30 – 19:30	<b>Opening Symposium</b> Hall: Mediterranean/Adriatic
18:30-18:50	Treating Gaucher disease in Serbia, both present and historical <b>Nada Suvajdzic Vukovic, Belgrade, Serbia</b>
18:50-19:10	Treating patients with neuropathic Gaucher disease in Serbia <b>Maja Djordjevic Milosevic, Belgrade, Serbia</b>
19:10-19:30	Immunoglobulin heavy chain gene rearrangements in patients with Gaucher disease <b>Predrag Rodic, Belgrade, Serbia</b>
19:30	Program at a Glance
19:40	<b>Welcome Reception</b> Garden: Hotel Crowne Plaza

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**Scientific Committee:** Predrag Rodic, Johannes Aerts, Derralynn Hughes, Carla Hollak, Marc Berger, Andrea Dardis, Shoshana Revel-Vilk

## Meeting Agenda

### Monday 20 May 2024 – Hall Exhibition

08:30 – 10:15	<b>Session 1: Patients' perspective of living with Gaucher disease</b> <b>Chairpersons: Jeremy Manuel, Tanya Collin-Histed</b>
08:30 – 09:30	GARDIAN – a prototype patient's lead registry <b>Tanya Collin-Histed, IGA</b>
09:30 – 10:15	Invited Speaker: Managing and leading an international network for a rare disorder <b>Biljana Jovanovic, IGA</b>
10:15 – 10:45	<b>Coffee Break</b>
10:45 – 12:15	<b>Session 1: Patients' perspective of living with Gaucher disease</b> <b>Chairpersons: Jeremy Manuel, Tanya Collin-Histed</b>
10:45 – 11:15	Invited Speaker: Challenges in access to orphan drugs <b>Carla Hollak, Netherlands</b>
11:15 – 12:15	<b>Selected Oral Abstracts</b>
11:15 – 11:30	Will rapid administration of enzyme replacement therapy impact patients' satisfaction? <b>Michal Becker-Cohen, Israel</b>
11:30 – 11:45	Preimplantation genetic testing (PGT) to reduce the risk for GBA-related Parkinson disease: Expanding of applications for embryo selection <b>Gheona Altarescu, Israel</b>
11:45 – 12:00	Assessing pediatric feeding in neuronopathic GD using remote smartphone video technology <b>Elin Haf-Davies, UK</b>
12:00 – 12:15	Never-treated, non-splenectomized patients with Gaucher Disease (GANT study): the prospective follow-up <b>Christine Serratrice, Switzerland</b>
12:15 – 13:30	<b>Lunch Break &amp; Poster Tour</b>
13:30 – 15:00	<b>Session 2: Laboratory: Genetics &amp; Biochemistry</b> <b>Chairpersons: Marc Berger, Andrea Dardis</b>
13:30 – 14:15	Invited Speaker: Activated proteins and mechanisms in Gaucher disease <b>Gregory Grabowski, USA</b>

## Meeting Agenda

### Monday 20 May 2024 – Hall Exhibition

14:15 – 15:30	<b>Selected Oral Abstracts</b>
14:15 – 14:30	Evaluation of technical practices used to measure CCL18 level in plasma from Gaucher disease patients, under the aegis of Biomarkers and Biomaterials IWGGD working group <b>Irene Serrano-Gonzalo, Spain</b>
14:30 – 14:45	When simultaneous detection becomes possible: A new screening method for lysosomal storage diseases <b>Amber Van Baelen, Belgium</b>
14:45 – 15:00	The role of miRNAs in bone involvement in Gaucher disease <b>Irene Serrano-Gonzalo, Spain</b>
15:00 – 15:15	Lyso-Gb1 as the driver of disease activity in neuronopathic Gaucher disease <b>Ozlem Goker-Alpan, USA</b>
15:30 – 15:45	<b>Coffee Break</b>
15:45 – 17:15	<b>Session 3: Gaucher Disease Clinical Spectrum</b> <b>Chairpersons: Derralynn Hughes, Shoshana Revel-Vilk</b>
15:45 – 16:15	Invited Speaker: Digital health in the diagnosis and management of Gaucher disease <b>Shoshana Revel-Vilk, Israel</b>
16:15 – 16:30	<b>Selected Oral Abstracts</b>
16:15 – 16:30	The phenotypic spectrum of type 2 Gaucher disease: time for new descriptors <b>Aimee Donald, UK</b>
16:30 – 16:45	The French Gaucher disease registry: clinical features, complications, and treatment trends of 688 patients <b>Nadia Belmatoug, France</b>
16:45 – 17:00	Is Gaucher disease in children a continuum of phenotype? A hypothesis or is it clinically evidence-based, analysis of a unique Egyptian cohort <b>Magy Abdelwahab, Egypt</b>
17:00 – 17:15	Phenotypic spectrum of Gaucher disease type 3 in Pakistani children and response to ERT <b>Huma Arshad Cheema, Pakistan</b>

## Meeting Agenda

### Tuesday 21 May 2024 – Hall Exhibition

08:30–11:00	<b>Session 4: New Developments and Young Researchers</b> <b>Chairpersons: Johannes Aerts, Predrag Rodic</b>
08:30 – 09:15	Invited Speaker: Developments in Basic Science: capita selecta <b>Johannes Aerts</b>
09:15 – 11:00	<b>Selected Oral Abstracts</b>
09:15 – 09:30	Exploring the transcriptional profile of GBA1 knock-out osteoblasts <b>Eleonora Pavan, Italy</b>
09:30 – 09:45	Molecular cell atlas of the brain in neuronopathic Gaucher disease reveals the brain stem as the major target of GBA1 deficiency involving microglia, astrocytes, and oligodendrocytes <b>Shiny Nair, USA</b>
09:45 – 10:00	Study of the development and implication of neutrophil extracellular traps in Gaucher disease <b>Irene Serrano-Gonzalo, Spain</b>
10:00 – 10:15	Comparative Analysis of Human and Zebrafish Glucocerebrosidase <b>Mats Bulterman, Netherlands</b>
10:15 – 10:30	Correlating LysoGb1 Levels with Clinical Phenotypes in Gaucher Disease: Insights from Newborn Screening and Biomarker Analysis <b>Leah Svarny, USA</b>
10:30 – 10:45	Biochemical and molecular characterization of patients affected by PSAP and LIMP-2 deficiencies: new insights into glucocerebrosidase impairment beyond Gaucher disease <b>Eleonora Pavan, Italy</b>
10:45 – 11:00	Deep learning-based quantification of osteonecrosis using magnetic resonance images in Gaucher disease <b>Patrick Deegan, UK</b>
11:00 – 11:30	<b>Coffee Break</b>
11:30 – 12:15	<b>Working Groups Updates and Panel Discussion</b>
12:15 – 13:30	<b>Session 5: GBA deficiency beyond Gaucher disease: Comorbidities</b> <b>Chairpersons: Mia Horowitz, Carla Hollak</b>

## Meeting Agenda

### Tuesday 21 May 2024 – Hall Exhibition

12:15 – 13:30	Selected Oral Abstracts
12:15 – 12:30	The Modifying Effect of Mutant LRRK2 on GBA1-Associated Parkinson Disease <b>Vera Serebryany, Israel</b>
12:30 – 12:45	Glucocerebrosidase mutations in a Serbian Parkinson's disease <b>Nikola Kresojević, Serbia</b>
12:45 – 13:00	Monitoring hepatic fibrosis in a cohort of patients with Gaucher disease type 1 from Rio Grande Do Sul Brazil <b>Ida Schwartz, Brazil</b>
13:00 – 13:15	Cancer among patients with Gaucher disease from the SZMC Gaucher Unit <b>Majdolen Istaiti, Israel</b>
13:15 – 13:30	Cancer risk in Gaucher disease using real-world data <b>Shoshana Revel-Vilk, Israel</b>
13:30 – 14:30	<b>Lunch Break &amp; Poster Tour</b>
14:30 – 16:30	<b>IWGGD Members Business Meeting</b>
18:00 – 23:00	<b>IWGGD Congress Dinner</b>

## Meeting Agenda

### Wednesday 22 May 2022 – Hall Exhibition

08:00 – 10:00	<b>Session 6: Therapies and clinic: present &amp; future</b> <b>Chairpersons: Timothy Cox, Gregory Grabowski</b>
08:00 – 08:40	Invited Speaker: Treatment application of RNA molecules <b>Andrea Dardis, Italy</b>
08:40 – 09:40	<b>Selected Oral Abstracts</b>
08:40 – 08:55	Safety and efficacy study of Taliglucerase alfa in type 3 Gaucher disease: A 12-month multicenter trial <b>Majdolen Istiti, Israel</b>
08:55 – 09:10	Outcomes of Eliglustat Substrate Reduction Therapy in Special Populations: Pediatric Patients and Adult Patients with Cardiac Co-morbidities <b>Armaan Saith, USA</b>
09:10 – 09:25	Gaucher Disease: A Contemporary Orthopedic Surgeon's Perspective <b>Ehud Lebel, Israel</b>
09:25 – 09:40	Outcomes of ERT/SRT monotherapy and combination therapy in neuronopathic Gaucher mouse model suggest transfer of peripheral ERT to brain <b>Nima Fattahi, USA</b>
09:40 – 10:00	<b>Coffee break</b>
10:00 – 12:00	<b>Session 7: Therapies and clinic: future</b> <b>Chairpersons: Timothy Cox, Gregory Grabowski</b>
10:00 – 10:40	<b>Selected Oral Abstracts</b>
10:00 – 10:20	AAV9-based gene therapy for Gaucher disease- Update from the PROVIDE and PROCEED studies <b>Sarah Neuhaus, USA</b>
10:20 – 10:40	Results from GALILEO-1, a first-in-human clinical trial of FLT201, an AAV-gene therapy, in adults with Gaucher disease type 1 <b>Derralynn Hughes, UK</b>
10:40 – 11:30	Invited speaker: Human Nature and Us <b>Timothy Cox, UK</b>
11.30 – 12:00	<b>Closing remarks</b>

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