

## **Agenda for the In-person “6<sup>th</sup> World Conference on CDG (WCCDG) for CDG families and professionals”.**



**This year the 6<sup>th</sup> World Conference on CDG (WCCDG) for CDG families and professionals.**

**Includes Virtual Pre-conference Sessions during May and early July 2023**

**and the In-person events in Caparica, at NOVA School of Science and Technology, FCT NOVA (Portugal).**



**Organized by the Portuguese Association for CDG and CDG and Allies**

**in collaboration with amazing Worldwide CDG Patients Advocacy Groups, advocates and awesome CDG Professionals!**

**An event endorsed by several prestigious entities and organizations devoted to rare diseases, including Congenital Disorders of Glycosylation, check [HERE](#).**

**The operations and activities of our event are a reality thanks to an active group of collaborators of the people-centric CDG and Allies International Network who kindly give their time in a volunteer manner and motivation to help us!**

**We are also grateful to our donors and supporters. Visit [HERE](#).**

## 6<sup>th</sup> World Conference on CDG (WCCDG) for CDG families and professionals.

This year's **theme** is “**Building a collaborative, community-centric strategy to boost drug development for all CDGs**”! and includes novelties like the “World CDG Advocacy, Policy and Leadership Academy” and the “World Think Metabolic, Think CDG Academy”!





As suggested by the theme for the 2023 conference – we are looking to create an environment where all attendees can interact and share insights, strategies, and techniques for either broadening or enabling access to CDG therapies, thanks to a coordinated collaborative, and community-centric strategy needed to boost drug development for all CDGs!



This is the most complete and resourceful international conference focused entirely on CDG. The content is delivered in a patient-friendly language that non-specialist audiences can understand. The ultimate goal is to help people to engage with clinicians and researchers more effectively and participate actively in their healthcare decisions. **Learn everything about the event [here](#).**



# Agenda:

This year the 6<sup>th</sup> World Conference on CDG (WCCDG) for CDG families and professionals include 3 educational paths:

- **The Online Pre-conference Sessions** “World Think Metabolic, Think CDG Academy” led from May to June 2023.
- **The In-person events**
  - “First World CDG Advocacy, Policy and Leadership Academy” from 20 July afternoon to 21 July morning 2023.
  - “6th World Conference on CDG”, from 21 July afternoon to 23 July 2023 lunch time.

Online Pre-conference Sessions.	In-person in Caparica, at NOVA School of Science and Technology, FCT NOVA (Portugal).	
		
<p><b>“World Think Metabolic, Think CDG Academy”</b> Audience: all participants</p> <p><b>What:</b> series of online videos <b>When:</b> released during May and June 2023 at <a href="https://worldcdg.org/">https://worldcdg.org/</a></p>	<p><b>“1<sup>st</sup> World CDG Advocacy, Policy and Leadership Academy”</b> Audience: leaders of CDG patient groups, their advocates and families interested to get upskilled to advocate for CDG.</p> <p><b>When:</b> Thursday afternoon 20 July- morning 21 July 2023</p>	<p><b>“6<sup>th</sup> World Conference on CDG”</b> Audience: all participants Theme: “Building a collaborative, community-centric strategy to boost drug development for all CDGs”!</p> <p>We offer simultaneous interpretation from</p> <ul style="list-style-type: none"><li>• English to Portuguese</li><li>• and English to Spanish.</li></ul> <p><b>When:</b> Friday afternoon 21 July – Sunday 23 July 2023</p>
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**\*\*\*About the In-Person Events under the 6<sup>th</sup> World Conference on CDG takes place in Caparica, at NOVA School of Science and Technology, FCT NOVA, Portugal\*\*\***

**They include:**

- o **“First World CDG Advocacy, Policy and Leadership Academy”** from 20 July afternoon to 21 July morning.
- o **“6th World Conference on CDG”**, from 21 July afternoon to 23 July 2023 lunch time.

**“World CDG Advocacy, Policy and Leadership Academy”** held in English. This is intended **only for leaders of CDG patient groups, their advocates** and families members interested to get involved in advocating for CDG. It helps you to be successful advocates. It has **limited vacancies**. We recommend that you don't wait too long as the registration for the World CDG Advocacy, Policy and Leadership Academy will be on a first-come, first-serve basis. **Learn everything about the event [here](#).**

**NOTE:** Certification is delivered for those attending the “World Think Metabolic, Think CDG Academy” and the “World CDG Advocacy, Policy and Leadership Academy”.

**“6th World Conference on CDG”**, offers simultaneous interpretation from English to Portuguese and from English to Spanish.

**The format of sessions includes:**

- **Title(s) of short talk(s) and name of the Speaker(s).**
- **Followed by a Round table Meet the experts (Q & A).**
- **Live interviews.**
- **And finally, we close the exchange of learnings with Think Tanks:** participants will gather together in groups to cover questions related to the major topics of the In-Person event. This represents a great opportunity to work collaboratively and meet each other.

## Donors and Supporters

We'd like to thank our donors and supporters, and recognize their unwavering dedication to our cause. It was this dedication that made the 6th World Conference on CDG possible. Thank you for your invaluable support.



**WORLD CDG ORGANIZATION**  
Awareness, Research, Education and Empowerment United  
FAMILIES AND PROFESSIONALS

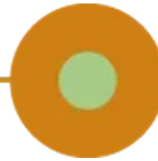


CDG & Allies – PPAIN  
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CDG & Allies - Professionals and Patient Associations International Network



PORTUGUESE ASSOCIATION

Foundation  
Glycosylation



NOVA SCHOOL OF  
SCIENCE & TECHNOLOGY



UCIBIO  
Applied Molecular Biosciences Unit



Institute for Health  
and Bioeconomy



VALE

**The In-Person and live transmitted “6<sup>th</sup> World Conference on CDG”** takes place from 21-23 July 2023 at the Great Auditorium in Caparica, at NOVA School of Science and Technology, FCT NOVA, Portugal, and is directed **to all participants**. **Learn everything about the event [here](#).**

### For those attending online:

Our workshops embrace the power of technology to facilitate an inclusive and engaging experience for participants, including those joining remotely. Through the use of Zoom breakout rooms, online participants will have the opportunity to actively participate in group discussions and collaborative activities, ensuring their voices and perspectives are heard and valued.

During the workshop sessions, participants will be divided into smaller groups using the Zoom breakout room feature. These breakout rooms serve as virtual spaces where participants can interact, exchange ideas, and engage in focused discussions with a smaller group of their peers. To ensure effective communication and promote active participation, a trained moderator will facilitate each breakout room.

Online participants will have the chance to contribute to group discussions, share their insights, and collaborate on various workshop activities, just like their in-person counterparts. The breakout rooms will enable meaningful interactions, fostering a sense of connection and camaraderie among all participants, regardless of their physical location.

**Note:** The think tanks mentioned in the agenda, are subject to slight modifications based on the flow and needs defined during the academy.

**EDUCATIONAL TRACK 3:” 6<sup>th</sup> World Conference on CDG”** takes place in person from 21-23 July 2023 at the Great Auditorium in Caparica in Caparica, at NOVA School of Science and Technology, FCT NOVA, Portugal.

**Audience:** is directed at all participants.

**How:** in person in Caparica, Portugal, at NOVA School of Science and Technology, FCT NOVA

**Hour(s):** are shown in Lisbon, Portugal local time (Consult the [World Time Buddy](#) (WTB)).

**Day 1 of the In-Person “6<sup>th</sup> World Conference on CDG”** takes place **Friday 21 July 2023 (Friday).**

**Where:** The Great Auditorium of NOVA School of Science and Technology | FCT NOVA, Campus of Caparica.

**Address:** Largo da Torre, 2829-516 Caparica, Portugal.

2:00- 3:00 PM

**Registration and gathering together.**

**Where:** The Great Auditorium of NOVA School of Science and Technology | FCT NOVA, Campus of Caparica.

3:00-3:30 PM

**Welcome. Setting the pillars for a 3 day conference!**

**World Conference on Congenital Disorders of Glycosylation (CDG) for Families and Professionals: the listening platform dedicated to collaboratively shape the research and drug development with and for the CDG Community.**

***Voices of CDG Families and Advocates: Inspiring stories and insights***

- **Sarah Aymar**, CDG Canada.
- **Sanja Juric**, CDG Croatia and CDG Germany.
- **Raquel Pinheiro and Juliana Mansur**, CDG Brasil.
- **Sanja Juric**, CDG Croatia and CDG Germany.

**Speakers:** *Vanessa Ferreira and Team CDG & Allies – Professionals and Patient Associations International Network (CDG & Allies-PPAIN), NOVA School of Science and Technology, NOVA University Lisbon, Caparica, Portugal.*

**Overview of research, drug development and post approval of therapies in CDG.**



<p>3:30-4:00 PM</p>	<p><b>Talk 1: Overview of the roadmap for CDG Research, Drug Development, Access, and Care and Management: a resource co-created with and for our CDG community during the "First World CDG Advocacy, Policy and Leadership Academy". (10 minutes)</b>  <b>Speaker: Marc Patterson and Eva Morava</b>, Mayo Clinic, and FCDGC USA together with participants of the "First World CDG Advocacy, Policy, and Leadership Academy".</p> <p><b>Talk 2: Patient and Public Involvement (PPI) within the roadmap for CDG Research, Drug Development, Access, and Care and Management: is it a new fashion? (15 minutes)</b>  <b>Speaker: Vanessa Ferreira and Ana Ferreira</b>, Portuguese Association for CDG (APCDG), World CDG Organization (WCDGO) CDG &amp; Allies - Professionals and Patient Associations International Network (CDG &amp; Allies-PPAIN), NOVA School of Science and Technology, Universidade NOVA de Lisboa, Caparica, Portugal.</p> <p><b>Panel of Q &amp; A (5 minutes):</b> More questions?  <b>Facilitated by:</b> Rita Francisco (Portugal), Pedro Granjo (Portugal) and Carlota Pascoal (Portugal).  <b>Panelist is formed by:</b> Andrew C. Edmondson (USA), Christin Johnsen (Germany), Peter Witters (Belgium).</p>
<p>4:00-4:30 PM</p>	<p><b>Cross-transversal topic(s): Tools and research areas with potential to advance breakthrough therapies and care across all CDGs.</b></p> <p>The activity will commence with a series of informative short talks (8 minutes), providing a concise overview of impactful tools and research areas that hold great promise in advancing breakthrough therapies and improving care for all CDGs. Through these talks, participants will acquire valuable insights into the advantages and importance of collaborations in the development of effective research and the enhancement of patient care.</p> <p><b>Talk 1: Learnings and opportunities from NGLY 1 with impact across all CDGs.</b>  <b>Speaker: Tadashi Suzuki</b>, RIKEN Institute, Japan.</p> <p><b>Talk 2: RNA-guided diagnosis for CDG patients: Updates, challenges, and opportunities.</b>  <b>Speaker: Lan Lin</b>, Children's Hospital of Philadelphia and FCDGC, USA.</p> <p><b>Talk 3: How are researchers translating CDG research into treatments? The GNE Myopathy (GNE-CDG) Case Study.</b>  <b>Speaker: Mariana Barbosa</b>, Glycoimmunology lab, CDG &amp; Allies - Professionals and Patient Associations International Network (CDG &amp; Allies-PPAIN), NOVA School of Science and Technology, Universidade NOVA de Lisboa, Caparica, Portugal.</p>

	<p><b>Panel of Q &amp; A (5 minutes):</b> More questions?  <b>Facilitated by:</b> Rita Francisco (Portugal), Pedro Granjo (Portugal) and Carlota Pascoal (Portugal).  <b>Panelist is formed by:</b> Andrew C. Edmondson (USA), Christin Johnsen (Germany), Peter Witters (Belgium).</p>
4:30-5:00 PM	<p><b>Coffee break and poster session is included.</b>  <b>Where:</b> The Great Auditorium of NOVA School of Science and Technology   FCT NOVA, Campus of Caparica.</p>
<p><b>Clinical trials for CDG: lessons, opportunities and best practices:</b> Patients are not only the ultimate beneficiaries of new treatments and therapies but also critical stakeholders in the clinical trial process. Several speakers will address how collaborative work with patients can improve the quality and relevance of clinical trials and ultimately improve patient outcomes.</p>	
5:00-5:40 PM	<p><b>Live interview about: What are the key ingredients to setting up clinical trials for rare diseases like CDG?</b>  Through interactive interviews and knowledge sharing, we aim to identify strategies and innovative approaches to overcome the challenges and leverage the opportunities in conducting effective and impactful clinical trials for CDG. We wish to bring to your attention learnings from other diseases that are easily transferable to CDG. Join us as we collaboratively explore the path to advancing CDG research through well-designed clinical trials.</p> <p><b>Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>● <b>Femke Van Der Maat-de-Deugd</b>, CDG Netherlands. (Part 1)</li> </ul> <p><b>Panel composed of:</b></p> <ul style="list-style-type: none"> <li>● <b>Tristen Moors</b>, Vice President, Clinical Operations at Glycomine, Inc., USA.</li> <li>● <b>Raquel Marques</b>, President Sanfilippo Association Portugal.</li> <li>● <b>Marc Patterson</b>, Mayo Clinic, and FCDGC USA.</li> <li>● <b>Christina Lam</b>, Seattle Children's Hospital, USA.</li> <li>● <b>Christin Johnsen</b>, University Medical Center Göttingen, Clinic for Pediatrics and Adolescent Medicine, Germany.</li> <li>● <b>Peter Witters</b>, Universitair Ziekenhuis Leuven, UZ Leuven, Department of Paediatrics, Belgium.</li> </ul>

	<ul style="list-style-type: none"> <li>● <b>Andrew C. Edmondson</b>, FCDGC, Metabolic Disease Program and the Division of Human Genetics at Children’s Hospital of Philadelphia, USA.</li> <li>● <b>Karen Morici</b>, CDG Care, USA.</li> <li>● <b>Eva Morava</b>, Mayo Clinic, and FCDGC USA.</li> </ul> <p><b>Panel of Q &amp; A (5 minutes):</b> More questions?  <b>Facilitated by:</b> <i>Rita Francisco (Portugal), Pedro Granjo (Portugal) and Carlota Pascoal (Portugal).</i></p>
5:40-6:30 PM	<p><b>Empowering change in the landscape of clinical trials for CDG.</b>  The following conference session brings together a diverse range of short talks (8 minutes) that collectively aim to empower change in the landscape of clinical trials for CDG. The session highlights the importance of reliable information, successful trial strategies, and promoting diversity, inclusion, and equity in CDG clinical trials. The talks featured in this session include:</p> <p><b>Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>● <b>Femke Van Der Maat-de-Deugd</b>, CDG Netherlands. (Part 2)</li> </ul> <p><b>Talk 1: Tips and resources for successful clinical trials in CDG across countries.</b>  <b>Speaker:</b> <i>Peter McWilliams</i>, Chief Business Officer (CBO) at Glycomine, Inc., USA.</p> <p><b>Talk 2: Patient-Driven innovation: Revolutionizing clinical trials with technological solutions, digital biomarkers, and collaborative data collection.</b>  <b>Speaker:</b> <i>Elisa Ferrer</i>, Patient Advocacy Manager at Aparito, UK.</p> <p><b>Talk 3: Empowering change: Building a future of Diversity, Inclusion, and Equity in Clinical Trials.</b>  <b>Speaker:</b> <b>Andrew C. Edmondson</b>, Frontiers in Congenital Disorders of Glycosylation Consortium (FCDGC), Metabolic Disease Program and the Division of Human Genetics at Children's Hospital of Philadelphia, USA.</p> <p><b>Talk 4: Breaking barriers: advancing Diversity, Inclusion, and Equity in Clinical Trials.</b>  <b>Speaker:</b> <b>Begoña Nafría Escalera</b>, Sant Joan de Déu, Research Institute, Barcelona, Spain.</p> <p>By combining these insightful talks, the conference session aims to inspire positive change in CDG clinical trials, fostering informed participation, successful trial strategies, and a more inclusive and equitable</p>

	<p>research landscape.</p> <p><b>Panel of Q &amp; A (5 minutes):</b> More questions?</p> <p><b>Facilitated by:</b> <i>Rita Francisco (Portugal), Pedro Granjo (Portugal) and Carlota Pascoal (Portugal).</i></p> <p><b>Panelist is formed by:</b> <i>Eva Morava (USA), Marc Patterson (USA).</i></p>
6:30-7:00 PM	<p><b>Think Tank 1: Collaborative Strategies: Shaping the roadmap for Clinical Trial solutions in CDG.</b></p> <p><b>Facilitated by:</b> <i>Ksenia Lebedeva (Portugal), Alison Slade (Switzerland), Cláudia de Freitas (Portugal) and Thomas Smith (UK), Rita Francisco (Portugal).</i></p> <p><b>Introduction by Vanessa Ferreira,</b> Portuguese Association for CDG (APCDG), World CDG Organization (WCDGO) CDG &amp; Allies - Professionals and Patient Associations International Network (CDG &amp; Allies-PPAIN), NOVA School of Science and Technology, Universidade NOVA de Lisboa, Caparica, Portugal.</p> <p><b>Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>● <b>Fiona Waddell,</b> CDG Netherlands. (Part 1)</li> </ul> <p>In this Think Tank session, we will delve into the realm of clinical trials for CDG and explore solutions to overcome current existing challenges. Major conclusions will be shared per group. <b>This session is subject to slight modifications based on the flow and needs identified during the academy.</b></p> <p><b>In parallel, you can pick up Refreshments.</b></p> <p><b>Where:</b> The Great Auditorium of NOVA School of Science and Technology   FCT NOVA, Campus of Caparica.</p>
7:00-7:05 PM	<p><b>Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>● <b>Fiona Waddell,</b> CDG Netherlands. (Part 2)</li> </ul>
7:05-7:25 PM	<p><b>Talk 1: Rarebarometer: Making the voice of People Living with Rare Diseases (PLwRDs) heard!</b></p>



	<p><b>Sub-title: EURORDIS-Rare Diseases Europe Global Survey Initiative for Evidence-Based Advocacy.</b>  <b>Speaker:</b> <i>Rita Francisco</i>, Survey Junior Manager EURORDIS, France.</p> <p><b>Panel of Q &amp; A (5 minutes):</b> More questions?  <b>Facilitated by:</b> <i>Rita Francisco (Portugal), Pedro Granjo (Portugal) and Thomas Smith (UK).</i>  <b>Panelist is formed by:</b> <i>Peter McWilliams (USA) and Christina Lam (USA).</i></p>
7:25-8:15 PM	<p><b>Series of Pitches at World Conference on CDG! Stay tuned!</b></p> <p>Several short presentations will share about community-centric ideas, projects, services and solutions that can help all CDG stakeholders!</p> <p><b>Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>• <b>Finding a treatment for PIGS-CDG.</b> <i>Jing Xu</i>, family research partner for PIGS-CDG, USA.</li> </ul> <p><b>Pitch 1: SumMed, the App that makes medical information accessible and understandable for all.</b>  <b>Speaker:</b> <i>Larisa Aragón Castro</i>, CEO and co-founder, SumMed, Switzerland and <b>Stefani Derzi</b>, Chief Scientific Officer, SumMed, Switzerland.</p> <p><b>Pitch 2: Buddy Service App, the solution to make travel and daily life accessible for all.</b>  <b>Speaker:</b> <i>Fabian Kramer</i>, CEO, Buddy Service Spain.</p> <p><b>Pitch 3: A rainbow is born of science with a touch of magic for PIGW-CDG: The path of a family - research partner.</b>  <b>Speaker:</b> <i>Kelsey Lents</i>, family research partner for PIGW-CDG, USA.</p> <p><b>Pitch 4: Empowering tomorrow's scientists: Building the Young Scientists for Congenital Disorders of Glycosylation (CDG) Network.</b>  <b>Speaker:</b> <i>Pedro Granjo</i>, APCDG, CDG &amp; Allies - Professionals and Patient Associations International Network (CDG &amp; Allies-PPAIN), NOVA School of Science and Technology, NOVA University Lisbon, Caparica, Portugal.</p> <p><b>Round table (10 minutes):</b> The goal of this round table is to explore and discuss how the presented ideas and projects can contribute to the advancement of CDG research, care, and accessibility. By bringing together experts from various fields, including medical information accessibility, travel and daily life accessibility, family research partnership, and young scientist networks, the round table aims to generate</p>

	<p>insights and perspectives on the potential impact of these initiatives on the CDG community. The discussion will focus on identifying opportunities for collaboration, sharing best practices, and discussing strategies to integrate these innovative approaches into the CDG ecosystem.</p> <p><b>Facilitated by:</b> <i>Rita Francisco (Portugal), Pedro Granjo (Portugal) and Thomas Smith (UK).</i></p> <p><b>Panelist is formed by:</b> <i>Eva Morava (USA), Marc Patterson (USA), Peter McWilliams (USA) and Christina Lam (USA).</i></p>
8:15 PM	<p><b>Finger Food Dinner represents an opportunity to enhance social interactions and network!</b></p> <p><b>See you there!</b></p> <p><b>Where:</b> The Great Auditorium of NOVA School of Science and Technology   FCT NOVA, Campus of Caparica.</p>
<p><b>End Day 1 In Person “6<sup>th</sup> World Conference on CDG!”</b></p>	

**Day 2 of the In-Person “6<sup>th</sup> World Conference on CDG” takes place 22 July 2023 (Saturday).**

**Where:** The Great Auditorium of NOVA School of Science and Technology | FCT NOVA, Campus of Caparica.

**Address:** Largo da Torre, 2829-516 Caparica, Portugal.

**Hour(s):** are shown in Lisbon, Portugal local time (Consult the [World Time Buddy](#) (WTB)).

**13.00-14.00 PM**

**Registration and gather together at The Great Auditorium of NOVA School of Science and Technology | FCT NOVA, Campus of Caparica. Those interested can visit the Posters.**

**Therapies for CDG (Part 1):** Currently, there are several nutritional replacement therapies for CDG and researchers continue exploring different therapeutic approaches to address CDG. Several speakers will share updates and learnings related to nutritional replacement therapies for CDG.

**2:00-2:50 PM**

**Talk 1: Overview of nutritional replacement therapies for CDG.**

**Speaker:** *Peter Witters*, Centre for Metabolic Diseases, UZ Leuven, Belgium.

**Talk 2: Update about clinical trial for dietary supplementation of Galactose in SLC35A2-CDG and PGM1-CDG and lessons transferable across all CDGs.**

**Speaker:** *Andrew C. Edmondson*, FCDGC and Children’s Hospital of Philadelphia (CHOP), USA.

**Talk 3: Efficacy of oral manganese and D-galactose therapy for a novel TMEM165-CDG patient.**

**Speaker:** *Zoé Durin*, CNRS, UMR 8576-UGSF-Structural and Functional Glycobiology Unit, University of Lille, Lille, France.

**Round table Meet the experts (Q & A) (5 minutes):** More questions? Take advantage of the round table!

**Facilitated by:** *Claudia de Freitas (Portugal), Rita Francisco (Portugal), Paula Videira (Portugal) and Dulce Quelhas (Portugal).*

**Panelist for the Round table Meet the experts (Q & A) is formed by:** *Tadashi Suzuki (Japan), Lan Lin (USA), Tamas Kozicz (USA).*

**CDG care, management and daily life (Part 1):** People living with CDG often face significant challenges in accessing timely diagnosis, appropriate treatment, and support. The care and management of CDG require a collaborative approach among healthcare providers, patient advocacy groups, regulatory agencies, and patients and their families. By working together, we can improve the lives of people living with CDG and ensure that they receive the care and support they need. We have

several sessions aimed at sharing updates about best practices, resources, and activities that can improve current care and management for CDG across countries and upskill all community.

2:50-3:30 PM

**Live interview: Empowering families: Preparing for medical appointments and amplifying their voice.**

During this inspiring interview, our panel of experts will delve into the crucial topic of empowering patients to be prepared for medical appointments and amplify their voice in healthcare. We will explore essential strategies and practical tips for patients to effectively navigate medical appointments, understand their rights, and create a personalized notebook for medical information. The panel will share insights on how patients can become active participants in their healthcare journey, advocate for their needs, and ensure their voices are heard. Join us as we empower patients to take charge of their health and advocate for the best possible care.

**Voices of CDG Families and Advocates: Inspiring stories and insights**

- **Fiona Waddell**, CDG Netherlands.

**Panel composed of:**

- **Christina Lam**, Seattle Children's Hospital, USA.
- **Mel McSweeney**, Great Ormond Street Hospital (GOSH), London, UK.
- **Karen Morici**, CDG Care, USA.
- **Stephanie Grünewald**, Great Ormond Street Hospital (GOSH), London, UK.
- **Eva Morava**, Mayo Clinic, and FCDGC USA.

**Round table Meet the experts (Q & A) (5 minutes):** More questions? Take advantage of the round table!

**Facilitated by:** Rita Francisco (Portugal), Paula Videira (Portugal) and Dulce Quelhas (Portugal).

**Panelist for the Round table Meet the experts (Q & A) is formed by:** Saadet Andrews (Canada), Peter Witters (Belgium) and Andrew C. Edmondson (USA).

3:30-4:20 PM

**Think Tank 2: Strengthening CDG knowledge, education, and fostering collaboration for enhanced care.**

**Facilitated by:** Ksenia Lebedeva (Portugal), Alison Slade (Switzerland), Cláudia de Freitas (Portugal) and Thomas Smith (UK), Rita Francisco (Portugal).



	<p><b>Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>• <b>Macarena Rodríguez</b>, CDG Chile.</li> </ul> <p>By bringing together the participants of our conference from different backgrounds, this think tank aims to generate insights, exchange experiences, and promote collaboration to advance CDG understanding, care, and communication. Major conclusions will be shared per group.</p> <p><b>This session is subject to slight modifications based on the flow and needs identified during the academy.</b></p>
4:20-4:50 PM	<p><b>Coffee break and poster session.</b></p> <p><b>Where:</b> at The Great Auditorium of NOVA School of Science and Technology   FCT NOVA, Campus of Caparica.</p>
4:50-5:10 PM	<p><b>Live interview: Emotional journeys: Navigating diagnosis and sibling experiences in the CDG community.</b></p> <p>In this live interview, we will delve into the emotional journeys of individuals and families within the CDG community as they navigate the challenges of diagnosis and the unique experiences of CDG siblings. Our guests will share their personal stories, insights, and coping strategies, offering a candid and heartfelt exploration of the emotional aspects involved in living with CDG. Join us as we delve into these important topics and gain a deeper understanding of the profound impact that CDG has on individuals and their families, while also uncovering sources of strength and resilience within the community.</p> <p><b>Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>• <b>Mariana Esquinca</b>, CDG México.</li> </ul> <p><b>Panel composed of:</b></p> <ul style="list-style-type: none"> <li>• <b>Marta Kadziolka</b>, SSR4-CDG Patient Ambassador, WCDGO, psychologist, Canada.</li> <li>• <b>Vanessa Ferreira</b>, APCDG, WCDGO and researcher at CDG &amp; Allies, Portugal.</li> </ul> <p><b>Panel of Q &amp; A (5 minutes):</b> More questions?</p>

	<p><b>Facilitated by:</b> <i>Rita Francisco (Portugal), Paula Videira (Portugal) and Dulce Quelhas (Portugal).</i></p>
<p><b>Mental well-being:</b> Mental health is an important aspect of overall health and well-being, and it is particularly important in the context of rare diseases (RDs) like CDG. The UN Resolution: “Addressing the challenges of persons living with a RD and their families”, urges the implementation of “effective programs to promote mental health and psychosocial support” for persons living with a RD. The various sessions within our conference aim at highlighting the needs from CDG related to mental health support, as well as to share existing resources and interventions that may support all our families.</p>	
<p>5:10-6:00 PM</p>	<p><b>Workshop: Nurturing Well-being: Empowering tips for all.</b>  <b>Facilitator:</b> <i>Vanessa Ferreira</i>, APCDG, CDG &amp; Allies - Professionals and Patient Associations International Network (CDG &amp; Allies-PPAIN), NOVA School of Science and Technology, Universidade NOVA de Lisboa, Caparica, Portugal.</p> <p><b>Set the scene with Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>• <i>Claudia Barbaro</i>, CDG Argentina.</li> <li>• <i>Lourdes Valenzuela</i>, CDG México.</li> <li>• <i>Femke Van Der Maat-de-Deugd</i>, CDG Netherlands.</li> <li>• <i>Sarah Aymar</i>, CDG Canada.</li> </ul> <p><b>Round table Meet the experts (Q &amp; A) (5 minutes):</b> More questions? Take advantage of the round table!  <b>Facilitated by:</b> <i>Rita Francisco (Portugal), Paula Videira (Portugal) and Dulce Quelhas (Portugal).</i></p>
<p>6:00-7:00 PM</p>	<p><b>Think Tank 3: Prioritizing behavior issues and mental health in CDG: addressing needs for CDG families.</b>  <b>Facilitated by:</b> <i>Ksenia Lebedeva (Portugal), Alison Slade (Switzerland), Cláudia de Freitas (Portugal) and Thomas Smith (UK), Rita Francisco (Portugal).</i></p> <p>Think Tank 3 focuses on prioritizing behavior issues and mental health in families affected by CDG. This session aims to raise awareness about the specific challenges faced by CDG families in relation to behavior issues and mental well-being. Experts and participants will come together to discuss and develop strategies to address these needs effectively. By emphasizing the importance of mental health support and interventions, this think tank aims to improve the overall quality of life for CDG families and enhance their access to appropriate care and resources. Major conclusions will be shared.</p>

	<p><b>Refreshments.</b>  <b>Where:</b> at The Great Auditorium of NOVA School of Science and Technology   FCT NOVA, Campus of Caparica.</p>
7:00-8:10 PM	<p><b>Series of Pitches at the World Conference on CDG! Stay tuned!</b>  <b>Several short presentations will share about community-centric ideas, projects, services and solutions that can help all CDG stakeholders!</b></p> <p>The main goal of the pitches is to showcase innovative ideas, initiatives, and personal experiences that contribute to the advancement of CDG research, inclusivity, and awareness. Each pitch aims to inspire the audience, whether through international collaboration in glycans research, sustainable and inclusive fashion, or sharing a remarkable journey of resilience and hope. The overall objective is to engage and inform attendees, highlighting the importance of collaboration, accessibility, and personal narratives in the CDG community.</p> <p><b>Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>• <b>Mariana Esquinca</b>, <i>CDG México</i>.</li> </ul> <p><b>Pitch 1: How to form an international team to advance understanding of the role of glycans in biology and impact CDG research: The GlycoTwinning.</b>  <b>Speaker: Paula Videira</b>, APCDG, CDG &amp; Allies - Professionals and Patient Associations International Network (CDG &amp; Allies-PPAIN), NOVA School of Science and Technology, NOVA University Lisbon, Caparica, Portugal.</p> <p><b>Pitch 2: Unveiling Valter's Journey: An inspirational life story.</b>  <b>Speaker: Anja Højte</b>, from VALE designs, Denmark.</p> <p><b>Pitch 3:VALE Designs, a brand of sustainable and fashionable adaptive clothing aiming to ensure equal opportunities for all.</b>  <b>Speaker: Hugo Perdigão and Tina Sørensen</b>, from VALE designs, Denmark.</p> <p><b>Pitch 4: I was the first CDG patient in the world who had a liver transplant and I wrote the book Survivor about it!</b></p>

	<p><b>Speaker:</b> <i>Fiona Waddell</i>, from CDG Netherlands.</p> <p><b>Round table (10 minutes).</b></p> <p>The goal of this round table is to explore and discuss how the presented ideas and projects can contribute to the advancement of CDG. These pitches will offer diverse perspectives and showcase innovative ideas, highlighting the determination and resilience of individuals and organizations in the CDG community.</p> <p><b>Facilitated by:</b> <i>Angelina Palma (Portugal), Rita Francisco (Portugal), and Dulce Quelhas (Portugal).</i></p> <p><b>Panelist for the Round table Q &amp; A is formed by:</b> <i>Tadashi Suzuki (Japan), Lan Lin (USA), Tamas Kozicz (USA), Begoña Nafría Escalera (Spain), Saadet Andrews (Canada), , Peter Witters (Belgium) and Andrew C. Edmondson (USA).</i></p>
8:10 - 10:00 PM	<p><b>Antunia (Portuguese Uni Band) &amp; Finger Food Dinner</b></p> <p><b>Where: Where:</b> at The Great Auditorium of NOVA School of Science and Technology   FCT NOVA, Campus of Caparica.</p>
<p><b>End Day 2 In Person “6<sup>th</sup> World Conference on CDG!”</b></p>	



**Day 3 of the In-Person “6<sup>th</sup> World Conference on CDG” takes place 23 July 2023 (Sunday).**

**Where:** The Great Auditorium of NOVA School of Science and Technology | FCT NOVA, Campus of Caparica.

**Address:** Largo da Torre, 2829-516 Caparica, Portugal.

**09:00-09.30 AM** Registration and gathering together at The Great Auditorium of NOVA School of Science and Technology | FCT NOVA, Campus of Caparica. Those interested can visit the Posters.

**Therapies for CDG (Part 2):** Currently, there are several non-nutritional replacement therapies for CDG and researchers continue exploring different therapeutic approaches to address CDG. Several speakers will share updates and learnings related to non-nutritional replacement therapies for CDG.

**09.00-09:30 AM** **Talk 1: Overview of non-nutritional replacement therapies (Part 1).**

**Speaker:** *Eva Morava*, FCDGC and Mayo Clinic (USA).

**Round of Q&A (5 minutes).**

**Facilitated by:** *Rita Francisco (Portugal), Pedro Granjo (Portugal) and Carlota Pascoal (Portugal).*

**Panelist formed by:** *Stephanie Grünewald (UK) Christina Lam (USA).*

**CDG care, management, and daily life (Part 2).**

**09:30-10:00 AM**

**Voices of CDG Families and Advocates: Inspiring stories and insights**

- **Maranda Martin**, CDG USA.

**Talk 1:** The role of the advanced clinical practitioner in the care and management of Congenital Disorders of Glycosylation (CDG).

**Speaker:** *Mel McSweeney*, Great Ormond Street Hospital (GOSH), London, UK.

**Talk 2:** The recipe to build excellence from scratch: Overcoming challenges in establishing a center of excellence for CDG.

**Speaker:** *Christin Johnsen, MD*, University Medical Center Göttingen, Clinic for Pediatrics and Adolescent Medicine (Germany).

	<p><b>Round of Q&amp;A (5 minutes).</b></p> <p><b>Facilitated by:</b> <i>Rita Francisco (Portugal), Pedro Granjo (Portugal) and Carlota Pascoal (Portugal).</i></p> <p><b>Panelist formed by:</b> <i>Andrew C. Edmondson (USA), Eva Morava-Kozicz (USA) and Ruqaiyah Altassan (Saudi Arabia).</i></p>
10:00-10:30 AM	<p><b>Live interview: Transcending borders: Best practices from CDG Centers of Excellence for global impact.</b></p> <p>In these live interviews, the experts will explore the best practices implemented by centers of excellence for CDG and highlight their potential for transferability across countries. Attendees will gain valuable insights into successful strategies and approaches used by these centers to enhance diagnosis, treatment, and care for individuals with CDG. By sharing experiences and lessons learned, this live interview aims to inspire collaboration and the adoption of effective practices worldwide, ultimately improving outcomes for CDG patients globally.</p> <p><b>Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>● <b>Dorota Cimiega</b>, <i>CDG Poland.</i></li> </ul> <p><b>Panel composed of:</b></p> <ul style="list-style-type: none"> <li>● <b>Andrew C. Edmondson</b>, Children’s Hospital of Philadelphia (CHOP) (USA).</li> <li>● <b>Erik Eklund</b>, Pediatrics at Lund University (Sweden).</li> <li>● <b>Christina Lam</b>, Seattle Children's Hospital (USA).</li> <li>● <b>Mercedes Serrano Gimaré</b>, Hospital Sant Joan de Deu (Spain).</li> <li>● <b>Eva Morava</b>, Mayo Clinic (USA).</li> <li>● <b>Marc C. Patterson</b>, Mayo Clinic (USA).</li> <li>● <b>Peter Witters</b>, UZ Leuven (Belgium).</li> <li>● <b>Tristen Moors</b>, Vice President, Clinical Operations at Glycomine, Inc., USA.</li> <li>● <b>Stephanie Grünewald</b>, Great Ormond Street Hospital for Children (GOSH), London,UK.</li> </ul> <p><b>Round of Q&amp;A (5 minutes).</b></p> <p><b>Facilitated by:</b> <i>Rita Francisco (Portugal), Pedro Granjo (Portugal) and Carlota Pascoal (Portugal).</i></p>

10:30-11:00 AM	<p><b>Coffee break 1 and poster session.</b>  <b>Where:</b> at The Great Auditorium of NOVA School of Science and Technology   FCT NOVA, Campus of Caparica.</p>
11:00-11:30 AM	<p><b>Live interview: Funding the future: Accelerating Research and Drug Development for CDG through successful project examples and funding strategies.</b></p> <p>This session will provide valuable insights into securing funding for CDG research and drug development. The talk will highlight various tips and examples of successfully funded projects that have had a significant impact across all CDGs. Attendees will gain a deeper understanding of the funding landscape, learn effective strategies to attract funding, and discover real-life examples of projects that have made a difference in the field of CDG. This session aims to empower the audience with the knowledge and tools they need to secure funding and advance CDG research and drug development.</p> <p><b>Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>● <b>Maranda Martin</b>, CDG USA.</li> </ul> <p><b>Panel composed of:</b></p> <ul style="list-style-type: none"> <li>● <b>Gert Matthijs</b>, Laboratory for Molecular Diagnostics at the Center for Human Genetics in Leuven, Belgium.</li> <li>● <b>Tadashi Suzuki</b>, RIKEN Institute, Japan.</li> <li>● <b>Lan Lin</b>, Children's Hospital of Philadelphia and FCDGC, USA.</li> <li>● <b>Tamas Kozicz</b>, Mayo Clinic (USA).</li> <li>● <b>Saadet Andrews</b>, Associate professor and biochemical geneticist at the University of Alberta, Canada.</li> <li>● <b>Peter McWilliams</b>, Chief Business Officer (CBO) at Glycomine, Inc., USA.</li> <li>● <b>Ethan Perlstein</b>, Perlara (USA).</li> <li>● <b>Holly Charmichael</b>, Patient Advocate, Chief Operating Officer of GT Independence, USA.</li> <li>● <b>Karen Morici</b>, CDG CARE, USA.</li> <li>● <b>Andrew C. Edmondson</b>, Children's Hospital of Philadelphia (CHOP), USA.</li> </ul> <p><b>Round of Q&amp;A (5 minutes).</b>  <b>Facilitated by:</b> <i>Rita Francisco (Portugal), Pedro Granjo (Portugal) and Carlota Pascoal (Portugal).</i></p>

<b>Access to treatments across countries.</b>	
11:30-12:00	<p><b>Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>• <i>Raquel Pinheiro and Juliana Mansur, CDG Brasil.</i></li> </ul> <p><b>Talk 1: The Power of Social Innovation for Rare Diseases.</b>  <b>Speaker: Tineke Kleinhout-Vliek,</b> Postdoctoral researcher Social Pharmaceutical Innovation   Copernicus Institute of Sustainable Development, Utrecht University, The Netherlands.</p> <p><b>Round of Q&amp;A (5 minutes).</b>  <b>Facilitated by:</b> <i>Rita Francisco (Portugal), Pedro Granjo (Portugal) and Carlota Pascoal (Portugal).</i></p>
12:00-12:10 AM	<p><b>Refreshments (10 minutes).</b>  <b>Where:</b> at The Great Auditorium of NOVA School of Science and Technology   FCT NOVA, Campus of Caparica.</p>
<p><b>Therapies for CDG (Part 3):</b> Currently, there are several non-nutritional replacement therapies for CDG and researchers continue exploring different therapeutic approaches to address CDG. Several speakers will share updates and learnings related to non-nutritional replacement therapies for CDG.</p>	
12:00-13:00 PM	<p><b>Voices of CDG Families and Advocates: Inspiring stories and insights</b></p> <ul style="list-style-type: none"> <li>• <i>Dorota Cimiega, CDG Poland.</i></li> </ul> <p><b>Overview of non-nutritional replacement therapies (Part 2).</b>  <b>Talk 1: GLM-101 in PMM2-CDG, results from phase 1 and phase 2 trials.</b>  <b>Speaker: Tristen Moors,</b> Vice President, Clinical Operations at Glycomine, Inc., USA.  <b>Talk 2: Drug repurposing in CDG: Update, challenges and opportunities.</b>  <b>Speaker: Ethan Perlstein,</b> Perlara (USA).</p> <p><b>Round of Q&amp;A (5 minutes).</b></p>

	<p><b>Talk 3: Acetazolamide CDG: Update and lessons for all CDGs.</b>  <b>Speaker: Christina Lam</b>, Seattle Children's Hospital (USA).</p> <p><b>Talk 4: Epalrestat repurposing in PMM2-CDG: Update and lessons for all CDGs.</b>  <b>Speaker: Eva Morava</b>, FCDGC and Mayo Clinic (USA).</p> <p><b>Round of Q&amp;A (5 minutes).</b></p>
13:00-13:10 PM	<p><b>Let's revisit The roadmap for CDG Research, Drug Development, Access, and Care and Management: a resource co-created with and for our CDG community.</b>  <b>Speaker: Marc Patterson and Eva Morava</b>, Mayo Clinic, and FCDGC USA together with participants of the "First World CDG Advocacy, Policy, and Leadership Academy".</p>
13:10-13:20 PM	<p><b>A Global Call to Action: Propelling CDG forward at the 7th World Conference and closing remarks.</b></p>
13:20-13:30 PM	<p><b>Special event dedicated to the "First World CDG Advocacy, Policy, and Leadership Academy" diploma ceremony.</b></p> <p>During this ceremony, participants in the academy will be honored and awarded their well-deserved diplomas. The academy, designed to empower individuals in CDG advocacy, policy, and leadership roles, will equip participants with essential knowledge, skills, and expertise. The diploma ceremony will serve as a recognition of their accomplishments and a celebration of their dedication to driving positive change in the field of CDG.</p>
13:30-15:30 PM	<p><b>Finger Food Lunch</b>  <b>Where: The Great Auditorium of NOVA School of Science and Technology   FCT NOVA, Campus of Caparica.</b></p>
15:30-17:30 PM	<p><b>Office Expert Hours:</b>  The Office Expert Hours is a dedicated time slot during the conference where families have the opportunity to speak with one of our CDG medical doctors. This aims to provide valuable insights and information to assist families in the daily care and management of their child with CDG. Families can seek guidance, ask questions, and receive expert advice directly from our medical professionals. The Office</p>



Expert Hours serve as a valuable resource, offering families a chance to discuss their specific concerns and receive personalized support in navigating the challenges associated with CDG.  
You only need to have signed up in advance and then show up!

**Where: The Great Auditorium of NOVA School of Science and Technology | FCT NOVA, Campus of Caparica.**

**End of the In Person “6<sup>th</sup> World Conference on CDG!”**