CDG HEROES!

CDG CARE is so excited about our newest series on Facebook, CDG HEROES.

By featuring one of our heroes every month with a picture and short bio, we hope to inspire and enlighten our community with each smiling face that is shared.

Be sure to follow us on Facebook so you never miss out on meeting some of our bravest.

If you are interested in sharing your CDG hero, please send an email to KMorici11@gmail.com.

CDG CONNECT - ENROLL TODAY!

Your participation will help ADVANCE TREATMENTS FOR CDG!

Why join the CDG Connect Patient Insight Network? Because your participation is important to collect critical information to understand the history and progression of CDG, to make it easier for researchers to study CDG, for patients and families to learn about CDG treatment options, and for advocates to speak on behalf of the CDG community.

The CDG Connect is a global patient registry that has been created to advance the understanding of Congenital Disorders of Glycosylation. Enroll in the registry to share your experiences, access data and insights and learn more about new CDG clinical trials. Your participation may help us discover findings to improve patient lives. Together, we can build the CDG Connect community!

You can read more about CDG Connect and what we've learned from participants so far on the following pages.
CDG CONNECT INSIGHTS

Who are we?

Patients: 93 Ages: <1 to >60

- 52% Male
- 44% Female

Impact on our lives

Frequent doctor visits

- 35% 2 - 6 times a year
- 20% 7 - 11 times a year

Major episodes of care

- 39% been admitted to hospital
- 39% been to the ER
- 23% had surgery

A combination of treatments

Prescription medication 29%

- Over-the-counter supplements, vitamins, herbs, remedies 23%
- None 17%

Getting diagnosed

Initial symptoms

- ☑ Developmental delays
- ☑ Gastrointestinal problems
- ☑ Growth delay

Average age at diagnosis

- 3 Year(s) old

- <1 Youngest
- 14

Age Range
- Oldest

Extensive testing

- Blood tests
- Genetic Testing
- Biopsy
- MRI
- Physical examination

Different types of tests

Diagnosing specialty

- Genetics
- Neurology
- Nephrology (kidney)

Patients everywhere are eager to help find better treatments

91% want to be contacted about trials
MAY 16TH - CONGENITAL DISORDERS OF GLYCOSYLATION (CDG) AWARENESS DAY!

It's that time of year again – time to paint the globe GREEN to raise awareness for CDG!

This year it’s easy for you to support CDG CARE’s global efforts to make this 4th Annual Campaign for Congenital Disorders of Glycosylation (CDG) Awareness Day our biggest success to date! Join our efforts to raise $75,000 to advance CDG awareness, education, research, and resources for families that will help to improve the lives of children and young adults diagnosed with CDG!

This year’s Campaign is running February 28th through May 16th. If you are organizing or running in a 5K run/walk, asking families to donate to your favorite cause for your birthday, or doing a tribute in honor of a loved one… we have got you covered! Crowdstreet is an easy use charity platform. Just visit our Donations Page, create your Fundraiser or Event, and join our efforts to raise CDG Awareness in your Community and Nationwide! We are also offering CDG Awareness Day wristbands and magnets while supplies last! These items are perfect to give to friends, share with schools, distribute amongst your community and show your support for CDG Awareness Day!

You can participate in our Campaign efforts in any of the following ways –
1 – Follow us on Facebook
2 – Like and Share our Posts
3 – Join the 2019 “Frame Our Heroes” Campaign and use our official CDG Frame for your favorite Facebook Profile image
3 – Plan an event to celebrate World CDG Awareness Day and let us know!
4 – Hold a fundraiser to help us reach our 2019 CDG Awareness Campaign Goal!
5 – Participate by going GREEN! Wear green, decorate your home green, paint your community green all in support of CDG!

Our 2019 CDG Awareness Proclamation Campaign is also in full swing and we already have 10 State Governors who have officially proclaimed May 16th be observed as CDG Awareness Day throughout their State! THANK YOU – Colorado, Delaware, Florida, Indiana, Kentucky, Michigan, New Hampshire, Pennsylvania, Texas and West Virginia! Follow our progress! Join our Campaign! As we Paint the US and the entire World GREEN showing support for May 16th – CDG Awareness Day!
MARK YOUR CALENDARS!

We are excited to invite you to the upcoming CDG Scientific & Family Conference, February 27 – March 1, 2020. This event is being organized by CDG CARE in partnership with the Sanford Burnham Prebys (SBP) Medical Discovery Institute. The Conference will be held at The Dana Hotel on Mission Bay in the beautiful city of San Diego, California.

The Conference program will be strategically designed to provide education and resources for CDG families by bridging scientific presentations with family-centered sessions. In addition to scientific sessions involving current models and therapies, an overview of the education behind this class of disorders will be presented, as well as efforts to make resources readily available for patients and families, and provide families with cutting edge interventional and therapeutic approaches to maximize the overall quality of life for their family member diagnosed with CDG.

This unique collaborative event will engage the scientific and medical community with CDG families in a productive forum and make a significant difference to our attendees by improving the overall awareness, diagnosis and prognosis for families living with CDG.

The conference agenda will encompass 1.5 days of current and groundbreaking scientific sessions for medical community professionals, and 1.5 days of educational sessions presented by a diverse panel of clinical, investigational, therapeutic and community experts from across the globe. This conference will also be bringing back and expanding upon the highly anticipated “Doctor-Is-In” sessions which are available for families to register for to speak to research and medical specialists about individual CDG questions and issues.

The conference program is designed to offer multiple opportunities for clinical, research and therapeutic rare disease experts to meet and interact with CDG children and families at breaks and luncheons. Activities will promote professional and family networking, increase the framework for information sharing and support, and enable families to develop meaningful and lasting relationships with fellow rare disease families.

While the planning for this highly anticipated event is still evolving, it is an opportunity that should not be missed! Registration will be available early Fall 2019 and we look forward to seeing everyone in sunny San Diego very soon!
GLY-000 Observational Study Update
We are currently recruiting PMM2-CDG patients for an observational study (GLY-000).

Although patients will not be receiving investigational drug, the data we collect is critical to understanding each patient’s unique disease profile and progression. We seek to truly understand the patient population, and to identify tests and biomarkers that are ideal for upcoming clinical trials with our investigational drug.

Patients will participate in the GLY-000 study for at least 6 months before being considered for later clinical trials with investigational drug and be seen every 6 months by the study doctors, for up to 3 years.

The GLY-000 study is open to approximately 48 patients of all ages and is already over halfway enrolled. We are hoping to open at least one more site in the US and several more in Europe. Interested patients can find full study details and participating doctors listed on the website https://clinicaltrials.gov and inserting the ID number NCT03173300.

Lipo-M1P Development Update
Progress is being made with the development of our investigational drug, Lipo-M1P. It is being designed as a replacement for the important protein glycosylation intermediate, mannose-1-phosphate (M1P) and thereby support normal protein glycosylation. The following video provides a simplified overview of our therapeutic approach:
https://youtu.be/olMqE5QBDNM.

During 2018, Glycomine has collaborated with experts in the field and finalized the manufacturing process for Lipo-M1P to ensure highest yield and quality of product. Larger scale manufacturing is being scheduled in parallel with currently ongoing laboratory and animal testing required to proceed with human dosing, estimated by end of 2019.

Upcoming Clinical Trials
Glycomine met with both the European and USA regulatory agencies last year and received valuable input about the safest approach to human dosing once the Lipo-M1P supply is available. We are required to dose a small number of healthy volunteers (people who do not have PMM2-CDG) and expect to do so by end of 2019. We will then proceed with dosing in PMM2-CDG patients as soon as possible after safety is shown in that first healthy volunteer study, anticipated for early 2020.

GLY-000 Study Contacts
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4TH WORLD CONFERENCE ON CDG

Title: Will you walk the CDG road - from diagnosis to therapies - with us?
This is the challenge for this very special edition of the conference, which invites all participants to walk “The CDG road: from diagnosis to therapies!”. Registration is now OPEN (and will remain so till 30th June).
The design of this 4th edition is based on the therapy development process. It is organized in 4 different workshops all ending with a moderated discussion (called Think Tank). The main aims of these conference are to:
• Identify the main obstacles to therapy development for CDG;
• Gather all of the CDG community stakeholders (professionals and families) to promote networking, to discuss, share experiences and find solutions together;
• Ultimately, to accelerate therapy discovery for CDG!
The conference is taking place in Lisbon (Portugal) from 26th to 27th July. More detailed information can be found at the official web page of the event [http://www.apcdg.com/events.html](http://www.apcdg.com/events.html).
This year there will also be a poster session. Find out more about it at [http://www.apcdg.com/poster-submission---world-conference-on-cdg-2019.html](http://www.apcdg.com/poster-submission---world-conference-on-cdg-2019.html)
Additional information and updates will be available in social media: Facebook, Twitter, LinkedIn and APCDG blog [HERE](http://www.apcdg.com/poster-submission---world-conference-on-cdg-2019.html).

If you have questions or need more information, please write to: [worldcdgconference2019@gmail.com](mailto:worldcdgconference2019@gmail.com)
Looking forward to seeing you all in Lisbon to walk the CDG road!

1ST SATELLITE MEETING
"CDG CLASSIFICATION AND NOMENCLATURE"

What is a CDG? This THE question that will be addressed at the 1st Satellite Meeting dedicated to this topic. This meeting is taking place on 25th July afternoon (pre-World Conference on CDG).

WHY was this topic selected? The number of CDG types has been growing steadily in recent years. Having a common, global and clear definition of what defines a CDG (in genetic, biochemical and clinical terms) is of the utmost importance for Professionals and Families; it is critical for research and diagnosis; it central for clinical care and quality of life!
Dr Carlos Ferreira is going to give a talk overviewing this problematic, which will be followed by a discussion moderated by Prof Jaak Jaeken, Prof Eva Morava and Dr Maurizio Scarpa. The aim is to reach an international consensus.

Join us in this debate!
If you have questions or need more information, please write to: [worldcdgconference2019@gmail.com](mailto:worldcdgconference2019@gmail.com)
DO YOU HAVE 2020 VISION?

By Hud Freeze PhD, Scientific and Medical Advisory Board Member

Oh, no, I’m not talking about the visual acuity test. I’d fail that one; without my contacts I’m legally blind at 20/400.

I’m talking about the Rare Disease Day 2020 here in San Diego, and there, I do have an evolving vision of what I would like this meeting to be and what it would encompass. To me the most important thing is that all the stake holders—families, scientists and physicians—come away enriched because of the meeting. So, unlike most scientific meetings, ours does not have a single “average” focus. Barriers and silos are keep us isolated. We know that well here in the US where conversations over sensitive topics can quickly get out of hand. My goal is to assemble a group of presenters who will bring up to the minute scientific results, interpretations and a vision (20/20) of where their work might lead. Of course, “it’s always hard to make predictions, especially about the future” (A. Berra, NY Yankees). But we do our best. I especially encourage every speaker to provide a medical or patient-centric element in their talks, including realistic hopes about where their work leads. Even in grant applications, scientists must think about their impact on patients and families. Because we know “from research…the Power to Cure”.

Our Rare Disease Day meeting has evolved over the last decade or so. Initially, we were more focused on a subset of “traditional” Type I and Type II disorders. We also drilled down on PMM2-CDG, and more recently on NGly1, a complicated disorder with many overlapping features with CDG as the NIH has demonstrated. Now that there are well over 130 different (primary and secondary) disorders that affect glycosylation. That is our unifying definition and primary starting point, but we cannot everything for everyone. But we can be more inclusive, and that’s what I want to do this coming year. Under the umbrella of glycosylation, I hope to erase some of the rather artificial boundaries. For instance, the dystroglycanopathies and other muscle diseases—now numbering 19 disorders—will have a presence in 2020. We hope those affected families will join us too. GPI-anchor defects—now numbering at least 18—will have a more prominent position. Our CDG-CARE grant recipient and ace-GPI researcher, Taroh Kinoshita, will be here. Our keynote presentation will be in the most capable hands of a Nobel Laureate. And like the 2018 meeting, our local mega philanthropists, T. Denny Sanford and Malin Burnham will welcome everyone. They love seeing all the kids and families. It makes their day. They are real fans of our meeting.

I want to highlight the basic science, that touches areas with promise or even the reality of therapy. It’s part of the “2020 Vision” I was talking about, and as the program solidifies in the near future, I hope the excitement of hearing about new possibilities and new approaches to the ever-broadening area of CDG will garner the enthusiasm and spirit of an “old time revival”. We’ll have some surprises too. Working on those also.

We’ll keep everybody updated as the time draws closer and I’m sure CDG CARE will send details for being here again in San Diego.

CDG CARE

Where CARE Grows...

Visit us on the web at www.cdgcare.com or send an e-mail to info@cdgcare.com.