



# THE CHILDREN'S FUND FOR GLYCOGEN STORAGE DISEASE RESEARCH

"... because every child deserves to be healthy."

## Hopes and Dreams

DIGITAL NEWSLETTER

December 2023



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**Catching up with Jerrod Watts, the first GSD1a patient to receive gene therapy treatment:** Find out how he is doing today.

**Super Bowl Raffle Fundraiser:** You could be going to Super Bowl LVIII in Las Vegas! Get your raffle tickets now!

**2022 GSD Gala Recap:** "A Night in Nashville" was a night to remember. Thank you to all who supported!

**AGSD Conferences:** Bringing the GSD community together.

**GSD1a Leadership Council:** Ultragenyx wants to hear from you!

**Patient Spotlight:** Congratulations to Jake Gordon who ran the the NYC Marathon in November. Get inspired!

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# GSD1a Research Update

Your contributions make a difference!

Almost 22 years ago *The Children's Fund for GSD Research, Inc.* was created, and thanks to you, we have raised over \$8,000,000 dollars! Most of these funds have been granted to researchers around the world, all working to make the lives of people with GSD1 and their families better, with the hopes of one day ultimately curing this disease. We are proud to have helped fund preclinical animal studies which were the gateway towards moving into clinical human trials!

Although we are pleased with the progress and outlook so far, we have not reached our goals yet. We still worry about the health of our children every second of every day and for this reason, we will continue to work hard raising funds so that we can support the world's most promising GSD1 research. We won't stop until a cure is found.



Ultragenyx completed a Phase 1/2 human clinical study and is currently more than halfway through the fully enrolled Phase 3 trial. "The Phase 3 GlucoGene study is underway to evaluate the ability of DTX401 (the company's moniker for the gene

therapy 'drug') to reduce the use of cornstarch while maintaining or improving glucose control as well as the therapy's impact on patients' quality of life." This is huge! Your generosity has and continues to make a difference. We are happy to share the exciting data from the [Phase 1/2 Study](#) as well as the press release from UConn Health about the [Phase 3 trials](#).



Moderna has been researching the use of mRNA for the potential treatment of GSD1a. The Balance Moderna Trial, which began at UConn Health in June

2022, aims to see if a new investigational medication, mRNA-3745, is safe and if it could potentially help correct the defect that causes GSD1a and restore function. If successful, mRNA-3745 would teach certain cells in the body to effectively break down glycogen, correct low blood glucose and avoid starch intake. To learn more, read the Uconn Health [press release](#) about the first patient dosed.

[Moderna Trials](#)



Beam Therapeutics, located in Cambridge, Mass., is dedicated to providing lifelong cures to patients suffering from serious diseases. Beam's technologies, which are based on gene editing, seek to address unmet medical needs so that people can reach their full potential. Beam-301 is focused on Glycogen Storage Disease Type 1a and is currently in its IND

enabling stage. It is focused on gene correction via a LNP in vivo modality. This study is a liver-targeting LNP formulation of base editing reagents designed to correct the R83C mutation, which is the most common mutation responsible for causing GSD1a.

To learn more about this exciting advancement in GSD research, please read Beam's October 25, 2023 [press release](#) addressing the status of new preclinical data demonstrating the ability of its drug candidate to directly correct the R83C mutation.

[Beam Trials](#)

## UCONN HEALTH

The GSD laboratory at the University of Connecticut (UCONN) has been created for Dr. Youngmok Lee where he and his team work towards developing cures through preclinical research using animal GSD models. The lab raises awareness about GSD and gene therapy among research communities, collaborating with research groups around the world. At UCONN there are both ongoing and future projects related to GSD1a. Read about them [HERE](#).

## Catching up with Jerrod Watts

A pioneer for GSD1a Gene Therapy Clinical Trials



Recently, board member Teri Reed sat down with Jerrod Watts, the first GSD1a gene therapy patient as he shared his inspirational experience being patient 1 of phase 1 human trials, which began July 24, 2018....

### **What made you decide to participate in the GSD1a gene therapy trial as patient 1?**

When I was a child, my goal in life was to bring about a change in the treatment of GSD so no one in the community would ever again fear dying in the middle of the night due to a missed cornstarch dose.

### **How has your life changed since your treatment?**

My life has been a complete 180 since the trial. Not only is my glucose under much better control, but I have also seen improvements in my body on a molecular level.

### **Did the results meet your expectations?**

Actually, I went into the trial with no expectations for my own treatment; my only hope was that it would not make me sick.

[Read the full interview HERE](#)

Gene Therapy Press Release

## **Super Bowl LVIII Raffle !!**

This is our TWENTY-FIRST annual Super Bowl raffle to support The Children's Fund for Glycogen Storage Disease (GSD) Research. Our motto has always been that we will not stop until "every child born with GSD is cured." As in past years we ask for your support to continue with our mission to cure GSD. Because we are a volunteer organization 100% of your contributions are used to fund the most promising research. Click [HERE](#) to purchase raffle tickets or make a donation.

# WIN 2 TICKETS TO THE SUPER BOWL

SPONSORED BY AND TO BENEFIT  
THE CHILDREN'S FUND FOR GLYCOGEN STORAGE DISEASE RESEARCH



## GRAND PRIZE INCLUDES:

- TWO TICKETS TO SUPER BOWL LVIII ON 2/11/2024 AT ALLEGIANT STADIUM IN LAS VEGAS, NEVADA
- 3 NIGHT ACCOMMODATIONS AT MGM GRAND, LAS VEGAS

A MAXIMUM OF 1,000 TICKETS WILL BE SOLD

DRAWING TO BE HELD:  
WEDNESDAY, JANUARY 7, 2024 AT 5PM PST

\$100 PER TICKET

**PURCHASE TICKETS**

OR, CHECKS PAYABLE TO:  
THE CHILDREN'S FUND FOR GSD RESEARCH  
C/O MIKE JULIUS  
5455 BROMELY DRIVE  
OAK PARK, CA 91377

THANK YOU FOR YOUR SUPPORT!

ONE PRIZE TO BE AWARDED. NO PURCHASE NECESSARY. SUGGESTED DONATION PER TICKET: \$100. TICKET HOLDERS NEED NOT BE PRESENT TO WIN. WINNER WILL BE NOTIFIED BY PHONE OR EMAIL IMMEDIATELY FOLLOWING THE DRAWING ON WEDNESDAY, JANUARY 7, 2024 AT 5 PM PST. PRIZES ARE NOT REDEEMABLE FOR CASH OR ALCOHOLIC BEVERAGES.

**CureGSD.org**

2022 GSD Gala brings in over \$300,000!



## A NIGHT IN NASHVILLE

2022 GSD Gala

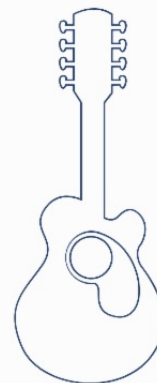
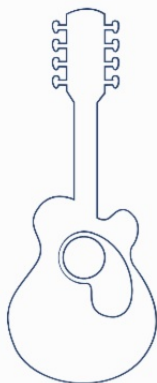
benefitting The Children's Fund for Glycogen Storage Disease Research



On November 5, 2022, *The Children's Fund for GSD Research* hosted our 13th fundraising gala, *A Night in Nashville*. Over 300 friends, family members, sponsors, scientists, researchers and colleagues gathered together at the Seminole Hard Rock Hotel & Casino in Hollywood, Florida to support one another and learn about the progress we have made in GSD Research. [Read more here.](#)

[View our Digital Event Program](#)

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## 2023 GSD Conferences

This past year our foundation was represented at two annual Association for Glycogen Storage Disease (AGSD) conferences: Orlando, Florida and Terrassa, Spain. It was amazing to meet so many strong advocates for GSD and connect with patients, caregivers and scientists around the world.



Jake Gordon (GSD1a) and Iris Ferrecchia share a moment at the conference in Spain.



Lisa Hodes at the AGSD conference in Orlando, where The Children's Fund had a table to share information about our foundation.

## GSD1a Leadership Council

Ultragenyx Pharmaceuticals is establishing a Global Leadership Council to learn more about the needs and challenges of the GSD1a community. If you are someone living with and/or caring for someone with GSD1a and would like to be considered, please click [HERE](#) to learn more.





# Announcing Ultragenyx's New Global GSDIa Leadership Council

Learn more and how to apply



Ultragenyx is establishing a **Global GSDIa Leadership Council** to build on the invaluable insights we have learned directly from the community, better understand the needs and challenges of the community, and use these insights to inform future Ultragenyx GSDIa activities



Ultragenyx would like the Council to **provide the diverse perspectives and feedback** of people living with and/or caring for someone with GSDIa and include representatives from different countries and across age groups



Ultragenyx aims for Council members to:

- **Provide** insights as someone living with and/or caring for someone with GSDIa
- **Communicate** individual needs and the needs of the GSDIa community
- **Represent and share** perspectives of the broader GSDIa community



The Council will **convene members up to 3 times a year** (virtually and possibly in-person) beginning in the first half of 2024. Individuals will serve up to 2-3 years on the Council, however, this timing may change depending on the specific needs of the group

If you are interested in being considered for Ultragenyx's Global GSDIa Leadership Council, please:

Send an email to

[gsdiacouncil@discoveryworldwide.com](mailto:gsdiacouncil@discoveryworldwide.com)  
with this information:

- Your name
- Your country/state
- Do you or your loved one(s) have GSDIa?
- What is your/their gender?
- What is your/their current age?
- At what age were you/they diagnosed with GSDIa?



Selected individuals will be invited to participate in a 1-hour discussion in November or December 2023 with a member of the Discovery team (an Ultragenyx partner)



Up to 8 people living with and/or caring for a loved one with GSDIa will be invited to participate on the Council

Please note:

- Due to the maximum number of individuals we can accommodate on the Council, not everyone will move forward to a discussion with Discovery
- Phase 1/2 study participants can apply, but Phase 3 study participants cannot apply to protect the integrity of the study
- Council meetings will be conducted in English

Ultragenyx is committed to the GSDIa community; there will be other opportunities to share your voice with us!

By contacting Discovery, you are consenting to Discovery using your personal information to contact you about participation in this program. Discovery will store and use your personal information in accordance with applicable laws and regulations.

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## Patient Spotlight: Jake Gordon

GSD patient doing extraordinary things!

Running a marathon is not an easy task for anyone. Running a marathon with GSD is something else entirely. When my son Jake first began to exercise



in middle school, his blood sugar would constantly drop to dangerously low levels. Making it just one lap around the school track was a difficult task, with rescue measures put in place to bring his blood sugar level back to stable numbers. But Jake was determined to make this work. Through hard work, determination, and communication with his care team, he turned himself into an accomplished runner. [Read the full story.](#)

## Do you have a GSD story to share?

Send us an email at [info@cureGSD.org](mailto:info@cureGSD.org)  
and you could be featured in the next edition of our  
Hopes and Dreams newsletter.

We started this foundation almost 22 years ago. At that time there was very little information available on Glycogen Storage Disease and there was virtually no research being done towards treatment or a cure. We have made tremendous strides in this area, however, GSD patients and caregivers continue to struggle and deal with GSD issues each and every day. The fears we had 22 years ago are just as real and intense as ever, and we remain hopeful that our GSD community of families will one day be able to live the healthy, carefree lives they deserve.

As we bid goodbye to 2023, we reflect back on the goodness of our friends, family, colleagues and loyal supporters who have helped get us to where we are today. Your kindness and generosity do not go unnoticed and we hope we can count on you to continue the momentum for funding of new treatments and an eventual cure. **We thank you, we appreciate you and we look forward to sharing more positive GSD news in the coming year.**

Wishing you good health, peace and happiness in 2024!

**Happy Holidays**

from all of us at

**The Children's Fund for Glycogen Storage Disease Research**

**[www.cureGSD.org](http://www.cureGSD.org)**

[Donate Now](#)

The Children's Fund for GSD Research Inc.  
info@curegsd.org | www.CureGSD.org



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