WINTER 2025

GALACTOSEMIA GAZETTE

Galactosemia Foundation
Linked for Life

President's Message

Always Moving Forward

BY: NICOLE CASALE

It hasn't been too long since we were all together in Charlotte, but so much has happened since then—some good, some not so great. What remains constant is our resilience. Time and time again, we prove that we are stronger when we stick together. As a Foundation and as a community, we are always moving forward.

We are kicking off 2025 with a renewed focus and a laundry list of goals that we're motivated to achieve. I was telling a friend recently that when I first joined the board, our monthly meetings would last about 45 minutes, maybe an hour. Now we're starting at 8 p.m. and lucky if we wrap up after two and a half hours. It's incredible how much we've grown—we're no longer just a grassroots organization. We're becoming something much bigger, and we are so excited about that.

But a growing to-do list and additional commitments means, you guessed it… we're always looking for volunteers. And here's the tough part—even though we need help, we don't always have the bandwidth to direct that help. It is challenging to guide others when we're learning as we go. But we are trying our best.
So, in an effort to be transparent, here's a list of what's to come and how you can support our short, medium, and long-term goals:

Immediate and Ongoing Goals
- Monthly webinars or virtual meetups
  - Volunteers to help with meetings
  - Speaker suggestions
- 4-5 outreach activities across the U.S., annually

Strategic Growth
- Create and implement a Scientific Advisory Board
- Develop a Newborn Welcome Package
- Research and implement a new Patient Registry

Enhancing Community Engagement
- Summer camp on off-conference years
- Revamp our bi-annual conference (we've heard your feedback—we know what's great and where we can improve)
- Advocacy support and travel stipends for community members
- Expand outreach and fundraising opportunities around Rare Disease Day and Giving Tuesday

We're excited about the road ahead, but we can't do it without you. Whether it's lending your expertise on an on-going basis, helping with events, or just spreading the word, every effort moves us closer to achieving these goals. And of course, continued fundraising to support conference, future education opportunities and research.

Let's keep moving forward—together.

Update From Applied Therapeutics

At Applied Therapeutics, we are continuing to advance our mission of creating transformative treatments for rare diseases like Galactosemia.

Our drug candidate, govorestat, is a once-daily oral compound that inhibits the enzyme Aldose Reductase, which plays a crucial role in the pathogenesis of Galactosemia. Govorestat has demonstrated rapid and sustained reductions in galactitol in clinical trials, resulting in meaningful clinical outcomes in pediatric patients.

Our commitment to the Galactosemia community is rooted in our belief that govorestat has the potential to change the lives of patients with Galactosemia, which we believe is evidenced by the breadth of efficacy and safety data demonstrating its ability to stop the decline on progressive clinical outcomes, including cognition and behavior.

We are continuing to work with the U.S. Food and Drug Administration (FDA) to determine next steps with regard to govorestat. In December 2024, we announced the appointment of John H. Johnson, a biopharmaceutical industry veteran with 40 years of transformational leadership experience at global healthcare organizations, as Executive Chairman of Applied Therapeutics. John’s top priority is ensuring the Company has the right groundwork in place to work toward our regulatory and clinical milestones. We also announced that Les Funtleyder has stepped in as Interim CEO. Les has a strong understanding of our business and operations and has fostered relationships across our industry and the Applied Therapeutics team since resuming the role of CFO last year.

While we complete the important work to best position govorestat, John and the rest of the Applied Therapeutics team remain committed to developing a new treatment option for Galactosemia and helping patients suffering from this rare disease.

APPLIED
THERAPEUTICS

Glow for Galactosemia on April 26

Planning for Glow for Galactosemia 2025 is underway. For the second year, the run will take place in Roanoke, VA under the leadership of Jamie Hiegel.

As in past years, there will be a virtual component for our community across the country to fundraise and participate from their location.

Do you want to create a team but running isn’t your thing? That’s okay! In previous years teams have done glow bowling, cycling classes, roller skating and more!

Many of the details are still in the works but we will communicate when more information is available.

Get registered today!

Laser Tag, Bumper Cars, Safe Food, Oh My!

Our first outreach event in Raleigh, North Carolina was a success. Members of the Galactosemia Board were joined by local galactosemia families for a night of fun at Frankies!

We started with a buffet of safe food items and then spent some time in the game room playing laser tag, bumper cars and some were even brave enough to try the outdoor go karts.

As a group, the Board doesn't do a lot of traveling, but we are making an effort to plan these types of outreach events when we do.

Thank you to the families who came out to spend time with us and make some memories! We look forward to spending time with more of you at future events.

Connect with Us

Follow Galactosemia Foundation on Social Media and visit our website.

Contact The Board

Nicole Casale, President
Brittany Cudzilo, Vice President/Outreach
Jessica Taylor, Treasurer
Jodie Solari, Communications
Mary Leimbach, Secretary
Gillian Sapia, Outreach
Keith Topper, Board Member at Large
Kelley Foley, Board Member at Large

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UPCOMING EVENTS 2025

FOR MORE INFO VISIT:
WWW.GALACTOSEMIA.ORG

FEBRUARY

OUTREACH ACTIVITY
FRANKIES OF RALEIGH
FRIDAY
2/21/25 | RALEIGH, NC

OUTREACH & ADVOCACY
RARE DISEASE WEEK ON CAPITAL HILL
MONDAY-WEDNSESAY
2/24/25- 2/26/25 | WASHINGTON, DC

MARCH

VIRTUAL WEBINAR
DIET GUIDELINES & BONE BASICS
THURSDAY 7:00PM EST
3/27/25 | DR. VAN CALCAR

SCAN TO GET INFORMATION OR REGISTER FOR EVENTS

APRIL

OUTREACH & FUNDRAISER
GLOW FOR GALACTOSEMIA 5K
SATURDAY
4/26/25 | ROANOKE, VA

FUNDRAISER
THE DENISE D'ASCENZO FOUNDATION'S WALK TO FIGHT RARE DISEASES
SATURDAY
4/26/25 | HAMDEN, CT

MAY

VIRTUAL GATHERING
MIX, MINGLE & MUNCH: A VIRTUAL BAKING PARTY
FRIDAY
5/9/2025 6:00 PM EST

OUTREACH ACTIVITY
TOUR OF BOSTON
SATURDAY
5/17/25 | BOSTON, MA

JUNE

VIRTUAL ROUND TABLES
THROUGHOUT THE MONTH OF JUNE BASED ON AGE GROUP

VIRTUAL GATHERING
BOARD MEET-UP & UPDATES
SUNDAY 8:30 PM PM EST
6/22/25

JULY

VIRTUAL WEBINAR
BACK TO SCHOOL; TEACHER LETTER; IEP; 504 & MORE
T.B.D. | TRACEY MILLER

AUGUST

OUTREACH ACTIVITY
DENVER ZOO
SUNDAY
8/3/25 | DENVER, CO

SEPTEMBER-DECEMBER
T.B.D.

MARK YOUR CALENDAR: July 16 - 18, 2026
Galactosemia Conference in Aurora, CO