

GALACTOSEMIA GAZETTE



Galactosemia Foundation

Linked for Life

President's Message

BY: NICOLE CASALE



Hello Galactosemia Community!

I can't believe how quickly time flies. It feels like we were just together in Orlando, and here we are only 6 months until the next conference. So now in the midst of conference planning, this is where my column is going. I know many have been asking about it, and I promise the information will be out soon!

What I can say, is that we have some great speakers scheduled, along with some awesome breakout topics. As always, we plan for the Thursday Social, Friday Talent Show, and the Saturday Dance. We've changed some things up this year, so stay tuned. For now, please mark your calendars for (in person) July 18-20, 2024, and we'll see you at the Embassy Suites in Concord, North Carolina! Although registration is not yet available you can visit the [Galactosemia Foundation website](#) to see dates, prices and a link for hotel reservations.

For those unable to attend the 2024 conference, unfortunately we will not be running a hybrid event. We attempted it in 2022 but did not have the bandwidth or resources to execute it successfully. We have however, been working on our website and are creating a member portal in hopes to connect more throughout the year. The goal is to host webinars, and virtual meet and greet, so stay tuned.

MARK YOUR CALENDAR: July 18 - 20, 2024
Galactosemia Conference in Charlotte, NC

We Want your Feedback

The Galactosemia Foundation, with support from Jaguar Gene Therapy, created a new resource for our community that launched in June of 2022. [Navigating Galactosemia Life Stages: A Handbook for the Galactosemia Community](#) presents information for each life stage of galactosemia to guide patients and caregivers from newborn diagnosis through adulthood as they navigate the lifelong complications of the disease.



TELL US WHAT
YOU THINK!

Complete a brief
survey!

SURVEY OPEN THROUGH
FEBRUARY 29

We are now seeking your feedback on the handbook! If you are interested, we ask you to fill out [this brief survey](#) by Feb. 29. The survey consists of 15 questions and will take no more than 30 minutes to complete. We plan to use your feedback to better understand the usefulness of the handbook to community members. Your feedback may also help inform future updates to this handbook.

Thank you in advance for your honest responses. If you have any questions, please reach out to Nicole Casale (nicole.casale@galactosemia.org) or Brian Moy (bmoy@jaguargenetherapy.com).

Update From Applied Therapeutics

Applied Therapeutics recently announced it has submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for govorestat (AT-007) for the treatment of Classic Galactosemia. The NDA was submitted in December 2023. The FDA has a 60-day filing review period to determine whether the NDA is complete and accepted for review. “There are currently no treatments available for Galactosemia, a serious progressive disease affecting over 3,000 patients in the United States,” said Brittany Cudzilo, Vice President of the Galactosemia Foundation. “Govorestat offers hope to many patients and families affected by Galactosemia. I saw the positive impact of govorestat first-hand in my daughter, Ansell, who participated in the ACTION-Galactosemia Kids pediatric study. As a community, we will do everything possible to ensure that the regulatory agencies understand the importance of approving this treatment for our loved ones with Galactosemia.”

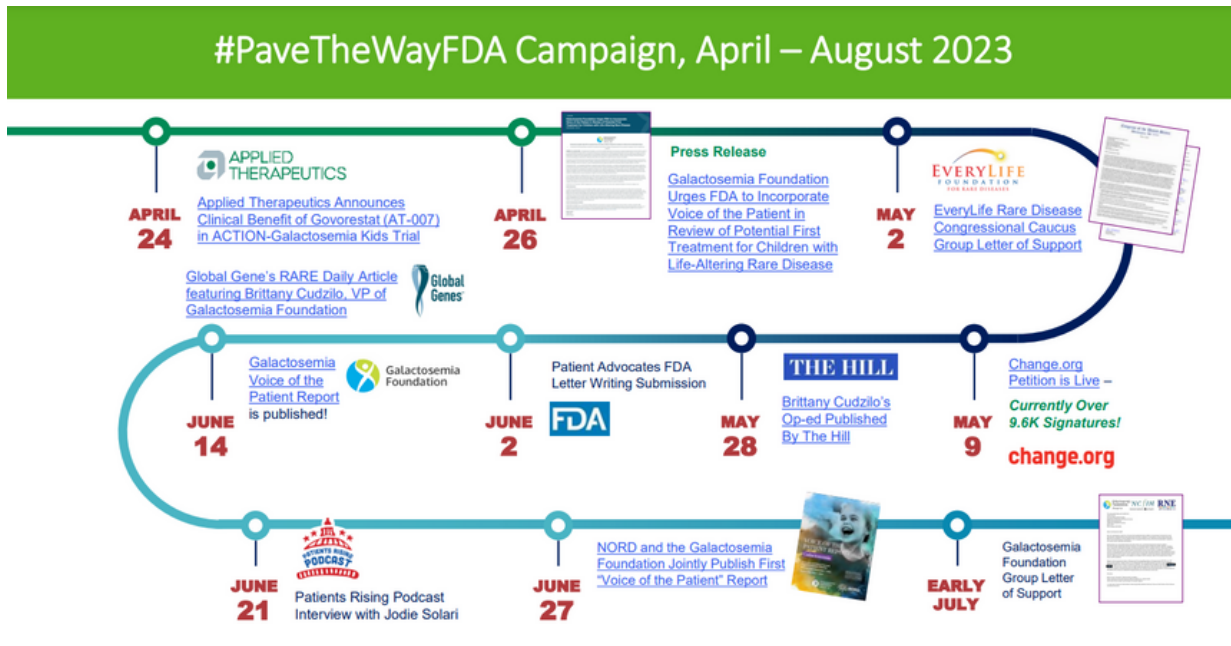
In addition, the Company submitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) in the fourth quarter of 2023, which was subsequently validated and accepted for review in December 2023, and will move to review by the EMA’s Committee for Medicinal Products for Human Use (CHMP).

You can find additional information in the Applied Therapeutics press release available [on our website](#).



#PaveTheWayFDA

The Galactosemia Foundation did a #PaveTheWayFDA campaign last year, which ran April - August. We published posts across our [social media channels](#) as well as participated in press releases, articles and interviews to raise awareness and ask the FDA to approve Govorestat, the first ever possible treatment for galactosemia. Click on the links below the image to learn more about this important campaign.



- April 24 - [Applied Therapeutics NR](#)
 - [Global Genes RARE Daily article](#)
- April 26 - [Galactosemia Foundation PR](#)
- May 2 - [EveryLife Group Letter](#)
- May 9 - [Change.org petition](#)
- May 28 - [Op-ed Published by The Hill](#)
- June 2 - [FDA letter writing submission](#)
- June 14 - [Galactosemia Voice of the Patient Report Published](#)
- June 21 - [Patients Rising Podcast](#)
- June 27 - [NORD and Galactosemia Foundation jointly publish First Voice of the Patient report](#)
- Early July - [Galactosemia Foundation sends group letter of support](#)

Interested in Advocacy?

While advocating for a treatment has been a huge effort, there are many other advocacy efforts underway. If you would like to learn more or volunteer to support the cause, please fill out [this form](#). You can learn more about our Advocacy team on the [Galactosemia Foundation website](#).



GLOW FOR GALACTOSEMIA on May 5

GLOW 2024 is ON and we are so excited to have added two new faces to the leadership of this event! If you haven't participated in GLOW, it's a great way to raise awareness around Galactosemia, fundraise for our foundation, and invite your family and friends to join you in some sort of physical activity (we've got lots of runners, but have had all kinds of exercise in the past, including ice skating, bowling, yoga, and more).

In the past 6 years, GLOW has raised over \$200,000 while connecting many families with Galactosemia! Because of GLOW's success, we have expanded and are grateful to welcome Jamie Heigel and Kayla Kloska to the team!

Jamie will be hosting the GLOW physical event in Roanoke, VA and Kayla will be helping with virtual teams + participants! This year GLOW will be taking place on May 5 to accommodate a new site and hopefully offer better weather for many of our virtual hosts! Many of the details are still in the works but we will communicate when more information is available.



Reminder about Lactose-free Dairy Milk

Recently, a young child with galactosemia experienced life-threatening liver dysfunction from consuming large quantities of lactose-free dairy milk. Fortunately, the child has recovered, but we want to reinforce the message that “lactose-free” dairy milk is not galactose free. To be clear, lactose is a 2-sugar compound that includes one molecule of glucose linked to one molecule of galactose. Most lactose-free dairy products have been processed to break down lactose into its components – glucose and galactose. So, the lactose is gone, but the galactose remains. Lactose-free dairy milk and dairy products are intended for people with lactose-intolerance, which is a common condition that is completely different from galactosemia. Lactose-free dairy milk and dairy products are as dangerous to children with galactosemia as the “regular” milk they came from. Please be careful and consult your healthcare provider if you are unsure of what foods are and are not safe for you or your child to consume. Thank you.

-Judith Fridovich-Keil, PhD, Michael Gambello, MD PhD, Hong Li, MD PhD

Connect with Us

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Nicole Casale, President
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