



Fall 2017

## Welcome to the Ultragenyx Patient Advocacy E-News Update!

Welcome to both returning visitors and new subscribers; we're glad you're here! This fall offers many opportunities to get involved in your rare disease community, have your voice heard and share your experience with others.

We're excited to announce that Ultragenyx has launched a **Facebook page**, featuring new resources, upcoming events, patient stories, and more! You can visit and "like" the page [here](#).

We look forward to connecting!

Best,  
The Ultragenyx Patient Advocacy Team

## Advocates in Action National MPS Society Annual Family Conference



Mark Dant, the CEO and President of the National MPS Society, and his son Ryan and girlfriend Silvia pose at the Ultragenyx table at the National MPS Society Family Conference.



Patients, caregivers, advocates and industry join together to release balloons at the Minneapolis Madness for MPS 5K in remembrance of those who have passed.

The 2017 National MPS Society Family Conference was the biggest and best one yet! This year, the conference was held from August 3-5 in Minneapolis, MN. Attendees came together for an exciting few days of meet and greets, and informative sessions on emerging therapies and disease coping strategies. The weekend was topped off with a challenging and picturesque 5K run across the Mississippi River.

Members of the Ultragenyx Patient Advocacy team were lucky enough to participate. Kim Mooney, Associate Director of Patient Advocacy at Ultragenyx, said, "The strength and positive attitude among the families we've met is an inspiration to continue research! The MPS community is truly special, and its members are pioneers for rare disease advocacy."

See other MPS conference highlights [here](#), and don't miss these upcoming conferences!

- **Global Genes' RARE Patient Advocacy Summit** will be held in Irvine, CA from Sept. 14-15, with the RARE Tribute to Champions of Hope Gala on Sept. 16. Last year, 98% of attendees felt more connected with the rare disease community after the summit. There is a discounted registration cost for patients and advocates. Breakfast, lunch, refreshments, snacks and evening receptions are included with registration. Time is running out to participate in this year's event, so don't forget to register [here](#).
- **XLH Day**, sponsored by the XLH Network Inc., will take place at Quinnipiac University in North Haven, CT this year from Oct. 6-7. This annual event brings XLH patients, caregivers and advocates together to connect with one another, discuss opportunities and challenges, and hear from doctors about potential new treatments. The first annual Walk/Run for XLH will be held during the weekend, and you can even participate virtually to help raise money for XLH education! Find out more [here](#).

To find out how to register or be involved in other patient advocacy events, visit our [event page](#).

## Featured Resources

What is a genetic counselor and what do they do?



Genetic counselors have the advanced training needed to guide and support people with inherited diseases, and their families. They can help families understand what genetic testing might be right for them and also help understand what genetic results mean. Interested in learning more, or how to find a genetic counselor near you? Check out the "About Genetic Counselors" website from the National Society of Genetic Counselors.

[Learn More](#)



The Child Neurology Foundation (CNF) Family Support and Empowerment Program (FSEP) was created so that no parent or caregiver in the child neurology community ever walks alone. This new program offers families a free, direct connection with a *Peer Support Specialist* who is trained to listen to you and support you on your journey with a neurologic condition. In just six months, the program has connected with families in 28 U.S. states and 24 countries from across the globe.

[Learn More](#)

## Patient Journeys



### Tasia: Learning to Manage my FAOD

Meet Tasia and learn more about what it's like to grow up with FAOD.

*"When I see kids and their parents struggling the same way that I did, and my parents did, being able to be a beacon of hope for them—that in itself has really helped me."*

[View the full story](#)

## What's Happening?

September 2017 - December 2017

Below are upcoming events and important dates that may be of interest to you or someone you know. Please consider attending or spreading the word.

### September

- Newborn Screening Awareness Month
- Global Genes RARE Patient Advocacy Summit: September 14-15 Irvine, CA
- Global Genes RARE Tribute to Champions of Hope Gala: September 16 Anaheim, CA
- International Ataxia Awareness Day: September 25

### October

- XLH Day 2017: October 6-7 North Haven, CT

### November

- Epilepsy Awareness Month
- 5<sup>th</sup> Glycoproteinoses International Conference: November 1-4 Rome, Italy
- TREAT-NMD 5<sup>th</sup> International Conference: November 27-29 Freiburg, Germany

### December

- International Day of Persons with Disabilities: December 3

[See the full calendar](#)

## Thank you!

Thank you for your continued support of rare disease communities. If you found this E-News Update helpful, please invite your friends and family to [join our mailing list!](#)

The patient advocacy department within a company serves as the point of connection to the patient community. It works to understand and represent patient and caregiver views within its organization.

Ultragenyx is a clinical-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic conditions.

Have a specific question? Don't hesitate to [contact us](#).

[Visit our Patient Advocacy Website](#)