Patient Advocacy at ultragenyx pharmaceutical

Winter 2017

Welcome to the Ultragenyx Patient Advocacy E-News Update!

As 2017 draws to a close, the Ultragenyx advocacy team is reflecting on an action-packed year filled with productive meetings, engaging events and strengthened partnerships. Together, we also made history with Ultragenyx's first FDA-approved product.

None of these moments could have happened without the participation and support from you and your fellow community members. Thank you for being involved and continuing to share your voice. Looking ahead to 2018, we are excited to both build on these existing connections and grow our community.

We wish you a safe and enjoyable holiday season.

Best,
The Ultragenyx Patient Advocacy Team
Don't forget to follow us on Facebook!

Ultragenyx Receives First Commercial Approval in the U.S.

Thank you to the individuals who participated in clinical trials for their invaluable contributions, and to the caregivers and families who supported their participation. Read the full news release here. This launch also includes UltraCare™, our new patient service program in the U.S. We encourage you to visit UltraCareSupport.com to learn more!

Advocates in Action

This year, Ultragenyx was privileged and honored to meet directly with members from four rare disease communities to gain insight into the patient journey and better understand issues important to patients, caregivers and families.

- In May and October, Ultragenyx brought together people impacted by long-chain fatty acid oxidation disorders (FAOD) to learn more about their experiences as patients and caregivers.
- During July, caregivers of people living with Glut1 deficiency syndrome joined Ultragenyx to discuss potential collaboration opportunities to improve patient care, treatment and education.
- In October, Ultragenyx also brought together patients and caregivers impacted by GNE Myopathy in Europe to share their experiences with one another.
- During November, a diverse group of patients, physician experts and leaders of patient organizations from the X-linked hypophosphatemia (XLH) and rare bone disease communities came together at an XLH Patient Advocacy Summit hosted by Ultragenyx.

Key topics discussed at all meetings included educational needs; opportunities to improve diagnosis, management and treatment for future generations; and appropriate ways for industry and advocacy groups to work together. We look forward to continuing the dialogue!

Welcome Our New Advocacy Team Member

We are happy to welcome Kristin Voorhees, Senior Manager of Patient Advocacy, to the Ultragenyx advocacy team. Kristin has a passion for helping patients and caregivers advocate for themselves within the healthcare system. To learn more about Kristin and other members of the advocacy team, visit our team page here.

"My daily goals are to make a positive difference in the lives of patients, caregivers and families and ensure that their voices are heard."
– Kristin Voorhees

Featured Resources

NIH's Talking Glossary of Genetic Terms

What does it mean when someone talks about DNA, chromosomes or epigenetics? You might hear these words from physicians or researchers, but might not always remember their definitions. The NIH's National Human Genome Research Institute (NHGRI) has a nifty glossary where specialists share definitions, images and animations to explain genetic terms in a unique way.

Learn More

Caring for Rare Disease Caregivers

Caring for someone with a rare condition can be an around-the-clock effort, but we sometimes forget caregivers need help, attention, and downtime too. The Caregiver Action Network created this guide for caregivers of loved ones with rare disease. It has lots of helpful hints and resources designed to help caregivers maintain balance.

Learn More

Patient Journeys

Cheryl:
Living with X-Linked Hypophosphatemia (XLH)

Meet Cheryl and learn how she stays positive while managing her XLH.

"Having a rare disease, I can't control my symptoms, but I can control my attitude."

View the full story

What's Happening?
January – February 2018

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.

January

- A Rare Affair 2018: January 7
  San Francisco, CA
- RARE in the Square 2018: January 8 – 10
  San Francisco, CA

February

- 14th Annual WORLD*Symposium*: February 5 – 9
  San Diego, CA
- EURORDIS Black Pearl Awards: February 20
  Brussels
- RDLA Rare Disease Week on Capitol Hill: February 25 – March 1
  Washington, D.C.
- International Rare Disease Day: February 28

See the full calendar

Thank you!

Thank you for your continued support for rare disease. If you found this E-News Update helpful, feel free to 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 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.

Visit our Patient Advocacy Website

Patient Advocacy at ultragenyx pharmaceutical

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