Patient Advocacy at Ultragenyx pharmaceutical

Spring 2018

Welcome to the Ultragenyx Patient Advocacy E-News Update!

News

Ultragenyx Receives Second Commercial Approval in the U.S.

Ultragenyx has been granted its second commercial approval – a therapy for x-linked hypophosphatemia (XLH)! XLH is a hereditary, progressive and lifelong disease that can affect children and adults.[1] More than 200 people with XLH participated in our clinical trials. We would like to thank those individuals, and their caregivers and families for being their support system throughout the trials. Without them, this progress for patients would not be possible. You can visit Ultragenyx.com (our corporate website) for more information.

Advocates in Action

Employees, Advocacy Leaders and Patients Gather for Ultragenyx Patient Day 2018

Patient Day speakers included Dr. Emil Kakkis, CEO of Ultragenyx (pictured left) and Kristin Smedley (pictured right), award-winning speaker and author, founder of the Curing Retinal Blindness Foundation, and mother to two boys with a rare form of blindness called CRB1 LCA/RP.

Every day, rare disease patients and their families prove to us that you don't have to fly through the air or save the world to be a hero. That's why we partnered with Audentes Therapeutics to honor rare disease patients and families superhero-style for Patient Day 2018!

"We were so happy to meet many new people and, of course, catch up with familiar faces at Patient Day," said Kristin Voorhees, Senior Manager, Patient Advocacy at Ultragenyx. "It was incredible to see members of the rare disease community mixing and mingling with the people working to develop treatments."

The action-packed, fun-filled day had something for heroes of all ages – food, games, activities, and even a surprise performance by Rachel Platten, who sang "Fight Song!"

"Events like Patient Day help remind everyone at our company why we do what we do," noted Kim Mooney, Associate Director, Patient Advocacy at Ultragenyx. "The patient communities continually inspire us to be courageous and relentless in researching solutions to medical issues."

Ultragenyx to Receive Rare Impact Award for Industry Innovation

The National Organization for Rare Disorders (NORD) will host its 35th Anniversary Celebration presenting the 2018 Rare Impact Awards on May 17. This event will celebrate those who work to help the 30 million Americans with rare diseases. Ultragenyx is honored to be receiving the Industry Innovation Award for its first commercial approval! For more information on the Rare Impact Awards and to see the other honorees, click here. Be sure to watch the live stream of the ceremony online here!

Don't miss it!

- **RARE on the Road 2018**, a rare disease leadership tour sponsored by the EveryLife Foundation and Global Genes, sets out to bring education to the rare disease community at the local level. This year, sessions will take place in Houston, Salt Lake City, and Nashville throughout June and July. Registration is now open!
- **The 15th International Symposium on MPS and Related Diseases** will take place in San Diego, CA this year. "At the symposium, you can expect to see everything from workshops geared toward best practices in MPS care to the latest advances in research from around the globe," says Stephanie Bozarth, Chairman, Board of Directors, National MPS Society. Learn more here!

Featured Resource

Share Your Rare Journey

Telling your story is a powerful way to inspire change and advocate for yourself or your loved one. But where do you begin? Download our new resource, Share Your Rare Journey, for an easy-to-follow checklist.

Learn More

Patient Journeys

David:
Living with XLH

Meet David, who did not find out he was living with XLH until he was 33 years old.

"When I spoke with physicians about XLH, the symptoms I had experienced since childhood suddenly made sense."

Read More

What's Happening?

May - September 2018

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

May

- RARE Patient Advocacy Symposium: May 19 in Philadelphia

June

- RARE on the Road Session #1: June 4 in Houston, TX
- 2nd European Conference on Glut1 Deficiency: June 22-23 in East Grinstead West Sussex, UK
- CISCRP Medical Heroes Appreciation 5k Run & Walk: June 25 in Boston, MA
- RARE on the Road Session #2: June 30 in Salt Lake City, UT

July

- Fatty Oxidation Disorders (FOD) Awareness Month
- 6th International Conference on Neurodegenerative Disorders and Stroke: July 5-6 in Vienna, Austria
- RARE on the Road Session #3: July 21 in Nashville, TN

August

- 15th International Symposium on MPS and Related Diseases: August 2-4 in San Diego, CA

September

- Newborn Screening Awareness Month
- Dystonia Awareness Month
- International Network for Fatty Acid Oxidation Research and Management (INFORM) Symposium 2018: September 2-3 in Athens, Greece
- 10th Annual Rare Disease Scientific Workshop: Conceptualizing an FDA Rare Disease Center of Excellence: September 13
- International Ataxia Awareness Day: September 25
- ASBMR 2018 Annual Meeting: September 28 – October 1

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! If you'd like to stay up-to-date with resources, events, patient stories and more, we hope you'll also follow us on Facebook!

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

Reference

1. Linglart A, Biosse-Duplan M, Briot K, et al. Therapeutic management of hypophosphatemic rickets from infancy to adulthood. Endocr Connect. 2014;3(1):R13-30.

Patient Advocacy at Ultragenyx pharmaceutical

Ultragenyx Pharmaceutical Inc.
60 Leveroni Court, Novato, CA 94949
(415) 483-8800