Patient Advocacy at Ultragenyx

Winter 2018/2019

Welcome to the Ultragenyx Patient Advocacy E-News Update!

Happy Holidays from the Ultragenyx Patient Advocacy Team!

As 2018 comes to a close, we reflect on the progress in rare disease advocacy and research this year, and look with hope to the year ahead.

Read on for a recap of the Global Genes 7th Annual RARE Patient Advocacy Summit, new insights from advocacy leaders, and a look ahead to exciting 2019 events in the rare disease community. Don't forget to follow us on Facebook to stay up-to-date with resources, events, patient stories, and more!

We wish you a safe and enjoyable holiday season with family and friends.

Sincerely,
The Ultragenyx Patient Advocacy Team
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Advocates in Action

Rare Communities Connect at RARE Patient Advocacy Summit

Rachel Callander, author of the *Super Power Baby Project*, empowers patients and families in her keynote address at the Global Genes 7th Annual RARE Patient Advocacy Summit.

From October 3-4, 2018, more than 800 members of the rare disease community met in Irvine, CA for the Global Genes 7th Annual RARE Patient Advocacy Summit, the largest educational event for rare disease patients, advocates, and thought leaders in the world. The two-day event brought together people from all backgrounds to connect and learn from one another, while equipping themselves with the tools needed to move forward with confidence and positivity. We enjoyed meeting and speaking with attendees at the Ultragenyx Advocacy Team's exhibitor booth!

This year's keynote speaker was Rachel Callander, who shared her efforts to engage with healthcare professionals to challenge the language they use with regards to disability. Keep reading for Rare Remarks from Rachel below!

The RARE Patient Advocacy Summit also included educational panels, with tracks dedicated to living with a life-altering condition, growing a disease advocacy community, and the role of patients as partners and drivers in research. For the first time ever, the Summit featured a workshop called "Data and Technological Innovation," where attendees heard from industry and patient advocacy leaders about technological innovations and the exciting applications of these new tools.

Did you miss this year's Summit? You can watch sessions from the 2018 event here. Mark your calendars – next year's RARE Patient Advocacy Summit will take place September 18-20 in San Diego. Registration opens in March 2019 on the Global Genes website.

Rare Remarks

Insights from Advocacy Leaders

This issue's Rare Remarks come from advocate Rachel Callander, who makes it her mission to raise awareness for the importance of empowering communication in healthcare. Inspired by her experience coping with a diagnosis of partial trisomy 9 and partial monosomy 6 in her daughter Evie, Rachel created the Super Power Baby Project, an internationally celebrated book featuring extraordinary portraits of brave children with chromosomal and genetic conditions.

In October, Rachel delivered the keynote address at the Global Genes 7th Annual RARE Patient Advocacy Summit. Here is what she had to say:

On explaining her daughter's condition to strangers:

"I didn't get to share anything about Evie that I really loved, and I felt like I was letting her down. But with this new 'superpower' language, the next time someone asked me, 'What's wrong with your child?,' I just said 'Nothing is wrong with my child. Actually, she has superpowers.'"

"[Evie] could communicate just by using a single sound and the tone of her voice. There was so much going on between us without words and without language."

"She got around by scooting on the floor on her back. She had incredible core strength. This was another one of her superpowers. She would scoot around to her heart's content. Completely independent. Completely free."

On creating "Evie's Awesomeness Form" and the power of emphasizing the unique abilities of people with rare disease:

"This [traditional ability assessment] form actually broke me because I couldn't tick a single answer. I had this sense of hopelessness and powerlessness because I thought, 'The people on the other end reading this form would think that Evie was a child with no value that couldn't do anything.' I decided that I was going to make up my own form; one with much better questions."

"I created 'Evie's Awesomeness Form," ten or so of my own questions, and I answered yes to every single one. With this form, I could see that Evie was changing and growing and developing and celebrate those things."

"In that moment, Evie became a human being to them instead of this collection of failing body parts. And in that moment, there was an acceptance of difference, of diversity."

"After answering questions like, 'Tell me something that you're proud of. What are you enjoying about your child? What makes them laugh?' parents were saying, 'I am now seeing my child through a different lens. I am seeing how my child is developing. Not only in the realms of speech and language, but of character and personality.'"

To watch Rachel's whole keynote address, click here.

User Experience Survey

Tell us what you think!

The Ultragenyx Patient Advocacy team invites you to share your feedback. Please take five (5) minutes to fill out our brief User Experience Survey to help us better understand what content is most useful and relevant for you and learn how we can improve our resources in the future.

Provide Feedback

Please note, by completing the survey, you agree to the terms of our Privacy Policy. This survey is not the appropriate channel to report any side effects, product complaints, or adverse events associated with any Ultragenyx clinical trial or commercial marketed product. If you believe that you have experienced a side effect (adverse event), contact your physician immediately. To appropriately report side effects, a product complaint or an adverse event:

- Contact Ultragenyx at ultragenyx@primevigilance.com,
- Contact the U.S. Food and Drug Administration at www.fda.gov/medwatch or call 1-800-FDA-1088. If you do not reside in the United States, consult your physician.

Featured Resources

Rare University's "Understanding Drug Development"

This recently-updated, 12-lesson "Understanding Drug Development" course introduces the drug development process specifically for rare disease patients and advocates. Visit the Rare University website to enroll for free.

Learn More

Patient Journey

Braylee:
Raising a child with LC-FAOD

"Even on her hardest days, Braylee had the ability to make everyone around her smile, laugh, and be happy."

Braylee was born with LCHAD, a type of fatty acid oxidation disorder.

Read More

What's Happening?

January – March 2019

Don't miss these exciting events:
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.

January

- RARE in the Square: January 7-9 in San Francisco, CA

February

- Cerebral Creatine Deficiency Syndrome Awareness Day: February 1
- 15th Annual WORLDSymposium: February 4 in Orlando, FL
- EURORDIS Black Pearl Awards: February 12 in Brussels, Belgium
- EURORDIS Round Table of Companies: February 13 in Brussels, Belgium
- Miami Facioscapulohumeral Dystrophy Society Family Day Conference: February 23 in Miami, FL
- Rare Disease Week on Capitol Hill: February 24-28 in Washington, D.C.
- Rare Disease Day: February 28

March

- ENQO 2019: March 23-26 in New Orleans, LA
- 62nd Annual Ataxia Conference: March 29-30 in Las Vegas, NV
- RARE on the Road Session #1: March 30 in Boston, MA
- XLH Network Regional Meetup: March 30 in Columbus, OH

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

Patient Advocacy at Ultragenyx

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