

THE RARE ROUNDUP

The official Newsletter of Rare Disease Research, LLC

"To make a difference in someone's life, you don't have to be brilliant, rich, beautiful, or perfect.
You just have to care" - Mandy Hale

A THANK YOU NOTE FROM ALL OF US AT RDR

Twenty-twenty was a difficult year for many of us; and yet, we all got through it TOGETHER! RDR is extremely grateful to have the support of many collaborators, sponsors, CROs, and vendors throughout the years. We are especially thankful to our RDR team—a group of dedicated and caring professionals who work tirelessly to ensure that there is little interruption to the myriad of research studies being conducted at our site. And the biggest “Thank you” goes to all of our patients and their families! **You are our inspiration** and the very reason that we are here! Despite the pandemic, you have continued to travel (many from across the country) to RDR to participate in all study-related procedures and assessments. Your commitment to participate in these studies provides hope for the rare disease community—not just here, but all over the globe. **Again, thank you from the bottom of our hearts, and cheers to a better 2021!**

RARE DISEASE DAY 2021!

This year, **Rare Disease Day will be on February 28, 2021.** This day represents a concerted effort from various organizations to raise awareness for the rare disease communities from all around the world. Please join us in advocating for rare disease patients and their families through participating in the following online events:

Georgia Rare Disease Day

The Georgia Rare Disease Day Virtual Event will occur on **Friday, February 26, 2021.** You can register for the event with this link: <https://rareaction.org/resources-for-advocates/state-profiles/georgia/>. For patients who are from out-of-state, please refer to the Rare Action Network in your state to participate in this important mission. Please visit <https://rareaction.org/resources-for-advocates/state-profiles/> for more information.

Jett Foundation 4th Annual Rare Disease Day Celebration

This year, the Jett Foundation's Rare Disease Day event on Friday, February 26, 2021 will feature community ambassadors, Duchenne medical professionals, rare disease researchers and others who will share personal stories about the challenges they have experienced with their work due to the coronavirus pandemic.

Look at who is the guest speaker for this special event! Our very own Dr. Phan!
You can register for the event at: <https://www.jettfoundation.org/rare-disease-day>.

FDA Rare Disease Day 2021

The FDA also plans to have a public meeting in recognition of the event__ on March 5, 2021. There will be various presentations and interactive panel discussions surrounding rare disease product development. The meeting will focus on rare disease partnerships and collaborations, scientific advancements, patient involvement, and strategies to support rare disease product development during COVID-19.

You can learn more about this meeting at <https://www.fda.gov/news-events/fda-meetings-conferences-and-workshops/public-meeting-fda-rare-disease-day-2021-03052021-03052021#Registration>. Registration for the meeting is through a third-party vendor at: <https://www.surveymonkey.com/r/FDARD2021>.



WELCOMING NEW STAFF

This month, RDR is pleased to introduce you to our newest clinical research coordinator, **Brittany Danielly**. Brittany came to us from the Winship Cancer Institute of Emory University where she was a coordinator for 1 ½ years. She completed her undergraduate study at Johnson C. Smith University in Charlotte, NC, and is currently pursuing a Master's in Public Health and Business Administration through Benedictine University. Brittany enjoys working with patients and their families. She also has extensive experience in working with the pathology lab at Winship. Please join us in welcoming Brittany. We are very excited to have her on our team!



POLICY ISSUES IMPACTING THE RARE DISEASE COMMUNITY



Since its establishment in 1983, the National Organization for Rare Disorders (NORD) has advocated for policy changes to improve the lives of Americans impacted by rare diseases at the federal and state levels. This year, NORD has identified the following topics as critically pertinent to the rare disease community:

- **Access to affordable, adequate coverage:** Rare disease patients need access to affordable, adequate, and accessible health care coverage to maintain their health
- **Access to Affordable Medicines:** More than 95% of rare disease patients lack an FDA approved treatment for their condition. Fortunately, scientific innovation continues and more treatments for rare diseases are becoming available. However, the high cost of some of these drugs can sometimes hinder access for rare disease patients. Addressing the affordability of existing and new drugs is critical to ensuring patients can access medically necessary treatments.
- **Access to Diagnostics:** many rare disease patients face years of difficult "diagnostic odyssey" before receiving their correct diagnosis. Obtaining an early and accurate diagnosis is critical for allowing patients to seek effective treatment sooner and preventing morbidity and mortality.
- **Access to Innovative Medicines and Therapies:** The rare disease community faces disparate challenges that require unique considerations throughout the drug and medical device development processes to ensure timely access to innovative treatment.
- **Advancing Rare Disease Research and Regulatory Science:** The vast majority of rare diseases have no FDA-approved treatment and hurdles in the development of these therapies are many. It is critical that the research and regulatory environment helps to address these barriers and foster a successful bench to bedside pathway for rare disease treatments.
- **COVID-19:** The pandemic has had a significant impact on nearly all aspects of our lives, particularly for the rare disease community. NORD continues to work in support of policies to help mitigate the risk of contracting COVID-19 and ensure patients have continued access to vital health care services.
- **Rare Disease Advisory Councils (RDACs):** RDAC acts as an advisory body that gives the rare community a stronger voice in state government and makes recommendations as to how their state's programs and policies should be
- **Telehealth:** Telehealth allows patients and physicians to exchange health information without being in the same room. While historically only available to patients living in the rural areas, the COVID-19 pandemic has significantly expanded the utilization of telehealth services. Both the federal government and states have the power to regulate aspects of telehealth, including telehealth licensure requirements, reimbursement rates and eligible services.

(Retrieved from <https://rarediseases.org/advocate/policy-priorities/policy-issues/> on February 1st, 2021)

RARE Staff Development

The weekly Lunch-and-Learn continues with more interesting topics to help our team operate better and hopefully gain more satisfaction from our day-to-day clinical work as well as personal growth. Here are a few sessions that you may be interested in:

- An Introduction to the Clinical Study Start-Up Process
- Maintaining the Investigator Site File (ISF) after Site Activation
- How to Maintain the Pharmacy Binder for a Study
- Neuroplasticity and How to Maximize Yours And many more...

All in a RARE Day's Work

It was an end-of-day huddle at RDR and we had a guest. We went around and each staff introduced themselves. It was our quietest team member's turn, and this was what he said:

"Hi, I'm Nicholas, and I'm the heartbeat of the organization!"

Needless to say, everyone after Nick claimed a different vital organ!

Yeah, you know who you are... :))

Just to see if you were paying attention...

Do you remember who among us said this? "I'm starting out the new year in the positive, positive?" The winner(s) will receive lunch courtesy of Trang. :)

Editors-in-Chief

The content and design of this newsletter are produced by our very own **Trang Vo and Genevieve Wilson**.

If you have any ideas you would like to see implemented, please let us know!

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