FEDERAL RIGHT TO TRY ACT—AN ALTERNATE PATHWAY FOR US PATIENTS WITH TERMINAL ILLNESS TO ACCESS EXPERIMENTAL THERAPIES

Once a drug enters clinical trials, it takes an average of 10 years to achieve market approval, and over 90% of drugs entering Phase I clinical trials fail to obtain approval. For patients with life-threatening or severely debilitating disease and limited treatment options, the right to access investigational drugs prior to approval has become a priority. US physicians have for decades used the Food and Drug Administration (FDA) Expand ed Access (EA) programs as the main method by which patients can request access to investigational products outside of participating in a clinical trial. The EA pathway requires physicians to get prior permission from the U.S. Food and Drug Administration (FDA) and, except in emergency cases, institutional review board (IRB) approval.

On May 30, 2018, the Right-To-Try (RTT) bill, allowing the use of experimental, non-FDA approved drugs as a last resort for those unable to participate in clinical testing who have also exhausted all other treatment options, was signed into law. At the time this bill was signed, 38 states had already passed similar RTT laws. Now, this law takes effect nation-wide.

The requirements for granting approval of experimental drug treatment under RTT laws are:

• a terminally ill patient has exhausted all other treatment options and is ineligible to participate in clinical trials,
• the experimental drug passes FDA phase I clinical testing,
• the patient’s health-care provider or treating physician must recommend and approve the experimental treatment,
• the pharmaceutical manufacturer must approve the drug for use as an experimental treatment, and
• patient’s written informed consent is required.

RTT offers an alternative pathway for patients who meet the above criteria the ability to access experimental drugs. However, patients may be charged the direct costs of making the investigational drug available for their use. The federal RTT does incentivize sponsors and manufacturers to make their investigational drugs available by protecting them against liability with respect to acts and omissions regarding the investigational drug. The Act also protects prescribers, dispensers, and other individuals from liability, unless the act or omission constitutes reckless or willful misconduct, gross negligence, or an international tort under state law. Finally, the Act expressly protects against liability to any person for not providing access to an investigational drug under the Act.
Federal Right to Try Act—continued

The RTT act does require sponsors and manufacturers who have made their investigational drugs, available under RTT to annually report to the FDA the number of doses supplied, the number of patients treated, the uses for which the drug was made available, and any known serious adverse events. In turn, the FDA must make this information publicly available on its website.

The federal law states that the patient’s physician must be in good standing with the licensing organization or board. The physician cannot be compensated directly by the sponsor or manufacturer for certifying a patient is eligible for right to try. The law also removes any physician liability for helping the patient gain access, except in situations of serious misconduct. While the RTT Act requires that a physician must receive “written informed consent regarding the eligible investigational drug,” no guidance has been given on the content of such informed consent or what other regulations might impact the consent process.

For patients or physicians considering which pathway to choose for accessing an experimental drug the first step is to start by contacting the sponsor/manufacturer to see if they will provide the investigational product. The company may prefer one pathway over the other; if so, this simplifies the decision. There is nothing in the law that requires patients and physicians choose EA over RTT or vice versa. However, it may be a good idea to choose one pathway and stick with it. Attempting both approaches simultaneously could create confusion as to which requirements and responsibilities apply and slow down the process overall. For sponsors and manufacturers, it is recommended to establish and publish policies regarding EA and RTT.

Critics worry that RTT puts vulnerable and desperate patients at risk by removing FDA oversight of drug access, and by limiting liability of drug manufacturers. Pharmaceutical companies face significant challenges in developing fair processes for patients seeking early access that do not expose vulnerable patients to potential harms, and preserves the sponsor’s ability to carry out clinical trials. Whether RTT efforts will substantially impact drug availability, manufacturer willingness to release drugs for compassionate use or the timeframe in which such requests are met, remains to be seen.

NEWLY AWARDED STUDIES:

- Hemophilia B – Seroprevalence Study
- Immune-Mediated Necrotizing Myopathy – Phase II
- Myasthenia Gravis – Phase III

MEETING HIGHLIGHTS

WORLD RARE DISEASE DAY @PRA

Each year the global community recognizes the last day of February as World Rare Disease Day—a day that drug developers, researchers, clinicians, patients, advocates, and caregivers raise awareness of rare diseases, in addition to celebrating progress and highlighting the challenges we have not yet solved. On February 28, 2019 the PRA Center for Rare Diseases hosted a World Rare Disease Day symposium in Blue Bell, PA.

The event included six patient advocates and two industry partners, all veterans in successful collaborations serving rare disease patient communities. Each speaker had the opportunity to talk about their journey and their respective organization’s role in the rare disease drug development ecosystem, and had the opportunity to map a path to a strengthened and more fruitful collaboration between stakeholders going forward.

Please visit http://my.prahs.com/RareDay19/ to see images and video from World Rare Disease Day—a list of speakers is on page 3.
WORLD RARE DISEASES DAY SPEAKERS INCLUDED:

**EMILY KRAMER-GOLINKOFF**  
Co-founder of Emily’s Entourage  
Emily charged the room with continuing to create awareness for rare diseases, especially for such “invisible” diseases as cystic fibrosis and for the many disease sub-types that still have no treatment available. She illuminated the incredible research being done and the involvement of cystic fibrosis patients throughout the process.

**KYLE BRYANT**  
Founder & Director of rideAtaxia and Spokesperson for Friedreich’s Ataxia Research Alliance (FARA)  
Kyle, a patient with Friedreich’s Ataxia, founded rideAtaxia to support FARA’s research efforts. In 2007, Kyle and his father rode their bicycles from San Diego, CA to the National Ataxia Foundation annual meeting in Memphis, TN. Kyle and his father raised over $800,000 during this initial ride and continue to raise funds and inspire other patients by hosting rides across the United States. He recently authored a book entitled *Shifting into High Gear: One Man’s Grave Diagnosis and the Epic Bike Ride That Taught Him What Matters*, that aims to reframe the language of disease through action and service.

**ALLISON MOORE**  
Founder & CEO of the Hereditary Neuropathy Foundation  
Allison shared insights with the room about how her organization collects patient data through a registry and shares that information to drug developers as needed. She stressed the important to other patient advocacy groups to make a concerted effort to collect and truly own their data.

**JENS OLTROGGE**  
Senior Director of Commercial Development in Hematology at CSL Behring  
Jens brought an additional and valuable voice as an industry partner. He showed how there is immeasurable value when drug developers and patients converge. Jens emphasized collaboration amongst industry and patients, noting the great progress that has been made while rooting us in the challenges that still lie ahead. He also illustrated CSL Behring’s commitment to patients and the communities CSL Behring serves through service and community building outside the drug development arena.

**ROB LONG**  
Executive Director of Uplifting Athletes  
Rob shared his diagnostic story through a powerful video and explained how Uplifting Athletes is working with collegiate athletes to bring awareness to rare diseases. Uplifting Athletes hosts a variety of programs to drive this awareness, such as the Young Investigator Draft, which recognizes scientific advancements in rare cancers, rare autoimmune and immunological disorders, rare blood disorders, rare genetic disorders, and rare muscular and neurological disorders.

**SHEELA SITARAMAN DAS**  
Executive Director and Medicine Development Lead at Amicus Therapeutics  
Sheela shared her thoughts from a drug development perspective and she talked about the Amicus model and approach to patient partnered drug development. She also spoke about how Amicus, during the development of a failed rare disease drug, shared data with other competitors to ensure that drug development for Epidermolysis Bullosa continued.

**MICHAEL HUND**  
Executive Director of Epidermolysis Bullosa (EB) Research Partnership  
Michael helped all attendees see the possibility of funding rare disease solutions through Venture Philanthropy. In this approach, the Patient Advocacy Group takes operational cues from the private sector. This strategy funds EB research with seed money, which earns a substantial return on EB research investment, resulting in additional funds that in turn support innovative new research—all part of a virtuous cycle that will end the disease.

**SHEEPA SMEDLEY**  
President for Curing Retinal Blindness Foundation  
Kristin was the final patient speaker for the day. She shared a powerful video detailing the painful journey of her son being diagnosed with a rare disease. Receiving a diagnosis for a rare disease is an extremely emotional experience, and Kristin detailed her memories and emotions after her son was diagnosed and the events that led her to shift her perspective and become an advocate.
MEETING HIGHLIGHTS

UPLIFTING ATHLETES

Recognizing that the challenges faced by the Rare Disease Community are bigger than any one individual, team or organization can tackle alone, Uplifting Athletes takes pride in providing opportunities for college football student-athletes and NFL players to use the platform they are afforded to shine a spotlight on rare diseases and support the cause. The Young Investigator Draft is the result of Uplifting Athletes’ ongoing commitment to cultivate resources that accelerate scientific advancements for rare disease treatments and potential cures while facilitating the next generation of rare disease researchers.

PRA was thrilled to return to the Second Annual Young Investigator Draft as an All-American sponsor this past March. Similar to the NFL Draft where teams select the top young prospects they feel can make the most impact on their future, at the Young Investigator Draft, Uplifting Athletes celebrated its 2019 draft class in five different rare disease categories: rare cancers, rare autoimmune and immunological disorders, rare blood disorders, rare genetic disorders, and rare muscular and neurological disorders.

The 2019 Young Investigator Draft class includes Dr. Shana McCormack, Dr. Elizabeth Harrington, Dr. Alberto Japp, Dr. Eugene Hwang, and Dr. Brian Sworder. Young Investigator Draft grants are intended to inspire collaborative and translational research that will benefit the entire Rare Disease Community. The Young Investigator Draft stage provided each doctor a platform to educate and inspire by sharing their research along with its impact on the Rare Disease Community. Several of the grant recipients said the process of being drafted was a “first” for them and despite being briefed and told how the evening would unfold, they said there were no words could fully prepare them for the energy and excitement the evening created.

In addition to the grants awarded to Young Investigator Draftees, Uplifting Athletes also awarded Dr. Brenda Gallie of Children’s Hospital in Toronto the Young Investigator Draft Collaborative Leadership Award for her decades of commitment to eradicating the rare eye disease retinoblastoma. She was introduced via video by former USC long snapper and 2016 Uplifting Athletes Rare Disease Champion Award winner Jake Olson, who is legally blind after losing both eyes to retinoblastoma.

Congratulations to all the recipients. PRA applauds Uplifting Athletes for enabling your continued efforts on behalf of rare disease patients around the globe!
MEETING HIGHLIGHTS

PATIENTS AS PARTNERS

Seven years ago, Greg Simon, who was the Head of Policy at Pfizer at the time and is now President of the Biden Cancer Initiative, asked our audience of peers when was the last time they thanked patients for being in a clinical trial. When no one raised a hand, he saw an urgent need to reshape the way drug developers and clinical trial participants engage. Thus, the Patients as Partners Conference series was initiated! Now in its sixth year, the Patients as Partners team has been guided by a dedicated group of advisors from pharma and biotech, patient advocacy, and the FDA whom meet quarterly to develop the conference agenda. This event focuses on demonstrating how to involve patients throughout the entire development life cycle of new treatments, driving greater efficiencies in clinical research.

PRA Health Sciences shares this mission and was proud to be an Executive Sponsor of the 2019 Patients as Partners conference March 11–12 in Philadelphia this year. The Center for Rare Diseases is grateful for the opportunity to connect and reconnect with our partners across the drug development landscape, and the opportunity to work together to reshape the clinical trial process to meet the needs of everyone at the table.

WORLD ORPHAN DRUG CONGRESS

PRA colleagues from the Center for Rare Diseases, Center for Pediatric Clinical Development, Marketing, and Business Development joined orphan drug stakeholders from around the globe to attend the World Orphan Drug Congress US at the Gaylord National Convention Center near Washington, DC on April 10-12, 2019. This meeting is designed to foster collaboration, enhance strategy, advance patient advocacy, and strengthen partnership in the global rare disease global research community. One of the many benefits of this international gathering is the opportunity to create a forum for patient advocacy groups to help shape drug development efforts. Amy was joined by Kendall to chair a roundtable discussion among drug developers and patient advocates on the topic of “Patient-focused research – new approaches to bring clinical trials directly to patients and change the patient-centric trial paradigm in rare diseases”; Jessica joined Amy to chair a second roundtable on the topic of “Gene therapy development – challenges, issues, and solutions from the new frontier of rare disease therapies”. Both conversations served as useful forums for developers and advocates to share best practices and brainstorm solutions to challenges unique to orphan drug development. The PRA booth was a hub of activity, and conversations continued during a lively client reception held in a private lounge featuring breathtaking views of the National Harbor, hosted by PRA. We were delighted to connect and reconnect with partners new and old, from all corners of drug development and patient advocacy!
IN THE PIPELINE:

- Pemphigus – Phase IB
- Amyotrophic Lateral Sclerosis – Phase I/II
- Batten Disease – Phase I/II
- Limb-Girdle Muscular Dystrophy – Phase I/II
- Mucopolysaccharidosis – Phase I/II
- Prader Willi Syndrome – Phase I/II
- Gaucher Disease – Phase I/II
- Achondroplasia – Phase I/II
- Beta-thalassemia – Phase II
- PKU – Phase II
- Pompe Disease – Phase III
- Hemophilia B – Phase III
- Cystic Fibrosis – Phase III
- Rare Genetic Epilepsy – Phase III

CONTACT INFO

If you need assistance with a rare disease study, have a personal interest in rare diseases, or would like more information, please contact us at PRACenterForRareDiseases@prahs.com or individually:

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