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By electronic mail

Re: Public Comment - Advancing Rare Disease Therapies Through a Food and Drug Administration Rare Disease Innovation Hub

Dear Ms. Goldberg and Dr. Rothblum-Oviatt,

The Goldwater Institute (GI or Institute) is grateful for the opportunity to provide comments on the establishment of the Food and Drug Administration (FDA or Agency) Rare Disease Innovation Hub.

GI was established in 1988 as a nonpartisan public policy and research foundation devoted to advancing the principles of limited government, individual freedom, and constitutional protection through litigation, policy research and legislative advocacy. Among the goals GI seeks to promote are individual liberty; patient autonomy; and the simplification, reduction, or elimination of barriers that federal and state governments erect against patient access to medical care.

Partnering with patients and doctors nationwide, the Institute drafted Right to Try legislation to protect the patients' right to access potentially lifesaving treatments. In a few short years, 41 states adopted Right to Try before it became federal law in 2018. Right to Try has given Americans with life-threatening illnesses new hope by allowing them to seek potentially life-saving treatments that were previously offered only to a few patients through clinical trials. As follow-up to that success, Goldwater again worked with patient advocates, medical professionals, and innovators to create the Right to Try for Individualized Treatments Act, which builds upon Right to Try by protecting rare and ultra-rare disease patients' right to access innovative, cutting-edge treatments that are specific to their genetic profile.

Both initiatives are rooted in the Institute's firm belief that patient autonomy is fundamental to individual liberty, as well as a bedrock principle of medical ethics. Respectfully, the Institute also shares the opinion that the Agency's current drug approval process is outdated and in disrepair, especially as it applies to the next generation of individualized medical treatments, because Congress has not significantly modified that process in over six decades. Thus, the current means for drug and device approval has not evolved contemporaneously with the rapid advancements in medical technology and innovation occurring over the past six decades. GI believes that this results in a system that is excessively paternalistic and at times seems obstructionist.

With that in mind, GI urges the FDA to use the Innovation Hub as a mechanism to meaningfully modernize and streamline the processes for making the products of advancements in medical innovation and technology, (specifically novel medications and devices), more readily available and with alacrity, to rare disease patients. GI believes FDA's plan for the Rare Disease Innovation Hub could well achieve its stated goal of enhanced collaboration and cooperation between the Center for Biologics Evaluation (CBER) and Research and the Center for Drug Evaluation and Research (CDER). However, we are also concerned that it could just as easily serve as a mechanism to further thicken the bureaucratic morass that encumbers patients with rare diseases from benefitting from novel treatments and advancements in care.

To enhance the prospect for achieving its stated goals of aligning review efforts, identifying innovative approaches to endpoints and enhancing communications with the rare disease community, the Institute advocates that the Rare Disease Innovation Hub Steering Committee specifically include representatives from the rare disease patient community and appropriate key stakeholders from industry who are attempting to revolutionize care for those with rare diseases. Incorporating patient representatives and industry will keep priority focus on patients and on the efforts of those who seek to provide treatments with the potential for valuable impact.

As the Institute documented in its advocacy efforts leading to the successful passage of Right to Try, time is of the essence for many patients with rare diseases.[1] In point of fact, some rare disease patients have little time for life remaining and eagerly seek therapeutic interventions that hold potential to positively impact their affliction. The right to advocate for possible means of saving one's own life is deeply rooted in our nation's history and tradition. That right is also among those crucial rights that are protected by due process principles, and thereby implicit to our concept of ordered liberty. Accordingly, it is imperative that the Innovation Hub include this critical voice so that it might be continuously updated and reminded of the downstream impacts of FDA's process, procedures, and actions.

The Institute believes that the Innovation Hub's Steering Committee should also include appropriate stakeholders who are leaders at the forefront of developing unique products to bring to market benefitting rare disease patients. The inclusion of these stakeholders will, at the very

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[1] https://arizonastatelawjournal.org/wp-content/uploads/2017/07/Sandefur_Final.pdf; See also https://righttotry.org/right-to-try-is-working/

least, facilitate more effective interaction between FDA and the innovators by providing a means for more direct communication. It is hoped that such will improve the efficiency of the processes and procedures mandated by FDA to make their novel products available.

Admittedly, the broadening of the roster to include representatives from patient interest groups and industry is not a suggestion of seismic proportion. That said, any small step with potential to enhance the efficiency of FDA's process that is so easily implemented should be pursued in the interest of improving the efficiency and keenness of the current process.

Lastly, GI advocates that the FDA utilize the Rare Disease Innovation Hub as a means by which to explore, initiate and implement rational processes that simply get out of the way of the trailblazing innovators who have dedicated their efforts to providing hope and relieving the suffering of patients with rare diseases. Federal regulations and superfluous layers of bureaucracy have slowed medical innovations for decades now. For rare and ultra-rare disease patients to benefit from the next generation of medicine, the federal government must develop a new and more responsive "lighter touch" regulatory approach toward groundbreaking individualized treatments. A paradigm shift in medicine deserves a paradigm shift in regulation. The FDA should use this opportunity to solidify the United States as the leader in treating rare diseases by simply giving market innovators room to do what they do best: solve problems.

Again, GI thanks the Agency for the opportunity to express its opinions and concerns. We sincerely hope that your efforts will be successful and would be pleased to serve as a resource for the FDA at any time.

Respectfully,

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