



Rare Disease Patients Need Access To Therapy Innovations

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One lesson we've learned from Covid-19 is that if innovative medical solutions, therapies and vaccines are available, accessible and affordable, then hundreds of millions of lives can be saved.

At one point in our not too distant history, it would have been unthinkable that science would allow us to find a way to alter cells to fight rare diseases. Yet, in 2021 these therapies and medical advancements are being developed and utilized to provide rare disease patients with better health management which can range from conducting the simple activities of life to prolonging their lives.

As we've most recently learned from our battle against Covid-19, innovation is critical in the fight against the disease. However, what is the value of that innovation if it doesn't reach those who need it most?

In the case of Rare Diseases, patients and their families face overwhelming challenges, waiting for a therapy to be discovered, to navigate clinical trials, to overcome potential failures and to be approved for their specific rare disease. Perhaps even more difficult is the reality a therapy may already be available, but the ability to access that therapy is beyond reach for a patient and their family.

In 1983, President Ronald Reagan and Congress enacted the Orphan Drug Act. According to the FDA, only 10 medical treatments for rare diseases were introduced in the entire decade before the Orphan Drug Act's enactment. Since the implementation of the Orphan Drug Act, therapies for over 900 rare disease indications have been approved, with over 25% of the approvals occurring in the last three years.

Without question, these therapies have brought hope to millions. But discovery is only one step in the patient's journey. It is imperative that patient access keeps pace with these breakthrough therapies. To date, that has not been the case and many rare disease patients, such as Leitha Brogan, have needlessly suffered financial, emotional and physical hardships.

Leitha suffers from homozygous familial hypercholesterolemia, or HOFH, an incredibly rare genetic disease that affects approximately 500 people in the entire country. HOFH causes uncontrolled production of bad cholesterol in a patient's blood that can lead to severe heart disease. For years Leitha tried everything but her condition did not improve.

That was until Leitha found a doctor familiar with the rare disease she suffers from and prescribed an effective therapy. A single pill that had dramatic positive results and was covered by her private insurance. Unfortunately, when Leitha's health insurance became unaffordable she had to go on Medicare.

Under Medicare, Leitha is required to cover 5% of the total cost of the drug when she reached the catastrophic portion of the benefit. For Leitha, living on a fixed income, the cost of this therapy that is used by less than 500 people was beyond her means. While a pharmaceutical manufacturer may be willing to assist Leitha with these payments, regulations governing Medicare Part D do not allow it.

Leitha's doctor offered her an alternative treatment known as apheresis. Along with her husband, who had to take time off from work, they traveled six hours every two weeks to receive this treatment. To support apheresis, Leitha endured a series of fistula surgeries to build up her blood vessels. Unlike the pill she was able to take at home, this new treatment requires a machine to administer the therapy, which Leitha is severely allergic to.

During one particular treatment, Leitha had an anaphylactic reaction to the procedure. As Leitha recounts, "I'm lying there thinking, while they're pumping me full of steroids and starting me on oxygen, I had chest pain and they gave me nitro glycerin. And I'm lying there thinking, isn't that great, that there is a pill, halfway across the country, that had been being shipped to me every month, and I was fine. And now I'm laying here, and I'm not fine. Nothing about this is fine."

Leitha's story is not rare like her disease. Millions of rare disease patients are denied the therapies and treatments that can help manage their disease due to financial hardship or lack of patient access through their health coverage. But there is a solution. Instituting a cap on patient out-of-pocket costs in Medicare Part D is long overdue, as well as addressing the culture of delay in other programs covering these treatments. Congress and the Administration can solve these issues without onerous price controls that will drive investment from rare diseases.

On this Rare Disease Day, it is finally time to realize that an innovation for a rare disease without patient access is no solution at all.