

Applying Operation Warp Speed to ultra rare diseases

The last day of February is International "Rare Disease Day." In the United States, a rare disease is defined as one that affects fewer than [200,000 people](#) nationwide. An estimated [25-30 million people in the United States](#) and [over 300 million worldwide](#) have a rare disease. So, rare diseases aren't really all that rare. Of the over 7,000 rare diseases that have been identified, [95 percent](#) have no treatment.

Imagine being told that your child has been diagnosed with a rare disease going through months and often years of testing, just to find out there are no known cures. It is a crisis for the families watching their children suffer. And, it's a global humanitarian question we should all care about.

The Food and Drug Administration (FDA) provides scientific oversight and regulatory advice to researchers seeking to bring vaccines, treatments, and cures to the medical community and, ultimately, to patients. The FDA is known for rigid adherence to process to ensure safe drugs are brought to patient communities, but it has also shown throughout its history that emergent issues require certain actions in which they can streamline regulatory processes to move faster.

This flexibility with which the FDA assesses and advances regulatory process changes was exemplified recently when the agency employed Emergency Use Authorization to accelerate the development and availability of vaccines in the face of the COVID-19 pandemic. This led to the fastest approval of vaccines in history.

Operation Warp Speed brought together government leaders, international agencies, nonprofits, and pharmaceutical companies to coordinate a plan to speed up the creation of the vaccines vital to addressing this global

emergency. Regulatory controls were modified to ensure faster development and distribution because our government knew that people would continue to die without an effective vaccine.

So, why could this expedited process not be deployed for other urgent medical issues? And, what constitutes an emergency?

While many of us focus on the hopefulness brought through the speed by which vaccine development has shown the potential to stem a pandemic that has threatened our way of life in immeasurable ways, parents of children with rare degenerative diseases live with this life-ending threat and critical sense of urgency daily. Every day, they wake up with the fear that their child may die if they are unable to access necessary medical support. Every day, their children battle to eat or sit up or even breathe on their own. Every day, more time slips away.

It would make sense for regulatory agencies to apply lessons learned from Operation Warp Speed to accelerate access to genetic research and treatments for ultra-rare diseases. Regulatory agencies could become catalysts to support access to lifesaving genetic medicine despite how rare the disease is — in fact, because of it.

- [Fauci donates personal COVID-19 virus model to Smithsonian](#)
- [DC Guard chief: Approval to deploy on Jan. 6 took more than 3 hours](#)

There is an urgent need, arguably an emergency, for ultra-rare disease research and therapies to advance quicker and save the lives of children around world. In the year since the last "Rare Disease Day," the FDA has shown the ability to move quickly and nimbly when the will is there, and there is certainly a path when it comes to addressing rare diseases.

Now, we should apply the lessons learned on a macro-level in the rapid creation of the COVID-19 vaccines to the micro-numbers of treatments

required for children born with ultra-rare genetic diseases. These kids deserve our focus.

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