The Goldwater Institute, a nonpartisan public policy foundation dedicated to advancing the principles of limited government, personal responsibility, and individual freedom—including the vital principles of healthcare freedom and medical autonomy—respectfully submits this comment to express concern over the Department of Commerce's investigation into foreign pharmaceuticals, insofar as this investigation may result in a proposal to impose tariffs on imports of pharmaceuticals into the United States.

It is true that American reliance on medicines owned or controlled by authoritarian regimes is risky for patients, leaving them dependent on the whims of foreign dictators. However, imposing tariffs on imported pharmaceuticals will not immediately solve these problems; they are far more likely to harm patients, instead. As Goldwater Institute Visiting Fellow Dr. Jeffrey Singer recently observed, "tariffs on pharmaceuticals won't just impact balance sheets—they will also affect patients. Whether through higher premiums, increased out-of-pocket costs, or fewer new treatments in the future, American health care consumers will bear the burden."[1]

There is one reform the federal government can adopt, however, which will reduce Americans' dependence on foreign medical treatments without imposing additional barriers to patients needing access to treatment, or additional costs on taxpayers or medical providers: Right to Try for Individualized Treatments, also known as "Right to Try 2.0."

Right to Try 2.0 aims to harness American innovation and enable patients to relieve their suffering. The original Right to Try—signed into law in 2018—guaranteed the right of terminally

[1] Jeffrey A. Singer, "Making Medicine Cost More Won't Make America Healthy Again," https://www.cato.org/blog/making-medicine-cost-more-wont-make-america-healthy-again.

ill patients to access medicines that have been approved for safety, but not yet fully approved for sale, by the Food and Drug Administration. That pathbreaking law has enabled countless Americans to receive treatments they need to alleviate their suffering. But today, the latest innovations in medicine are individualized treatments: medicines made specifically for each patient, based on their genetics. These individualized treatments hold immense promise for Americans with devastating diseases. They are especially critical for the 30 million Americans who have a rare disease,[2] as 95% of these diseases have no FDA-approved treatment.[3] But by virtue of the very fact that they are individualized, these treatments by definition cannot go through the FDA's outdated regulatory processes in a timely manner.

To appreciate the promise of Right to Try 2.0, consider the story of Diego Morris. When he was 10, he was diagnosed with Osteosarcoma, a rare form of potentially fatal bone cancer. Diego's family learned of a bone cancer medication, Mifamurtide, that had been approved for use in countries such as Israel, Mexico, and England, but was not yet approved in the United States. There was no way for Diego's family to have the medicine sent to them, either: for Mifamurtide to successfully treat Osteosarcoma, it must be taken immediately after chemotherapy. Thus, because they could not secure government permission to try the treatment

[2] Zebras Among Us: Advocating for the 30 Million Americans Living with Rare Disease, https://pubmed.ncbi.nlm.nih.gov/37886282/#:~:text=Nearly%2030%20million%20(about%201,specialists%20along%20their%20diagnostic%20journey.

[3] National Institutes of Health, Delivering Hope for Rare Diseases, https://ncats.nih.gov/sites/default/files/NCATS_RareDiseasesFactSheet.pdf.

in time, Diego and his family were forced to move to England for the treatment—which was successful.[4]

Diego and his family were fortunate. Every year, countless Americans suffer and die while a slow and expensive bureaucratic process bars their access to treatments that could help them. And most Americans don't have the means to travel to a foreign country to get treatments there. In response to that horrifying reality, the Goldwater Institute worked with patients, doctors, and lawmakers across the country to develop the Right to Try law, which protects terminally ill patients' right to try safe investigational treatments that have been prescribed by their physician and that have passed basic safety screening by the federal government, but that the government has not yet approved for market.

That reform helped desperate Americans receive lifesaving treatments at home. After Texas enacted its Right to Try law, Houston-based oncologist Dr. Ebrahim Delpassand successfully treated hundreds of terminally ill neuroendocrine cancer patients using LU-177 (or Lutetium Dototate), a drug that had completed three phases of the FDA-approved clinical trials, and was widely available in European countries for years, but had not yet received final FDA approval for sale within the U.S., and was thus only available to the small number of Americans who could afford to travel overseas. Many of these patients were only given three to six months to live, but after receiving treatment under Right to Try, they remained alive years later.[5] Dr. Delpassand's patients were exceedingly grateful. One said that without Right to Try, he "would

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[4] *Exploring a Right to Try for Terminally Ill Patients: Hearing before the S. Comm. on Homeland Sec. & Gov't Affairs*, 114th Cong. (2016) (statement of Diego Morris) http://www.hsgac.senate.gov/hearings/exploring-a-right-to-try-for-terminally-ill-patients.

[5] *Exploring a Right to Try for Terminally Ill Patients: Hearing before the S. Comm. on Homeland Sec. & Gov't Affairs*, 114th Cong. (2016) (statement of Dr. Ebrahim Delpassand, Oncologist) http://www.hsgac.senate.gov/hearings/exploring-a-right-to-try-for-terminally-ill-patients.

have had to go on disability to make trips to Switzerland." Another said he "would have traveled to Switzerland for this same treatment and follow-up appointments every three months." But thanks to Right to Try and to Dr. Delplassand, he could stay in the U.S. and spend time with his wife and kids.

In a few short years, forty-one states enacted Right to Try laws before the federal version was signed into law, allowing terminally ill Americans to access investigational treatments that can improve or even save their lives.

But rare disease patients still find themselves in the same boat that others were in before the adoption of Right to Try. Consider the story of Arizona mother Kendra Riley. In 2020, she was devastated to learn that two of her young children had metachromatic leukodystrophy (MLD), a rare progressive disease that attacks the white matter in the brain and then the nervous system. Kendra's middle daughter, Olivia, lost the ability to walk and talk due to MLD. Then came the devastating news that Olivia's sister Keira had the same gene mutation for the disease.

Kendra's doctors had good news and bad news for her. The good news is that there was an individualized gene therapy treatment that could save Keira's life. Like so much of today's innovative tailor-made treatments, this therapy is specifically designed for particular patients—and this holds great promise. The bad news was that the treatment was not available in the United States, where onerous regulations force patients to wait months or even years to access potentially live-saving personalized treatments. Determined to save Keira, Kendra and her family were forced to fundraise half a million dollars to relocate to Italy to secure the treatment. Fortunately, Keira is thriving under the treatment—she's now swimming and attending school. But sadly, her sister Olivia is now in hospice care.

Americans should not have to ask strangers for hundreds of thousands of dollars to travel another country to save their lives—or the lives of their children. The United States rightly prides itself as the capital of medical innovation—as the place where ingenious new treatments are invented that can save and improve our lives and the lives of our loved ones. But bureaucratic red tape is still holding us back. Now, with Right to Try 2.0, our government can reduce dependence on foreign care and help to grow the next generation of medical treatments right here at home, allowing patients suffering from rare and genetic diseases to try personalized treatments—all without raising prices or restricting competition. Unlike imposing costly tariffs, this common-sense measure will boost innovations in medicine and help get treatments to Americans who need them the most.