

## Pfizer's Position on Federal Right to Try Legislation

Pfizer respects the strong desire on the part of patients and families facing life threatening illnesses to access experimental treatments. Pfizer supports policies that protect the integrity of clinical trials and FDA oversight to maintain the best interests of patients, with an emphasis on safety. Clinical trials follow strict scientific standards, and help produce reliable data and results. Wherever possible, use of an investigational medicine within a clinical trial is preferable because clinical trials can generate data that may lead to the approval of products, and consequently, to wider availability. At the same time, Pfizer supports efforts to rapidly provide access to investigational medicines for patients diagnosed with a serious or immediately-life threatening disease or condition, that have exhausted approved treatment options, and who are not eligible for clinical trials. Pfizer is guided by principles of patient safety, expedited medical review, and access to clear information for patients and physicians. Consistent with 21<sup>st</sup> Century Cures Act, passed in 2016 to accelerate the discovery, development and delivery of new cure and treatments, Pfizer catalyzed its efforts to clearly communicate its Compassionate Access policies on [Pfizer.com](https://www.pfizer.com). In 2018, Pfizer Medical received 5,544 requests for pre-approval access to 25 investigative medicines from 62 countries; approximately 98 percent of requests were granted. We supported the launch of the Reagan-Udall Foundation of the FDA Expanded Access Navigator<sup>1</sup> and helped develop physician education tools in collaboration with the Multi Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard.<sup>2</sup>

Therefore, Pfizer's leading policy principle with respect to Federal Right to Try Legislation is to protect the FDA oversight of expanded access, safety, and the integrity of clinical trials. We support PhRMA and BIO efforts to work with the Senate and House sponsors of legislation so that the FDA pathway is preserved, and patient protections afforded by the Federal Food, Drug, and Cosmetics Act, the Code of Federal Regulations, and the Public Health Service Act are maintained and subject to enforcement of the FDA. Pfizer supports protecting companies from liability for granting pre-approval access requests, and clarifying FDA's utilization of expanded outcomes data in the regulatory process.

Pfizer believes that recent FDA efforts to streamline its processes have addressed many of the concerns with past FDA processes that led to initiation of Right to Try legislation at the state level a few years ago. The July 2017 Government Accountability Office analysis found that FDA currently approves 99 percent of all expanded access requests submitted by physicians and companies for patients with immediately life-threatening illnesses who cannot participate in clinical trials and typically responds to emergency single-patient requests within hours and other types of requests within the allotted 30 days.<sup>3</sup> Pfizer applauds the FDA's efforts to streamline the application process; to simplify their website; provide factsheets for patients and physicians; and to provide updated expanded access guidance including the streamlining of IRB review requirements and clarifying its approach to evaluating adverse events arising in pre-approval access settings when making approval decisions for new drugs.

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<sup>1</sup> See: [navigator.reaganudall.org](https://navigator.reaganudall.org).

<sup>2</sup> See: [mrcctcenter.org/wp-content/uploads/2017/12/2017-12-01-Bioethics-Collaborative-Expanded-Access-to-Investigational-Products.pdf](https://mrcctcenter.org/wp-content/uploads/2017/12/2017-12-01-Bioethics-Collaborative-Expanded-Access-to-Investigational-Products.pdf).

<sup>3</sup> See: [www.gao.gov/products/GAO-17-564](https://www.gao.gov/products/GAO-17-564).