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Expanded Access and Right to Try: Access to Investigational Drugs

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SUMMARY

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Hassan Z. Sheikh
Analyst in Health Policy

Expanded Access and Right to Try: Access to Investigational Drugs

The Food and Drug Administration (FDA) regulates the safety and effectiveness of drugs and biological products under its authorities in the Federal Food, Drug and Cosmetic Act (FFDCA) and Public Health Service Act (PHSA). In general, a manufacturer may not sell a drug or biologic in the United States until FDA has reviewed and approved its marketing application (i.e., a new drug application [NDA] or biologics license application [BLA]).

The primary route for an individual to obtain an investigational (i.e., unapproved) drug is to enroll in a clinical trial testing that new drug. However, an individual may be excluded from the clinical trial because its enrollment is limited to patients with particular characteristics (e.g., in a particular stage of a disease, with or without certain other conditions, or in a specified age range), or because the trial has reached its target enrollment number. In certain circumstances, FDA may allow an individual to obtain an investigational drug outside of a clinical trial through its expanded access procedures. Another option, the pathway created by the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017 ("Right to Try Act," P.L. 115-176), does not require FDA permission.

Right to Try Act

The Right to Try Act became federal law on May 30, 2018. Prior to its passage, 40 states had enacted related legislation. The goal of these legislative efforts was to allow individuals with imminently life-threatening diseases or conditions to seek access to investigational drugs directly from the manufacturer without the step of procuring permission from FDA. Another goal—held by the Goldwater Institute, which led the initiative toward state bills, and some of the legislative proponents—was focused more on the process: to eliminate government's role in an individual's choice.

The Right to Try Act offers eligible individuals and their physicians a pathway other than FDA's expanded access procedures to obtain investigational drugs. It defines an *eligible patient* as one who (1) has been diagnosed with a life-threatening disease or condition, (2) has exhausted approved treatment options and is unable to participate in a clinical trial involving the eligible investigational drug (as certified by a physician who meets specified criteria), and (3) has given written informed consent regarding the drug to the treating physician.

It defines an *eligible investigational drug* as an investigational drug (1) for which a Phase 1 clinical trial has been completed, (2) that FDA has not approved or licensed for sale in the United States for any use, (3) that is the subject of an NDA or BLA pending FDA decision or is the subject of an active investigational new drug application and is being studied in a clinical trial that is intended to support the drug's effectiveness, and (4) for which the manufacturer has not discontinued active development or production and for which the FDA has not placed on clinical hold.

The Right to Try Act also has provisions that limit how the Secretary of Health and Human Services (through the FDA) can use data regarding clinical outcomes of patients who get these drugs through this pathway; require a drug's sponsor or manufacturer to report annually to FDA on use of the pathway; and require FDA to post certain annual summaries. Finally, the Right to Try Act states that the sponsor or manufacturer has "no liability" for actions under these provisions. The no-liability provision applies also to a prescriber, dispenser, or "other individual entity" unless there is "reckless or willful misconduct, gross negligence, or an intentional tort."

Before the Right to Try Act was enacted, observers discussed several obstacles to access to investigational drugs through FDA's expanded access procedures. These included some that were FDA-related: the reportedly difficult process to request FDA permission, concern about FDA use of adverse event data, and the role of FDA as gatekeeper. Some related to why a manufacturer might decline to provide an investigational drug: limited available supply, liability, limited staff and facility resources, and concerns about use of outcomes data. The Right to Try Act directly eliminates some of these concerns, addresses some others, and leaves others unaddressed.

Opponents of the law have expressed concern about the erosion of protections for patients who may be exposed to drugs that are unsafe or ineffective. For example, in taking FDA out of the equation, the Right to Try Act limits the agency's ability to make suggestions to the protocols under which investigational drugs are provided, potentially compromising patient safety.

Congressional Considerations

While the Right to Try Act aimed to remove certain perceived obstacles to obtaining investigational drugs, unknowns remain regarding its impact on patients, drug manufacturers, and FDA. These unknowns include (1) whether more patients have received investigational drugs than prior to the law's enactment, (2) whether manufacturers are granting more requests for investigational drugs under the Right to Try Act pathway than previously under expanded access, and (3) FDA's role in implementing certain Right to Try Act requirements when the purpose of the law was to remove FDA from the situation. Congress may consider whether the law has had the effect its sponsors intended or whether legislative changes are necessary.

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Introduction

The Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017 ("Right to Try Act," P.L. 115-176) became federal law on May 30, 2018. Prior to its passage, 40 states had enacted related legislation. The law's goal was to allow individuals with imminently life-threatening diseases or conditions to seek access to investigational drugs without the step of procuring permission from the Food and Drug Administration (FDA). Another goal—held by the Goldwater Institute, which led the initiative toward state bills, and some of the legislative proponents—was focused more on the process: to eliminate government's role in an individual's choice.[1]

The effort to publicize the issue and press for a federal solution involved highlighting the poignant situations of individuals who sought access. For example, in March 2014, millions of Americans heard about the plight of a seven-year-old boy with cancer. He was battling an infection following a bone marrow transplant that no antibiotic had been able to treat.[2] His physicians thought an experimental antiviral drug might help. Because FDA had not yet approved that experimental drug, it was not available in pharmacies. FDA did have the authority to permit the use of an unapproved drug in certain circumstances—a process referred to as *expanded access*. For FDA to grant that permission, however, the manufacturer must have agreed to provide the drug. The manufacturer, which was still testing the drug, declined. Other stories often pointed toward FDA as an obstacle.

During this time, certain groups—for example, the Goldwater Institute—encouraged Congress to act on right-to-try legislation (i.e., legislation that would allow patients to access investigational drugs without FDA permission). The institute framed the issue as one of individual freedom and circulated model legislation.[3] After 33 states[4] enacted legislation reflecting the Goldwater Institute-provided model bill, in January 2017, some Members of Congress introduced a bill to try to address the issue. The Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017—named for several individuals facing amyotrophic lateral sclerosis (ALS, Lou Gehrig's disease) or Duchenne muscular dystrophy—sought to remove what proponents saw as FDA obstacles to patient access. On May 30, 2018, President Trump signed the bill into law (P.L. 115-176).

This report discusses

- how FDA regulates investigational drugs;

[1] Goldwater Institute, "President Trump Signs Right to Try Act into Law," May 30, 2018, https://goldwaterinstitute.org/article/president-trump-signs-right-to-try-act-into-law/. The Goldwater Institute's website describes itself as "a leading free-market public policy research and litigation organization that is dedicated to empowering all Americans to live freer, happier lives … the Institute focuses on advancing the principles of limited government, economic freedom, and individual liberty" (Goldwater Institute, https://goldwaterinstitute.org/about/).

[2] Steve Usdin, "Josh Hardy chronicles: How Chimerix, FDA grappled with providing compassionate access to Josh Hardy," *BioCentury*, March 31, 2014, https://www.biocentury.com/biocentury/regulation/2014-03-31/how-chimerix-fda-grappled-providing-compassionate-access-josh-hardy; Kim Painter, "Drug company changes course, gives drug to sick boy," *USA Today*, March 12, 2014, http://www.usatoday.com/story/news/nation/2014/03/11/chimerix-josh-hardy-drug/6308891/; and David Kroll, "Josh Hardy Going Home After Getting Chimerix Anti-Viral Drug," *Forbes*, July 17, 2014, http://www.forbes.com/sites/davidkroll/2014/07/17/josh-hardy-going-home-after-getting-chimerix-anti-viral-drug/.

[3] Goldwater Institute, "Right to Try Model Legislation," https://goldwaterinstitute.org/wp-content/uploads/cms_page_media/2016/1/5/GoldwaterInstituteRighttoTryModel.pdf.

[4] Starlee Coleman, "Ohio becomes 33rd state to adopt right to try law for terminally ill," Goldwater Institute, January 5, 2017, https://goldwaterinstitute.org/article/ohio-33rd-state-to-adopt-right-to-try-law-terminally-ill/.

- FDA's expanded access procedures and the perceived obstacles to individuals accessing experimental drugs through this mechanism;
- a summary of the provisions in the Right to Try Act and how they are meant to address those obstacles; and
- selected provisions in the Right to Try Act and what questions remain unresolved.

FDA Regulation of Investigational Drugs

The FDA regulates the safety and effectiveness of drugs and biological products ("biologics") under its authorities in the Federal Food, Drug and Cosmetic Act (FFDCA) and Public Health Service Act (PHSA).[5] In general, a manufacturer may not sell a drug or biologic in the United States until FDA has reviewed and approved its marketing application (i.e., a new drug application [NDA] or biologics license application [BLA]). That application for a new drug or biologic must include data from clinical trials as evidence of the product's safety and effectiveness for its stated purpose(s).[6]

After laboratory and animal studies have identified a potential drug or biologic, the sponsor of the clinical trial, usually its manufacturer, may submit an investigational new drug (IND) application to FDA for permission to begin testing the drug in humans.[7] An IND must include information about the proposed study design, chemistry and manufacturing of the drug, and the investigator's qualifications, among other information.[8] The investigator also must provide assurance that an Institutional Review Board (IRB) will provide initial and continuous review and approval of each of the studies in the clinical investigation to ensure that participants are aware of the drug's investigative status and that any risk of harm will be necessary, explained, and minimized.[9] Sponsors of clinical trials also must comply with FDA regulations governing protection of human subjects (e.g., informed consent),[10] adverse event reporting,[11] and charging for investigational new drugs,[12] among other requirements.

FDA has 30 days to review an IND, after which a sponsor may begin clinical testing if the agency has not objected and imposed a clinical hold.[13] In reviewing an IND, FDA's primary objective is to assure the safety and rights of human subjects, and with respect to Phase 2 and 3 trials specifically, to ensure that the quality of the scientific investigations and evaluations is adequate to permit an evaluation of the drug's safety and effectiveness.[14]

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[5] Whereas the FFDCA (§505) authorizes FDA to approve and regulate drugs, the Public Health Service Act (PHSA §351) authorizes FDA to license biological products (e.g., monoclonal antibodies, vaccines). Most FDA procedures regarding drugs also apply to the agency's regulation of biological products.

[6] FFDCA §505(b) [21 U.S.C. §355(b)], PHSA §351(a) [42 U.S.C. §262(a)], 21 C.F.R. §314.50, §601.2. For an overview of the general process of drug approval in the United States, see CRS Report R41983, *How FDA Approves Drugs and Regulates Their Safety and Effectiveness*. See, also, FDA, "How Drugs are Developed and Approved," http://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/default.htm.

[7] FFDCA §505(i) [21 U.S.C. §355(i)], PHSA §351(a)(3) [42 U.S.C. §262(a)(3)], 21 C.F.R. Part 312.

[8] 21 C.F.R. §312.23.

[9] 21 C.F.R. §312.23(a)(1)(iv) and 21 C.F.R. Part 56.

[10] 21 C.F.R. Part 50.

[11] 21 C.F.R. §312.32.

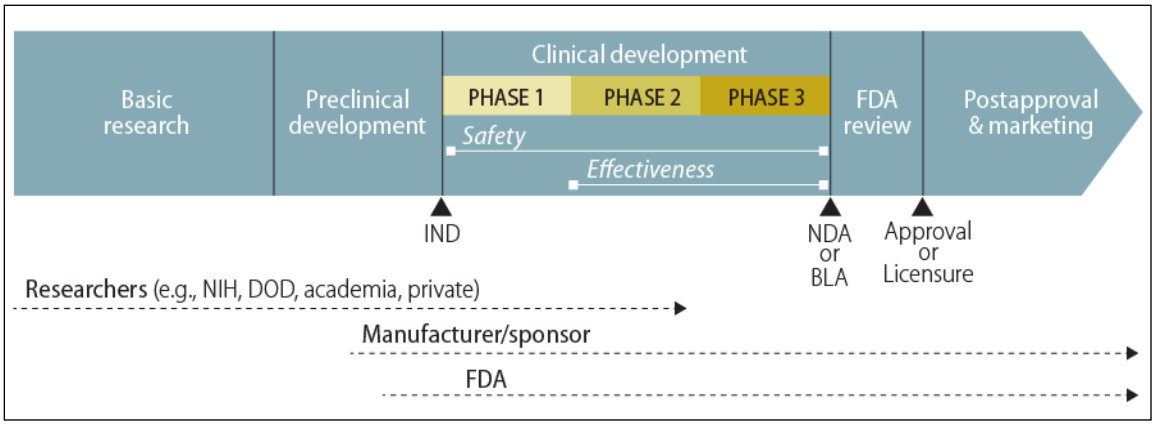
[12] 21 C.F.R. §312.8.

[13] 21 C.F.R. §312.20(c).

[14] 21 C.F.R. §312.22(a).

Once the IND application is approved, the sponsor may then start the first of three major phases of clinical—human—trials. (**Figure 1** illustrates the general path of a pharmaceutical product.) Researchers first test in a small number of human volunteers the *safety* they had previously demonstrated in animals. These trials, called Phase 1 clinical trials, attempt "to determine dosing, document how a drug is metabolized and excreted, and identify acute side effects."[15] If a sponsor considers the product still worthy of investment based on the results of a Phase 1 trial, it continues with Phase 2 and Phase 3 trials. Those trials look for evidence of the product's *effectiveness*—how well it works for individuals with the particular characteristic, condition, or disease of interest.[16] Phase 2 is a first attempt at assessing effectiveness and its experience helps to plan the subsequent Phase 3 clinical trial, which the sponsor designs to be large enough to statistically test for meaningful differences attributable to the drug.

Figure 1. Standard Drug Development Path



Source: Created by CRS.

Notes: The figure does not show the elements of the path to scale.
BLA = biologics license application. DOD = Department of Defense. FDA = Food and Drug Administration. IND = investigational new drug application. NDA = new drug application. NIH = National Institutes of Health.

The primary route for an individual to obtain an investigational drug is to enroll in a clinical trial testing that new drug. However, an individual may be excluded from the clinical trial because its enrollment is limited to patients with particular characteristics (e.g., in a particular stage of a disease, with or without certain other conditions, or in a specified age range), or because the trial has reached its target enrollment number. In certain circumstances, FDA may allow an individual to obtain an investigational drug outside of a clinical trial through its expanded access procedures. Another option, the pathway created by the Right to Try Act, does not require permission from FDA. **Table 1** summarizes selected differences in criteria for access to investigational drugs through participation in clinical trials, expanded access, and right to try.[17]

[15] FDA, "Inside Clinical Trials: Testing Medical Products in People," http://www.fda.gov/drugs/resourcesforyou/consumers/ucm143531.htm.

[16] 21 C.F.R. §312.21(b) & (c).

[17] Under certain emergency circumstances, FDA may issue an emergency use authorization (EUA) to allow the use of an unapproved medical product or the unapproved use of an approved product. The EUA mechanism is beyond the scope of this report but is discussed in other CRS products. See, for example, CRS In Focus IF10745, *Emergency Use Authorization and FDA's Related Authorities*.

Table 1. Access to Investigational Drugs

Clinical Trials, Expanded Access, and Right to Try

	Clinical Trials	Expanded Access	Right to Try
Who is eligible?	Individual who meets the trial's requirements for inclusion and exclusion	Individual must have a serious or *immediately* life-threatening disease or condition, be unable to participate in a clinical trial, and have no comparable therapeutic options	Individual must have a serious or life-threatening disease or condition, be unable to participate in a clinical trial, and have exhausted approved treatment options
When can patients gain access?	May enroll in Phase 1, 2, or 3 trials	During or after Phase 1, 2, or 3 trials	After Phase 1 trials have been completed
Who must provide permission?	FDA, IRB, and drug manufacturer	FDA, IRB, and drug manufacturer	Drug manufacturer
Is informed consent from the individual required?	Yes, in accord with 21 C.F.R. Part 50 "Protection of Human Subjects"	Yes, in accord with 21 C.F.R. Part 50	Yes, but not defined and exempt from 21 C.F.R. Part 50

Source: FFDCA §§561 & 561B, 21 C.F.R. §312.305, FDA, "Expanded Access to Investigational Drugs for Treatment Use—Questions and Answers," Guidance for Industry, June 2016, updated October 2017, https://www.fda.gov/media/85675/download.

Expanded Access and Obstacles

FDA Requirements

The primary purpose of expanded access is to provide investigational drugs as treatment for patients who lack therapeutic alternatives. This is in contrast to clinical trials, which are designed primarily to generate evidence of safety and effectiveness to support approval of an NDA or BLA.[18]

Through FDA's expanded access procedure, a person, acting through a licensed physician, may request access to an investigational drug—through either a new IND or a revised protocol to an existing IND—if[19]

- a *licensed physician* determines (1) the patient has "no comparable or satisfactory alternative therapy available to diagnose, monitor, or treat" the serious disease or condition; and (2) "the probable risk to the person from the investigational drug or investigational device is not greater than the probable risk from the disease or condition";

[18] FDA, "Expanded Access to Investigational Drugs for Treatment Use—Questions and Answers," Guidance for Industry, June 2016, updated October 2017, pp. 2-3, https://www.fda.gov/media/85675/download.

[19] FFDCA §561(b) [21 U.S.C. §360bbb(b)]. See, also, FDA, "Expanded Access: Information for Patients," https://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/ucm20041768.htm. In addition to the individual IND or protocol, regulations describe other categories of expanded use of investigational drugs: intermediate-size patient populations, with one IND or protocol that consolidates several individual access requests, and treatment IND or treatment protocol for "widespread treatment use" when a drug is farther along the clinical trial and marketing application process. See FFDCA §561(c) [21 U.S.C. §360bbb(c)]; and 21 C.F.R. §§312.305, 312.310, 312.315, and 312.320.

- the *Secretary* (FDA, by delegation of authority) determines (1) "that there is sufficient evidence of safety and effectiveness to support the use of the investigational drug" for this person; and (2) "that provision of the investigational drug ... will not interfere with the initiation, conduct, or completion of clinical investigations to support marketing approval"; and
- the *sponsor* of the investigational drug, or clinical investigator, submits to FDA a clinical protocol consistent with the requirements of FFDCA Section 505(i) and related regulations.

FDA makes most expanded access IND and protocol decisions on an individual-case basis. Consistent with the IND process under which the expanded access mechanism falls, it considers the requesting physician as the investigator. The investigator must comply with informed consent and IRB review of the expanded use.[20] The sponsor of the IND must make required safety reports to FDA.[21] FDA may permit a sponsor to charge a patient for the investigational drug, but "only [for] the direct costs of making its investigational drug available"[22] (i.e., not for development costs or profit).

Expanded access could apply outside of the clinical trial arena in these situations:

> (1) use in situations when a drug has been withdrawn for safety reasons, but there exists a patient population for whom the benefits of the withdrawn drug continue to outweigh the risks; (2) use of a similar, but unapproved drug (e.g., foreign-approved drug product) to provide treatment during a drug shortage of the approved drug; (3) use of an approved drug where availability is limited by a risk evaluation and mitigation strategy (REMS) for diagnostic, monitoring, or treatment purposes, by patients who cannot obtain the drug under the REMS; or (4) use for other reasons.[23]

Obstacles to Access

The widespread use of expanded access is limited by an important factor: whether the manufacturer agrees to provide the drug, which—because it is not FDA-approved—cannot be obtained otherwise. FDA does not have the authority to compel a manufacturer to participate. In addition, some manufacturers have expressed concern regarding how FDA would use adverse event data from expanded access when reviewing drug applications. Many highly publicized accounts of specific individuals' struggles with life-threatening conditions and efforts by activists influenced public debate over access. Examples of public attitudes included news accounts of specific individuals' struggles with life-threatening conditions. Some found the process of asking FDA for a treatment IND too cumbersome. Others questioned FDA's right to act as a gatekeeper

[20] 21 C.F.R. §312.305(c)(4).

[21] 21 C.F.R. §312.305(c)(5).

[22] 21 C.F.R. §312.8 and FDA, "Guidance for Industry: Charging for Investigational Drugs Under an IND—Questions and Answers," Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research, June 2016, https://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm351264.pdf.

[23] FDA, "Expanded Access to Investigational Drugs for Treatment Use—Questions and Answers," Guidance for Industry, June 2016, updated October 2017, p. 3, https://www.fda.gov/media/85675/download.

at all.[24] Some pointed to manufacturers' refusal to provide their experimental drugs.[25] Most critics, therefore, see solutions as within the control of FDA or pharmaceutical companies. This section lays out key perceived obstacles and issues—both FDA- and manufacturer-related—with respect to expanded access prior to the enactment of the Right to Try Act.

FDA-Related Issues

Difficult Process to Request FDA Permission

In February 2015, FDA issued draft guidance (finalized in June 2016 and updated in October 2017) on individual patient expanded access applications, acknowledging difficulties with requesting permission for access to investigational drugs from the agency.[26] FDA developed a new form that a physician could use when requesting expanded access for an individual patient. It reduced the amount of information required from the physician by allowing reference (with the sponsor's permission) to the information the sponsor had already submitted to FDA in its IND.[27]

In October 2017, FDA modified its expanded access IRB review policy to allow one IRB member to concur with the treatment use rather than the full IRB.[28] This policy change was made pursuant to a statutory directive that FDA streamline IRB review of individual patient expanded access requests.[29] A September 2019 report published by the Government Accountability Office (GAO) found that the IRB update was helpful for physicians and patients, for example, by reducing the amount of time for patients to obtain access to investigational drugs.[30]

In instances where a patient needs emergency treatment with the investigational product before a physician can submit a written request, FDA can authorize expanded access for an individual patient by phone or email, and the physician or sponsor must agree to submit an IND or protocol

[24] The Abigail Alliance, formed by the father of a young woman with cancer who had unsuccessfully attempted to get an investigational drug, subsequently went to court, claimed "as a fundamental aspect of constitutional due process, the right to choose to take medication of unknown benefit and risk that might potentially be lifesaving" (Linda Greenhouse, "Justices Won't Hear Appeal on Drugs for Terminally Ill," *New York Times*, January 15, 2008, http://www.nytimes.com/2008/01/15/washington/15appeal.html?_r=0). The U.S. Court of Appeals for the District of Columbia Circuit 2007 opinion found "that there is no Constitutional right to access to experimental drugs for terminally ill patients"; in 2008, the Supreme Court declined to consider an appeal (FDA, "Court Decisions, Fiscal Year 2008," http://www.fda.gov/downloads/iceci/enforcementactions/enforcementstory/ucm129820.pdf).

[25] Jonathan J. Darrow, Ameet Sarpatwari, Jerry Avorn, M.D., and Aaron S. Kesselheim, "Practical, Legal, and Ethical Issues in Expanded Access to Investigational Drugs," *New England Journal of Medicine*, January 2015, vol. 372, pp. 279-286.

[26] FDA, "Individual Patient Expanded Access Applications: Form FDA 3926," Guidance for Industry, June 2016, Updated October 2017, p. 4, https://www.fda.gov/media/91160/download.

[27] FDA estimated that it would take a physician about 45 minutes to complete the proposed new form rather than the 8 hours estimated for the original form (or 16 hours when the request was for emergency access) (80 FR 7318). FDA, "Guidance for Industry: Individual Patient Expanded Access Applications: Form FDA 3926."

[28] FDA, "Statement from FDA Commissioner Scott Gottlieb, M.D., on new efforts to strengthen FDA's expanded access program," November 8, 2018, https://www.fda.gov/news-events/press-announcements/statement-fda-commissioner-scott-gottlieb-md-new-efforts-strengthen-fdas-expanded-access-program. FDA, "Expanded Access to Investigational Drugs for Treatment Use—Questions and Answers," Guidance for Industry, p. 6.

[29] P.L. 115-52, §610(b).

[30] GAO, "Investigational Drugs: FDA and Drug Manufacturers Have Ongoing Efforts to Facilitate Access for Some Patients," GAO-19-630, September 2019, pp. 18-19, https://www.gao.gov/assets/710/701243.pdf.

within 15 working days.[31] In such emergency circumstances, treatment with the investigational drug may begin prior to IRB approval, but the IRB must be notified within five working days.[32]

Coincident with discussions preceding passage of the Right to Try Act, FDA had commissioned an independent report on its expanded access program. Citing that report,[33] in November 2018, then-FDA Commissioner Gottlieb announced several actions to improve its program.[34] These included an enhanced webpage to help applicants navigate the application process and establishing an agency-wide Expanded Access Coordinating Committee. In July 2019, FDA launched the Oncology Center of Excellence Project Facilitate, which provides a single point of contact through which FDA oncology staff help physicians through the process of submitting an expanded access request for an individual patient with cancer.[35] According to a 2019 GAO report, officials from one drug manufacturer indicated that Project Facilitate may help reduce the burden on oncologists seeking expanded access to investigational drugs for their patients. However, other officials from the same manufacturer "raised concerns about the potential for FDA to intentionally or unintentionally pressure companies to make their investigational drugs available to patients, should FDA have increased involvement with drug manufacturers as part of the pilot program."[36]

Use of Adverse Event Data from Expanded Access

In October 2017, FDA updated its guidance to address how the agency reviews adverse event data in the expanded access context. In the guidance, FDA explains that reviewers are aware of the context in which adverse event data are generated—for example, that patients who receive a drug through expanded access may have a more advanced stage of the disease than those enrolled in a clinical trial—and evaluate adverse events in that context. The guidance further states that "FDA is not aware of instances in which adverse event information from expanded access has prevented FDA from approving a drug."[37] However, FDA officials have indicated to GAO that "efficacy and safety data from the expanded access program have been used to support drug approvals in several instances."[38] Further, expanded access use may allow for the detection of rare adverse events or may contribute to information about use of the drug in certain populations that are not exposed to the drug in clinical trials.[39] While some drug manufacturers have indicated that they

[31] 21 C.F.R. §312.310(d). FDA "For Physicians: How to Request Single Patient Expanded Access ("Compassionate Use")," https://www.fda.gov/drugs/investigational-new-drug-ind-application/physicians-how-request-single-patient-expanded-access-compassionate-use.

[32] FDA, "Expanded Access to Investigational Drugs for Treatment Use—Questions and Answers," Guidance for Industry, p. 5.

[33] FDA, "Expanded Access Program Report," May 2018, https://www.fda.gov/media/119971/download.

[34] FDA, "Statement from FDA Commissioner Scott Gottlieb, M.D., on new efforts to strengthen FDA's expanded access program," November 8, 2018, https://www.fda.gov/news-events/press-announcements/statement-fda-commissioner-scott-gottlieb-md-new-efforts-strengthen-fdas-expanded-access-program.

[35] FDA, "Project Facilitate," https://www.fda.gov/about-fda/oncology-center-excellence/project-facilitate. GAO, "Investigational Drugs: FDA and Drug Manufacturers Have Ongoing Efforts to Facilitate Access for Some Patients," pp. 18-19.

[36] GAO, "Investigational Drugs: FDA and Drug Manufacturers Have Ongoing Efforts to Facilitate Access for Some Patients," p. 19.

[37] FDA, "Expanded Access to Investigational Drugs for Treatment Use—Questions and Answers," Guidance for Industry, p. 18.

[38] GAO, "Investigational Drugs: FDA and Drug Manufacturers Have Ongoing Efforts to Facilitate Access for Some Patients," p. 22.

[39] FDA, "Expanded Access to Investigational Drugs for Treatment Use—Questions and Answers," Guidance for Industry, p. 18.

view FDA's updated guidance as an improvement, others maintained that they still had significant concerns about adverse event data from expanded access use negatively affecting development of their investigational new drugs.[40]

FDA as Gatekeeper

FDA action is not the final obstacle to access, as the manufacturer still needs to agree to provide their product. Between FY2010 through FY2020, FDA received 16,380 expanded access requests and granted 16,258 (99.3%) of them.[41]

Leading up to passage of the Right to Try Act, in August 2014, a *USA Today* editorial had called the FDA procedures that patients must follow for compassionate use access "bureaucratic absurdity," "daunting," and "fatally flawed." Echoing much of the criticism that FDA had received regarding the issue, it called for one measure that would "cut out the FDA, which now has final say."[42] The solution the editorial proposed involved what proponents term "right to try" laws. By spring 2018, 40 states had passed right to try laws in the absence of federal legislation.[43] The laws varied on the detail required in informed consent and liability issues of the manufacturer and the patient's estate.[44] However, several experts had suggested that this state law approach is unlikely to directly increase patient access.[45] Before passage of the federal Right to Try Act, analysts raised questions about how federal law (the FFDCA), which required FDA approval of such arrangements, might preempt this type of state law.[46] After the enactment of the federal Right to Try Act, some legal analysts had predicted that the issue of federal preemption of state laws would "likely be determined on a case-by-case basis."[47]

Manufacturer-Related Issues

The manufacturer faces a complex decision in determining whether or not to give its experimental drug to a patient who requests it. In making a decision in each case, the manufacturer considers

[40] GAO, "Investigational Drugs: FDA and Drug Manufacturers Have Ongoing Efforts to Facilitate Access for Some Patients," pp. 21-22.

[41] Reports for 2010 through 2020 are at FDA, "Expanded Access INDs and Protocols," https://www.fda.gov/drugs/ind-activity/expanded-access-inds-and-protocols.

[42] The Editorial Board, "FDA vs. right to try: Our view," *USA Today*, August 17, 2014, http://www.usatoday.com/story/opinion/2014/08/17/ebola-drugs-terminally-ill-right-to-try-editorials-debates/14206039/.

[43] National Conference of State Legislatures, "'Right to Try' Experimental Prescription Medicines State Laws and Legislation for 2014-2017," March 7, 2018, http://www.ncsl.org/research/health/state-laws-and-legislation-related-to-biologic-medications-and-substitution-of-biosimilars.aspx#Right_to_Try.

[44] For example: House Bill 14-1281, State of Colorado, Sixty-ninth General Assembly, http://www.leg.state.co.us/clics/clics2014a/csl.nsf/fsbillcont/CE8AAA4FAF92567487257C6F005C8D97?Open&file=1281_enr.pdf; House Bill No. 891, Enrolled, Louisiana, https://www.legis.la.gov/Legis/ViewDocument.aspx?d=902583; Conference Committee Substitute No. 2 for Senate Substitute for House Committee Substitute for House Bill No. 1685, Truly Agreed To and Finally Passed, Missouri, 97th General Assembly, 2014, http://www.house.mo.gov/billtracking/bills141/billpdf/truly/HB1685T.PDF; Public Act Numbers 345 and 346 of 2014, State of Michigan, 97th Legislature, http://www.legislature.mi.gov/(S(gb2onn55vxkuylrvqmn3axrp))/mileg.aspx?page=PublicActs.

[45] Arthur Caplan, "Bioethicist: 'Right to Try' Law More Cruel Than Compassionate," NBC NEWS, May 18, 2014, http://www.nbcnews.com/health/health-news/bioethicist-right-try-law-more-cruel-compassionate-n108686; and David Kroll, "The False Hope Of Colorado's 'Right To Try' Investigational Drug Law," *Forbes*, May 19, 2014, http://www.forbes.com/sites/davidkroll/2014/05/19/the-false-hope-of-colorados-right-to-try-act/.

[46] See, generally, Elizabeth Richardson, "Health Policy Brief: Right-to-Try Laws," *Health Affairs*, March 5, 2015, http://www.healthaffairs.org/healthpolicybriefs/brief.php?brief_id=135.

[47] Phoebe Mounts, Kathleen Sanzo, and Jacqueline Berman, "A Closer Look At New Federal 'Right To Try' Law," Law 360, June 1, 2018, https://www.law360.com/articles/1048871/a-closer-look-at-new-federal-right-to-try-law.

available supply of the drug, liability, safety, and whether adverse event or outcome data will affect FDA's consideration of a new drug application in the future.

Available Supply

If a manufacturer has only a tiny amount of an experimental drug, that paucity may limit distribution, no matter what the manufacturer would like to do.[48] Sponsors of early clinical research make small amounts of experimental products for use in small Phase 1 safety trials, and progressively more for Phase 2 and 3 trials. Although one or two additional patients may not cause supply problems, a manufacturer does not know how many expanded access requests it will receive. Investment in building up to large-scale production usually comes only after reasonable assurance that the product will get FDA approval. For a company to redirect its current manufacturing capacity involves financial, logistic, and public relations decisions.

Liability

In discussing expanded access, some manufacturers have raised liability concerns if patients report injury from the investigational products.[49] Whether these concerns become illustrated by court cases and how any issues may be resolved in future laws are beyond the scope of this discussion.[50]

Limited Staff and Facility Resources

Any energy put into setting up and maintaining an expanded access program could take away from a company's focus on completing clinical trials, preparing an NDA, and launching a product into the market. While this delay would have bottom-line implications, one CEO, in denying expanded access, portrayed the decision as an equity issue, saying, "We held firm to the ethical standard that, were the drug to be made available, it had to be on an equitable basis, and we couldn't do anything to slow down approval that will help the hundreds or thousands of [individuals]." Pointing to ways granting expanded access might divert them from research tasks and postpone approval, he said, "Who are we to make this decision?"[51]

Data for Assessing Safety and Effectiveness

By distributing the drug outside a carefully designed clinical trial, it may be difficult, if not impossible, to collect the data that would validly assess safety and effectiveness. Clinical trials are structured to assess the safety of a drug as well as its effectiveness. The trial design may exclude subjects who are so ill from either the disease or condition for which the drug is being tested or another disease or condition. This allows, among other reasons, the analysis of adverse

[48] GAO, "Investigational Drugs: FDA and Drug Manufacturers Have Ongoing Efforts to Facilitate Access for Some Patients," p. 25.

[49] For example, see Sam Adriance, "Fighting for the 'Right To Try' Unapproved Drugs: Law as Persuasion," *Yale Law Journal Forum*, vol. 124, December 4, 2014, http://www.yalelawjournal.org/forum/right-to-try-unapproved-drugs; Darshak Sanghavi, Meaghan George, and Sara Bencic, "Individual Patient Expanded Access: Developing Principles For A Structural And Regulatory Framework," *Health Affairs Blog*, July 31, 2014, http://healthaffairs.org/blog/2014/07/31/individual-patient-expanded-access-developing-principles-for-a-structural-and-regulatory-framework/; and Elizabeth Richardson, "Health Policy Brief: Right-to-Try Laws," *Health Affairs*, March 5, 2015, http://www.healthaffairs.org/healthpolicybriefs/brief.php?brief_id=135.

[50] CRS Legal Sidebar LSB10115, *Federal "Right-to-Try" Legislation: Legal Considerations*.

[51] Steve Usdin, "Josh Hardy chronicles: How Chimerix, FDA grappled with providing compassionate access to Josh Hardy," BioCentury, March 31, 2014, https://www.biocentury.com/biocentury/regulation/2014-03-31/how-chimerix-fda-grappled-providing-compassionate-access-josh-hardy.

events in the context of the drug and disease of interest. The patients who would seek a drug under a right to try pathway are likely to be very ill and likely to experience serious health events. Those events could be a result of the drug or those events could be unrelated. They would present difficulties both scientific and public relations-wise to the manufacturer. A manufacturer may avoid those risks by choosing to not provide a drug outside a clinical trial.

As mentioned, FDA has indicated that it is not aware of any instances in which safety and effectiveness data obtained from expanded access have prevented approval of a drug, but there are instances in which such data have been used to support approval (see the section "Use of Adverse Event Data from Expanded Access").

Disclosure

It is unclear how many people request and are denied expanded access to experimental drugs by manufacturers. This lack of information makes devising solutions to manufacturer-based obstacles difficult. Although FDA reports the number of requests it receives, manufacturers do not (nor does FDA require them to do so). The number of individuals who approach manufacturers is unknown.

In December 2016, the 21st Century Cures Act amended the FFDCA to require a manufacturer or distributor of an investigational drug intended for a serious disease or condition to make its policies on evaluating and responding to compassionate use requests publicly available.[52] However, the law does not require manufacturers to disclose how many requests they receive, grant, or deny.

A 2019 GAO study surveyed 29 drug manufacturers regarding their policies for individual patient access to investigational drugs.[53] Of those surveyed, 23 reported using their websites to communicate whether they considered individual requests for access to investigational drugs outside of clinical trials; the remaining 6 were in the process of developing this content for their websites. Of those 23 manufacturers, 19 stated they were willing to consider requests, while 4 stated they were not. Of the 19 drug manufacturers willing to consider requests, 13 indicated that they require the relevant regulatory authority to review requests, of which 6 specified that they require FDA to review requests for access in the United States.

The Right to Try Act

On January 24, 2017, Senator Johnson introduced S. 204, the Trickett Wendler Right to Try Act of 2017, and the bill had 43 cosponsors at that time. On August 3, 2017, the Senate Committee on Health, Education, Labor, and Pensions discharged the bill by unanimous consent. The same day, the Senate passed S. 204, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act (P.L. 115-176) with a substantial amendment also by unanimous consent.

On March 13, 2018, Representative Fitzpatrick introduced a related bill, H.R. 5247, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2018, and the bill had 40 cosponsors at that time. On March 21, the House passed the bill (voting 267-149). The House accepted the Senate bill on May 22, 2018 (voting 250-169), and President Trump signed it into law on May 30, 2018.

[52] FFDCA §561A [21 U.S.C. §360bbb-0], as added by P.L. 114-255, §3032.

[53] GAO, "Investigational Drugs: FDA and Drug Manufacturers Have Ongoing Efforts to Facilitate Access for Some Patients," pp. 24-26.

This section of the report first summarizes the provisions in the Right to Try Act. It then discusses how those provisions address some of the obstacles described in the previous section.

Provisions in the Right to Try Act

The Right to Try Act added FFDCA Section 561B, Investigational Drugs for Use by Eligible Patients. It has a separate paragraph that is not linked to an FFDCA section to limit the liability to all entities involved in providing an eligible drug to an eligible patient. It concludes with a "Sense of the Senate" section.

FFDCA Section 561B has several provisions that mirror many steps in FDA's expanded access program. A major difference is that the new section is designed to exist wholly outside the jurisdiction and participation of FDA. These provisions

- define an *eligible patient* as one who (1) has been diagnosed with a life-threatening disease or condition, (2) has exhausted approved treatment options and is unable to participate in a clinical trial involving the eligible investigational drug (as certified by a physician who meets specified criteria), and (3) has given written informed consent regarding the drug to the treating physician;[54]
- define an *eligible investigational drug* as an investigational drug (1) for which a Phase 1 clinical trial has been completed, (2) that FDA has not approved or licensed for sale in the United States for any use, (3) that is the subject of an NDA or BLA pending FDA decision or is the subject of an active IND and is being studied in a clinical trial that is intended to form the primary basis of the drug's effectiveness, and (4) for which the manufacturer has not discontinued active development or production and which the FDA has not placed on clinical hold;[55] and
- exempt use under this section from parts of the FFDCA and FDA regulations regarding misbranding, certain labeling and directions for use, drug approval, investigational new drug regulations, protection of human subjects, and IRBs.[56]

FFDCA Section 561B includes provisions that address use of clinical outcomes and reporting of certain information to FDA. These provisions

- prohibit the Secretary (FDA) from using clinical outcome data related to use under this section "to delay or adversely affect the review or approval of such drug" unless the FDA determines its use is "critical to determining [its] safety," at which time the FDA must provide written notice to the sponsor to include a public health justification, or unless the sponsor requests use of such clinical outcome data;[57]
- require the sponsor to submit an annual summary to FDA to include "the number of doses supplied, the number of patients treated, the uses for which the drug was made available, and any known serious adverse events";[58] and

[54] FFDCA §561B(a)(1) [21 U.S.C. §360bbb-0a(a)(1)].

[55] FFDCA §561B(a)(2) [21 U.S.C. §360bbb-0a(a)(2)].

[56] FFDCA §561B(b) [21 U.S.C. §360bbb-0a(b)].

[57] FFDCA §561B(c) [21 U.S.C. §360bbb-0a(c)].

[58] FFDCA §561B(d)(1) [21 U.S.C. §360bbb-0a(d)(1)].

- require FDA to post an annual summary on its website to include the number of drugs for which (1) FDA determined the need to use clinical outcomes in the review or approval of an investigational drug, (2) the sponsor requested that clinical outcomes be used, and (3) the clinical outcomes were not used.[59]

The act has an uncodified section titled "No Liability," which does not correspond to the FDA's expanded access program. The provision states that, related to use of a drug under the new FFDCA Section 561B,

- "no liability in a cause of action shall lie against ... a sponsor or manufacturer; or ... a prescriber, dispenser, or other individual entity ... unless the relevant conduct constitutes reckless or willful misconduct, gross negligence, or an intentional tort under any applicable State law"; and
- no liability, also, for a "determination not to provide access to an eligible investigational drug."[60]

Discussion of Selected Provisions in the Right to Try Act

Eligible Patients

The Right to Try Act defines eligibility, in part, as a person diagnosed with a "life threatening disease or condition." That definition differs from many of the state-passed laws, as well as from what FDA preferred: that the definition make clear patients were eligible only if they faced a "terminal illness."[61] FDA Commissioner Gottlieb noted that "[many] chronic conditions are life-threatening, but medical and behavioral interventions make them manageable."[62] Examples of such diseases or conditions are diabetes and heart disease.

Speaking in support of right to try bills, supporters told of people facing death who, with no alternatives remaining, would be willing to risk an experimental drug that might even hasten their death.[63] By not limiting eligibility to those at the end of options, the Right to Try Act could allow people with chronic conditions to take extreme risks rather than live a normal lifespan with treatments now available. Because of the broad eligibility, manufacturers could see a significant increase in requests. If Congress revisits the Right to Try Act, Members might consider the definition and clarify what they want for patients and manufacturers.

Informed Consent

The Right to Try Act makes it mandatory that before eligible patients receive an investigational drug, they give the treating doctor their informed consent in writing—but it does not define "informed consent."[64] Other right to try bills, including the House-passed H.R. 5247 (115th

[59] FFDCA §561B(d)(2) [21 U.S.C. §360bbb-0a(d)(2)].

[60] P.L. 115-176, §2(b).

[61] Statement of Scott Gottlieb, M.D., Commissioner of Food and Drugs, before the Subcommittee on Health, Committee on Energy and Commerce, U.S. House of Representatives, October 3, 2017, https://www.fda.gov/NewsEvents/Testimony/ucm578634.htm.

[62] Statement of Scott Gottlieb, M.D., Commissioner of Food and Drugs, before the Subcommittee on Health, Committee on Energy and Commerce, U.S. House of Representatives, October 3, 2017, https://www.fda.gov/NewsEvents/Testimony/ucm578634.htm.

[63] For example, Rep. Barton during House floor debate on S. 204, Congressional Record, May 22, 2018, p. H4359, https://www.congress.gov/crec/2018/05/22/CREC-2018-05-22-pt1-PgH4355.pdf.

[64] FFDCA §561B(a)(1)(C) [21 U.S.C. §360bbb-0a(a)(1)(C)].

Congress), included more specific direction for consent, such as criteria already laid out in 21 CFR Part 50.[65] The Right to Try Act neither provides nor requires the development of such criteria. It thus may weaken patient protections that FDA's expanded access program provides. The Right to Try Act also eliminates the requirement that an IRB review the investigational use of a drug.[66]

If Congress decides to revisit the Right to Try Act, it may seek to create a more explicit informed consent requirement and some outside oversight to reduce the risk to patients either by well-meaning but less knowledgeable physicians or by unscrupulous actors some opponents of the law anticipate.[67]

Data to FDA

Clinical Outcomes

It sometimes takes thousands of patients to establish an accurate evaluation of a drug's safety and effectiveness. Researchers exclude from the clinical trial patients who—for reasons other than the drug's effectiveness—may not show evident benefit from the drug. Those are the patients who would get access through the Right to Try Act pathway.

The Right to Try Act prohibits FDA from using clinical outcome data related to use under this section "to delay or adversely affect the review or approval of such drug."[68] This might make a sponsor more likely to approve the use of its investigational drug under this pathway. The Right to Try Act, however, includes two exceptions. It allows FDA to use those data if the agency determines their use is "critical to determining [the drug's] safety" or if the sponsor requests use of such outcomes.[69] If drug sponsors find that this remains an obstacle to their permitting access to investigational drugs, Congress could work with them, FDA, and patient advocacy groups to devise another approach.

Adverse Events

The Right to Try Act requires the manufacturer to report once a year to FDA, including an account of all serious adverse events that occurred in the preceding 12 months.[70] It does not require immediate reporting of adverse events.[71] This is less than what FDA requires of sponsors of approved drugs and investigational drugs provided in clinical trials or under expanded access. All must periodically inform FDA of such events—and immediately if the event is "serious and

[65] 21 C.F.R. 312.305(c)(4); Rep. Walden, during House debate on S. 204, May 22, 2018, pp. H4357-4358, https://www.congress.gov/crec/2018/05/22/CREC-2018-05-22-pt1-PgH4355.pdf; and Letter to Speaker Ryan and Minority Leader Pelosi, dated May 21, 2018, from 104 advocacy groups, including the American Cancer Society Cancer Action Network, the American Lung Association, the Cystic Fibrosis Foundation, and the Leukemia & Lymphoma Society, as entered into the record by Rep. Castor during House debate on S. 204, May 22, 2018, p. H4358, https://www.congress.gov/crec/2018/05/22/CREC-2018-05-22-pt1-PgH4355.pdf.

[66] FFDCA §561B(b) [21 U.S.C. §360bbb-0a(b)].

[67] Rep. Pallone, during House floor debate on S. 204, Congressional Record, May 22, 2018, p. H4360, https://www.congress.gov/crec/2018/05/22/CREC-2018-05-22-pt1-PgH4355.pdf.

[68] FFDCA §561B(c)(1) [21 U.S.C. §360bbb-0a(c)(1)].

[69] FFDCA §561B(c)(1)(A) & (B) [21 U.S.C. §360bbb-0a(c)(1)(A)&(B)].

[70] FFDCA §561B(d)(1) [21 U.S.C. §360bbb-0a(d)(1)].

[71] Letter to Speaker Ryan and Minority Leader Pelosi, dated May 21, 2018, from 104 advocacy groups, including the American Cancer Society Cancer Action Network, the American Lung Association, the Cystic Fibrosis Foundation, and the Leukemia & Lymphoma Society, as entered into the record by Rep. Castor during House debate on S. 204, May 22, 2018, p. H4358, https://www.congress.gov/crec/2018/05/22/CREC-2018-05-22-pt1-PgH4355.pdf.

unexpected."[72] An adverse event may not be clearly attributable to a drug. A clustering of such reports, though, could signal FDA that this might be something worth exploring.

If Congress were to reconsider the Right to Try Act, it could explore with stakeholders—FDA, drug sponsors, and physicians and patients who use this pathway—ways to make data available to advance the goal of developing safe and effective drugs while protecting the legitimate business interests of manufacturers and the access of seriously ill individuals to try risky drugs.

Disclosure

The Right to Try Act requires the manufacturer or sponsor to submit an annual summary to FDA to include "the number of doses supplied, the number of patients treated, the uses for which the drug was made available, and any known serious adverse events."[73] FDA has issued a proposed rule to implement this annual reporting requirement, which will not become effective until FDA promulgates a final rule and establishes a deadline for such reports.[74] The Right to Try Act also requires FDA to post an annual summary on its website to include the number of drugs for which (1) the agency has determined the need to use clinical outcomes in the review or approval of an investigational drug, (2) the sponsor requested that clinical outcomes be used, and (3) the clinical outcomes were not used.[75]

Congress may choose to revisit these reporting requirements, to require the manufacturer or sponsor to provide more information to FDA, to require FDA to make public additional information, or both.

Financial Cost to Patient

FDA's expanded use process permits a sponsor to charge a patient for the investigational drug, but only to recover the direct costs of making the drug available, as defined under 21 C.F.R. 312.8(d).[76] This includes costs to manufacture the drug in the quantity needed or costs to acquire the drug from another source (e.g., shipping, handling, storage).[77] The sponsor cannot charge for development costs or to make a profit. The Right to Try Act extends this requirement to drugs that sponsors may provide under this pathway.[78] However, it does not require insurers to pay for the drug—or pay for doctor office visits or hospital stays associated with its use or potential adverse outcomes—and these costs may therefore fall on the patient. Congress may consider examining the effect of the Right to Try Act on costs incurred by patients.

Liability Protections

Manufacturers may see liability costs as an obstacle to providing an investigational drug to patients. The no-liability provision in the Right to Try Act seems to remove that obstacle, although it may leave the patient with limited legal recourse. In the past, Congress has sometimes tried to protect both recipients and the manufacturer from harm (e.g., the National Childhood Vaccine Injury Act of 1986 and the Smallpox Emergency Personnel Protection Act of 2003). In

[72] 21 C.F.R. §314.80(c)(1)(i), 21 C.F.R. §312.32(c)(1).

[73] FFDCA §561B(d)(1) [21 U.S.C. §360bbb-0a(d)(1)].

[74] FDA, "Annual Summary Reporting Requirements Under the Right to Try Act," 85 *Federal Register* 44803, July 24, 2020.

[75] FFDCA §561B(d)(2) [21 U.S.C. §360bbb-0a(d)(2)].

[76] 21 C.F.R. §312.8(d)(1).

[77] FDA, "Guidance for Industry: Charging for Investigational Drugs Under an IND—Questions and Answers," p. 6.

[78] FFDCA §561B(b) [21 U.S.C. §360bbb-0a(b)].

those cases, where Congress felt the public health benefit to the larger group outweighed the smaller risk to some, the federal government accepted responsibility for compensating injured patients and indemnifying manufacturers from lawsuits.[79] That has not been the motivating force behind the Right to Try Act. Discussions of earlier versions of liability protections raised concerns that they might not fully protect the manufacturer.[80] As patients use drugs under the Right to Try Act pathway, it is possible that they will test such protections in the courts. This is yet another issue that Congress might pursue.

Concluding Comments

Several questions remain regarding the impact of the Right to Try Act on patients, drug manufacturers, and FDA.

- **First: Will more patients get investigational drugs?** The Right to Try Act requires manufacturers or sponsors to report each year on the number of doses supplied and patients treated as a result of the law, as well as what the drugs were used for and any known serious adverse events.[81] Over time—and perhaps with requesting other data—Congress could determine whether the law has had the effect its sponsors intended.
- **Second: Has the law removed the obstacles to access to investigational drugs?** While the Right to Try Act achieves proponents' objective of removing the FDA application step in a patient's quest for an investigational drug, it does not address other obstacles—such as a limited drug supply or limits on staff and facility resources—that could lead a manufacturer to refuse access to its drugs. Further, it is not clear whether it sufficiently deals with the obstacles it does address—use of clinical outcomes data and liability protection. While the reporting required by the Right to Try Act was not designed to answer those questions, Congress could ask GAO to evaluate the law's impact on manufacturers' willingness to provide investigational drugs under this pathway.
- **Third: How will this affect FDA?** One news article referred to the Right to Try Act's "bizarre twist," as FDA must determine its role in implementing a law whose function is to remove FDA from the situation.[82] Writing in opposition to the bill, four former FDA commissioners warned that it would "create a dangerous precedent that would erode protections for vulnerable patients."[83] That is something Congress may choose to address.

[79] The National Childhood Vaccine Injury Act of 1986 (P.L. 99-660) established the National Vaccine Injury Compensation Program. The Smallpox Emergency Personnel Protection Act of 2003 (P.L. 108-20) established the Smallpox Vaccine Injury Compensation Program.

[80] Bexis, "Federal Right to Try Legislation—Is It Any Better?" Drug & Device Law, September 5, 2017, https://www.druganddevicelawblog.com/2017/09/federal-right-to-try-legislation.

[81] FFDCA §561B(d)(1) [21 U.S.C. §360bbb-0a(d)(1)].

[82] For almost a decade, the Goldwater Institute has been working toward the goal it achieved with the signing of the Right to Try Act. It says that "people have a fundamental right to try to save their own lives without applying to the federal government for permission." (Goldwater Institute quoted in Erin Mershon, "Drug makers have to post policies for patients seeking experimental medicines. Not all do." Stat+, April 5, 2018, https://www.statnews.com/2018/04/05/drug-makers-compassionate-use-policies/.)

[83] Laurie McGinley, "Former FDA commissioners say right-to-try bills could endanger 'vulnerable patients,'" *Washington Post*, March 18, 2018, https://www.washingtonpost.com/news/to-your-health/wp/2018/03/18/former-fda-commissioners-say-right-to-try-bills-could-endanger-vulnerable-patients/?utm_term=.3fe265fa04eb.

The Right to Try Act concludes with a "Sense of the Senate" section that appears to acknowledge that this legislation offers minimal opportunity to patients. It is explicit in asserting that the new law "will not, and cannot, create a cure or effective therapy where none exists." The legislation, it says, "only expands the scope of individual liberty and agency among patients." The drafters realistically end that phrase with "in limited circumstances."

Author Information

Hassan Z. Sheikh
Analyst in Health Policy

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This report was originally authored by Susan Thaul, former CRS Specialist in Drug Safety and Effectiveness, and Agata Bodie, former CRS Analyst in Health Policy.

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