NOTE

Prescribing a Cure for Right-to-Try Legislation

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ABSTRACT

In the past four years, thirty-eight states have passed legislation creating a right for terminal patients to try investigational drugs without approval by the Food and Drug Administration. Despite the popularity of the laws, this legislation has functioned as a legal placebo because manufacturers are hesitant to distribute treatments under the authority of these laws due to concerns about liability, cost, and other administrative burdens. A solution is pending in Congress, but even if it is passed it is unlikely that the federal bill, as written, would actually facilitate access to these treatments.

This Note proposes a federal approach to solve the problems individuals with terminal illnesses face when seeking investigational drugs. This Note proposes that Congress (1) pass a federal right-to-try law to eradicate preemption concerns and (2) add language to the law to incentivize manufacturers to provide the drugs to those with terminal illnesses. This approach would go beyond providing terminal patients a nominal right to try by instituting pragmatic procedures that make it easier for patients to access potentially lifesaving treatments.

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INTRODUCTION

Joshua Thompson was thirty-one when he was diagnosed with Amyotrophic Lateral Sclerosis ("ALS"), also known as Lou Gehrig's disease.[1] The health of this father of two was rapidly weakening, and he was in search of any medication that could possibly help.[2] After researching treatments, his mother Kathy learned about an investigational drug called Iplex.[3] The drug showed promising results in its clinical trial, but the company halted the trial after another company sued for patent infringement of its competing drug.[4] It later became clear that the competing drug was not as effective as Iplex in treating ALS, and after months of lobbying and negotiations, the drug companies reached an agreement that allowed ALS patients to petition the Food and Drug Administration ("FDA") for the treatment through the Agency's "Expanded Access"[5] program.[6] When Joshua petitioned the FDA, however, the Agency declined his application, citing "safety concerns."[7] "Safety," Kathy said, "and what, exactly, is safe about A.L.S.?"[8]

Although the FDA reversed itself two months later, and Joshua was eventually able to access the medication,[9] examples of FDA bureaucracy keeping terminal patients from potentially lifesaving drugs has started a state-level movement to create a "right to try"—a right that provides individuals with terminal illnesses the ability to access investigational drugs prior to FDA approval.[10] Currently, pharmaceutical manufacturers can only provide treatments to consumers either (1) after they have received FDA approval by completing at least three phases of clinical

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1 Amy Harmon, *Fighting for a Last Chance at Life*, N.Y. TIMES (May 16, 2009), http://www.nytimes.com/2009/05/17/health/policy/17untested.html.

2 *Id*.

3 *Id*.

4 *See id*.

5 The FDA's Expanded Access program allows certain individuals to petition the FDA for access to investigational drugs. *Expanded Access (Compassionate Use)*, U.S. FOOD & DRUG ADMIN., https://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/default.htm [https://perma.cc/Y2A2-98TW] (last updated Feb. 21, 2018). It is also known as "compassionate use." *See id*.

6 *See* Harmon, *supra* note 1.

7 *Id*.

8 *Id*.

9 *Id*.

10 *See* Stephen Fee, *'Right to Try' Law Gives Terminal Patients Access to Drugs Not Approved by FDA*, PBS NEWSHOUR, (June 21, 2014, 11:59 AM), http://www.pbs.org/newshour/bb/right-try-law-gives-terminal-patients-access-non-fda-approved-drugs/.

trials[11] or (2) through the FDA's Expanded Access program, which allows companies to provide investigational drugs to terminal patients after receiving FDA permission.[12] State right-to-try bills seek to lessen FDA bureaucracy by allowing pharmaceutical companies that have completed the first phase of clinical trials to provide the treatments to terminal patients without any further FDA involvement.

Despite this push from the public for a right to try, state or federal legislation might not be as effective as lawmakers promise. State-level legislation faces preemption by the Federal Food, Drug, and Cosmetic Act ("FDCA"),[13] and, even if granted the legal authority to distribute the treatments, there are still reasons manufacturers are reluctant to do so. For example, costs, fear of negative impact on ongoing clinical trials, and possibility of adverse inferences from unsuccessful treatment are all concerns that may prevent a manufacturer from providing a treatment.

The time may be ripe, however, for federal action to aid patients in accessing investigational drugs. One of President Barack Obama's final accomplishments in office was passing the bipartisan 21st Century Cures Act.[14] The Act includes a provision that requires pharmaceutical companies to be more transparent about their investigational drug policies, a step toward making experimental drugs more accessible.[15] In his first speech before Congress, President Trump criticized the FDA bureaucracy for keeping treatments from patients, saying "our slow and burdensome approval process at the [FDA] keeps too many advances . . . from reaching those in need."[16] The President has since explicitly supported the legislation by including it in his first State of the Union address:

> We also believe that patients with terminal conditions should have access to experimental treatments that could potentially save their lives.
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> People who are terminally ill should not have to go from country to country to seek a cure—I want to give them a chance right here at home. It is time for the Congress to give these wonderful Americans the "right to try."[17]

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[11] *See* Investigational New Drug Application (IND), 21 C.F.R. § 312.21 (2017).

[12] *See* U.S. FOOD & DRUG ADMIN., EXPANDED ACCESS TO INVESTIGATIONAL DRUGS FOR TREATMENT USE—QUESTIONS AND ANSWERS 2, 11 (2017).

[13] 21 U.S.C. § 301 (2012).

[14] *See* 21st Century Cures Act, Pub. L. No. 114-255, 130 Stat. 1033 (2016).

[15] *See id.*

[16] *Trump's Address to Joint Session of Congress, Annotated*, NPR (Feb. 28, 2017, 8:53 PM), http://www.npr.org/2017/02/28/516717981/watch-live-trump-addresses-joint-session-of-congress.

[17] *President Donald J. Trump's State of the Union Address*, WHITEHOUSE.GOV (Jan.

To address these issues, this Note recommends that Congress pass a federal right-to-try law to satisfy public demand for access to experimental drugs for terminal patients. It also suggests that Congress include provisions in the legislation that take further pragmatic steps to ensure patients actually have a chance at securing the treatments. These steps would authorize the FDA to take a more active role in helping patients access treatments—empowering the FDA to facilitate rather than hinder—and allow more flexibility in the way companies can charge for these treatments.

Part I outlines the history of federal regulation of drugs in the United States and explains the current drug approval process. It also details the journey of the grassroots movement advocating for a right to try and the FDA's attempt to increase access to investigational drugs through its Expanded Access program. Part II analyzes the shortcomings of current state right-to-try laws, while Part III explores the issues that would not be corrected under pending federal legislation. Finally, Part IV proposes a legislative solution that recommends Congress not only pass a federal right-to-try bill but also take further steps to incentivize manufacturers to provide pharmaceuticals to the most vulnerable patients imaginable.

I. SETTING THE STAGE FOR A RIGHT TO TRY: FDA OVERSIGHT AND THE PUSH FOR SPEEDY ACCESS

Although the right-to-try movement has only gained traction in state legislatures during the past four years, demand for faster access to pharmaceuticals is not a new concept and has molded FDA regulatory processes.

A. FDA Regulation of Pharmaceuticals: Historical Context and Current Processes

In evaluating the modern right-to-try movement, it is necessary to understand the history of federal oversight of drugs that has shaped the current drug approval process. The public demand for government oversight to both ensure the safety of pharmaceuticals and facilitate efficient access to treatments has impacted FDA procedures and may continue to do so in the future.

1. History of Federal Oversight of Pharmaceuticals

Federal regulation of pharmaceuticals is a relatively recent occurrence in the history of the United States, and public demand that the government

30, 2018), https://www.whitehouse.gov/briefings-statements/president-donald-j-trumps-state-union-address/ [https://perma.cc/8L28-XAW4].

regulate drugs to protect the population from unsafe treatments has largely formed the role of the FDA as it exists today. During the first 100 years of American history, the federal government played an almost nonexistent role in the production and distribution of medications.[18] State governments oversaw regulation of food and medicine, and the Bureau of Chemistry within the Department of Agriculture, the predecessor to the FDA, existed in a research capacity without any regulatory function.[19] Near the end of the nineteenth century, this system of local regulation began to show fundamental flaws as states were unable to enforce regulations against out-of-state companies, and there were increasing instances of consumers purchasing medications that contained dangerous ingredients.[20] In this time without federal regulation, drifters would travel from town to town selling goods to consumers—most famously "snake oil"—promising to cure a range of maladies, despite the fact that the "drugs" were often useless.[21]

It was not until Congress passed the Pure Food and Drug Act of 1906[22] that Congress empowered the executive branch to increase its oversight of food and drugs entering the market.[23] Congress passed the legislation with overwhelming support due to public cries for oversight after Upton Sinclair's *The Jungle* shed light on the unsanitary conditions in the manufacturing industry.[24] Under the Pure Food and Drug Act, the federal government could impose fines or criminal punishment on companies selling "unadulterated" or "misbranded" drugs or foods in interstate commerce.[25] The legislation charged the Bureau of Chemistry at the

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[18] *See* Abigail All. for Better Access to Developmental Drugs v. von Eschenbach, 445 F.3d 470, 483 (D.C. Cir. 2006).

[19] *See* A HISTORICAL GUIDE TO THE U.S. GOVERNMENT 249 (George Thomas Kurian et al., eds., 1998).

[20] *See* Oscar E. Anderson, Jr., *Pioneer Statute: The Pure Food and Drugs Act of 1906*, 13 EMORY J. PUB. L. 189, 189–90 (1964).

[21] *See, e.g.*, *Arrested for Counterfeiting: Peculiar Crimes Which Attack the Public Health and Pocketbook*, THE MILWAUKEE J., Aug. 9, 1897, at 8 (recounting the story of two men arrested in Chicago in 1897 for creating counterfeit pills that the men claimed were "Dr. Williams' Pink Pills for Pale People"); *Rattlesnake Joe: The Odd Character Who Peddles Snake Oil in Philadelphia*, ST. LOUIS GLOBE-DEMOCRAT, May 30, 1882, at 12; *Rhoads on the Road*, ROCKY MOUNTAIN NEWS (Denver), Dec. 11, 1896, at 5 (describing a missing pseudo eye doctor who was suspected of circulating fliers in the mail advertising fake medicines).

[22] Pure Food and Drug Act of 1906, Pub. L. No. 59-384, 34 Stat. 768, *repealed by* Federal Food, Drug, and Cosmetic Act of 1938, 21 U.S.C. § 301 (2012).

[23] *See id.*

[24] *See* Oscar E. Anderson, Jr., *The Pure-Food Issue: A Republican Dilemma, 1906–1912*, 12 FOOD DRUG COSM. L.J. 30, 32 (1957); *see also* Anderson, *supra* note 20, at 193.

[25] *See* Pure Food and Drug Act of 1906 § 1.

Department of Agriculture with implementing the law and determining when a food or drug was "unadulterated" or "misbranded."[26]

This manner of federal regulation of pharmaceuticals continued through the first third of the twentieth century, but in 1927 the nonregulatory responsibilities of the Bureau of Chemistry were moved to another department, prompting the Bureau of Chemistry's renaming to the Food, Drug and Insecticide Administration.[27] In 1930, the agency changed its name a final time to become what it is known as today: the Food and Drug Administration.[28] Despite the name changes during that time, the regulatory scheme from 1906 has generally remained the same, with only five regulatory changes.[29]

By the 1930s, it was evident that the 1906 law still left consumers unprotected,[30] and legislators sought to replace it with a more effective bill.[31] By 1937, after five years of debate with no resolution in sight,[32] Congress had reached an impasse that was only overcome by a health crisis concerning a drug called Elixir Sulfanilamide.[33] After a pharmaceutical company converted the drug Elixir Sulfanilamide from powder to liquid form, between seventy-three and ninety-three individuals who consumed the liquid died.[34] Prior to introducing the drug to market, the manufacturer tested the liquid form for taste, appearance, and fragrance, but it failed to test whether the new form was safe to consume.[35] After an investigation of the incident, it became clear that a simple animal-based test would have demonstrated the toxic nature of the drug, and the tragedy would have been avoided.[36] Under the 1906 law, however, the FDA did not have the

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26 *See id*. § 5.

27 *See* A HISTORICAL GUIDE TO THE U.S. GOVERNMENT, *supra* note 19, at 249.

28 *See id*.

29 *See* David F. Cavers, *The Food, Drug, and Cosmetic Act of 1938: Its Legislative History and Its Substantive Provisions*, 6 LAW & CONTEMP. PROBS. 2, 5 (1939).

30 Limitations of the Pure Food and Drug Act were revealed to the public in *Your Money's Worth*, a Consumer's Research publication that highlighted dangers to consumers in the marketplace. Cavers, *supra* note 29, at 5–6 (citing STUART CHASE & F.J. SCHLINK, YOUR MONEY'S WORTH: A STUDY IN THE WASTE OF THE CONSUMER'S DOLLAR, (1932)). The publication noted that although the legislation was supposed to protect consumers, it did not require companies to note dangerous ingredients, allowed certain misrepresentations on labels, and provided no protection against products that lacked value. CHASE, *supra* note 30, at 46–47.

31 *See* Cavers, *supra* note 29, at 5–6.

32 *See id*. at 8–20.

33 *See id*. at 20; *see also* SEC'Y OF AGRIC., ELIXIR SULFANILAMIDE-MASSENGILL, S. DOC. NO. 75-124 (1937) [hereinafter ELIXIR SULFANILAMIDE-MASSENGILL REPORT].

34 ELIXIR SULFANILAMIDE-MASSENGILL REPORT, *supra* note 33, at 1.

35 *Id*. at 3.

36 *Id*. at 9.

authority to require testing for toxicity either prior to or after distribution of the drug.[37] Instead, the FDA only had the authority to remove the drug from the market because it was mislabeled—the drug was labeled an "elixir," indicating it contained alcohol, which it did not.[38] This incident, and the FDA's legal inability to detect the drug's toxicity, provided Congress the momentum it needed to pass the FDCA in 1938,[39] repealing the previous law and broadening the federal government's regulatory authority by requiring manufacturers to test the drugs prior to introducing them to market.[40]

This regulatory scheme remained largely in place until the 1960s.[41] At that time, the country suffered another drug-related crisis: babies of mothers who used Thalidomide to treat morning sickness during pregnancy were born with birth defects.[42] Although FDA investigators discovered the problem and heroically worked to remove the drug from the market,[43] almost 10,000 babies had already been impacted after mothers ingested samples that doctors provided.[44] In response to the public outcry, Congress passed the Kefauver-Harris Amendments in 1962.[45] These reforms charged the FDA with reviewing drugs for safety and effectiveness.[46] The law

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37 *See id*.

38 *See id*.

39 21 U.S.C. § 301 (2012); 82 CONG. REC. 355 (1937) (statement of Rep. Rees) ("If there ever was need for legislation on food and drugs for this country, that time is right now. . . . [A company] in Tennessee was permitted to sell a drug known as elixir of sulfanilamide that has resulted in not only the illness of numbers of people[,] . . . at least 73 innocent people have died from using this misbranded and misrepresented drug.").

40 *See* Abigail All. for Better Access to Developmental Drugs v. von Eschenbach, 445 F.3d 470, 482 (D.C. Cir. 2006).

41 *See id*.

42 *See Drug Industry Act of 1962: Hearing on H.R. 11581 and H.R. 11582 Before the H. Comm. on Interstate & Foreign Commerce*, 87th Cong. 204 (1962) (statement of Chester S. Keefer, M.D., Senior Member, Massachusetts Memorial Hospitals) ("The widespread publicity on congenital malformations in infants . . . which have been attributed to the drug thalidomide [] has intensified public interest in our drug laws."); Tony Long, *Oct. 1, 1957: Thalidomide Cures Morning Sickness, But . . .*, WIRED (Oct. 1, 2008, 12:00 PM), https://www.wired.com/2008/10/dayintech-1001-2/.

43 *See* Adam Bernstein & Patricia Sullivan, *Frances Oldham Kelsey, FDA Scientist Who Kept Thalidomide off U.S. Market, Dies at 101*, WASH. POST, (Aug. 7, 2015), https://www.washingtonpost.com/national/health-science/frances-oldham-kelsey-heroine-of-thalidomide-tragedy-dies-at-101/2015/08/07/ae57335e-c5da-11df-94e1-c5afa35a9e59_story.html?utm_term=.fe54e84d0084.

44 *See* Long, *supra* note 42.

45 *See* Drug Amendments of 1962, Pub. L. No. 87-781, 76 Stat. 780; Margaret Hamburg, *50 Years After Thalidomide: Why Regulation Matters*, U.S. FOOD & DRUG ADMIN.: FDA VOICE (Feb. 7, 2012), http://blogs.fda.gov/fdavoice/index.php/2012/02/50-years-after-thalidomide-why-regulation-matters/ [https://perma.cc/C45Y-WNBL].

46 Hamburg, *supra* note 45.

required manufacturers to conduct "adequate and well-controlled investigations" and submit the data from those investigations to the FDA.[47] The legislation empowered the FDA to approve the drugs based on the information the company submitted.[48] Additionally, the amendments required that manufacturers report to the FDA any adverse effects the drugs had on individuals during the testing periods.[49]

Although public outcry for safer drugs in response to health crises fueled the early- and mid-twentieth century expansion of the FDA, by the end of the twentieth century, public outcry from the AIDS crisis drove reforms for faster access to investigational drugs. From the late 1980s until the mid-1990s, in response to demand for experimental drugs as a result of the AIDS crisis, the FDA created accelerated avenues for earlier access to certain treatments.[50] This was the first time the FDA created a "fast track" for certain drugs, acknowledging that there are circumstances when the consequences of not receiving a pharmaceutical outweigh the necessity of a comprehensive clinical trial.[51]

In 2009, again in response to public demand, the FDA went even further by issuing regulations that created an avenue where individuals with terminal illnesses could access experimental drugs that had only completed Phase I clinical tests—a process outlined in detail below.[52] In 2016, the FDA further streamlined the process by creating a more user-friendly application that reduced completion time from eight hours to a mere forty-five minutes.[53]

The FDA and the procedures it follows today—which are discussed in detail in the next section—exist largely because the public either demanded

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47 Abigail All. for Better Access to Developmental Drugs v. von Eschenbach, 445 F.3d 470, 482 (quoting 21 U.S.C. § 355(d) (2012)).

48 *See id*.

49 *See id*. at 482–83.

50 *See, e.g*., New Drug, Antibiotic, and Biological Drug Product Regulations; Accelerated Approval, 57 Fed. Reg. 58,954 (Dec. 11, 1992) (codified at 21 C.F.R. § 314.500); Suzanne White Junod, *FDA and Clinical Drug Trials: A Short History*, U.S. FOOD & DRUG ADMIN., (last updated Feb. 1, 2018), https://www.fda.gov/aboutfda/whatwedo/history/overviews/ucm304485.htm [https://perma.cc/TCA4-58Y6].

51 *See* New Drug, Antibiotic, and Biological Drug Product Regulations, 52 Fed. Reg. 8798 (Mar. 19, 1987).

52 *See* 21 C.F.R. § 312.1 (2009); U.S. FOOD & DRUG ADMIN., OMB CONTROL NO. 0910-0814, INDIVIDUAL PATIENT EXPANDED ACCESS APPLICATIONS: FORM FDA 3926 (2016) [hereinafter FDA EXPANDED ACCESS GUIDANCE].

53 *See Exploring a Right to Try for Terminally Ill Patients: Hearing Before the S. Comm. on Homeland Security and Gov't Affairs*, 114th Cong. 3 (2016) [hereinafter Lurie Statement] (statement of Peter Lurie, Associate Comm'r for Public Health Strategy and Analysis, FDA); FDA EXPANDED ACCESS GUIDANCE, *supra* note 52.

oversight to ensure safer drugs or demanded quicker access to treatments for patients for whom time is critical.

2. The Current FDA Drug Approval Process

The FDCA[54] prohibits a manufacturer from introducing a new drug into interstate commerce without FDA approval.[55] Although development of a drug at the initial stages does not involve the FDA, once a pharmaceutical company begins testing on humans it must coordinate with the FDA to proceed.[56] In its regulatory function, the FDA is primarily concerned with the safety and efficacy of new drugs, which it monitors by requiring companies to engage in clinical trials.[57] To begin these trials, a manufacturer must file an Investigational New Drug Application ("IND") with the FDA containing information about the drug, data from animal testing, plans for clinical testing—including indications of the drug to be tested[58]—and the credentials of the lead investigator.[59] The manufacturer may begin clinical testing (using the procedures set forth in its IND) thirty days after filing the application as long as the FDA has not placed a hold[60] on the company's application.[61]

All drugs undergo three phases of clinical trials.[62] Phase I focuses on whether the drug is safe for humans.[63] During this phase, researchers observe the drug's impact on the participants, particularly looking for any side effects.[64] After a successful first phase, the drug enters Phase II trials, which are larger (usually involving 100 to 300 individuals) and controlled.[65] In Phase II, the FDA evaluates early signs of the drug's safety

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54 21 U.S.C. § 301 (2012).

55 *See id.* § 355.

56 *See* 21 C.F.R. § 312.40(b) (2016).

57 *See id.* § 312.21.

58 *See id.* § 201.57(c)(2) (explaining that a pharmaceutical label "must state that the drug is indicated for the treatment, prevention, mitigation, cure, or diagnosis of a recognized disease or condition, or of a manifestation of a recognized disease or condition, or for the relief of symptoms associated with a recognized disease or condition").

59 *See id.* § 312.23(a)(3)(iv).

60 The FDA may place a hold on an application for several reasons, including that the risks to human subjects are too high, the investigators are unqualified, or there is a concern about information in the IND. *See id.* § 312.42.

61 *See* 21 C.F.R. § 312.40(b).

62 *See id.* § 312.21.

63 *See id.*

64 *See id.*

65 *See id.* When a clinical trial is "controlled," observers compare the results of individuals testing the treatment with a "control group" who either do not receive the treatment, take a placebo, or receive a different effective treatment. U.S. DEP'T HEALTH & HUMAN SERVS. ET AL, GUIDANCE FOR INDUSTRY E10 CHOICE OF CONTROL GROUP AND

and efficacy as well as how to improve testing methods for future phases.[66] Phase III testing is similar to Phase II, including the use of a control group, but it involves a larger testing pool.[67] Generally, there are at least 1,000 participants during Phase III, although some trials can involve up to several thousand individuals, and the phase can last for a significantly longer period of time than a Phase II trial.[68] During Phase III, the manufacturer expects to glean insight into the efficacy and side effects of the drug, which may manifest themselves over a longer period of time, and seeks to compare the results of the trial to comparable approved treatments to guarantee that the drug is at least as efficacious as any already available treatments.[69] After the drug reaches the market, a company may be required to conduct a Phase IV trial to gain further information about the long-term safety or efficacy of the drug.[70] After the clinical trials are completed, the manufacturer must submit a New Drug Application ("NDA") to the FDA explaining the results of the trials and providing other information about the drug, its label, and the proposed manufacturing process.[71] The FDA has 180 days to review the NDA and either approve or reject the drug based on whether the trials demonstrated "substantial evidence" of drug safety and effectiveness, whether the labeling of the packaging is appropriate, and whether the manufacturing method meets Good Manufacturing Practices.[72] The Agency, however, can increase the time for review if the company amends the NDA or agrees to an extension.[73]

3. Expanded Access Process

The FDA Expanded Access program creates a back door for patients with a serious illness to access certain treatments that have not yet received final FDA approval. It allows patients to access approved treatments that have been withdrawn for safety reasons, or those that have not been approved by the FDA because they are in the early stages of clinical testing or have failed for safety reasons that are not necessarily a risk to a terminal

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RELATED ISSUES IN CLINICAL TRIALS 3 (2001).

66 *See* 21 C.F.R. § 312.21.

67 *See id*.

68 *See id*.; *see also The Drug Development Process, Step 3: Clinical Research*, U.S. FOOD & DRUG ADMIN., https://www.fda.gov/ForPatients/Approvals/Drugs/ucm405622.htm#Clinical_Research_phase_Studies [https://perma.cc/B8BZ-X69E] (last updated Jan. 4, 2018).

69 *See* 21 C.F.R. § 312.21.

70 *See id*. § 312.85.

71 *See* SUSAN THAUL, CONG. RESEARCH SERV., R41983, HOW FDA APPROVES AND REGULATES DRUGS AND THEIR SAFETY AND EFFECTIVENESS 5 (2012).

72 *See* 21 U.S.C. § 355 (2012); 21 C.F.R. § 314.105 (2016).

73 *See* 21 C.F.R. § 314.100.

patient.[74] Expanded Access regulations create an exception for distribution of investigational treatments to groups of patients with fewer participants than the number participating in a clinical trial or to individuals on a case-by-case basis.[75]

a. Requirements for Individual Expanded Access

To be eligible for FDA Expanded Access, an individual must have an illness that is serious or immediately life threatening, and there cannot be an alternative treatment available, as determined by the FDA.[76] A physician must determine that there is a reasonable balance between the risk of using the unapproved treatment and the potential benefit,[77] and the manufacturer of the pharmaceutical must support the patient's request by either amending a pending IND to grant permission or by filing a new IND.[78] Under the regulations, manufacturers can only charge the patient for the direct costs of providing the treatments.[79] An individual is only eligible for Expanded Access if granting permission to the individual to access the drug does not interfere with ongoing clinical investigations.[80]

b. The Individual Expanded Access Process

An individual begins the Expanded Access process when her physician submits an application to the FDA.[81] That application must include information sufficient to demonstrate that the individual is eligible for

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74 *See* U.S. FOOD & DRUG ADMIN., EXPANDED ACCESS TO INVESTIGATIONAL DRUGS FOR TREATMENT USE—QUESTIONS AND ANSWERS 3, 17 (2016).

75 *See* 21 C.F.R. § 312.300 (2016).

76 *See id.* § 312.305(a)(1); *see also id.* § 312.300(b) (defining an illness as serious if it is "associated with morbidity that has substantial impact on day-to-day functioning" and life threatening if it is "a stage of disease in which there is reasonable likelihood that death will occur within a matter of months or in which premature death is likely without early treatment"). For a drug to be eligible for this program, FDA regulations state that there cannot be a "comparable or satisfactory alternative therapy to diagnose, monitor, or treat the patient's disease or condition." *Id.* § 312.300(a). There is no clear standard for what constitutes a satisfactory alternative treatment, but the FDA has indicated that it makes determinations on a case-by-case basis. *See* Expanded Access to Investigational Drugs for Treatment Use, 70 Fed. Reg. 40,900, 40,910 (Aug. 13, 2009). For example, in response to a comment from a cancer patient concerned that alternative treatments are more toxic than the investigational drug, the FDA noted that it would evaluate "[t]he potential lower toxicity of an experimental therapy[,] . . . the patient's ability to tolerate the approved therapy, and other clinical factors." *Id.* at 40,910.

77 *See* 21 C.F.R. § 312.305(a)(2).

78 *See id.* § 312.305(b).

79 *Id.* § 312.8(d)(1).

80 *Id.* § 312.305(a)(3).

81 *See id.* § 312.310(b).

Expanded Access.[82] Additionally, if the pharmaceutical is already the subject of a pending IND, either the manufacturer must amend the IND with a protocol amendment for individual Expanded Access or a physician must get the manufacturer's permission to allow the FDA to refer to the manufacturer's IND for information to support the Expanded Access project.[83] In certain emergency situations, the FDA allows a physician or manufacturer to request Expanded Access over the phone, and the physician or manufacturer is required to follow up with a written report within fifteen days.[84] The FDA only allows this treatment for a specific period of time, and after the treatment period has ended, the manufacturer or physician must report the results of the treatment to the FDA, including adverse effects.[85] An FDA reviewing official has thirty days to review and respond with a decision,[86] although in practice the FDA frequently responds expediently.[87]

B. History of the Push for a Right to Try

Despite the FDA's recent expansion of its Expanded Access program, a grassroots movement has grown over the past four years leading states to enact right-to-try laws that allow manufacturers to provide investigational drugs to individuals with terminal illnesses without approval by the FDA.[88] Although right-to-try legislation is still in its infancy, patients with serious illnesses have sought unapproved treatments for decades.[89] In 1979, cancer patients sued the United States to permit the importation of an unapproved drug, but the Supreme Court held that there was no express or implied exception to FDCA requirements for individuals with terminal illnesses.[90] Consistent with this decision, in 2007 the D.C. Circuit held that there is no due process right to access unapproved drugs.[91] These judicial setbacks

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82 *Id*.

83 *Id*. § 312.310(b)(1)–(3).

84 *Id*. § 312.310 (d)(1)–(2).

85 *Id*. § 312.310 (c)(1)–(2).

86 *See id*. § 312.305(d).

87 *See* Lurie Statement, *supra* note 53; U.S. GOV'T ACCOUNTABILITY OFF., GAO-17-564, INVESTIGATIONAL NEW DRUGS: FDA HAS TAKEN STEPS TO IMPROVE THE EXPANDED ACCESS PROGRAM BUT SHOULD FURTHER CLARIFY HOW ADVERSE EVENTS DATA ARE USED 19–21 (2017).

88 *See* John Meyers, *Californians with Terminal Diseases Are Now Cleared to Try Experimental Drugs*, L.A. TIMES (Sept. 27, 2016, 2:37 PM), https://www.latimes.com/politics/essential/la-pol-sac-essential-politics-updates-californians-with-terminal-diseases-are-1475011903-htmlstory.html.

89 *See id*.

90 *See* United States v. Rutherford, 442 U.S. 544, 558–59 (1979).

91 *See* Abigail All. for Better Access to Developmental Drugs v. von Eschenbach, 495 F.3d 695, 697 (D.C. Cir. 2007) (holding terminally ill patients represented by the Abigail

have not deterred right-to-try advocates though, as in recent years the movement has turned to local legislatures to guarantee that terminal patients can access investigational drugs through state law.

C. State-Level Right-to-Try Bills

Despite judicial decisions holding that there is no constitutional right to try, state legislatures began enacting bills in 2014 that, on paper, provided terminal patients that very right. Colorado was the first state to pass such a law,[92] and in the spring of 2017, Ohio became the thirty-third state to pass a right-to-try law.[93] In general, these laws allow manufacturers to provide investigational drugs to a person with an immediately life-threatening disease who is not eligible to participate in a clinical trial.[94] The legislation shields physicians, manufacturers, or any other person involved from tort liability for any harm from distribution of the investigational drug.[95]

The popularity of right-to-try legislation is evident from the number of state legislatures willing to approve such provisions in the past four years. Over this time, virtually every state has introduced a version of the legislation.[96] As discussed below,[97] however, there is no evidence that any individual has been able to access medication under these bills that would not have otherwise been available.[98]

D. Federal Right-to-Try Legislation

In May 2016, Senator Ron Johnson of Wisconsin introduced the Trickett Wendler Right to Try Act of 2016,[99] a federal counterpart to state right-to-try legislation.[100] The bill prohibits the federal government from

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Alliance for Better Access to Developmental Drugs did not have a fundamental right to access investigational drugs, and thus the FDA policy satisfied rational basis review).

92 Fee, *supra* note 10.

93 *See* Denise Grant, *'Right-to-Try' Law Received Local Support*, COURIER (Jan. 16, 2017), http://thecourier.com/local-news/2017/01/16/right-to-try-law-received-local-support/.

94 *See, e.g.*, Assemb. B. 1668, 2015–2016 Assemb., Reg. Session, (Cal. 2016).

95 *See, e.g.*, *id.*

96 *See* Brenda Huneycutt, *Right-to-Try Bills Grow in Popularity Yet Success Is Unclear*, AVALERE HEALTH (Mar. 17, 2016), http://avalere.com/expertise/life-sciences/insights/right-to-try-bills-grow-in-popularity-yet-success-is-unclear [https://perma.cc/6HT2-LUKM].

97 *See infra* Section II.B.

98 *See* Arthur Caplan, *Medical Ethicist Arthur Caplan Explains Why He Opposes 'Right-to-Try' Laws*, 30 ONCOLOGY 8 (2016), http://dc.cn.ubm-us.com/i/628608-oncology-january-2016.

99 Trickett Wendler Right to Try Act of 2016, S. 2912, 114th Cong. (2016).

100 Although Senator Johnson attempted to pass the bill by unanimous consent, then-Senate Majority Leader Reid blocked the procedure, citing lack of bipartisanship and other

interfering with distribution of experimental drugs to individuals with terminal illnesses in right-to-try states, empowering manufacturers to distribute investigational treatments in compliance with state right-to-try laws.[101] The federal legislation defines experimental drugs as drugs still under review at the FDA that have, at minimum, passed a Phase I clinical trial.[102] The legislation leaves the determination of what constitutes a terminal illness to each of the individual states.[103] The federal bill also shields manufacturers or any other participants in the distribution process from tort liability to placate companies concerned with products liability class actions resulting from provision of the drugs.[104] Because the individuals receiving treatments have terminal illnesses, and are higher-risk than those in clinical trials, the legislation also prevents the FDA from considering any adverse effects of the treatment in its eventual review of the drug's associated NDA.[105] The conservative contingency in the Senate overwhelmingly supported the legislation, with forty Republican cosponsors, but the bill only found liberal support in its two Democratic cosponsors, leaving it to stall in the Senate.[106]

Right-to-try legislation finally gained momentum during the 115th Congress. On January 24, 2017, Senator Johnson reintroduced the bill,[107] and the Senate passed the legislation by unanimous consent on August 3, 2017.[108] In contrast to the legislation from the 114th Congress that simply resolved the preemption issue by allowing eligible patients to access investigational drugs in accordance with state right-to-try legislation, the legislation in the 115th Congress allows all eligible patients to access

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political issues. *See* Bill Glauber, *Johnson's Right-to-Try Bill Blocked*, J. SENTINEL, http://www.jsonline.com/story/news/politics/2016/09/28/johnsons-right—try-bill-blocked/91225922/ (last updated Sept. 28, 2016, 7:42 PM); Press Release, U.S. S. Comm. on Homeland Sec. & Gov't. Affairs, Johnson Introduces Trickett Wendler Right to Try Act (May 10, 2016), https://www.hsgac.senate.gov/media/majority-media/johnson-introduces-trickett-wendler-right-to-try-act [https://perma.cc/7XG6-UFXR].

101 S. 2912 § 2(a).

102 *Id*. § 2(c)(3).

103 *Id*. § 2(c)(5).

104 *Id*. § 2(b)(1).

105 *Id*. § 2(b)(2).

106 Glauber, *supra* note 100.

107 Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, S. 204, 115th Cong. (2017); Laurie McGinley, *Senate Passes 'Right to Try' Bill to Help Terminally Ill Patients Get Experimental Drugs*, WASH. POST (Aug. 3, 2017), https://www.washingtonpost.com/news/to-your-health/wp/2017/08/03/senate-passes-right-to-try-bill-to-help-terminally-ill-patients-get-experimental-drugs/?utm_term=.be12d9b23a93.

108 McGinley, *supra* note 107.

treatments regardless of state law authorization.[109] In the late hours of March 9, 2018, the House Energy and Commerce Committee released its version of the right-to-try bill.[110] The proposed legislation includes a narrower definition of "eligible illness" than the Senate bill: it only applies to illnesses where there is a "reasonable likelihood that death will occur within a matter of months; [] or a disease or condition that would result in significant irreversible morbidity that is likely to lead to severely premature death."[111] On March 21, 2018, the House of Representatives passed its version of the Right-to-Try bill.[112] Once Congress reconciles the two bills, the final legislation will be presented to President Trump for his signature.[113] Given the President's explicit support of the legislation in his State of the Union address, it is likely that he will sign the bill into law.[114]

II. CRITICISMS OF STATE-LEVEL RIGHT-TO-TRY LAWS

Despite the popularity of right-to-try legislation at the state level, there are plenty of reasons to be skeptical of its actual effectiveness. The laws create preemption concerns because they effectively overwrite the FDCA, and even after the laws are enacted, there are barriers preventing manufacturers from providing the drugs, and barriers preventing patients from obtaining the drugs. The significance of these concerns, which are addressed below, is evidenced by the fact that no individual has accessed an investigational treatment under a state right-to-try law.

A. State-Level Right-to-Try Laws Are Likely Preempted by the Food, Drug and Cosmetic Act

Perhaps the most significant concern with state-level right-to-try laws is they are likely preempted by the FDCA and likely would not withstand a constitutional challenge. The Supremacy Clause provides the authority for a constitutional federal law to preempt a state law.[115] The Court in *Gade v.*

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109 *See* S. 204, 115th Cong. (2017).

110 Thomas M. Burton, *House Introduces 'Right to Try' Legislation to Permit Use of Unproven Drugs for Dying Patient*s, WALL ST. J., https://www.wsj.com/articles/house-introduces-right-to-try-legislation-to-permit-use-of-unproven-drugs-for-dying-patients-1520697934 (Mar. 10, 2018, 4:12 PM).

111 Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2018, H.R. 5247 (as reported by the H. Comm. on Energy & Commerce Mar. 9, 2018).

112 Sarah Karlin-Smith, *House Passes Right-to-Try on Second Try*, POLITICO (Mar. 21, 2018 10:16 PM), https://www.politico.com/story/2018/03/21/drugs-right-to-try-congress-434677.

113 *See id.*

114 *See President Donald J. Trump's State of the Union Address*, *supra* note 17.

115 U.S. CONST. art. VI ("This Constitution, and the Laws of the United States which

National Solid Wastes Management Ass'n[116] explained that preemption can occur because the federal law either expressly limits state action or impliedly limits state action.[117] There are two types of implied preemption: (1) field preemption, where the "federal regulation is 'so pervasive as to make reasonable the inference that Congress left no room for the States to supplement it,'"[118] and (2) conflict preemption, where "compliance with both federal and state regulations is a physical impossibility."[119] In 2009, the Supreme Court analyzed both conflict and field preemption when it evaluated whether FDA labelling requirements preempt state tort law claims.[120] Specifically, the drug manufacturers challenging the requirements argued that it was impossible for companies to comply with both federal regulations and state law duties of care.[121] The Court ruled that the state law was not preempted because the plaintiff failed to prove that it was impossible for manufacturers to comply with both state and federal labelling laws.[122]

Although there is no evidence that the plain language of the FDCA expressly preempts right-to-try laws, there may be a case for implied preemption due to conflict preemption. The FDCA states that "[n]o person shall introduce or deliver for introduction into interstate commerce any new drug, unless an approval of an application [NDA] . . . is effective with respect to such drug,"[123] a provision which is in direct conflict with the right-to-try legislative goal of allowing manufacturers to provide pharmaceuticals prior to FDA approval.[124] Some state laws even appear to acknowledge this conflict.[125] For example, the preamble of the California law concedes that federal law requires pharmaceuticals to be approved by the FDA, but proceeds to permit manufacturers to provide such pharmaceuticals anyway.[126]

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shall be made in Pursuance thereof; and all Treaties made, or which shall be made, under the Authority of the United States, shall be the supreme Law of the Land.").

116 505 U.S. 88 (1992).

117 *Id*. at 98.

118 *Id*. (quoting Fid. Fed. Sav. & Loan Ass'n v. de la Cuesta, 458 U.S. 141, 152–53 (1982)).

119 *Id*. (quoting Fl. Lime & Avocado Growers, Inc. v. Paul, 373 U.S. 132, 142–43 (1963)).

120 *See* Wyeth v. Levin, 555 U.S. 555, 556, 560, 565 (2009).

121 *See id*. at 563.

122 *See id*. at 573.

123 21 U.S.C. § 355(a) (2012).

124 *See, e.g.*, Assemb. B. 1668, 2015–2016 Assemb., Reg. Session, (Cal. 2016).

125 *See, e.g.*, *id*.

126 *See id*.

Despite this conflict, advocates have argued that right-to-try laws are similar to "right-to-die" laws,[127] noting that the Supreme Court's holding in *Gonzales* v. *Oregon*[128] that right-to-die laws are not preempted, may apply directly to right-to-try laws. In *Gonzales*, however, the Court held that Oregon's law—which provided protections from criminal liability to doctors who provided life-ending drugs to patients—was not preempted by the Controlled Substances Act[129] because the language of the Act only focused on "conventional drug abuse."[130] This argument is unlikely to gain traction in the context of right-to-try laws because these patients are seeking access to drugs for their indicated use, which is exactly what the language of the FDCA covers.[131] Nevertheless, in a 2017 Frequently Asked Questions page, the FDA took no position concerning whether the FDCA preempts right-to-try laws.[132] Even though the question of preemption has not been addressed by the courts or the FDA, the state legislation would likely be found preempted by the FDCA.

Although the distribution of unapproved pharmaceuticals under a state right-to-try law is likely preempted by the FDCA and would be subject to FDA enforcement procedures, it is possible that the immunity granted by these laws to manufacturers against state tort liability would be effective.[133] The Supreme Court has been hesitant to hold that state tort liability is preempted by the FDCA, only finding preemption where a portion of the legislation expressly calls for preemption.[134] This will likely give little comfort to a manufacturer hoping to provide the drugs under state right-to-try laws because that manufacturer could still face criminal liability under federal law.

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127 *See* Benjamin A. Cohen-Kurzrock, Philip R. Cohen & Razelle Kurzrock, *The Right to Try is Embodied in the Right to Die*, 13 NATURE REVS. CLINICAL ONCOLOGY 399, 399 (2016).

128 546 U.S. 243 (2006).

129 21 U.S.C. §§ 801–971 (2012).

130 *See id*. at 275; Cohen-Kurzrock, *supra* note 127, at 399.

131 *See* 21 U.S.C. § 355(a) (2012).

132 *See FDA and Marijuana: Questions and Answers*, U.S. FOOD & DRUG ADMIN (Aug. 15, 2017), http://www.fda.gov/NewsEvents/PublicHealthFocus/ucm421168.htm#Q10 [https://perma.cc/2Y3A-WKY7] ("The FDA has not taken a position on any particular state 'Right to Try' bill.").

133 *See* Jan Murray & Lindsey E. Gabrielsen, *Right-to-Try Laws: Safe and Effective or Placebo Legislation?* 9 J. HEALTH & LIFE SCI. L. 54, 67 (2016).

134 *See* Riegel v. Medtronic, Inc., 552 U.S. 312, 330 (2008) (holding that plaintiff's tort claim for failure to warn concerning a medical device was preempted because the FDCA expressly preempted state requirements for such devices); *see also* Wyeth v. Levine, 555 U.S. 555, 574–75 (2009) (holding that plaintiff's claim that the drug label's failed to warn of side effects was not preempted by the FDCA, because Congress did not provide an express preemption provision concerning labeling prescription drugs).

State right-to-try laws are likely preempted by the FDCA, creating confusion that begs Congress to take action and pass a federal statute, thus avoiding any concern about preemption.

B. State Right-to-Try Laws Are Ineffective Because Patients Are Not Using Them to Access Treatments

Another issue with the state right-to-try legislation is that the laws provide a right to try in name only, and they do not actually increase access to investigational drugs for those with terminal diseases.[135] Data supports these claims.[136] In a 2015 survey, Modern Healthcare found that there was no evidence that any individual had successfully acquired developmental drugs under the authority of a state right-to-try bill.[137] Despite the popularity of the laws, patients and manufacturers still face roadblocks in achieving their goals even after they are passed.

State right-to-try laws specifically state that manufacturers are not required to provide treatments,[138] and many are still hesitant to do so because providing these drugs (1) is expensive, (2) may have a negative impact on ongoing clinical trials, and (3) may open the drug companies up to penalties from the FDA.

Providing drugs that have only passed one phase of clinical trials to individuals with terminal diseases creates expenses for pharmaceutical companies that they will be unlikely to recoup under state legislation. Some state laws allow pharmaceutical companies to bill direct costs to the patients, while others prohibit the companies from receiving any compensation for providing the drugs.[139] One concern with this is that manufacturers are likely to incur costs beyond just the production of the individual treatment, which could prevent smaller pharmaceutical companies from providing the treatments.[140] A larger concern is that

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135 *See* Caplan, *supra* note 98, at 3.

136 *See* Steven Ross Johnson, *Despite Political Support, State Right-to-Try Bills Show No Takeup*, MODERN HEALTHCARE (Oct. 17, 2015), http://www.modernhealthcare.com/article/20151017/MAGAZINE/310179969 [https://perma.cc/RR59-R7YZ].

137 *See id.*

138 *See, e.g.*, Assemb. B. 1668, 2015–2016 Assemb., Reg. Session, (Cal. 2016).

139 *Compare* TEX. HEALTH & SAFETY CODE ANN. § 489.053(c) (West 2017) ("If a manufacturer makes available an investigational drug, biological product, or device to an eligible patient under this subchapter, the manufacturer must provide the investigational drug, biological product, or device to the eligible patient without receiving compensation."), *with* CAL. HEALTH & SAFETY CODE § 111548.2(b)(2) (2017) ("A manufacturer . . . may . . . [r]equire an eligible patient to pay the costs of, or associated with, the manufacture of the investigational drug, biological product, or device.").

140 *See* Vital Therapies, Comment Letter on Proposed Rules regarding Expanded Access to Investigational Drugs for Treatment Use and Charging for Investigational Drugs (Jan. 30, 2007). Vital Therapies noted that only allowing a pharmaceutical company to

pharmaceutical companies consider data concerning drug costs to be a closely guarded trade secret, and calculating the direct costs of a pharmaceutical would expose the cost information of the pharmaceuticals, which companies are unwilling to do.[141] Some companies have suggested that allowing drug companies to set a "reasonable administrative fee" that is negotiated with patient groups and reflects market prices of comparable treatments would alleviate some of these concerns because it would allow pharmaceutical companies to avoid disclosing proprietary cost information and would prevent a third party from dictating which cost calculations are appropriate.[142]

Pharmaceutical companies may not receive direct financial benefit for participating in these programs, and there is no evidence at this point that they are gaining positive publicity for providing these medicines.[143] At the end of the day, these companies seek to make a profit, and due to the potential of exposing the company to liability by providing these drugs (as explained below), it is likely not worth the subsequent potential losses.

Pharmaceutical manufacturers may also be hesitant to provide drugs under right-to-try laws out of fear that doing so will adversely impact their ongoing clinical trials. As outlined above, in order for a drug to secure FDA approval, the company must demonstrate its efficacy and safety through clinical trials.[144] If an individual does not have to participate in a controlled clinical trial—where there is a chance she will receive the placebo drug—and can instead take the drug directly from the manufacturer, pharmaceutical companies may not have enough individuals willing to participate in clinical trials.[145] This concern does not arise under the Expanded Access program because the FDA requires that no alternative treatments exist to qualify for the program, and a clinical trial would constitute an alternative treatment.[146] This dilution of the clinical trial base through right-to-try laws may not be a significant concern in practice, however, because there are many other barriers to participation in clinical

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charge for direct costs is a disincentive to small companies and recommended the FDA broaden the category to include "allocation of production fixed costs," "cost of drug delivery," and "research and development." *See id*.

141 *See* Jonathan J. Darrow, et al., *Practical, Legal, and Ethical Issues in Expanded Access to Investigational Drugs*, 372 NEW. ENG. J. MED. 279, 282 (2015).

142 *See id*.

143 In fact, the opposite may be the case. *See id*. at 281 (arguing that when a drug company charges direct costs before FDA approval they may suffer public pushback when the drug goes to market at a higher cost).

144 21 C.F.R. § 312.21 (2016); *see supra* Section I.A.2.

145 *See* Darrow, et al., *supra* note 141, at 281.

146 *See* 21 C.F.R. § 312.305 (2016).

trials—for example, eligibility requirements.[147] And, this Expanded Access loophole would only apply to the narrow pool of patients with terminal illnesses.[148]

Another concern is that the adverse events[149] patients could experience while taking drugs issued under right-to-try laws may influence the FDA when it determines whether to approve an NDA. Although the state legislation does not require reporting of adverse events to the FDA,[150] pharmaceutical companies are still concerned about the subjective impression such an event may have on the FDA if negative patient reactions were publicized, particularly the possibility that a negative reaction may impact ongoing clinical trials.[151] Pharmaceutical companies invest significant time and resources into the development of a drug, and clinical trials to approve the drug, so companies may be hesitant to provide treatments out of concern that they will negatively impact the ongoing FDA approval process.

Additionally, although a manufacturer can feel confident that it will not face penalties at the state level for providing the drugs under right-to-try bills, the threat of retribution from the federal government may prevent manufacturers from providing the drugs. As outlined above, there is a strong possibility that these laws are preempted by the FDCA.[152] This uncertainty, as well as potential severe penalties for violating the FDCA, likely prevent manufactures from providing pharmaceuticals under these laws.

Another possible reason why individuals are not accessing treatments under right-to-try laws is that such treatments are not covered by insurance, and without insurance coverage, patients cannot afford the treatments even when the manufacturer can only charge the direct costs.[153] Although each of the state laws differs in its specific provisions, none of the laws mandate that health insurers cover these treatments.[154] In states where companies are able to bill direct costs to patients, medical bills add up quickly and may

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147 *See* Cohen-Kurzrock, *supra* note 127, at 400.

148 *See id*.

149 An adverse event is "any untoward medical occurrence associated with the use of a drug in humans, whether or not considered drug related." 21 C.F.R. § 312.32 (2016).

150 *See, e.g.*, CAL. HEALTH & SAFETY CODE § 111548.2 (2017).

151 *See infra* Section III.A.1.

152 *See supra* Section II.A.

153 *See, e.g.*, CAL. HEALTH & SAFETY CODE § 111548.2(c)(1) ("This article does not expand the coverage provided under . . . Sections 10145.3 and 10145.4 of the Insurance Code . . . .").

154 *See, e.g.*, *id*.

prevent an individual from accessing treatments.[155] This reasoning, however, fails to explain why individuals do not access treatments in the states that explicitly forbid manufacturers from charging a patient to access the treatments.[156]

Although thirty-eight states have adopted right-to-try laws in the past four years, these state laws have failed to assist patients in accessing investigational drugs. The laws are likely preempted by the FDCA and there are significant barriers preventing manufacturers from providing the drugs and patients from accessing them. Without further action, it is unlikely that state-level legislation will ever effectively help a terminal patient access an investigational drug.

III. CRITICISMS OF THE CURRENT PROPOSED FEDERAL LEGISLATION

Because state right-to-try legislation faces issues of preemption and does not appear to facilitate access to investigational drugs, it is tempting to turn to a federal right-to-try bill to solve this problem. The current federal right-to-try legislation, however, presents its own challenges because manufacturers have little incentive to provide treatments under a federal bill and because patients may be unable to access the treatments.

A. The Federal Legislation Does Not Require Manufacturers to Provide Treatments, and Patients Are Often Unable to Access the Drugs

The proper solution to the insufficiencies of state-level right-to-try legislation is a federal bill that would solve any preemption issues and provide manufacturers with the assurance that the FDA will not seek criminal penalties against the companies for providing these treatments to terminal patients. The Trickett Wendler Right to Try Act that is currently pending in Congress would fail in practice, however, because it does not adequately incentivize manufacturers to provide experimental drugs to patients and may not significantly aid patients in securing the treatments they need.

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155 *See* Steven Ross Johnson, *For the Dying, State Laws Offer Hope that Critics Call Hollow*, MODERN HEALTHCARE (Nov. 5, 2014), http://www.modernhealthcare.com/article20141105/NEWS/311059922?AllowView=VDl3UXk1SzRDdkdCbkJiYkY0M3hla0twaWtVZENPUT0%3D [https://perma.cc/6YBY-GT5L].

156 *See* Carrie Feibel, *Patients Demand the 'Right to Try' Experimental Drugs, but Costs Can Be Steep*, NPR (Mar. 3, 2017, 2:17 PM), http://www.npr.org/sections/health-shots/2017/03/03/517796956/patients-demand-the-right-to-try-experimental-drugs-but-costs-can-be-steep ("Although nearly three dozen right to try laws are now on the books, researchers at New York University who have been looking for evidence of the laws' usefulness haven't yet found a single substantiated case of a patient getting a drug by using a state law.").

1. Why Are Manufacturers Unwilling to Provide the Drugs?

Even if Congress enacts a federal law giving manufacturers the legal authority to provide investigational drugs to individuals with terminal illnesses prior to FDA approval, there are barriers that prevent manufacturers from doing so. Manufacturers may be reluctant to grant patients these treatments due to concerns about costs, adverse incidents, liability, and supplies.

First, manufacturers may be unwilling to provide investigational drugs even after Congress passes legislation because companies may only be able to bill the costs associated with manufacturing the drug. Although the Trickett Wendler Right to Try Act does not specify how much manufacturers can charge to provide the treatments, it states that the drugs should be provided "in accordance[] with State law."[157] As discussed above, state laws either allow companies to charge direct costs or prohibit charges altogether, which may discourage manufacturers from providing the drug.[158]

Second, manufacturers may be concerned that providing the treatments outside of a clinical trial may negatively impact the ongoing clinical trials because doing so may result in adverse events that FDA officials consider when evaluating a pending NDA.[159] Because individuals with terminal diseases are more ill than the general population and more likely to react negatively to the pharmaceuticals, the FDA may make an adverse inference about the company's pending NDA in light of the adverse side effects that arise for the right-to-try population.[160] When determining whether to allow a drug to reach market, the FDA considers adverse events that occur during the clinical trial.[161] By providing these drugs to high-risk patients, manufacturers increase the odds that a negative reaction will occur.[162] Although, in theory, the language of the pending federal legislation prohibits the FDA from making such an inference,[163] manufacturers may

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157 Trickett Wendler Right to Try Act of 2016, S. 2912, 114th Cong. § 2(a)(1)(B) (2016).

158 *See supra* Section II.B.

159 It is worth noting that the same concerns manufacturers might have with providing the treatments under state law—that providing individuals with access to the treatments outside a clinical trial may create a disincentive for individuals to participate in clinical trials—would be applicable under the federal legislation. *See* Darrow, et al., *supra* note 141, at 281; *supra* Section II.B.

160 *See* Darrow, et al., *supra* note 141, at 281.

161 *See* 21 C.F.R. § 312.32 (2016).

162 *See* Lurie Statement, *supra* note 53, at 3–4.

163 *See* Trickett Wendler Right to Try Act of 2016, S. 2912, 114th Cong. § 2(b)(2) (2016) ("[T]he outcome of any production, manufacture, distribution, prescribing, dispensing, possession, or use of an experimental drug, biological product or device . . . .

still be wary that subjective impressions may impact the decision of whether to approve a drug.[164] A former GlaxoSmithKline vice president illustrated this dilemma when describing the company's experience in deciding whether to provide an influenza treatment that was in the third stage of its clinical trial to critically ill patients:

> There was some resistance [to provide the drug] because when you're treating patients who are very ill and with end-stage congestive heart failure or who are immunocompromised, and the patient dies, your label will have all this bad stuff on it. . . . They wanted to save these lives, but they also wanted to get the drug approved.[165]

Third, manufacturers may be unwilling to provide treatments because of concerns about potential liability if a patient suffers a negative reaction to the pharmaceutical. The FDA does not allow a pharmaceutical company to exculpate itself from liability in Expanded Access treatment.[166] Although the current federal legislation would explicitly shield manufacturers from liability,[167] this does not prevent a plaintiff from filing a nuisance suit in state court. A federal defense is not enough to move the case to federal court,[168] and the company would be subject to state tort law and the vagaries of the state court systems. In the thirty-eight states that have passed right-to-try laws,[169] this would not be a concern, but the inconsistencies between the states regarding tort immunity could present concerns for manufacturers.

Finally, manufacturers may not provide these treatments to terminal patients because they have only produced enough supplies for clinical trials. Creating product beyond what is required for the trials would

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shall not be used by a Federal agency reviewing the experimental drug . . . or otherwise adversely impact review or approval of such experimental drug . . . .").

164 *See* Lurie Statement, *supra* note 53, at 2.

165 George S. Mack, *Expanded Access Rules Pose Quandary for Drug Developers*, 27 NATURE BIOTECHNOLOGY 871, 872 (2009), https://www.nature.com/articles/nbt1009-871.pdf.

166 21 C.F.R. § 50.20 (2016).

167 *See* S. 2912 § 2(b)(1) ("Notwithstanding any other provision of law, no liability shall lie against a producer, manufacturer, distributor, prescriber, dispenser, possessor or user of an experimental drug . . . .").

168 *See* Louisville & Nashville R.R. v. Mottley, 211 U.S. 149, 153–54 (1908) (holding that a defense that implicates federal law is not a proper basis for removing a case to federal court).

169 Marilyn J. Heine & Bruce E. Johnson, *What Patients Should Know About Pa.'s New 'Right to Try' Legislation*, INQUIRER (Oct. 19, 2017, 8:06 AM), http://www.philly.com/philly/opinion/commentary/what-patients-should-know-about-pa-s-new-right-to-try-legislation-20171019.html.

reallocate resources away from the trials, which may stifle testing and slow down sales and marketing to the general public.[170]

Manufacturers are hesitant to provide treatments under currently written federal legislation because they are concerned about costs, the impacts on clinical trials, and liability, but there are also barriers preventing individuals who desperately need these treatments from accessing them.

2. Barriers for Individuals

Not only do roadblocks exist preventing manufacturers from efficiently providing treatments under a federal right-to-try bill, but individuals seeking the experimental drugs also face challenges in overcoming information gaps and affording treatments that are not covered by insurance. Historically, there has been little transparency concerning who the patient should contact in order to request the treatments, which can result in patients and physicians often finding themselves having to send multiple requests to the drug company to little avail.[171] Some manufacturers recognize this disconnect and independently provide information on their websites or in other public forums.[172] Congress took a step in the right direction in December of 2016 when it passed the 21st Century Cures Act, which required that manufacturers of investigational drugs publish on their websites the contact information for the manufacturer, procedures for making requests, and what information will result in a successful request.[173] Despite these reforms, there is still room for the government to further facilitate communications between patients, doctors, and pharmaceutical companies.

B. The Federal Right-to-Try Bill is Duplicative and Fails to Solve Any Problem Not Already Solved by FDA Expanded Access

A common criticism of the federal right-to-try bill is that it does not help patients with terminal illnesses to any greater extent than the current FDA Expanded Access program.[174] Expanded Access allows patients to access investigational drugs if the patients receive permission from the FDA.[175] Additionally, official FDA data indicates that the Agency approves ninety-nine percent of all requests for investigational drugs filed with the Expanded Access program,[176] and the Agency constantly works to improve the program by lessening the clerical burden on manufacturers, physicians, and patients.[177] Advocates respond that this data may not show the full

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170 *See* Ana Swanson, *How Companies Decide Whether to Give Experimental Drugs to Dying Patients*, WASH. POST (May 7, 2015), https://www.washingtonpost.com/news/wonk/wp/2015/05/07/how-drug-companies-decide-whether-to-give-experimental-drugs-to-dying-patients/?utm_term=.608134bff06b.

picture, as only around 1000 applications are issued each year.[178] Whether these numbers provide a true indication of the number of people who require access to promising yet unapproved drugs, the amount of support behind right-to-try laws from almost two-thirds of the states is evidence that there are still problems Expanded Access has yet to solve.

C. Removing the FDA from the Process Opens Desperate Patients Up to Potential Manipulation from Drug Manufacturers

Another criticism of right-to-try laws is that removing the very FDA oversight that Congress enacted to protect the population opens the door for persons with a terminal illness to be manipulated by drug companies.[179] As the FDA Associate Commissioner for Public Health Strategy and Analysis testified before the Senate Committee on Homeland Security and Governmental Affairs ("HSGAC"), "[the] FDA is concerned about the ability of unscrupulous individuals to exploit such desperate patients."[180] Advocates have dismissed such concerns, noting, as voiced by Senator Johnson during the HSGAC hearing, that it is paternalistic for the federal government to tell an individual they are protecting them by not letting them access the drugs they seek when death is the likely outcome.[181] This criticism is not compelling because it strikes at the core of the what the

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171 *See* Andy Taylor & Alison Bateman-House, *Right to Try Misses the Real Issue. There is Another Solution.*, HILL (Dec. 20, 2016, 4:50 PM), http://thehill.com/blogs/congress-blog/healthcare/311259-right-to-try-misses-the-real-issue-there-is-another-solution.

172 Reem Nasr, *The Right to Try Experimental Drugs Gets More Transparent*, CNBC (May 7, 2015, 3:57 PM), http://www.cnbc.com/2015/05/07/the-right-to-try-experimental-drugs-gets-more-transparent.html.

173 *See* 21st Century Cures Act, Pub. L. No. 114-255, 130 Stat. 1033 (2016).

174 *See* Taylor & Bateman-House, *supra* note 171.

175 *See supra* Section I.A.3.

176 *See* U.S. FOOD & DRUG ADMIN, CBER AND CDER EXPANDED ACCESS IND SUBMISSION AND PROTOCOLS, FY 2010–2015, http://www.fda.gov/downloads/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/UCM471305.pdf (last visited Mar. 1, 2018).

177 *See* Steven Ross Johnson, *FDA Streamlines Application Process for 'Compassionate Use*,' MODERN HEALTHCARE (June 2, 2016), http://www.modernhealthcare.com/article/20160602/NEWS/160609977 [https://perma.cc/N2XY-426T] (noting changes to the FDA program, including cutting the time required to complete an application from 100 hours to forty-five minutes).

178 *See* Cohen-Kurzrock, *supra* note 127.

179 *See* Lurie Statement, *supra* note 53, at 2–3.

180 *See id.* at 3.

181 *See Exploring a Right to Try for Terminally Ill Patients: Hearing Before the S. Comm. on Homeland Security and Gov't Affairs*, 114th Cong. 3 (2016) (statement of Sen. Ron Johnson).

right-to-try movement is arguing: it should be up to the person facing death, and not the government, to decide what treatment is too risky.[182]

Because there are issues with both the state-level right-to-try bills and the current federal proposal, Congress needs to pass legislation that removes the threat of preemption but also works to incentivize manufacturers to actually provide the treatments to patients.

IV. CREATING A GENUINE RIGHT-TO-TRY BILL WILL REQUIRE FEDERAL LEGISLATION, BUT THE SOLUTION NEEDS TO EXPAND UPON THE BILL CURRENTLY PENDING IN CONGRESS

Congress should listen to the demand for federal right-to-try legislation and pass an amended version of the Trickett Wendler Right to Try Act.[183] It should capitalize on the momentum and popular support for right-to-try legislation and add provisions to the bill enabling the FDA to incentivize manufacturers to actually provide the pharmaceuticals. Passing a comprehensive bill that seeks to go beyond a nominal right to try and actually increase access to treatments is the political solution patients truly need.

A. Congressional Action: A Right-to-Try Bill that Enables the FDA to Act as a Facilitator Could Be a Win for Patients and a Win for Bipartisanism

Congress has historically reformed the FDA in response to demands from the public, balancing the public's desire for more safety oversight with the demand for faster access to treatment.[184] Here, Congress faces a grassroots movement where, in just a few years, almost two-thirds of states spanning across the political and economic spectrum have come out in support of this cause.[185] There is political support for legislation that allows manufacturers to provide preapproved pharmaceuticals to those with terminal illnesses without seeking special permission from the FDA.[186] Congress should therefore pass an amended Trickett Wendler Right to Try Act to satisfy this demand. As currently written, the Act allows pharmaceutical companies to distribute investigational drugs in states which have passed "right to trial" legislation and prohibits the FDA from

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182 *See* Lurie Statement, *supra* note 53, at 1.

183 Trickett Wendler Right to Try Act of 2016, S. 2912, 114th Cong. (2016).

184 *See supra* Part I.

185 For example, states that have passed the legislation include highly urbanized, liberal states such as California and rural, conservative states such as Mississippi. *See, e.g.*, CAL. HEALTH & SAFETY CODE § 111548.2(a) (2017); MISS. CODE. ANN. § 41-131-1 (2017).

186 *See* CAL. HEALTH & SAFETY CODE § 111548.2(a); MISS. CODE. ANN. § 41-131-1(2)(a)(i), (b).

considering adverse events resulting from this distribution when evaluating a company's NDA.[187] Prior to passing the Act, Congress should amend the legislation to: (1) direct the FDA to use funds to create an office that works to mediate between patients seeking treatments and pharmaceutical companies creating the investigational drugs and (2) allow companies more flexibility in charging for treatments.

Passing a federal right-to-try bill is necessary to resolve concerns about preemption (and tort liability for manufacturers) by explicitly authorizing pharmaceutical companies to provide the drugs while granting immunity from legal recourse and providing cover for manufacturers concerned about the FDA's use of adverse events, thus encouraging manufacturers to provide the drugs in light of the positive publicity they would receive for their actions.[188] Additionally, by amending the legislation to authorize the FDA to work as an intermediary between the patients and sponsors, Congress would incentivize manufacturers to actually provide the pharmaceuticals instead of just giving them the ability to do so. The 21st Century Cures Act was a step in the right direction in increasing transparency for patients seeking drugs by requiring pharmaceutical companies to publish their Expanded Access policies, but taking a further step to empower the FDA to act as an information intermediary between patients/doctors and pharmaceutical companies would further assist patients in accessing treatments.[189] The FDA already has access to information concerning pharmaceutical companies' investigational drugs through the INDs.[190] The Agency should maintain that information in a centralized database, so the patient or his doctor could simply contact the Agency rather than seeking out the information on various pharmaceutical websites.[191] Additionally, Congress should empower the FDA to facilitate communication between patients/doctors and pharmaceutical companies by setting up meetings and phone calls. This would not only help individuals with the information gap, but it would provide additional assurance that the FDA is involved in distribution of preapproved pharmaceuticals, assuring manufacturers that their actions will be highly unlikely to lead to legal consequences.

Additionally, Congress should give companies who wish to charge to provide the treatments the option to charge a "reasonable administrative

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187 S. 2912 § 2(a)(1)(B), (b)(2).

188 *See supra* Sections II.A., III.A.1.

189 *See* 21st Century Cures Act, Pub. L. No. 114-255, 130 Stat. 1033 (2016).

190 *See supra* Section I.A.2.

191 *See supra* Section III.A.2.

fee"[192] instead of direct costs.[193] The fee can either be negotiated with patient groups or based on the price of comparable treatments.[194] To further protect companies who are sensitive about proprietary cost information leaking before the drug goes to market, the FDA should set up an interest-bearing escrow account.[195] The patient would pay into that account, and the pharmaceutical company would only be able to collect if its product reaches the market. When a product fails to pass approvals, the FDA could use the money collected to subsidize individuals with financial barriers who are seeking these treatments yet unable to pay the reasonable administrative fee.[196]

There is some concern that passing a federal law authorizing state right-to-try laws is superfluous when the FDA Expanded Access program already exists specifically to allow access to unapproved drugs. In other words, the federal and state right-to-try laws are redundant in light of the Expanded Access program. The federal law, however, is necessary for two reasons: (1) it would ensure that pharmaceutical companies do not face liability for tort causes of action, and (2) it would provide the political momentum for Congress to appropriate funds to enable the FDA to facilitate access. Coupling right-to-try legislation, which is popular amongst Republicans, with funding to enable the FDA to act as a facilitator between companies and patients, which would likely draw Democrats,[197] would provide political momentum to pass a bipartisan solution that not only grants a nominal right to try but actually works to provide access to these treatments for terminal patients.

Passing a federal right-to-try law that empowers the FDA to take a greater role in facilitating access and provides pharmaceutical companies more flexibility with price will give patients more than a right to try in name only.

B. Congressional Legislation Is More Appropriate to Solve the Problem than Executive Action

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192 *See supra* Section II.B.

193 *See supra* Section III.A.1.

194 *See supra* Section II.B.

195 *See* Darrow, et al., *supra* note 141, at 285.

196 *See id.* at 284.

197 Providing funding to empower the FDA to help patients may draw the support of Democrats who support the FDA's role in pharmaceutical access. *See*, *e.g.*, Sheila Kaplan, *Republicans Reach Deal to Pass Cures Act by End of Year, but Democrats Pushing for Changes*, STAT NEWS (Nov. 27, 2016), https://www.statnews.com/2016/11/27/cures-act-deal/ (describing initial concerns about the 21st Century Cures Act, including Democrats' fears that expediting the FDA approval processes might weaken the Agency's ability to regulate the safety of the pharmaceuticals).

The President could address some of the issues laid out above through an executive order; however, that is not the ideal course of action because the limitations of executive power, which are noted below, would leave some concerns unaddressed. For example, the President could issue an executive order that prohibits the FDA from prosecuting companies that provide treatments under the authority of state right-to-try laws even though the state provisions would be preempted by the FDCA, or he could issue an executive order authorizing the FDA to act as an intermediary between patients and drug manufacturers. Such an order would skip the arduous legislative process and provide quicker access to investigational drugs for those with serious illnesses.[198]

Although the expediency of an executive order is appealing, particularly for patients with terminal illnesses where time is short, an executive order would be ill-considered. For example, an executive order instructing the FDA not to bring criminal charges against these companies would create serious confusion for pharmaceutical companies. The next president could easily reverse the order, or Congress could itself challenge the President's constitutional authority to essentially rewrite the FDCA.[199] Because an executive order is only in force for as long as the President fails to unilaterally repeal it,[200] drug manufacturers will always lack the predictability necessary to embark on a course of conduct that arguably violates a federal statute with criminal penalties. Taking such actions solely on the goodwill and lark of the President is perilous at best. Additionally, in order to play the role of right-to-try facilitator, the FDA will likely require additional appropriation that an executive order cannot provide.[201] Without a bipartisan solution in Congress, the FDA will lack the necessary funds to assist patients, thus allowing this program to wither on the vine. Because executive orders can be undone by the legislative, judicial, and even executive branch (under a different administration), an executive solution to the right-to-try problem is unpredictable and should be disregarded in favor of a Congressional solution.

CONCLUSION

198 *See* John C. Duncan, Jr., *A Critical Consideration of Executive Orders: Glimmerings of Autopoiesis in the Executive Role*, 35 VT. L. REV. 333, 400 (2010) ("Executive orders are a quicker remedy than congressional legislation . . . .").

199 *See* Todd F. Gaziano, *The Use and Abuse of Executive Orders and Other Presidential Directives*, 5 TEX. REV. L. & POL., 267, 281, 291 (2001).

200 *See id.* at 281.

201 The Appropriations Clause in the Constitution exclusively grants Congress the ability to allocate money. U.S. CONST. art. I, § 9, cl. 7.

Although a federal right-to-try law should lead to quicker access to life-changing medication for Joshua and other terminally ill patients, others in this same situation may face significant barriers to treatment even with a federal law in place. To avoid this deadly consequence, Congress should pass a federal right-to-try law that allows states with their own local right-to-try laws to move forward in immunizing drug manufacturers that distribute unapproved treatments to these patients. To make the law effective in practice, however, Congress should also amend the legislation to empower the FDA to connect patients with pharmaceutical companies and incentivize manufacturers to provide the treatments to terminal patients. This solution would ensure that the government supports patients, like Joshua, in their battles with terminal illness rather than blocking them from the medication that may save their lives.