

Symposium

Science Challenges for Law and Policy

The “Right to Try” Investigational Drugs: Science and Stories in the Access Debate

Rebecca Dresser*

In 2014, states began enacting laws giving terminally ill patients a “right to try” investigational drugs. Right-to-try laws are the latest policy development in a decades-long struggle between advocates of liberal access to investigational drugs and defenders of access restrictions. According to access advocates, physician opinion and minimal safety testing are an adequate scientific basis for allowing terminally ill patients to try investigational drugs. But science and policy experts are virtually unanimous in criticizing right-to-try laws. According to the experts, more rigorous scientific and regulatory oversight is necessary to justify wide patient access. In defense of their position, experts cite data on investigational drug risks and low success rates, as well as the public interest in a rigorous drug-evaluation system. Access advocates use a different strategy, however—one that highlights stories of patients and families pleading for investigational drugs. These stories strongly influence legislative and public opinion on access policy. To mount an effective response, experts must tell stories illustrating the harm that liberal access can produce. In this arena, experts must convey their concerns in ways that are meaningful to lay decision makers.

Introduction

In 2014, several state legislatures confronted a novel policy proposal. They were asked to consider bills recognizing the terminally ill patient’s right to try investigational drugs.¹ The bills sought to allow patients to use investigational drugs without the United States Food and Drug Administra-

* J.D. Daniel Noyes Kirby Professor of Law, Professor of Ethics in Medicine, Washington University in St. Louis.

1. Right-to-try laws define terminal illness, with minor variations in language, as “a disease that without life-sustaining procedures will result in death in the near future or a state of permanent unconsciousness from which recovery is unlikely.” MO. ANN. STAT. § 191.480.1(3) (West Supp. 2015). *Accord* ARIZ. REV. STAT. ANN. § 36-1311.4 (Supp. 2014) (defining “terminal illness” for the purposes of the right-to-try law); COLO. REV. STAT. ANN. § 25-45-103(3) (West Supp. 2014) (same); LA. REV. STAT. ANN. § 40:1300.423(3) (Supp. 2015) (same); MICH. COMP. LAWS ANN. § 333.26451.1(2)(a) (West Supp. 2014) (defining “advanced illness” for the purposes of Michigan’s right-to-try law).

tion's (FDA's) permission.² Five states adopted right-to-try laws in 2014, and by early 2015, right-to-try bills had been introduced in many other states.³

Right-to-try laws are the latest policy development in a decades-long struggle between advocates of liberal access to investigational drugs and defenders of access restrictions. According to liberal-access advocates, physician opinion and minimal safety testing are an adequate scientific basis for allowing terminally ill patients to try investigational drugs. But scientific and other experts dispute this claim. Right-to-try laws would do more harm than good, experts say, by exposing patients to risky and ineffective agents. The laws would also pose an unacceptable threat to the larger group of patients who benefit from receiving drugs that have undergone thorough human testing. Experts predict that if terminally ill patients can easily obtain investigational drugs, fewer patients will be willing to participate in the clinical trials that determine which drugs can actually help patients live longer and better lives.

Experts are nearly unanimous in opposing right-to-try laws. In defense of access oversight, scientists, FDA officials, and policy experts cite data on investigational-drug risks and low success rates, as well as the need for a rigorous drug-evaluation system. But in the access debate, data and abstract policy considerations go only so far. Access advocates use a different strategy, one that highlights individual patients' stories. To support their cause, access advocates offer heartrending accounts of terminally ill patients seeking investigational drugs and deceased patients who were denied such drugs. These stories strongly influence legislative and public opinion on the access question.⁴

It's inevitable that patients' stories will shape public and legislative opinion on access policy. But policy decisions should take into account the full range of patient experiences with investigational drugs. Stories illustrating the harm that can come from liberal access belong in the debate too. An adequate response to the right-to-try campaign will require experts to vividly describe the negative impact that liberal access can have on patients.

2. See Editorial, *Quicker Access to Experimental Drugs*, N.Y. TIMES, Feb. 12, 2015, <http://nyti.ms/1Mfk0GW>, archived at <http://perma.cc/6FG8-3Y95> (reporting that right-to-try laws and bills require that drugs have passed at least the first phase of FDA testing, but give patients access to drugs that "are still years away from reaching pharmacy shelves").

3. By late March 2015, a total of twelve states had adopted right-to-try laws. Press Release, Starlee Coleman, Goldwater Institute, Mississippi Becomes 12th State to Allow Terminally Ill to Access Investigational Medications (Mar. 1, 2015), available at <http://www.goldwaterinstitute.org/en/work/topics/healthcare/right-to-try/mississippi-becomes-12th-state-to-allow-terminally/>, archived at <http://perma.cc/PE8G-V7YG> (noting that eleven other states had already passed right-to-try laws when Mississippi enacted its own Mississippi Right to Try Act).

4. As one contributor to the access debate observed, patients' stories "have the power to reach a public who has little understanding of the research enterprise." Musa Mayer, *When Clinical Trials Are Compromised: A Perspective from a Patient Advocate*, 2 PLOS MED. 1060, 1062 (2005).

In this Article, I examine the right-to-try controversy and the role patient stories play in the debate. Part I reviews the history of investigational drug regulation and current rules governing investigational drug access. In Part II, I describe ethical and policy arguments for and against liberal access rules. To illustrate the potential benefits and harms of liberal access, Part III presents a variety of patients' access stories. Part III also considers interview and survey data examining patients' attitudes toward and experiences with investigational drugs. I conclude by urging experts to address access in ways that are meaningful to legislators and the public. Experts should use stories and other information on patients' experiences to describe the full range of effects that investigational drugs can produce. Such information is essential to developing access policies that truly promote patients' interests.

I. Investigational Drug Regulation

A. Drug Evaluation

In 1938, Congress passed legislation requiring manufacturers to file applications with the FDA attesting to a drug's safety prior to marketing the drug.⁵ To gain approval, manufacturers had to submit a New Drug Application for FDA review, giving the FDA the opportunity to bar the drug from being sold if it concluded that the drug was unsafe.⁶ In 1962, Congress strengthened the FDA approval standard, demanding evidence that drugs were both safe and effective for medical use.⁷ Federal law prohibits manufacturers from shipping drugs in interstate commerce without FDA permission.⁸

Evidence from human trials is necessary to demonstrate drug safety and effectiveness.⁹ But before drug sponsors¹⁰ may conduct a trial, they must obtain from the FDA an Investigational New Drug (IND) exemption allowing them to ship drugs for testing purposes.¹¹ Agency officials review clinical-

5. *Abigail Alliance for Better Access to Developmental Drugs v. von Eschenbach (Abigail Alliance II)*, 445 F.3d 470, 482 (D.C. Cir. 2006), *rev'd*, 495 F.3d 695 (D.C. Cir. 2007) (en banc).

6. *Id.*

7. *Id.*

8. 21 U.S.C. § 355(a) (2012).

9. *See* 21 U.S.C. 355(d) (2012) (requiring substantial evidence of a drug's efficacy prior to FDA approval and defining substantial evidence to include clinical investigations of the safety and efficacy of the drug).

10. Sponsors are normally drug companies hoping to market the drugs but may also be research institutions or nonprofit organizations. *The FDA's Drug Review Process: Ensuring Drugs Are Safe and Effective*, U.S. FOOD & DRUG ADMIN., <http://www.fda.gov/drugs/resourcesforyou/consumers/ucm143534.htm>, archived at <http://perma.cc/3E7S-AW9Q> (defining sponsors as "companies, research institutions, and other organizations that take responsibility for developing a drug").

11. *Investigational New Drug (IND) or Device Exemption (IDE) Process (CBER)*, U.S. FOOD & DRUG ADMIN., <http://www.fda.gov/BiologicsBloodVaccines/DevelopmentApprovalProcess/In>

trial proposals to ensure that they are based on adequate scientific evidence and will not expose subjects to undue risk.¹²

The FDA divides investigational drug trials into three phases. Phase I trials are typically conducted on twenty to eighty human subjects.¹³ These trials supply initial information about a drug's adverse effects in humans and help researchers determine a reasonably safe drug dosage.¹⁴ Phase I trials are not designed to evaluate effectiveness, although they sometimes generate early information suggesting that a drug might be effective.¹⁵

If a drug appears to have an acceptable safety profile, the FDA will permit a phase II study.¹⁶ Up to several hundred subjects with the disease the drug is targeting participate in phase II trials.¹⁷ In these trials, researchers continue to collect data on side effects and risks but also look for preliminary evidence of the drug's potential effectiveness.¹⁸ If phase II-trial evidence suggests that the drug presents an acceptable balance of risks and potential benefits, officials allow the sponsor to conduct phase III trials evaluating safety and effectiveness in a larger number of patients.¹⁹ If phase III trials yield acceptable results, sponsors may apply for FDA approval to market the drug.²⁰

About 70% of the investigational drugs undergoing phase I testing are found safe enough to advance to phase II trials.²¹ But many of those drugs fail to demonstrate sufficient safety and effectiveness to advance to phase III

vestigationalNewDrugINDorDeviceExemptionIDEProcess/, archived at <http://perma.cc/KR8G-45HW>.

12. *The FDA's Drug Review Process: Ensuring Drugs Are Safe and Effective*, *supra* note 10.

13. *Id.*

14. *See id.* (noting that the goal of phase I trials is to determine a drug's most frequent side effects and that phase II trials do not begin if phase I trials show that the drug is unacceptably toxic).

15. 21 C.F.R. § 312.21(a) (2014) (“[Phase I] studies are designed to determine the metabolism and pharmacologic actions of the drug in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness.”).

16. *See id.* (stating that phase I studies should produce “sufficient information about the drug’s pharmacokinetics and pharmacological effects” to allow sponsors to design “well-controlled, scientifically valid” phase II studies).

17. *See* § 312.21(b) (commenting that phase II trials usually involve no more than several hundred subjects and are conducted to evaluate the “effectiveness of the drug for a particular indication or indications”).

18. *See id.* (“Phase [II] includes the . . . studies conducted to evaluate the effectiveness of the drug for a particular indication or indications in patients with the disease or condition under study and to determine the common short-term side effects and risks associated with the drug.”).

19. *See* § 312.21(c) (describing phase III studies as “expanded . . . trials . . . performed after preliminary evidence suggesting effectiveness of the drug has been obtained, and . . . intended to gather the additional information about effectiveness and safety that is needed to evaluate the overall benefit-risk relationship of the drug”).

20. *See* 21 C.F.R. § 312.22(a) (2012) (explaining that phase III is aimed at assessing whether the “investigations will yield data capable of meeting statutory standards for [FDA] marketing approval”).

21. *Overview of Clinical Trials*, CENTERWATCH, <http://www.centerwatch.com/clinical-trials/overview.aspx>, archived at <http://perma.cc/QEU7-U9BT>.

testing. Only about one-third of investigational drugs are successful in both phase I and II trials,²² and as one expert put it, "[t]he bottleneck is in Phase II."²³

Not many investigational drugs make it through the full evaluation process. In 2013, an expert group estimated that about one in six drugs entering human testing is approved for clinical use.²⁴ A 2014 study was even less encouraging, putting the figure at one in ten.²⁵ Cancer drugs, which are often the subject of patient-access requests, have a lower-than-average success rate.²⁶ And even FDA-approved cancer drugs help just a small segment of patients.²⁷

B. History of FDA Access Policy

Not all seriously ill patients are eligible to participate in clinical trials evaluating investigational drugs. Because sponsors test drugs for a limited number of medical uses, patients must meet specific eligibility criteria to enter a trial.²⁸ Excluded from trials are patients whose illness differs from the condition under study.²⁹ Some patients live too far away from trial centers to participate.³⁰ A patient's age or exposure to previous treatments can lead to trial ineligibility too.³¹

Other patients are eligible for trials but decide against enrolling. Some are unwilling to accept extra hospital visits, tests, and other study demands.³²

22. *Id.* See also Benjamin P. Falit & Cary P. Gross, Commentary, *Access to Experimental Drugs for Terminally Ill Patients*, 300 JAMA 2793, 2793 (2008) (explaining that 34% of oncology drugs entering phase II trials are eventually approved).

23. Gregory A. Petsko, *When Failure Should Be the Option*, 8 BMC BIOLOGY, art. no. 61, at 2 (2010), <http://www.biomedcentral.com/content/pdf/1741-7007-8-61.pdf>, archived at <http://perma.cc/HU82-LUBG>.

24. JA DiMasi et al., *Trends in Risks Associated with New Drug Development: Success Rates for Investigational Drugs*, 87 CLINICAL PHARMACOLOGY & THERAPEUTICS 272, 276 (2010).

25. Michael Hay et al., *Clinical Development Success Rates for Investigational Drugs*, 32 NATURE BIOTECHNOLOGY 40, 41 (2014).

26. See *id.* at 42, 44 (finding that oncology drugs had the lowest likelihood of approval, at around one in fifteen drugs being approved).

27. See Manish Agrawal & Ezekiel J. Emanuel, Special Communication, *Ethics of Phase I Oncology Studies*, 290 JAMA 1075, 1075-76 (2003) (noting that only around 5% of cancer drugs going through phase I testing have clinical benefits, but they have significant toxic side effects); Falit & Gross, *supra* note 22, at 2793 (observing that "only a fraction" of patients receiving approved cancer drugs experience a benefit).

28. See Stephen L. George, *Reducing Patient Eligibility Criteria in Cancer Clinical Trials*, 14 J. CLINICAL ONCOLOGY 1364, 1365 (1996) (remarking that eligibility criteria aimed at the objectives of a clinical trial are necessary because a "clinical trial is a scientific study of medical interventions").

29. *Id.*

30. *Id.*

31. *Id.* at 1370 tbl.4.

32. Julie Brintnall-Karabelas et al., *Improving Recruitment in Clinical Trials: Why Eligible Participants Decline*, 6 J. EMPIRICAL RES. ON HUM. RES. ETHICS 69, 70 (2011).

And some decline to participate because they fear they will be assigned to a control group that will receive an inactive placebo or a standard therapy with low success rates.³³

At times, patients unable or unwilling to enroll in clinical trials seek to receive investigational drugs from their treating physicians. During the 1960s, the FDA operated an informal program to allow “compassionate use” of investigational drugs by selected patients.³⁴ The HIV/AIDS epidemic gave rise to the first broad expanded-access program.³⁵ Activists seeking measures to combat this deadly disease refused to accept a testing process that kept novel drugs out of the hands of patients for many years.³⁶ By 1987, activists had convinced FDA officials to allow more patients to obtain investigational drugs outside of trials.³⁷

The 1987 expanded-access regulations applied to patients with serious or immediately life-threatening conditions who were unable to enroll in clinical trials and had no reasonable treatment alternatives.³⁸ Physicians and drug sponsors could submit applications justifying the use of investigational drugs to treat qualified patients.³⁹ The FDA would issue “treatment protocols” and “treatment INDs” if certain criteria were met.⁴⁰ To ensure that treatment access did not interfere with drug evaluation, the agency would grant access only if clinical trials on the drug were in process or completed and the sponsor was “actively pursuing marketing approval.”⁴¹

The access regulations said that FDA officials would ordinarily make investigational drugs available to patients during or following completion of phase III trials.⁴² But in “appropriate circumstances,” including the presence of an immediately life-threatening condition, drugs could be made available

33. Manik Chahal, *Off-Trial Access to Experimental Cancer Agents for the Terminally Ill: Balancing the Needs of Individuals and Society*, 36 J. MED. ETHICS 367, 368 (2010). See also Brintnall-Karabelas et al., *supra* note 32, at 70 (reporting “disinterest in a placebo-controlled study” as a reason for nonparticipation).

34. *Clinical Trial Subjects: Adequate FDA Protections?: Hearing Before the H. Comm. on Gov't Reform & Oversight*, 105th Cong. 58 (1998) (statement of Michael A. Friedman, M.D., Lead Deputy Comm'r, Food and Drug Administration).

35. See REBECCA DRESSER, *WHEN SCIENCE OFFERS SALVATION: PATIENT ADVOCACY AND RESEARCH ETHICS* 48–49 (2001) (describing the HIV/AIDS emergency as the “driving force” for FDA action).

36. *Id.* at 48.

37. *Id.* See generally Jonathan J. Darrow et al., *New FDA Breakthrough-Drug Category—Implications for Patients*, 370 NEW ENG. J. MED. 1252, 1253 (2014) (reviewing access-promoting FDA policies).

38. *Investigational New Drug, Antibiotic, and Biological Drug Product Regulations; Treatment Use and Sale*, 52 Fed. Reg. 19,466, 19,476 (May 22, 1987) (to be codified at 21 C.F.R. pt. 312).

39. *Id.* at 19,477.

40. *Id.*

41. *Id.* at 19,476.

42. *Id.*

during phase II testing.⁴³ Officials would deny a patient's request if the existing scientific evidence failed to provide a reasonable basis for concluding that the drug could be effective or if the drug presented "an unreasonable and significant additional risk of illness or injury."⁴⁴

The access regulations required documentation of patients' informed consent.⁴⁵ An Institutional Review Board (IRB) had to approve treatment uses as well.⁴⁶ In emergency situations, the FDA could permit treatment use without a formal submission, but only if the drug sponsor agreed to submit a formal request soon after the emergency authorization.⁴⁷ The regulations allowed sponsors to recover the costs of supplying investigational drugs but did not allow them to profit from the transaction.⁴⁸

Although hundreds of patients received investigational drugs through the FDA program, critics complained that it was applied unfairly and inconsistently.⁴⁹ They also said that the process was so demanding and time-consuming that many terminally ill patients were unable to benefit from it.⁵⁰

Eventually the Abigail Alliance for Better Access to Developmental Drugs, a patient-advocacy organization, challenged the regulations. The Alliance was founded by the father of Abigail Burroughs, a young woman who had sought to obtain two different investigational drugs for cancer treatment.⁵¹ Burroughs was ineligible for clinical trials evaluating the drugs, and the sponsors refused to include her in their limited compassionate-use programs.⁵² Although the FDA was not involved in the denial, the Alliance argued that the agency's failure to allow sponsors to market unapproved drugs created an unjustified impediment to patient access.⁵³

43. *Id.*

44. *Id.*

45. *Id.* at 19,476–77.

46. *Id.* Institutional Review Boards are interdisciplinary committees that review human-study proposals to ensure that they meet ethical and regulatory research standards. *Institutional Review Boards Frequently Asked Questions*, U.S. FOOD & DRUG ADMIN., <http://www.fda.gov/regulatoryinformation/guidances/ucm126420.htm>, archived at <http://perma.cc/U4QK-3K6Q>.

47. 21 C.F.R. § 312.36 (1987).

48. Investigational New Drug, Antibiotic, and Biological Drug Product Regulations; Treatment Use and Sale, 52 Fed. Reg. at 19,476.

49. Expanded Access to Investigational Drugs for Treatment Use, 71 Fed. Reg. 75,147, 75,149 (proposed Dec. 14, 2006) (to be codified at 21 C.F.R. pt. 312).

50. Expanded Access to Investigational Drugs for Treatment Use, 71 Fed. Reg. at 75,149–50.

51. *Our Story*, ABIGAIL ALLIANCE, <http://www.abigail-alliance.org/story.php>, archived at <http://perma.cc/V2EP-7R3K>.

52. Judy Foreman, *Dying Patient's Fight to Access Trial Drugs Met with Resistance*, BALTIMORE SUN, Sept. 29, 2003, http://articles.baltimoresun.com/2003-09-29/news/0309290141_1_experimental-drugs-abigail-burroughs-new-drugs, archived at <http://perma.cc/3KGA-DRU9>.

53. See *Abigail Alliance for Better Access to Developmental Drugs v. von Eschenbach (Abigail Alliance III)*, 495 F.3d 695, 699 (D.C. Cir. 2007) (noting that the Abigail Alliance asked "the FDA to promulgate new regulations that would allow sponsors to market experimental drugs, under some circumstances, after the completion of Phase I trials").

The Alliance initially filed a citizen petition asking the FDA to allow sponsors to market drugs to certain patients after phase I testing.⁵⁴ The Alliance had previously made similar requests to the FDA, but officials had concluded that the Alliance's proposals "would upset the appropriate balance [in drug regulation] . . . by giving almost total weight to the goal of early availability and giving little recognition to the importance of marketing drugs with reasonable knowledge for patients and physicians of their likely clinical benefit and their toxicity."⁵⁵

Expecting another negative FDA response, the Alliance turned to the federal courts for relief. The organization filed a complaint asserting that the FDA's expanded-access rules violated the terminally ill patient's constitutionally protected right of access to investigational drugs that have undergone phase I testing.⁵⁶ The D.C. district court dismissed the complaint, finding that no such constitutional right existed.⁵⁷ Later, a three-judge panel of the D.C. Circuit Court of Appeals reversed that finding.⁵⁸ On rehearing, however, a majority of the en banc court of appeals rejected the Alliance's claim that the Due Process Clause of the Fifth Amendment protects "the right of a terminally ill patient with no remaining approved treatment options to decide, in consultation with his or her own doctor, whether to seek access to investigational medications that the [FDA] concedes are safe and promising enough for substantial human testing."⁵⁹

The court majority cited an extensive record of state and federal drug regulation to counter the Alliance's argument that the nation's history and legal traditions protected the terminally ill patient's right to access experimental drugs.⁶⁰ Although government regulatory efforts initially focused on drug safety, effectiveness standards emerged as soon as scientists developed the randomized clinical trial methods that could discern whether drugs actually worked.⁶¹ Contrary to the Alliance's claim, the government's earlier failure to regulate drug effectiveness did not signify respect for patients' access rights; instead, the lack of regulation was a by-product of the inability to detect which drugs were effective.⁶²

The Alliance also contended that the terminally ill patient's constitutional right to use investigational drugs was grounded in three common law

54. *Id.*

55. *Id.* at 700 (internal quotation marks omitted).

56. *Abigail Alliance for Better Access to Developmental Drugs v. McClellan*, No. 03-1601, 2004 WL 3777340, at *2 (D.D.C. Aug. 30, 2004), *aff'd*, *Abigail Alliance III*, 495 F.3d 695 (D.C. Cir. 2007) (en banc).

57. *Id.* at *1.

58. *Abigail Alliance II*, 445 F.3d 470, 472 (D.C. Cir. 2006).

59. *Abigail Alliance III*, 495 F.3d at 701 (alteration in original).

60. *Id.* at 703-06.

61. *Id.* at 703-06, 706 n.12.

62. *Id.* at 706 n.12.

doctrines protecting individuals in life-threatening situations. According to the Alliance, the right to self-defense, the necessity defense, and the tort of intentional interference with lifesaving efforts support recognition of a constitutional right to self-preservation, one that should extend to terminally ill patients seeking investigational drugs.⁶³ But the appellate court rejected these claims, emphasizing the lack of evidence that post-phase I drugs qualify as necessary and reasonable lifesaving measures.⁶⁴

C. Current Access Regulations

The D.C. Circuit ruled that investigational-drug access issues should be resolved through the democratic process.⁶⁵ Although members of Congress sympathetic to the Alliance's position sponsored bills to widen access, none of them were enacted.⁶⁶ Eventually, however, congressional and advocacy-group pressure led FDA officials to issue revised expanded access regulations.⁶⁷

In 2009, the FDA adopted new rules establishing criteria for treatment use of investigational drugs in three situations: individual patients, intermediate-sized patient groups, and larger patient populations.⁶⁸ Most of

63. *Id.* at 703. The doctrine of necessity is a common law defense that applies when a person engages in otherwise criminal conduct to avoid a greater harm or evil. Self-defense principles cover people using reasonable force to protect themselves from aggressors. See O. Carter Snead, *Unenumerated Rights and the Limits of Analogy: A Critique of the Right to Medical Self-Defense*, 121 HARV. L. REV. F. 1, 6–12 (2007) (comparing traditional self-defense and necessity justifications to the theory of self-defense and necessity in the medical setting). The tort of intentional interference applies when one person intentionally prevents another from saving the life of a third person. See *Abigail Alliance III*, 495 F.3d at 708–09 (discussing the Restatement of Tort's definition of intentional interference and its application to medical lifesaving efforts).

64. *Abigail Alliance III*, 495 F.3d at 705–06. The U.S. Supreme Court denied the Abigail Alliance's petition for certiorari. *Abigail Alliance for Better Access to Developmental Drugs v. von Eschenbach*, 552 U.S. 1159, 1159 (2008), *denying cert. to Abigail Alliance III*, 495 F.3d 695 (2004). In an earlier case, the Supreme Court had upheld the FDA's power to prohibit distribution of Laetrile, an alleged cancer treatment that had not undergone any clinical testing. *United States v. Rutherford*, 442 U.S. 544, 549, 551 (1979). The Court said it was permissible for the agency to apply safety and effectiveness standards to protect terminally ill patients from toxic and ineffective remedies like Laetrile. *Id.* at 554–55.

65. *Abigail Alliance III*, 495 F.3d at 713–14.

66. See, e.g., Access, Compassion, Care, and Ethics for Seriously Ill Patients Act, S. 1956, 109th Cong. (2005) (attempting to amend the Federal Food, Drug, and Cosmetic Act to create a new approval program that would be responsive to the needs of seriously ill patients).

67. See Expanded Access to Investigational Drugs for Treatment Use, 71 Fed. Reg. 75,147, 75,149 (proposed Dec. 14, 2006) (to be codified at 21 C.F.R. pt. 312) (stating that the proposed rule was "intended to further address the concerns that motivated Congress to include in the act specific provisions on expanded access to investigational drugs").

68. Expanded Access to Investigational Drugs for Treatment Use, 74 Fed. Reg. 40,900, 40,942–45 (Aug. 13, 2009) (to be codified at 21 C.F.R. pt. 312, 316).

the substantive requirements in the new rules resemble those in the 1987 policy, but there are a few notable changes.⁶⁹

The 2009 regulations permit investigational-drug access early in the testing process. In most cases, FDA officials will make drugs available to patients with an “immediately life-threatening disease” during phase II trials.⁷⁰ In certain cases, however, officials will make drugs available before phase I testing is complete.⁷¹ And in exceptional circumstances, officials will make an investigational drug available to patients in the absence of any data on its human effects.⁷² Thus, the current FDA policy is in some respects more liberal than the Abigail Alliance’s proposal to permit treatment access after phase I testing.

D. State Right-to-Try Laws

Despite its liberal-access provisions, the current FDA policy fails to satisfy some access advocates. Frustrated by their lack of success at the federal level, advocates seeking less restrictive rules have turned to state legislatures. In 2014, the Goldwater Institute, a nonprofit organization whose mission is to protect freedom and prosperity,⁷³ developed a model bill “to protect the fundamental right of people to try to save their own lives.”⁷⁴ By January 2015, legislatures in Colorado, Missouri, Louisiana, Michigan, and Arizona had adopted versions of the model legislation.⁷⁵

State right-to-try laws vary, but they all purport to give terminally ill patients access to investigational drugs without the FDA’s permission. The laws permit manufacturers to supply investigational drugs that have completed phase I testing to patients whose physicians have recommended

69. The regulations still do not allow companies to profit from providing investigational drugs. 21 C.F.R. § 312.8(c) (2014). A 2013 FDA guidance document describes how officials determine whether treatment access would interfere with the clinical trial process. FOOD & DRUG ADMIN., GUIDANCE FOR INDUSTRY: EXPANDED ACCESS TO INVESTIGATIONAL DRUGS FOR TREATMENT USE—QS & AS 9–10 (2013).

70. Expanded Access to Investigational Drugs for Treatment Use, 74 Fed. Reg. at 40,945.

71. *See id.* at 40,912–13 (arguing for “flexibility in the evidentiary standards” applied to individual patient requests).

72. *See id.* at 40,912 (noting that treatment use of unapproved drugs “may be sought quite early in a drug’s development, and at any point during the development”).

73. *About*, GOLDWATER INSTITUTE, <http://www.goldwaterinstitute.org/en/about/>, archived at <http://perma.cc/DTD9-W974>.

74. CHRISTINA CORIERI, GOLDWATER INST., EVERYONE DESERVES THE RIGHT TO TRY: EMPOWERING THE TERMINALLY ILL TO TAKE CONTROL OF THEIR TREATMENT 1 (2014), available at https://goldwater-media.s3.amazonaws.com/cms_page_media/2015/1/28/Right%20To%20Try.pdf, archived at <https://perma.cc/RRZ2-WSHV?type=pdf>.

75. ARIZ. REV. STAT. ANN. §§ 36-1311 to -1312 (Supp. 2014); COLO. REV. STAT. ANN. §§ 25-45-101 to -104 (West Supp. 2014); LA. REV. STAT. ANN. §§ 40:1300.421–.426 (Supp. 2015); MICH. COMP. LAWS ANN. §§ 333.26451–.26457 (West Supp. 2014). In 2014, members of the U.S. House of Representatives introduced the Compassionate Freedom of Choice Act, a bill with aims similar to those of state right-to-try laws. H.R. 4475, 113th Cong. (2014).

the drugs.⁷⁶ Patients must provide written informed consent⁷⁷ and manufacturers may charge patients for the costs of supplying drugs.⁷⁸

All right-to-try laws bar state licensing boards from taking disciplinary action against physicians prescribing investigational drugs.⁷⁹ Some of the laws confer a degree of protection from civil liability on clinicians and manufacturers providing investigational drugs to patients.⁸⁰ But right-to-try laws do not require manufacturers to supply investigational drugs to patients, nor do they require insurers to cover the costs associated with use of those drugs.⁸¹

II. The Policy Debate

State right-to-try laws seek to dispense with FDA review of terminally ill patients' requests for access to investigational drugs. Access advocates contend that patients and doctors can make good decisions on their own and that regulatory-review requirements are unjustifiably paternalistic.⁸² Defenders of access restrictions argue that both patient and societal interests warrant FDA oversight. They also characterize right-to-try laws as "nothing but feel-good placebos" that will have no real impact on drug access.⁸³

A. Individual Autonomy vs. Patient Protection

According to access advocates, the choice to gamble on a novel intervention is not a scientific or public-health concern but a decision for patients and their doctors.⁸⁴ Once a drug has undergone phase I testing, access advocates believe that physicians are competent to determine whether

76. *E.g.*, ARIZ. REV. STAT. ANN. §§ 36-1311 to -1312; COLO. REV. STAT. ANN. §§ 25-45-103 to -104; MICH. COMP. LAWS ANN. §§ 333.26451-.26452; MO. ANN. STAT. § 191.480 (West Supp. 2015).

77. Some right-to-try laws include specific disclosure requirements. For example, the Missouri statute requires consent documents to be "at least as comprehensive as the consent used in clinical trials for the use of the investigational drug." MO. ANN. STAT. § 191.480.1(1)(d). In Colorado, patients must sign a document that "describes the potentially best and worst outcomes of using the investigational drug, . . . with a realistic description of the most likely outcome, including the possibility that new, unanticipated, different, or worse symptoms might result, and that death could be hastened by the proposed treatment." COLO. REV. STAT. ANN. § 25-45-103(4)(d).

78. *E.g.*, ARIZ. REV. STAT. ANN. § 36-1312(b)(2).

79. *E.g.*, MO. ANN. STAT. § 191.480.5.

80. *E.g.*, COLO. REV. STAT. ANN. § 25-45-107 (limiting civil liability of manufacturers and clinicians).

81. *E.g.*, COLO. REV. STAT. ANN. § 25-45-104(1), (3)(b).

82. See CORIERI, *supra* note 74, at 15 (arguing that despite the FDA's concerns about the risks of expanded access, patients understand and are realistic about those risks).

83. David Gorski, "Right to Try" Laws and Dallas Buyers' Club: Great Movie, Terrible for Patients and Terrible Policy, SCI. BASED MED. (Mar. 8, 2014), <http://www.sciencebasedmedicine.org/right-to-try-laws-and-dallas-buyers-club-great-movie-terrible-public-policy/>, archived at <http://perma.cc/M8VY-XW7P>.

84. CORIERI, *supra* note 74, at 1.

investigational drugs are promising enough for patients to try.⁸⁵ Claims that patients need FDA protection because they are desperate and vulnerable demean individuals who are fully capable of making treatment decisions.⁸⁶ To the contrary, terminally ill patients are rational and realistic people dealing with difficult circumstances.⁸⁷ To access advocates, “no-one is better placed than the person with the condition, or their family, to make a judgment about the level of risk that is worthwhile.”⁸⁸

Access advocates criticize the FDA’s substantive standards governing patient access, but what they really object to is the review requirement itself. Officials at the FDA grant nearly every request for treatment access and do so quickly in urgent cases.⁸⁹ But preparing a request can be time-consuming.⁹⁰ Right-to-try advocates say that the burden of preparing a

85. See, e.g., Nicole E. Lombard, Note, *Paternalism vs. Autonomy: Steps Toward Resolving the Conflict over Experimental Drug Access Between the Food and Drug Administration and the Terminally Ill*, 3 J. HEALTH & BIOMEDICAL L. 163, 186 (2007) (arguing that it is better for a “patient’s own doctor to determine whether his or her terminally ill patient is a suitable candidate for [an] experimental treatment”). With the possible exception of leading experts, it is hard to see how physicians could make good decisions about early-phase drugs when so little is known about their safety and effectiveness. See David W. Borhani & J. Adam Butts, Letter, *Rethinking Clinical Trials: Biology’s Mysteries*, 334 SCIENCE 1346, 1347 (2011) (questioning whether physicians should prescribe “unproven drugs . . . when faced with volumes of uncertain data”).

86. See, e.g., Les Halpin et al., *Improving Access to Medicines: Empowering Patients in the Quest to Improve Treatment for Rare Lethal Diseases*, J. MED. ETHICS (ONLINE FIRST), July 9, 2013, at 2 (describing it as “excessively paternalistic” to bar informed, terminally ill patients from trying unproven interventions); Eugene Volokh, *Medical Self-Defense, Prohibited Experimental Therapies, and Payment for Organs*, 120 HARV. L. REV. 1813, 1829–30 (2007) (challenging paternalistic restrictions on an individual’s right to use potentially lifesaving investigational drugs).

87. See Vicki Brower, *Food and Drug Administration Responds to Pressure for Expanded Drug Access*, 106 J. NAT’L CANCER INST. dju171, dju172 (2014) (quoting a doctor who asserted that patients seeking expanded access “well understand that their risk of toxicity is not trivial”); Chahal, *supra* note 33, at 368 (noting that terminally ill patients are considered competent to consent to participate in phase I trials evaluating drugs with unknown risks and little chance of benefit); John A. Robertson, *Controversial Medical Treatment and the Right to Health Care*, HASTINGS CENTER REP., Nov.–Dec. 2006, at 15, 17 (suggesting that there is no reason to believe a cancer patient’s choice to use an untested drug is irrational or any different than a choice to participate in a phase I or phase II trial of that same drug).

88. Simon Woods & Pauline McCormack, *Disputing the Ethics of Research: The Challenge from Bioethics and Patient Activism to the Interpretation of the Declaration of Helsinki in Clinical Trials*, 27 BIOETHICS 243, 248 (2013).

89. See Kelly Servick, “Right to Try” Laws Bypass FDA for Last-Ditch Treatments, 344 SCIENCE 1329, 1329 (2014) (commenting that the FDA received almost one thousand access requests in 2013 and granted all but three); Jerome Groopman, *The Right to a Trial*, NEW YORKER, Dec. 18, 2006, <http://www.newyorker.com/magazine/2006/12/18/the-right-to-a-trial>, archived at <http://perma.cc/GKZ2-R2HJ> (stating that an FDA official recalled just one access denial, involving parents who had refused standard cancer treatment for their child).

90. Until recently, the FDA used a submission form that demanded extensive information from physicians and sponsors requesting access. See Individual Patient Expanded Access Applications: Form FDA 3926, 80 Fed. Reg. 7318, 7320 (Feb. 10, 2015) (to be codified at 21 C.F.R. pt. 312) (estimating that the previous expanded-access process required approximately eight hours for the physician to complete). But in February 2015, in “an effort to streamline the submission process

submission deters overworked physicians and drug-company officials from seeking patient access.⁹¹ According to access advocates, many patients who could benefit from trying investigational drugs are deprived of help because the FDA insists on being involved in the decision.⁹²

Experts defending FDA oversight paint a different picture. They point to empirical evidence showing that informed-consent requirements fail to ensure that terminally ill patients understand the harms and burdens that can accompany investigational-drug use. According to the evidence, patients commonly overestimate the odds that novel interventions will extend or improve their lives.⁹³ Experts also say access advocates ignore the suffering that can come from trying novel drugs. To the advocates who ask, “What’s the harm?” in allowing unrestricted access, one expert responded, “If there’s anything worse than dying of a terminal illness, it’s dying of a terminal illness and suffering unnecessary complications or pain for no benefit and having to pay for the medications causing the complications yourself.”⁹⁴

Experts supporting access restrictions characterize terminally ill patients as a vulnerable population—one that needs more, not less, regulatory protection.⁹⁵ Thus, federal regulations should require researchers and physicians offering patients investigational drugs to discuss palliative care and hospice options, too; the rules should also mandate heightened monitoring of patients receiving unapproved drugs and clear criteria for stopping drug administration.⁹⁶ As one access critic put it, instead of adopting access rules that expose more terminally ill patients to harm and disappointment, “the gate to access experimental treatments must be closed enough to prevent medical interventions that impose excessive harm.”⁹⁷

for individual patient expanded access,” the agency proposed a much simplified form that officials estimated would take about forty-five minutes to complete. *Id.* at 7319, 7320.

91. CORIERI, *supra* note 74, at 9–10, 11. Right-to-try advocates also say the IRB review requirement creates an undue obstacle to patient access. *Id.* at 11.

92. *See, e.g., id.* at 18 (“The FDA’s long, costly, and burdensome process makes it difficult for patients to get the medications that may save their lives.”).

93. *See infra* notes 174–76 and accompanying text for discussion of this evidence. One medical expert says the right-to-try campaign reflects a similarly unrealistic conception of investigational drugs as “miracle” treatments. *See Gorski, supra* note 83.

94. Gorski, *supra* note 83.

95. Michael J. Malinowski, *Throwing Dirt on Doctor Frankenstein’s Grave: Access to Experimental Treatments at the End of Life*, 65 HASTINGS L.J. 615, 645 (2014).

96. *Id.* at 646, 651. *See also* George J. Annas, *The Changing Landscape of Human Experimentation: Nuremberg, Helsinki, and Beyond*, 2 HEALTH MATRIX 119, 138–39 (1992) (arguing for strict limits on research participation of terminally ill patients).

97. Malinowski, *supra* note 95, at 657.

B. *Individual Versus Societal Interests*

Besides disagreeing on the proper balance of liberty and protection in access policy, access advocates and critics disagree on the proper balance of individual and societal interests. A rigorous clinical-trial system is essential to determining whether investigational drugs are sufficiently safe and effective to be approved for the general patient population. Making drugs easily available outside trials could threaten that system. Because relatively few patients enroll in trials, it often takes many years to determine the quality of investigational drugs.⁹⁸ If more patients can obtain drugs outside trials, it will become even harder to conduct the studies that reveal which investigational drugs actually help patients.⁹⁹

Under existing FDA regulations, officials may approve treatment access only if it “will not interfere with the initiation, conduct, or completion of clinical investigations that could support marketing approval of the expanded access use.”¹⁰⁰ But access advocates say that excluding trial-eligible patients is an unethical sacrifice of their interests to promote the greater good.¹⁰¹ According to access advocates, a rule that “forces people to go into clinical trials if they want access to the only possibly lifesaving drugs” is unacceptably coercive.¹⁰²

98. See VICTORIA WEISFELD ET AL., INST. OF MED. OF THE NAT'L ACADS., PUBLIC ENGAGEMENT AND CLINICAL TRIALS: NEW MODELS AND DISRUPTIVE TECHNOLOGIES 2 (2012) (identifying “the increasing difficulty of recruiting and retaining an appropriate human subject population for specific clinical trials” as a “significant problem”).

99. Chahal, *supra* note 33, at 368.

100. 21 C.F.R. § 312.305(a)(3) (2014). The FDA treatment access regulations require doctors to report information about adverse events to sponsors and the FDA, which promotes public health interests by adding to the evidentiary basis for approval decisions. Compare 21 C.F.R. § 312.35(a)(3) (2014) (defining physicians who obtain access to experimental drugs for their patients as “investigators”), with 21 C.F.R. § 312.305(c)(4) (2014) (requiring investigators to report adverse drug events to investigational-drug sponsors), and 21 C.F.R. § 312.32(c)(i) (2014) (requiring investigational-drug sponsors to report adverse reactions to the FDA that are suspected of being related to the investigational drug). Right-to-try laws include no reporting requirement. See, e.g., COLO. REV. STAT. ANN. §§ 25-45-101 to -108 (West Supp. 2014) (containing no requirement that physicians report adverse drug reactions for investigational drugs being used through Colorado’s expanded-access program).

101. See Michael Kean, *It’s Time for Change*, J. MED. ETHICS (ONLINE FIRST), Feb. 28, 2014, at 1 (arguing that a more ethical approach would be to speed up the drug approval process by adopting different methods of collecting evidence about new drugs). Colorado’s right-to-try law limits investigational drug access to patients who have “been unable to participate in a clinical trial for the terminal illness within one hundred miles of the patient’s home address, . . . or not been accepted to the clinical trial within one week of completion of the clinical trial application process.” COLO. REV. STAT. ANN. § 25-45-103(1)(a)(III). None of the other laws includes a provision to exclude patients with reasonable opportunities to enroll in trials. See, e.g., LA. REV. STAT. ANN. §§ 40:1300.423–.424 (lacking a requirement that a patient have no opportunities to enroll in clinical trials before gaining expanded access to experimental drugs).

102. Volokh, *supra* note 86, at 1830 n.81. Other access supporters think the public-health threat has been exaggerated. E.g., Robertson, *supra* note 87, at 17.

Experts supporting access restrictions see the situation quite differently. They argue that the individual terminally ill patient’s desire to try investigational drugs is insufficient to outweigh the public’s interest in preserving the integrity of the drug-review system.¹⁰³ If access decisions are left to patients and their doctors, they say many more patients will be harmed.¹⁰⁴ Because it will become harder to conduct clinical trials, the drug-review process will be extended.¹⁰⁵ This will in turn deprive physicians of the information they need to make evidence-based treatment recommendations to their seriously ill patients.¹⁰⁶

Commentators defending access limits believe that “the public, as a body, merits protection from interference by individual members of society.”¹⁰⁷ The FDA’s access rules allow many patients to gain access to investigational drugs, striking a reasonable balance between individual and public health needs.¹⁰⁸ Supporters of access restrictions also point out that the law limits individual claims in many other contexts to protect the common good.¹⁰⁹

C. Doubts About Impact

According to right-to-try advocates, eliminating FDA oversight will greatly increase patients’ access to investigational drugs, giving them real opportunities to save their lives.¹¹⁰ Yet others predict that right-to-try laws will have little impact on access.

103. See, e.g., Seema Shah & Patricia Zettler, *From a Constitutional Right to a Policy of Exceptions: Abigail Alliance and the Future of Access to Experimental Therapy*, 10 YALE J. HEALTH POL’Y L. & ETHICS 135, 171 (2010) (explaining that one significant reason for protecting the integrity of clinical trials is that doing so prevents delay of wider access to approved drugs).

104. See, e.g., *id.* at 181 (contending that terminally ill people “may suffer more or even die sooner” if they are given drugs with uncertain and possibly severe risks, and this is an important reason to limit access to experimental therapies).

105. See Elizabeth Weeks Leonard, *The Public’s Right to Health: When Patient Rights Threaten the Commons*, 86 WASH. U. L. REV. 1335, 1352–53 (2009) (arguing that drug companies’ goal of FDA approval could be undermined if expanded access is granted for experimental drugs).

106. See, e.g., Leonard, *supra* note 105, at 1385–86 (deeming drug access prior to full clinical trials a “public bad” because it prevents development of medical knowledge of actual effectiveness of new drugs); Shah & Zettler, *supra* note 103, at 184 (stating that expanded access will delay FDA approval of effective drugs, negatively affecting public health).

107. Leonard, *supra* note 105, at 1384. See also Brower, *supra* note 87, at dju172 (citing one expert’s view of the Abigail Alliance’s access position as an “aggressively individualistic view, one that breathtakingly slights the public’s interest in drug safety”).

108. Leonard, *supra* note 105, at 1386–87.

109. See Shah & Zettler, *supra* note 103, at 194–95 (giving the example of a witness being forced to testify despite fears that the defendant will threaten his or his family’s safety); Snead, *supra* note 63, at 11 (noting that there is “no history or tradition of courts privileging the preferences of patients (including those suffering from terminal illnesses) for a particular prohibited medical intervention over governmental concerns about public health”).

110. See, e.g., Eleanor Clift, *The ‘Dallas Buyers Club’ Bill*, DAILY BEAST (Mar. 4, 2014), <http://www.thedailybeast.com/articles/2014/03/04/the-dallas-buyers-club-bill.html>, archived at

In the Abigail Burroughs case and other high-profile access cases it was drug sponsors, not FDA officials, who blocked patients' access to investigational drugs.¹¹¹ Because right-to-try laws fail to require drug sponsors to satisfy patient requests, the laws do nothing to help with this problem. Indeed, removing the FDA from involvement in patient access could inadvertently make things worse. In a 2014 interview, FDA Commissioner Margaret Hamburg reported that agency officials often help patients and physicians negotiate with drug companies to secure access agreements.¹¹² Without FDA involvement, fewer companies might agree to provide patients with investigational drugs.

Experts also point out that the FDA's regulatory requirements have not prevented drug companies from providing investigational drugs to many patients.¹¹³ When companies say no to patient requests, FDA access regulations are not the reason. Because the drug sponsors' overriding goal is to gain FDA approval of their drugs, sponsors prefer to devote their limited resources to conducting the clinical trials that will make approval possible.¹¹⁴ Producing investigational drugs for patients outside trials can be costly, as

<http://perma.cc/WF6B-MA7W> (citing a right-to-try advocate who claims that "government red tape" interferes with patients' right to save their lives).

111. See Jerry Menikoff, *Beyond Abigail Alliance: The Reality Behind the Right to Get Investigational Drugs*, 56 U. KAN. L. REV. 1045, 1059–60 (noting that none of the four plaintiffs in the *Abigail Alliance* case offered facts suggesting that the FDA interfered with their access efforts); Brady Dennis & Ariana Eunjung Cha, 'Right to Try' Laws Spur Debate Over Dying Patients' Access to Experimental Drugs, WASH. POST, May 16, 2014, http://www.washingtonpost.com/national/health-science/right-to-try-laws-spur-debate-over-dying-patients-access-to-experimental-drugs/2014/05/16/820e08c8-dcfa-11e3-b745-87d39690c5c0_story.html, archived at <http://perma.cc/L9ZF-WZDW> (giving one example of a patient denied an investigational drug by two drug companies, not the FDA); David Kroll, *The False Hope of Colorado's 'Right to Try' Investigational Drug Law*, FORBES (May 19, 2014, 5:26 PM), <http://www.forbes.com/sites/davidkroll/2014/05/19/the-false-hope-of-colorados-right-to-try-act/>, archived at <http://perma.cc/AGL3-6NQS> (describing one patient who was denied access by drug manufacturers).

112. *The Diane Rehm Show: Debate Over 'Right-to-Try' Laws* (WAMU 88.5 radio broadcast May 27, 2014), available at <http://thedianerehmshow.org/shows/2014-05-27/debate-over-right-try-laws/transcript>, archived at <http://perma.cc/8FYM-3GTF> (presenting Hamburg's comments that right-to-try laws could lead to the loss of FDA advocacy encouraging companies to make investigational drugs available). See also Ariana Eunjung Cha, *Crowdsourcing Medical Decisions: Ethicists Worry Josh Hardy Case May Set Bad Precedent*, WASH. POST, Mar. 23, 2014, http://www.washingtonpost.com/national/health-science/crowdsourcing-medical-decisions-ethicists-worry-josh-hardy-case-may-set-bad-precedent/2014/03/23/f8591446-ab81-11e3-adbc-888c8010c799_story.html, archived at <http://perma.cc/T37Q-JQGT> (discussing a case in which the FDA helped a boy obtain access to an investigational drug by negotiating with a sponsor).

113. For discussion of the potential business advantages for companies providing access, see Brower, *supra* note 87, at dju172. Companies also grant access for public-relations purposes. See, e.g., Cha, *supra* note 112 (citing examples of drug companies responding to public-relations pressure by granting access).

114. See Brower, *supra* note 87, at dju172 (quoting one company's executive's statement that the "priority is to help the greatest number of patients possible by helping drugs receive approval").

can diverting employees to administer patient-access programs.¹¹⁵ Even though the FDA permits them to recover their costs, companies may be unable to manage the logistics involved in operating a treatment-access program.

Experts doubt that right-to-try laws will do much to change this situation. They also warn that the FDA could challenge unauthorized investigational drug distribution as a violation of the Food, Drug and Cosmetic Act.¹¹⁶ Companies distributing investigational drugs without FDA permission could damage their efforts to win FDA approval of those drugs. It isn't surprising that the Pharmaceutical Research and Manufacturers of America trade group has "serious concerns with any approach to make investigational medicines available that seeks to bypass the oversight of the Food and Drug Administration and clinical trial process."¹¹⁷ Drug companies would be acting against their self-interest if they provided drugs outside the agency's access program.¹¹⁸

A further problem is that right-to-try laws fail to address existing inequities in patient access to investigational drugs. Under right-to-try laws, access would continue to be available only to patients whose physicians succeeded in persuading drug companies to provide investigational drugs. And right-to-try laws, like FDA regulations, allow sponsors to charge patients for drugs.¹¹⁹ Thus, only patients able to cover the charges would gain access. As the mother of a patient seeking access complained, right-to-try laws "ignore the elephant in the room, which is cost."¹²⁰

115. See Arthur L. Caplan, *Why "Right to Try" Laws Won't Help Desperately Ill Patients*, MEDSCAPE (June 19, 2014), <http://www.medscape.com/viewarticle/826708>, archived at <http://perma.cc/G9T8-ZJNS> (explaining that companies often lack resources needed to provide drugs for patient access). Companies also worry that negative drug effects detected in patients could make the agency more cautious about approving a drug, but FDA officials say this fear is unfounded. Servick, *supra* note 89, at 1329. See also *The Diane Rehm Show*, *supra* note 112 (interviewing Commissioner Margaret Hamburg, who said that the FDA has never denied approval due to adverse events in expanded-access patients).

116. See Patricia J. Zettler & Henry T. Greely, *The Strange Allure of State "Right-to-Try" Laws*, 174 J. AM. MED. ASS'N INTERNAL MED. 1885, 1885 (2014) (noting that federal law trumps state law).

117. John Tozzi, *Do Dying Patients Have a Right to Try Experimental Drugs? Libertarians Say Yes*, BUSINESSWEEK, Aug. 21, 2014, <http://www.bloomberg.com/bw/articles/2014-08-21/do-dying-patients-have-a-right-to-try-experimental-drugs-libertarians-say-yes>, archived at <http://perma.cc/HJ2M-F6FF>.

118. Gorski, *supra* note 83.

119. *Id.*

120. Mary Lou Byrd, *Third State Passes "Right to Try" Legislation*, WASH. FREE BEACON (July 17, 2014), <http://freebeacon.com/issues/third-state-passes-right-to-try-legislation/>, archived at <http://perma.cc/JG6U-CVT5>. Critics of the efforts to remove or lessen FDA access oversight propose what they see as better alternatives, such as changes that would allow more patients to participate in clinical trials. See, e.g., Chahal, *supra* note 33, at 369–70 (proposing ways to improve compassionate-use programs); Shah & Zettler, *supra* note 103, at 189–95 (proposing changes to the clinical trials process that would improve access for terminally ill patients).

III. Patient Perspectives

In the right-to-try debate, supporters depict liberal access as the best approach for patients. Advocates tell stories of patients helped by investigational drugs, as well as stories of patients who suffered and died without them. Advocates work with patients and their families to publicize these compelling accounts.

But access advocates leave out other kinds of patient stories. They omit stories of patients who tried investigational drugs but paid a price for doing so. Investigational drugs failed to give these patients longer or more comfortable lives; instead, the drugs hastened death and increased suffering. Access advocates also omit stories of when wide patient access made it harder to complete the clinical trials needed to determine whether experimental approaches were truly beneficial. These stories show that liberal access is not always the pro-patient position it purports to be.

A. *Stories in the Access Debate*

1. *Stories Supporting Access.*—Patients' stories are a staple of right-to-try and other access campaigns. Most famous is the story of Abigail Burroughs—a college student whose head-and-neck cancer failed to respond to standard therapies.¹²¹ She sought access to two investigational drugs under study, but the trials were enrolling only patients with different kinds of cancer. The companies making the drugs refused to include her in their compassionate-access programs, and she died when she was just twenty-one.¹²² One of the investigational drugs Burroughs wanted to try was later approved to treat head-and-neck cancer.¹²³ Her father, founder of the Abigail Alliance, is convinced that she would have survived if she had been able to obtain the drug.¹²⁴

Right-to-try law advocates tell other distressing stories. The Goldwater Institute's right-to-try proposal begins with the story of Kianna Karnes, a nurse and mother of four who died of kidney cancer the very day that the FDA and two drug companies agreed to cooperate with her request for compassionate access.¹²⁵ The proposal concludes with the plea of another terminally ill patient who claimed she was denied an investigational drug because the manufacturer was afraid of "a government hassle."¹²⁶

121. See *supra* text accompanying note 56.

122. Foreman, *supra* note 52.

123. Kimberly Leonard, *Seeking the Right to Try*, U.S. NEWS & WORLD REP. (Nov. 18, 2014, 12:01 AM), <http://www.usnews.com/news/articles/2014/11/18/right-to-try-laws-allowing-patients-to-try-experimental-drugs-bypass-fda>, archived at <http://perma.cc/U48X-PBVV>.

124. Byrd, *supra* note 120.

125. CORIERI, *supra* note 74, at 1. The first federal bill promoting liberal access was nicknamed "Kianna's Law." Groopman, *supra* note 89.

126. CORIERI, *supra* note 74, at 22.

Stories have played a central role in legislative proceedings on right-to-try laws too. The lead sponsor of Missouri’s bill was Jim Neely, a physician–legislator whose stepdaughter had been diagnosed with advanced colon cancer.¹²⁷ His stepdaughter was reportedly ineligible for any clinical trials but wanted to try investigational drugs.¹²⁸ Neely and two other men with children in similar situations told their stories at a legislative hearing on the bill, which was later unanimously approved.¹²⁹ One of the most vocal supporters of Colorado’s right-to-try bill was the wife of Nick Auden, a patient who died of melanoma after failing to convince two drug companies to provide him with promising investigational drugs.¹³⁰ Although the companies said their decisions were based on safety concerns and an inadequate drug supply, Auden’s wife blamed the FDA rules for their refusal.¹³¹

As one journalist observed, the “frustration of tragedy” drives right-to-try supporters.¹³² Some are people “motivated to honor ones they have lost to illness; others are racing to save sick family who are still living.”¹³³ They are people who have seen one side of the access situation, and this experience shapes their policy position.

2. *Cautionary Access Stories.*—Yet access advocates are not the only ones with tragic stories to tell. Law Professor Michael Malinowski describes the plight of his father, who had terminal cancer and “aggressively sought and received . . . experimental treatments—from drugs to a series of surgeries. The drugs worsened his health immediately; the side effects were horrific.”¹³⁴ For the remaining months of his life, Malinowski’s father was

127. *PBS Newshour: ‘Right to Try’ Law Gives Terminal Patients Access to Drugs Not Approved by FDA* (PBS television broadcast June 21, 2014), available at <http://www.pbs.org/newshour/bb/right-try-law-gives-terminal-patients-access-non-fda-approved-drugs/>, archived at <http://perma.cc/59JE-RXP3>.

128. Mike Sherry, *Missouri Becomes Third State to Enact ‘Right to Try’ Drug Law*, KBIA (Oct. 8, 2014, 4:56 PM), <http://kbia.org/post/missouri-becomes-third-state-enact-right-try-drug-law>, archived at <http://perma.cc/85JS-GDN6>.

129. Virginia Young, *Missouri Could Join Push for Experimental Drugs for Terminally Ill*, ST. LOUIS POST-DISPATCH, June 3, 2014, http://www.stltoday.com/news/local/govt-and-politics/missouri-could-join-push-for-experimental-drugs-for-terminally-ill/article_c213ab2a-9612-5661-8d75-951c84c271eb.html, archived at <http://perma.cc/YNR5-5633>.

130. Kurtis Lee, *“Right to Try” Aims to Limit Bureaucracy for Colorado’s Terminally Ill*, DENVER POST, Apr. 1, 2014, http://www.denverpost.com/politics/ci_25463368/right-try-aims-limit-bureaucracy-colorados-terminally-ill, archived at <http://perma.cc/H8L4-QDJN>.

131. *Id.*

132. Amity Shlaes, *The Right to Try*, NAT’L REV. (May 14, 2014, 4:00 AM), <http://www.nationalreview.com/article/377907/right-try-amity-shlaes>, archived at <http://perma.cc/6TEZ-MEFG>.

133. *Id.*

134. Malinowski, *supra* note 95, at 616–18.

on high doses of morphine for pain.¹³⁵ He lost more than one hundred pounds before he died.¹³⁶

Malinowski believes his father was “in a state of denial” about his impending death, a denial that no clinician was willing to challenge.¹³⁷ Physicians were eager to provide his father with experimental measures, but none brought up the palliative-care options that could have increased his comfort and extended his life.¹³⁸ In his father’s case, investigational interventions led to a painful and distressing death rather than a longer and better life.

Former cancer patient Musa Mayer tells a particularly instructive story about the downsides of access.¹³⁹ During the 1990s, Mayer worked to help two women with advanced breast cancer gain access to an experimental regimen that combined high-dose chemotherapy with bone marrow transplantation.¹⁴⁰ The regimen was supported by phase II trial evidence but had not been evaluated in phase III trials.¹⁴¹ The chemotherapy drugs used in the regimen had been approved for other treatment uses, so the FDA investigational drug restrictions did not apply in this case.¹⁴² But many insurers were unwilling to cover the unproven and costly regimen.¹⁴³ In this campaign, it was the insurance companies that were the target of access claims.

Despite a lack of solid evidence on treatment effectiveness, many women wanted to try high-dose chemotherapy and bone marrow transplantation.¹⁴⁴ They wanted to “go out fighting” and show their families they had done everything to survive.¹⁴⁵ But the women Mayer helped did not have good outcomes. One had a fatal hemorrhage soon after her bone marrow

135. *Id.* at 618.

136. *Id.*

137. *Id.* at 619.

138. *Id.* at 618–19. See also Jennifer S. Temel et al., *Early Palliative Care for Patients with Metastatic Non-Small-Cell Lung Cancer*, 363 *NEW ENG. J. MED.* 733, 739–40 (2010) (finding early integration of palliative care prolonged survival by approximately two months and resulted in “clinically meaningful improvements in quality of life and mood,” compared with standard care).

139. Musa Mayer, *Listen to All the Voices: An Advocate’s Perspective on Early Access to Investigational Therapies*, 3 *CLINICAL TRIALS* 149, 150 (2006).

140. *Id.* at 150–51.

141. *Id.* at 150.

142. Once a drug is approved for one medical use, physicians may prescribe it for other “off-label” uses. Erika P. Hamilton et al., *Availability of Experimental Therapy Outside Oncology Randomized Clinical Trials in the United States*, 28 *J. CLINICAL ONCOLOGY* 5067, 5067 (2010). Off-label use of cancer drugs can occur without good evidence that the drugs are safe and effective for the off-label application. See *id.* at 5071–72 (concluding that the increasing availability of experimental and “off-protocol” interventions outside clinical trials creates valid concerns over patient safety and drug efficacy).

143. Mayer, *supra* note 139, at 150.

144. *Id.*

145. Mayer, *supra* note 4, at 1060–61.

transplant, and treatment side effects led to the other woman’s death.¹⁴⁶ After that, Mayer wrote, “I finally understood what later seemed obvious—that there were other voices we had not been listening for being drowned out by the clamor for access, the voices of those who had died from the treatment itself”¹⁴⁷

Mayer realized the access campaign was neglecting other voices too. There were the “voices of those women who suffered from temporary or permanent disabilities as a result of their transplants.”¹⁴⁸ There were the voices of women whose cancer progressed despite the treatment.¹⁴⁹ And there were the voices of women “whose future treatments for advanced disease had been compromised by the massive doses of chemotherapy they had already endured.”¹⁵⁰ Many of these women kept quiet about their situations because they did not want “to demoralize the others, or second guess their own choices.”¹⁵¹

Because women were often able to obtain the unproven regimen through their physicians, few were willing to enroll in the phase III randomized clinical trials evaluating it.¹⁵² Women who wanted to try the regimen were unwilling to take the chance of being assigned to standard therapy, which had low success rates.¹⁵³ Once trial results became available, however, it was clear that what had been labeled a promising treatment actually delivered no survival benefit.¹⁵⁴

Mayer sees history repeating itself in expanded-access campaigns. “With legal liability off the table, FDA out of the picture, and the efficacy bar lowered as far as it will go,” she asks, “how long would it take to reach the first disaster relating to drug toxicity? How many savings accounts would be emptied in the vain pursuit of hope?”¹⁵⁵

3. *Stories on Both Sides.*—A few people tell both kinds of access stories. Physician Darshak Sanghavi explains his ambivalence about access by describing what happened to two different patients.¹⁵⁶ One patient was

146. Mayer, *supra* note 139, at 150.

147. *Id.*

148. *Id.*

149. *Id.*

150. *Id.*

151. *Id.*

152. *Id.*

153. *Id.*

154. *Id.* at 150–51.

155. *Id.* at 151.

156. Darshak M. Sanghavi, *The Pills of Last Resort: How Dying Patients Get Access to Experimental Drugs*, N.Y. TIMES MAG., Oct. 31, 2013, <http://www.nytimes.com/2013/11/03/magazine/how-dying-patients-get-access-to-experimental-drugs.html>, archived at <http://perma.cc/3NY7-3MVW>.

Sarah Broom, a thirty-five-year-old mother of three with advanced lung cancer.¹⁵⁷ She lived in New Zealand but was an Oxford University graduate with influential friends around the world.¹⁵⁸ Through her connections, she was able to locate and participate in an investigational drug trial.¹⁵⁹ She did well for two years, then her tumors began growing again.¹⁶⁰ Her last hope was a different investigational drug, but the manufacturer turned down her request for compassionate access.¹⁶¹ Broom and her physician refused to give up, however, and company officials eventually agreed to give her the drug. She lived another year before finally succumbing to cancer.¹⁶²

Investigational drugs gave Sarah Broom a few valuable years with her family—years in which she felt well and could enjoy her remaining life.¹⁶³ But for Sanghavi's father, access to an experimental measure was disastrous. In his father's case, an insurance company agreed to cover the costs of an expensive FDA-approved drug that had not been fully evaluated in patients with his father's illness, a fatal condition called idiopathic pulmonary fibrosis.¹⁶⁴ In a small preliminary study, nine patients with the condition had shown substantial improvement after receiving the drug.¹⁶⁵

Sadly, those early study results were misleading. Injections of the drug did not help Sanghavi's father. Instead, they "caused raging fevers that left him confined to bed in terrible pain."¹⁶⁶ A few years later, results from a larger and more rigorous study showed that the drug was both ineffective and risky for patients with idiopathic pulmonary fibrosis, producing lung infections in the patients who took it.¹⁶⁷ Sanghavi believes "[i]t would have been better if [his] father never took it. And because [they] had found a backdoor way of getting it—he never joined any study—no drug company or regulator learned anything constructive from his death."¹⁶⁸

These stories reveal the multifaceted impact of treatment access. No single story can present a full picture of patients' interests in the access debate. Expanded access gives some patients the relief they are seeking, but

157. *Id.*

158. *Id.*

159. *Id.*

160. *Id.*

161. *Id.*

162. *Id.*

163. *Id.*

164. *Id.*

165. Rolf Ziesche et al., *A Preliminary Study of Long-Term Treatment with Interferon Gamma-1b and Low-Dose Prednisolone in Patients with Idiopathic Pulmonary Fibrosis*, 341 *NEW ENG. J. MED.* 1264, 1268–69 (1999).

166. Sanghavi, *supra* note 156.

167. Ganesh Raghu et al., *A Placebo-Controlled Trial of Interferon Gamma-1b in Patients with Idiopathic Pulmonary Fibrosis*, 350 *NEW ENG. J. MED.* 125, 131–32 (2004).

168. Sanghavi, *supra* note 156. For other stories of patients who fared well and fared poorly after obtaining access to investigational drugs, see Groopman, *supra* note 91.

leaves others with shorter lives and more suffering. For patients, the freedom to try investigational drugs is at best a mixed blessing.

B. Empirical Data on Patients' Experiences

Patients' stories are graphic and powerful, conveying the real urgency, joy, and misery surrounding the quest to obtain investigational drugs. Empirical studies of patients receiving investigational drugs are another source of information relevant to access policy. Through interviews, surveys, and other methods, researchers explore patients' experiences with and beliefs about investigational drugs. Not enough of this research has been done, but the studies that exist offer insights that belong in the right-to-try debate.

A study published in 2007 is one example. Acting on their belief that "patients' views and experiences should inform the debate about the appropriate way to provide access to investigational agents," a research team asked a group of terminally ill patients for their views on access criteria.¹⁶⁹ The team surveyed one hundred patients who were hoping to participate in one of the first human tests of a potential anticancer drug widely publicized in the popular media.¹⁷⁰

In their survey responses, patients most often endorsed two criteria for deciding who should have access to investigational drugs when supplies are limited. Their preferred criteria were: (1) patients with the greatest need or most chance of benefit; and (2) patients in trials, because trials would generate the best information about drug safety and effectiveness.¹⁷¹ Few of the survey respondents agreed that investigational drugs should go to any patient who wanted them.¹⁷² At the same time, a majority of the respondents agreed that it was too hard for patients to get access to investigational drugs and that "knowing the right people" and "being persistent" would increase a patient's access chances.¹⁷³

Patients responding to the survey sympathized with terminally ill individuals seeking access, but they also recognized the public's interest in discovering which new agents are truly safe and effective. As the study authors observed, the patients' responses "reflect the core ethical tension between maximizing scientific advancement and making investigational agents available to ailing individuals."¹⁷⁴ Most patients believed in a right to

169. Rebecca D. Pentz et al., *Who Should Go First in Trials with Scarce Agents?*, IRB: ETHICS & HUM. RES., July–Aug. 2007, at 1, 2.

170. *Id.* The drug was called endostatin. Phase II trials found that the drug had had very little effect on patients' tumors. Ariel Whitworth, *Endostatin: Are We Waiting for Godot?*, 98 J. NAT'L CANCER INST. 731, 731 (2006).

171. Pentz et al., *supra* note 169, at 3.

172. *Id.* at 2.

173. *Id.*

174. *Id.* at 4.

access but also supported constraints that prioritize certain patients over others and take into account the public's interest in a rigorous drug-evaluation system.¹⁷⁵

Other studies relevant to access policy consider the quality of terminally ill patients' decisions to participate in early-phase trials. Most of these patients have the same mind-set as patients seeking investigational drugs outside of trials—they are hoping that a novel drug will be more effective than the standard therapies that have failed them.

Despite rigorous requirements for information disclosure and informed consent in clinical trials, study after study shows that many trial subjects overestimate the possibility of medical benefit and underestimate the possibility of harm from investigational-drug exposure. Some patients join trials without understanding that the trials' primary purpose is to generate knowledge about investigational drugs, not to provide the best treatment to individual trial participants.¹⁷⁶ Other trial subjects realize that early-phase drug trials rarely produce therapeutic benefits yet remain unrealistically optimistic about their own chances.¹⁷⁷ Contrary to the objective evidence, they believe they have a "greater likelihood of experiencing positive outcomes or avoiding negative outcomes compared with others in the same or similar situation."¹⁷⁸

Researchers have also found that social expectations lead some patients to join early-phase trials. In one interview study, a majority of subjects enrolled in phase I or II cancer trials said they expected a therapeutic benefit from their participation.¹⁷⁹ More than a third said their optimism was related to the expectations of others.¹⁸⁰ The optimistic patients in this group "sought to be model patients, pleasing the medical community, their families, and even their faith networks."¹⁸¹ Some linked optimism to their duty to "reassure or help loved ones in dealing with the patient's own struggle with

175. *Id.* at 2–3.

176. This misunderstanding is known as the therapeutic misconception. *See, e.g.,* Rebecca D. Pentz et al., *Therapeutic Misconception, Misestimation, and Optimism in Participants Enrolled in Phase I Trials*, 118 *CANCER* 4571, 4572, 4574 (2012) (describing surveys and interviews finding that nearly 70% of participants in phase I cancer trials could not correctly answer questions about purpose of research and how treatment is chosen in trial).

177. *Id.* at 4575.

178. Joshua Crites & Eric Kodish, *Unrealistic Optimism and the Ethics of Phase I Cancer Research*, 39 *J. MED. ETHICS* 403, 404 (2013). *See also* Don Swekoski & Deborah Bambaum, *The Gambler's Fallacy, the Therapeutic Misconception, and Unrealistic Optimism*, *IRB: ETHICS & HUM. RES.*, Mar.–Apr. 2013, at 1, 5 (describing how patients' lack of understanding or acceptance of low chance of benefit interferes with informed consent).

179. Daniel P. Sulmasy et al., *The Culture of Faith and Hope: Patients' Justifications for Their High Estimations of Expected Therapeutic Benefit When Enrolling in Early Phase Oncology Trials*, 116 *CANCER* 3702, 3705 (2010).

180. *Id.* at 3707.

181. *Id.*

cancer.”¹⁸² Yet patients’ loved ones are prone to the same misunderstandings and unrealistic hopes that patients harbor. Studies have shown that even physicians and researchers tend to overestimate the chance that investigational drugs will benefit patients in early-phase trials.¹⁸³

Studies about patients’ experiences while they are taking investigational drugs are relevant to access policy too. One such study involved in-depth interviews with patients who had advanced cancer, after their participation in early-phase drug trials. As the trials progressed, the patients reported “an increasing sense of being burdened.”¹⁸⁴ Some of the burdens were related to the trials, such as the extra hospital visits and tests required to generate study data.¹⁸⁵ But patients were also burdened by the side effects of the drugs—side effects that they had not anticipated.¹⁸⁶ Over time these patients developed “a feeling that the harm was too great, and a sense of disillusionment with what was on offer took over.”¹⁸⁷ Researchers eventually took 70% of the patients off the investigational drugs because the side effects became too severe or their cancer progressed.¹⁸⁸ At that point patients expressed disappointment and a sense of abandonment.¹⁸⁹

Studies like these shed light on patient access, but there are not enough of them. Particularly problematic is the scarcity of data on the experiences of patients whose disease fails to respond to investigational drugs. The majority of terminally ill patients receiving investigational drugs are in this group.¹⁹⁰ But researchers have been hesitant to study these patients, on the assumption that they are too vulnerable to participate in research.¹⁹¹

182. *Id.*

183. See, e.g., Christopher Daugherty et al., *Perceptions of Cancer Patients and Their Physicians Involved in Phase I Trials*, 13 J. CLINICAL ONCOLOGY 1062, 1036, 1065 tbl.4 (1995) (noting that phase I trials typically show therapeutic benefits in only 4% to 6% of cases, yet the oncologists surveyed had a median estimate of an 18% likelihood that phase I trials would provide an overall improvement for their patients); Victoria A. Miller et al., *Hope and Persuasion by Physicians During Informed Consent*, 32 J. CLINICAL ONCOLOGY 3229, 3234 (2014) (finding that the authors’ study of doctor–patient communications was consistent with prior research showing that doctors in phase I trials tend to emphasize hope with respect to therapeutic outcomes).

184. Karen Cox, *Enhancing Cancer Clinical Trial Management: Recommendations from a Qualitative Study of Trial Participants’ Experiences*, 9 PSYCHO-ONCOLOGY 314, 317 (2000).

185. *Id.*

186. *Id.*

187. *Id.*

188. *Id.* at 318.

189. Most said they did not regret their choice to participate, however, because trial participation gave them a chance to help themselves and others. *Id.*

190. See Bruce Patsner, *Dying Patients and Their “Right” to Unapproved Drugs: Did the D.C. Circuit Finally Get it Right in Abigail Alliance?*, HEALTH L. PERSP., Nov. 13, 2007, at 1, 1–2, available at [http://www.law.uh.edu/healthlaw/perspectives/2007/\(BP\)%20Fatal%20ill.pdf](http://www.law.uh.edu/healthlaw/perspectives/2007/(BP)%20Fatal%20ill.pdf), archived at <https://perma.cc/V2W2-P2NV> (discussing the risks of new anticancer agents and noting that less than 4% of patients respond to these experimental drugs).

191. Marjolein H. Gysels et al., *Patient, Caregiver, Health Professional and Researcher Views and Experiences of Participating in Research at the End of Life: A Critical Interpretive Synthesis*

The concern about patient vulnerability is understandable, but without data from patients in this position we will have an incomplete picture of patients' investigational-drug experiences. Without such data, it will be impossible to evaluate the benefits and harms associated with access to investigational drugs. Moreover, the desire to protect patients with poor outcomes from opportunities to participate in interview and survey research may be misplaced. A review of evidence on terminally ill patients' attitudes toward participation in research on end-of-life care found that a majority of patients had positive attitudes toward this form of research.¹⁹² It is likely that some patients with poor outcomes would be willing and able to discuss their access experiences with researchers.

Also absent from the literature is information about patients receiving investigational drugs outside of trials. Thousands of terminally ill patients have tried investigational drugs through the FDA's expanded-access program,¹⁹³ but to my knowledge no one has systematically examined what happened to them. How many had their lives lengthened or improved? How many experienced toxic side effects that made them feel worse? How many died as a result of those side effects? How did their experiences affect their views of expanded access? We ought to know much more about how patients evaluate their access experiences.

Conclusion

Right-to-try advocates claim they are on a mission of mercy, seeking to give terminally ill patients a last opportunity to postpone mortality. Time will tell whether right-to-try measures have any real impact on access to investigational drugs. At this point, it is uncertain how many states will enact these measures. Also unknown is whether patients in right-to-try states will actually succeed in obtaining investigational drugs without FDA permission.¹⁹⁴

Going forward, the legislative debate over right-to-try laws should be more informed than it has been. To achieve this end, scientists and other

of the Literature, 12 BMC MED. RES. METHODOLOGY, art. No. 123, at 2 (2012), <http://www.biomedcentral.com/content/pdf/1471-2288-12-123.pdf>, archived at <http://perma.cc/3978-Z5PH>. One research team wanted to include cancer trial participants with poor treatment outcomes in an interview study but was prevented from doing so by a research ethics committee. Committee members were concerned that the interviews would be too burdensome for patients. S. M. Madsen et al., *Participating in a Cancer Clinical Trial? The Balancing of Options in the Loneliness of Autonomy: A Grounded Theory Interview Study*, 46 ACTA ONCOLOGICA 49, 50 (2007).

192. Gysels et al., *supra* note 191, at 13.

193. Expanded Access to Investigational Drugs for Treatment Use, 71 Fed. Reg. 75,147, 75,148 (proposed Dec. 14, 2006) (to be codified at 21 C.F.R. 312) (“[The] FDA estimates that more than 100,000 patients have received investigational drugs through treatment INDs.”).

194. See Editorial, *supra* note 2 (noting that as of February 2015, there were no known cases in which state right-to-try laws helped patients to obtain investigational drugs without FDA authorization).

experts must address right-to-try laws in ways that are meaningful to legislators and the public. Experts should present a full picture of what happens to patients who succeed in gaining access. The selective storytelling that has dominated right-to-try campaigns presents a distorted picture of patient experiences, contributing to policies that could actually disserve patients.

A more informed debate could produce better legislative decisions about right-to-try laws. Such a debate could influence access advocacy as well. It could encourage advocates to develop policy proposals addressing the broad interests of terminally ill patients and their families—proposals that focus less on improbable treatment outcomes and more on patients' common medical and social needs. A debate like this could also help people understand how difficult it is to develop effective drugs for life-threatening conditions. It could clarify how drug development works, challenging popular conceptions of miracle cures held up by a heartless FDA bureaucracy.

The right-to-try campaign may be a small policy development, but it raises fundamental questions about our nation's attitudes toward death and dying. Right-to-try laws portray unproven interventions as desirable, even praiseworthy, responses to life-threatening illness. A more informed debate could reveal the human costs of this approach, drawing attention to alternative policies offering more meaningful help to people near the end of their lives.

Copyright of Texas Law Review is the property of University of Texas at Austin School of Law Publications and its content may not be copied or emailed to multiple sites or posted to a listserv without the copyright holder's express written permission. However, users may print, download, or email articles for individual use.