THE LATEST "FEDERAL MOVEMENT" IN THE FOOD AND DRUG LAW ARENA: THE FEDERAL RIGHT-TO-TRY *OR* RATHER RIGHT-TO-KNOW AND THUS REQUEST INVESTIGATIONAL THERAPIES FOR INDIVIDUALS WITH A LIFE-THREATENING DISEASE OR CONDITION

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I. INTRODUCTION

The national state movement regarding Right-to-Try state legislation spurred the enactment of the Federal Right-to-Try legislation passed in 2018.[1] Yet, even prior to the enactment of the Federal Right-to-Try law, the United States Federal Food and Drug Administration (FDA) has had mechanisms in place for those terminally ill who do not qualify for a clinical trial.[2] Does the Federal Right-to-Try Act provide improved access for the desperately ill? Will insurance companies provide reimbursement for a patient to undergo such investigational therapies? Is the manufacturer protected in terms of lawsuits? That is, does the patient relinquish the right to bring a legal action? Will physicians comprehend the pathway and advocate for their patients? Does this new law guarantee "any novel federal right"?

This article provides a Federal Primer on the Investigational Drug, Biologic and Device Process,[3] details a similar national right-to-know movement in the

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1. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018). Although titled Right to Try Act of 2017, this law was passed on May 30, 2018.

2. 21 U.S.C. § 360bbb (2012) (expanding access to unapproved therapies and diagnostics).

3. ROSEANN B. TERMINI, FOOD AND DRUG LAW: FEDERAL REGULATION OF DRUGS, BIOLOGICS, MEDICAL DEVICES, FOODS, DIETARY SUPPLEMENTS, PERSONAL CARE, VETERINARY AND TOBACCO PRODUCTS 131-34 (9th ed. 2017).

food and drug law arena that led to federal legislation perhaps comparable to how the Federal Right-to-Try Act[4] was enacted and includes a discussion about the state right to try movement which conceivably led to the enactment of the Federal Right-to-Try Act.[5] There are more queries than unambiguous answers regarding the recently enacted Federal Right-to-Try Act.[6] The federal law in essence could prove troublesome and confusing with both the state Right-to-Try measures due to, for instance, issues of national uniformity and preemption.[7] The recently enacted Federal Right-to-Try Act[8] adequate safeguards and perhaps a false unrealistic sense of hope?[9]

A look back provides insight to the current matter. In 2006, the federal courts grappled with this issue.[10] Appellate Court was faced with deciding if there was a constitutionally protected right to potentially lifesaving investigational medical therapies in the federal case of *Abigail Alliance for Better Access to Developmental Drugs and Washington Legal Foundation v. von Eschenbach (Abigail Alliance)*.[11]

In *Abigail Alliance,* the Court of Appeals held that terminally ill patients do not have a constitutional right to obtain investigational drugs prior to the Federal Food and Drug Administration (FDA) approval.[12] The United States Supreme Court declined to review the federal appeals court decision.[13] However, according to the Goldwater Institute, in the last few years, approximately 41 states have enacted legislation that addresses investigational drug access by the terminally ill.[14] These states include: Alabama, Alaska, Arizona, Arkansas, California,

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4. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018).

5. *Id.*

6. *Id.*

7. U.S. CONST. art. VI, cl. 2. The doctrine of preemption emanates from the Supremacy Clause (Article VI, Clause 2) of the United States Constitution. Article VI, Clause 2 prohibits states from enacting laws that conflict with federal law unless the federal law contains explicit preemption language.

8. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018).

9. *See, e.g.*, Marilyn J. Heine & Bruce E. Johnson, *Caution to Patients on 'Right to Try*,' PHILA. INQUIRER, Oct. 20, 2017, at A15. *See also* AMANDA BENNETT, THE COST OF HOPE: A MEMOIR (2012).

10. *Abigail All. for Better Access to Dev. Drugs v. Von Eschenbach,* 469 F.3d 129 (D.C. Cir. 2006), *aff'd*, 495 F.3d 695 (D.C. Cir. 2007).

11. *Id.*

12. *Id.*

13. *Abigail All. for Better Access to Dev. Drugs v. Von Eschenbach,* 469 F.3d 129 (D.C. Cir. 2006), *aff'd*, 495 F.3d 695 (D.C. Cir. 2007), *cert. denied*, 552 U.S. 1159 (2008).

14. *Alaska Becomes 41st State to Enact Right to Try Legislation*, GOLDWATER INST. (July 13, 2018), http://righttotry.org/alaska-becomes-41st-state-to-enact-right-to-try-legislation/ [https://perma.cc/6SUN-MC73]. The Goldwater Institute is a public policy conservative based organization located in Phoenix, Arizona.

Colorado, Connecticut, Florida, Georgia, Idaho, Illinois, Indiana, Iowa, Kentucky, Louisiana, Maine, Maryland, Michigan, Minnesota, Mississippi, Missouri, Montana, Nebraska, Nevada, New Hampshire, North Carolina, North Dakota, Ohio, Oklahoma, Oregon, Pennsylvania, South Carolina, South Dakota, Tennessee, Texas, Utah, Virginia, Washington, West Virginia, Wisconsin, and Wyoming.[15] Colorado was the first state to enact Right-to-Try legislation back in 2014.[16] The Colorado law Right-to-Try Act includes investigational medical devices.[17] The Federal Right-to-Try Act[18] does not address and is silent regarding investigational devices. However, prior legislative bills indicate devices were considered.[19] This could prove problematic in terms of preemption issues.[20] Yet, do these laws provide those who are desperately ill patients with "unrealistic false hope" by promising access to therapies that may not work or may cause harm, and that they may not even receive?[21] Are the state Right-to-Try laws usurping FDA authority?[22] Does the recently enacted Federal Right-to-Try Act provide additional benefits to the patient than what is already provided by the FDA expanded use? Alternatively, does the Federal Right-to-Try Act deny access as access is contingent on the physician, manufacturer and insurance companies to acquiesce? Who pays for access?

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15. *Id.*

16. Access to Treatments for Terminally Ill Patients, COLO. REV. STAT. ANN. § 25-45 (West 2014) (this article is known and may be cited as the "Right to Try Act").

17. § 25-45-102 (West 2014).

18. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018).

19. *See, e.g.*, H.R. 878, 115th Cong. (2017-2018) (Obligating the federal government to permit unrestricted manufacturing, "distribution, prescribing, or dispensing of an experimental drug, biological product, or [medical] device that is intended to treat a patient who has been diagnosed with a terminal illness."); *see also* H.R. 2368, 115th Cong. (2017-2018) ("This bill requires the federal government to allow unrestricted manufacturing, distribution, prescribing, and dispensing of experimental drugs, biological products, and medical devices that are authorized by state law and intended to treat terminally ill patients.") CONG. RES. SERV., *H.R. 2368: Right to Try Act*, GOVTRACK, https://www.govtrack.us/congress/bills/115/hr2368/summary [https://perma.cc/GKR4-23AW].

20. U.S. CONST. art. VI, cl.2. The doctrine of preemption emanates from the Supremacy Clause (Article VI, Clause 2) of the United States Constitution. Article VI, Clause 2 prohibits states from enacting laws that conflict with federal law unless the federal law contains explicit preemption language.

21. *See* Alison Bateman-House & Arthur Caplan, *Drug-Right Bills Give Patients False Hope*, PHILA. INQUIRER, Nov. 16, 2014, at B1 (concluding that state "right to try" laws could actually harm a terminally ill patient.

22. U.S. Const. art. VI, cl.2. The doctrine of preemption emanates from the Supremacy Clause (Article VI, Clause 2) of the United States Constitution. Article VI, Clause 2 prohibits states from enacting laws that conflict with federal law unless the federal law contains explicit preemption language.

Is informed consent adequately explained or in reality a protection mechanism for physicians?[23] These are complex questions without straightforward answers and there are various participants involved in this process besides the patient and the health care provider. Drug and medical device manufacturers, distributors, insurers and the health care providers have a significant role.

To establish the tone of how the national state movement spurred federal legislation in the Food and Drug law realm, the Bioengineered Act[24] provides insight. Next, a federal primer provides a synopsis of the federal investigation drug and medical device process. Following this, by way of illustration, is an overview of the Federal Right-to-Try legislation and a state comparison using the Right-to-Try legislation enacted in Maryland as an illustration.[25] Finally, the focus of this article is on the individual patient's right to try or request an investigational product.

II. A FEDERAL RUSH TO ENACTMENT—PERHAPS A LESSON LEARNED FROM THE RIGHT-TO-KNOW AND THE NATIONAL BIOENGINEERED FOOD DISCLOSURE STANDARD

For years, consumers advocated for a right-to-know on food labelling of bioengineered food.[26] Finally, The National Bioengineered Food Disclosure Standard (*Bioengineered Food Disclosure*), was passed July 29, 2016 which amended the Agricultural Marketing Act.[27] As background, several states passed Genetically Modified Organisms (GMO) laws.[28] For example, in 2013, Connecticut passed a law with a "trigger clause" which means the GMO law would only go into effect if nearby states also passed GMO labeling laws.[29] Similarly, in 2014, Maine passed a GMO labeling law that required foods containing (GMO) to be labeled as such however it contained a "trigger clause".[30] In 2015, the Maine legislature proposed a new law that would remove the trigger

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23. 21 U.S.C. § 360bbb-0a (2018); Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018); MD. CODE ANN., HEALTH-GEN. § 21-2B-01 (West 2018) (See Appendix V).

24. National Bioengineered Food Disclosure Standard, Pub. L. No. 114-216, 130 Stat. 834 (2016).

25. MD. CODE ANN., HEALTH-GEN. § 21-2B-01 (West 2018).

26. National Bioengineered Food Disclosure Standard, Pub. L. No. 114-216, 130 Stat. 834 (2016); *see* Lawrence O. Gostin, *Genetically Modified Food Labeling: A "Right to Know"?*, JAMA NETWORK (Dec. 13, 2016), https://jamanetwork.com/journals/jama/fullarticle/2592487 [https://perma.cc/G427-N7UM].

27. *Id.*

28. *See, e.g.*, CONN. GEN. STAT. § 21a-92c (2013); ME. REV. STAT. ANN. tit. 22, § 2593 (2014); VT. STAT. ANN. tit. 9, § 3043 (West 2014).

29. CONN. GEN. STAT. § 21a-92c (2013).

30. ME. REV. STAT. ANN. tit. 22, § 2592 (2014).

clause requirement that relies on other states.[31] Finally, Vermont enacted a GMO law without a trigger clause.[32]

Possibly the federal *Bioengineered Food Disclosure* law was enacted in response to state (GMO) legislation such as the Vermont GMO law.[33] The United States Department of Agriculture is tasked with the responsibility of promulgating regulations to implement the *Bioengineered Food Disclosure*.[34] Yet, prior to the *Bioengineered Food Disclosure* law, companies initiated proactive disclosure and either eliminate GMO ingredients or disclose GMO ingredients. For example, in July 2016, the Dannon yogurt company removed GMO ingredients in some products and disclosed GMO ingredients in the labeling of other products.[35] Yet, with any law that is shepherded through and

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31. ME. REV. STAT. ANN. tit. 22, §§ 2591-2595 (2015). "This bill requires disclosure of genetic engineering at the point of retail sale of food and seed stock and provides that food or seed stock for which the disclosure is not made is considered to be misbranded and subject to the sanctions for misbranding. The bill provides that food or seed stock may not be labeled as natural if it has been genetically engineered. The bill exempts products produced without knowledge that the products, or items used in their production, were genetically engineered; animal products derived from an animal that was not genetically engineered but was fed genetically engineered food; and products with only a minimum content produced by genetic engineering. The bill also provides that the disclosure requirements do not apply to restaurants, alcoholic beverages or medical food. The disclosure provisions are administered by the Department of Agriculture, Conservation and Forestry." *An Act to Protect Maine Food Consumers' Right to Know about Genetically Engineered Food and Seed Stock: Summary,* OFFICE OF LEGISLATIVE INFORMATION, https://www.mainelegislature.org/legis/bills/bills_126th/billtexts/HP049001.asp [https://perma.cc/LF5H-MCL3].

32. VT. STAT. ANN. tit. 9, § 3043 (West 2014).

33. VT. STAT. ANN. tit. 9 § 3043 (West 2014); National Bioengineered Food Disclosure Standard, Pub. L. No. 114-216, 130 Stat. 834 (2016).

34. National Bioengineered Food Disclosure Standard, Pub. L. No. 114-216, 130 Stat. 834 (2016). "Public Law 114-216 amended the Agricultural Marketing Act of 1946 (7 U.S.C. 1621 *et seq.*), as amended (amended Act), by adding Subtitles E and F. Subtitle E of the amended Act directs the Secretary to establish the National Bioengineered Food Disclosure Standard (NBFDS) for disclosing any BE food and any food that may be bioengineered. Subtitle E also directs the Secretary to establish requirements and procedures necessary to carry out the new standard. Additionally, the amended Act directs the Secretary to conduct a study to identify potential technological challenges related to electronic or digital disclosure methods. *See* 7 U.S.C. 1639b(c)(1). Subtitle F addresses Federal preemption of State and local genetic engineering labeling requirements. Subtitle F also specifies that certification of food under the U.S. Department of Agriculture's (USDA) National Organic Program (NOP) (7 CFR part 205) shall be considered sufficient to make claims about the absence of bioengineering in the food." National Bioengineered Food Disclosure Standard, 83 Fed. Reg. 19860 (proposed May 4, 2018) (to be codified at 7 C.F.R. pt. 66).

35. *See Dannon Announces Breakthrough Sweeping Commitment for Sustainable Agriculture, More Natural Ingredients and Greater Transparency*, DANNON (Apr. 27, 2016),

reactive to state legislation, as the federal *Bioengineered Food Disclosure* law, questions remain such as exemptions and implementation time.[36]

A comparative analogy is applicable to the Federal Right-to-Try Act in terms of reactive legislation to state right to try legislation. For example, why are medical foods not included nor addressed in the Federal Right-to-Try Act?[37] These are unanswered queries. Does the federal law provide anything new to a terminally ill patient? Does the patient understand the right to bring a lawsuit is thwarted?[38] Was there political pressure to enact Federal Right-to-Try legislation? Did the state Right-to-Try acts spur the federal legislation? Unequivocally, the state Right-to-Try legislative movement did spur federal action. Perhaps uniformity is preferred rather than various state laws. However, the state Right-to-Try certainly fostered Congress to Act in 2018.[39] Prior to delving into the Federal Right-to-Try Act,[40] the following primer details the investigational drug schemata.

III. FEDERAL PRIMER—INVESTIGATIONAL HUMAN DRUGS AND BIOLOGICAL DRUG PRODUCTS AND EXPANDED ACCESS

The Food and Drug Administration is the oldest comprehensive consumer

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http://www.dannon.com/the-dannon-pledge-on-sustainable-agriculture-naturality-and-transparency/ [https://perma.cc/U5B9-NG77].

36. *See* JENNIFER A. STAMAN, CONG. RESEARCH SERV., R43705, LEGAL ISSUES WITH FEDERAL LABELING OF GENETICALLY ENGINEERED FOOD: IN BRIEF (2016). http://nationalaglawcenter.org/wp-content/uploads/assets/crs/R43705.pdf [https://perma.cc/PW4H-Y982]; *see also* Greg Jaffe, *The ABCs of GMO Disclosure in the United States*, CTR FOR SCIENCE PUB. INT. (SEPT. 25, 2017), https://cspinet.org/news/abcs-gmo-disclosure-united-states-20170925 [https://perma.cc/2LWA-5P9K].

37. Medical foods is defined under the Federal, Food, Drug and Cosmetic Act in section 5(b) of the Orphan Drug Act 21 (U.S.C. § 360ee(b)(3)) as follows: "a food which is formulated to be consumed or administered enterally under the supervision of a physician and which is intended for the specific dietary management of a disease or condition for which distinctive nutritional requirements, based on recognized scientific principles, are established by medical evaluation." 21 U.S.C. §360ee(b)(3) (2017).

38. 21 U.S.C. § 360bbb-0a(b) (2017)

> (1) ALLEGED ACTS OR OMISSIONS. With respect to any alleged act or omission with respect to an eligible investigational drug provided to an eligible patient pursuant to section 561B of the Federal Food, Drug, and Cosmetic Act and in compliance with such section, no liability in a cause of action shall lie against
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> (A) a sponsor or manufacturer; or
>
> (B) a prescriber, dispenser, or other individual entity (other than a sponsor or manufacturer), unless the relevant conduct constitutes reckless or willful misconduct, gross negligence, or an intentional tort under any applicable State law.

39. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018).

40. *Id.*

protection agency in the United States federal government.[41] Striking the appropriate balance between free enterprise principles and obligatory government intervention is one of the most debated components of FDA regulation.[42] At the forefront, it is critical to understand the mission of the FDA as follows: "The Food and Drug Administration is responsible for protecting the public health by ensuring the safety, efficacy, and security of human and veterinary drugs, biological products, and medical devices; and by ensuring the safety of our nation's food supply, cosmetics, and products that emit radiation."[43] Yet, public protection is paramount.[44] Keeping the mission of the FDA in the forefront, the FDA aims to foster and protect public health through prompt and efficient review of clinical research and taking appropriate action on the marketing of regulated products in a timely manner.[45] Yet, this could be deemed insufficient for those who are desperately ill and seeking treatment for experimental products in a timely manner.[46]

Access to unapproved drugs includes participation in a clinical trial detailed below.[47] Eligibility parameters are specified in a protocol.[48] However, even if the eligibility criteria in a study protocol is not appropriate for a specific patient treatment, it may be possible to obtain the investigational treatment as a special exception termed expanded access generally referred to as compassionate use.[49] Expanded access, or as indicated, popularly referred to as compassionate use, is the use outside of a clinical trial[50] of an investigational medical product that has

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41. *The FDA History Office*, U.S. FOOD & DRUG ADMIN., https://www.fda.gov/AboutFDA/History/FDAHistoryOffice/default.htm [https://perma.cc/3JVA-B8FE] (last updated Feb. 1, 2018); Ben Panko, *Where Did the FDA Come From, and What Does It Do*?, SMITHSONIAN.COM (Feb. 8, 2017), https://www.smithsonianmag.com/science-nature/origins-FDA-what-does-it-do-180962054/ [https://perma.cc/M2PE-687D].

42. ROSEANN B. TERMINI, FOOD AND DRUG LAW: FEDERAL REGULATION OF DRUGS, BIOLOGICS, MEDICAL DEVICES, FOODS, DIETARY SUPPLEMENTS, PERSONAL CARE, VETERINARY AND TOBACCO PRODUCTS 5 (9th ed. 2017).

43. *What We Do*, U.S. FOOD & DRUG ADMIN., https://www.fda.gov/aboutfda/whatwedo [https://perma.cc/42XL-EP2B] (last updated Mar. 28, 2018).

44. *Id.*

45. *FDA Center for Drug Evaluation and Research (CDER) Strategic Plan 2013-2017*, U.S. FOOD & DRUG ADMIN., 3, https://www.fda.gov/downloads/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/UCM376545.pdf [https://perma.cc/N3W9-UQCX].

46. Clinical trials have eligibility criteria and at times patients are ineligible. *See, e.g.*, *Expanded Access (sometimes called "Compassionate Use")*, U.S. FOOD & DRUG ADMIN., https://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/default.htm [https://perma.cc/KQK7-8Q7X] (last updated June 19, 2018).

47. *Learn About Clinical Studies*, U.S. NAT'L LIBR. OF MED., https://clinicaltrials.gov/ct2/about-studies/learn [https://perma.cc/L6F2-P9KQ] (last updated Jan. 2017).

48. *Id.*

49. 21 C.F.R. § 312.300 (2018).

50. The U.S. National Library of Medicine's website, ClinicalTrials.gov, contains

not been yet approved by the FDA.[51] The FDA approval rate for compassionate use therapy is ninety-nine percent.[52] Treating a patient as a special exception entails modifying the informed consent form, sending the request to the FDA, and acquiring permission from an Institutional Review Board (IRB).[53] However, when treatment involves the emergency use of an investigational drug and approval from an IRB cannot be obtained before treatment, treatment may begin without prior IRB approval provided the IRB is notified of the emergency expanded access use within five working days of treatment.[54]

It also requires agreement of the manufacturer to provide the requested

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information about publicly and privately funded trials concerning safety and efficacy studies for Phase II drug approval process, Phase III drug approval process and post market Phase IV initiatives to require increased disclosure of clinical trial information remain in the forefront. As of July 21, 2018, there were 278,399 research studies in all 50 states and in 204 countries. U.S. NAT'L LIBR. OF MED., https://clinicaltrials.gov [https://perma.cc/8269-62RT].

51. *Expanded Access Categories for Drugs (Including Biologics)*, U.S. FOOD & DRUG ADMIN., https://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/ucm431774.htm [https://perma.cc/57NW-TQV6] (last updated Jan. 4, 2018).

52. *Expanded Access INDs and Protocols 2009-2017*, U.S. FOOD & DRUG ADMIN., https://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/ucm443572.htm [https://perma.cc/74K3-G75Z] (last updated Feb. 21, 2018). *See* Appendix I at the conclusion of this article. *See also Expanded Access INDs and Protocols 2009-2015*, U.S. FOOD & DRUG ADMIN., https://www.fda.gov/drugs/developmentapprovalprocess/howdrugsaredevelopedandapproved/drugandbiologicapprovalreports/indactivityreports/ucm373560.htm [https://perma.cc/Q9RT-2A2L].

53. *Id*. *See also Expanded Access to Investigational Drugs for Treatment Use —Questions and Answers, Guidance for Industry*, U.S. DEP'T OF HEALTH AND HUMAN SERVICES, FOOD & DRUG ADMIN., CTR. FOR DRUG EVALUATION AND RES., CTR. FOR BIOLOGICS EVALUATION AND RES. (OCT. 2017), https://www.fda.gov/downloads/drugs/guidances/ucm351261.pdf. "A physician submitting an individual patient expanded access IND using Form FDA 3926 may select the appropriate box on that form to request a waiver under § 56.105 of the requirements in § 56.108(c), which relate to full IRB review. FDA concludes that such a waiver is appropriate for individual patient expanded access INDs when the physician obtains concurrence by the IRB chairperson or another designated IRB member before treatment use begins. A physician submitting an individual patient expanded access IND using Form FDA 1571 may include a separate waiver request with the application." *Id*. at 6. Further, the Guidance defines and institutional review board (IRB) as follows. "An institutional review board (IRB) means any board, committee, or other group formally designated by an institution to review, to approve the initiation of, and to conduct periodic review of biomedical research involving human subjects. The primary purpose of IRB review is to assure that the rights and welfare of human subjects are protected, including by determining that informed consent is obtained in accordance with and to the extent required by Federal requirements. Institutions may have their own IRB to oversee human subjects research conducted within the institution or by the staff of the institution. If the patient's physician does not have access to a local IRB, an independent IRB may be used. The Department of Health and Human Services' Office for Human Research Protections maintains a database of registered IRBs." *Id*. at 5, n. 11.

54. 21 C.F.R. § 56.104 (2018).

investigational medical product. The health care practitioner is involved as well as the insurance company. As noted, several entities must acquiesce to the expanded use.

IV. FEDERAL PRIMER MECHANISMS INVESTIGATIONAL NEW DRUGS (IND) AND DEVICES INVESTIGATIONAL NEW DRUGS (INDS)

A review of the parameters for investigational new drugs is daunting. Yet, in a sense it should be in order to protect the human being. What should not be formidable is the process to obtain a potential lifesaving treatment. That is, the issue and perhaps the state right-to-try and now the Federal Right-to-Try Act is the panacea in terms of a patient's right-to-know that there is possibly an experimental drug. The panacea is a right-to-request a potentially life extending treatment with the caveat that the treatment is not necessarily a cure and the patient may not receive the treatment for a variety of reasons such as cost and manufacturer willingness to supply the product. In brief, the types of Investigator New Drugs (IND) include the following: Investigator IND, Emergency Use IND, and Treatment IND.[55] Once a sponsor submits an IND application, there is a thirty-day waiting period before commencing any clinical trials, for safety reasons.[56] Thirty days is too lengthy for those who are desperately ill. That is why FDA has a compassionate expanded use program available.[57]

Investigator IND Submissions—by a physician and the investigation is under direct control of the physician. For example, a physician could propose a research IND to suggest studying an unapproved drug. A physician could also propose a research IND for a new indication or for a new patient population.[58]

Emergency Use INDs—FDA permits use of an experimental drug in an emergency situation. This means that there is insufficient time for submission of an IND.[59] An emergency IND can be used for those patients who do not satisfy the conditions of an existing study protocol or possibly if there is no approved study protocol.[60]

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55. 21 C. F. R. § 312.34 (2018) (treatment use of an investigational new drug).

56. 21 C.F.R. § 312.305 (2018).

57. *Id.* (Requirements for all expanded access uses).

58. *Id.*

59. 21 C.F.R. § 312.310(d) (2018).

60. *Expanded Access to Investigational Drugs for Treatment Use —Questions and Answers, Guidance for Industry*, *supra* note 52 "In an emergency situation (either an emergency use IND or emergency use protocol) when there is not sufficient time to secure IRB review prior to beginning treatment, the emergency use of the investigational drug must be reported to the IRB within 5 working days of emergency use, as required under § 56.104(c)." *Id.* at 8. Further, "[a] physician submitting an individual patient expanded access IND using Form FDA 3926 may select the appropriate box on that form to request a waiver under § 56.105 of the requirements in § 56.108(c), which relate to full IRB review. FDA concludes that such a waiver is appropriate for individual patient expanded access INDs when the physician obtains concurrence by the IRB chairperson or

Treatment INDs—for experimental drugs showing potential in clinical testing for serious or life-threatening conditions and conducted during the final clinical work and during FDA review. Once an IND is filed, *several years* transpire prior to full approval. However, there are programs that facilitate and expedite development and review of new drugs that address unmet medical needs in the treatment of serious or life-threatening conditions are as follows:[61] fast track which means unmet medical need must be established; priority review which means a six month review time period where no sufficient therapy exists; accelerated approval involving initial clinical trials disclose disease reduction and survival lengthened; and a breakthrough therapy designation which is part of FDA Safety and Innovation Act.[62]

V. FEDERAL PRIMER—INVESTIGATIONAL MECHANISMS FOR MEDICAL DEVICES INVESTIGATIONAL MEDICAL DEVICE EXEMPTIONS AND CLINICAL TRIALS

The Federal Right-to-Try Act[63] does not specifically include medical devices yet arguably perhaps it does in the preamble and was expressly incorporated in prior legislative proposals.[64] Nevertheless, it is noteworthy to, at a minimum, recognize investigational devices. Similar to investigational drugs, investigational device exemptions (IDE) permit the use of a device on human subjects in clinical

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another designated IRB member before treatment use begins. A physician submitting an individual patient expanded access IND using Form FDA 1571 may include a separate waiver request with the application." *Id*. at 6.

61. *Fast Track, Breakthrough Therapy, Accelerated Approval, Priority Review*, U.S. FOOD & DRUG ADMIN., (Feb. 23, 2018), https://www.fda.gov/forpatients/approvals/fast/default.htm [https://perma.cc/8HCH-NTDC.]

62. Food and Drug Administration Safety and Innovation Act, Pub. L. No. 112-144, 126 Stat. 993 (2012) (codified as amended at 21 U.S.C.A. § 301(1938)). *See also* 21st Century Cures Act, Pub. L. No. 114-255, 130 Stat. 1033 (2016) (which is intended to accelerate drug and medical device approvals).

63. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018).

64. The prior legislative history details that medical devices were included, however, the law passed does not address medical devices. *See, e.g*., Right to Try Act of 2017, H.R. 878, 115th Cong. (2017-2018) (requiring the federal government to allow unrestricted manufacturing, distribution, prescribing, and dispensing of experimental drugs, biological products, and medical devices that are: "[1] intended to treat a patient who has been diagnosed with a terminal illness; and [2] authorized by . . . state law." *Id.*); *see also* 21 USC 360bbb-0 (b) (2017). In the preamble, of the Right-to-Try Act, the term "medical product" is used. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017. Pub. L. No. 115-176, 130 Stat. 834 (2018). Medical product is defined under 42 U.S. Code § 287a. "The term 'medical product' means a drug, device, biological product, or product that is a combination of drugs, devices, and biological products." 42 U.S.C. § 287a (a)(4) (2018).

trials.[65] An approved IDE stipulates the maximum amount of clinical sites and the maximum number of human subjects that may be enrolled in a study. FDA has the authority to exempt investigational use devices from particular requirements that other devices must abide by.[66] An approved IDE application permits shipment of a device, otherwise subject to marketing clearance.[67] An approved IDE permits use in a clinical trial to gather safety and efficacy data necessary to substantiate some 510(k) submissions and for PMA's.[68] Clinical studies are a requisite for a PMA and in some instances a 510(k) and require: an approved IDE by an institutional review board (IRB); informed consent from patients; labeling that details investigational use only; and records and reports.[69]

VI. EXPANDED ACCESS TO MEDICAL DEVICES

Some patients might not be eligible for an investigational device clinical trial. Fortunately, similar to investigational drugs, there are methods available to those patients who have a life-threatening disease for which there are no currently approved medical device treatments. These include emergency use, treatment use, and compassionate or humanitarian use, detailed below.

A. Emergency Use of Unapproved Medical Devices[70]

An unapproved device may provide the single potential life-saving alternative. However, what happens when there is no IDE or perhaps the proposed use is not approved under an existing IDE? Additionally, the physician or institution may not be approved under the IDE. FDA will use enforcement discretion, if a physician utilizes an unapproved device on an emergency basis. FDA prior approval is not required as long as the following criteria for emergency use are met:

> (1) The human subject is confronted by a life-threatening situation necessitating the use of the test article;
> (2) Informed consent cannot be obtained from the subject because of an inability to communicate with, or obtain legally effective consent from, the subject;

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65. 21 C.F.R. § 812 (2018).

66. *Id.*

67. *Id.*

68. *Id.*

69. Protection of Human Subjects, 21 C.F.R. § 50 (2018); Financial Disclosure by Clinical Investigators, 21 C.F.R. § 54 (2018); Institutional Review Boards, 21 C.F.R. § 56 (2018); Quality System Regulation, Design Controls, 21 C.F.R. § 820(C) (2018).

70. *Exception from General Requirements, 21 C.F.R. § 50.23(a) (2018). See also Expanded Access for Medical Devices*, U.S. FOOD & DRUG ADMIN., https://www.fda.gov/medicaldevices/deviceregulationandguidance/howtomarketyourdevice/investigationaldeviceexemptionide/ucm051345.htm [https://perma.cc/T53G-BBHE] (last updated Sept. 25, 2018).

> (3) Time is not sufficient to obtain consent from the subject's legal representative; and
> (4) There is available no alternative method of approved or generally recognized therapy that provides an equal or greater likelihood of saving the life of the subject.[71]

B. Treatment Use of Investigational Devices

Treatment use means that an IDE trial may be expanded to include additional patients with life-threatening or serious diseases if the data indicates that the device is effective.[72] The following parameters apply to treatment use of investigational devices:[73]

> the device is intended to treat or diagnose a serious or immediately life-threatening disease or condition; there is no comparable or satisfactory alternative device available to treat or diagnose the disease or condition in the intended patient population; the device is under investigation in a controlled clinical trial for the same use under an approved IDE, or all clinical trials have been completed; and the sponsor of the controlled clinical trial is pursuing marketing approval/clearance of the investigational device with due diligence.[74]

C. Humanitarian or Compassionate Use Devices

Similar to human drugs, compassionate use devices[75] provide an option for a patient that has a life- threatening or serious disease or condition. A humanitarian device, (HUD) also referred to as a compassionate use device, provides patients with a method to use an investigational device not yet approved. Compassionate use devices provide a possible access to those investigational devices that have not received FDA approval or clearance. Further, the patient's physician must be of the medical opinion the device may provide a benefit in treating or perhaps even diagnosing the disease or condition.

Compassionate use can be for patients who do not meet the requirements for inclusion in the clinical investigation and for devices that are in a clinical trial under an investigational device exemption.[76] Further, a compassionate use device may not be in a clinical investigation and is for a small group or an individual patient. The criteria include the following:[77]

> The patient has a life-threatening or serious disease or condition;

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71. Exception from General Requirements, 21 C.F.R. § 50.23(a) (2018).
72. Treatment Use of an Investigational New Device, 21 C.F.R. § 812.36 (2018).
73. *Id.*; *See also Expanded Access for Medical Devices*, *supra* note 69.
74. *Expanded Access for Medical Devices*, *supra* note 69.
75. *Id.*
76. *Id.*
77. *Id.*

> No generally acceptable alternative treatment for the condition exists; and
> Time is of essence which does not permit obtaining an investigational device exemption.[78]

Submission of a humanitarian device exemption application is necessary, and the approval rate is approximately 99%.[79] The Federal Right to Try Act does not address compassionate use for medical devices. [80]

VII. FDA ACTION PRIOR TO THE ENACTMENT OF THE FEDERAL RIGHT-TO-TRY LEGISLATION

As detailed above, despite state legislation, a patient who is ineligible to obtain an experimental therapy through a clinical trial has had the option to request experimental therapy through his or her physician to apply to the FDA to obtain experimental therapy under expanded access or compassionate use. According to FDA, expanded access or as indicated compassionate use is the "use of investigational drugs, biologics or medical devices outside the clinical trial setting for treatment purposes."[81] Further, to determine the suitability for expanded access the following are all requisite:[82]

> [The] patient has a serious disease or condition, *or* [his or her] life is immediately threatened by [the] disease or condition; [t]here is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; enrollment in a clinical trial is not possible; [any] potential patient benefit justifies [or outweighs] the potential risks of treatment; [using] the investigational medical product will not [impede the] investigational trials that could support [the] . . . development or marketing approval for the treatment indication.[83]

All of the above parameters are straightforward. However, the issue becomes how to obtain the potentially life-extending product. Perhaps that is why the state legislation[84] and now the Federal Right to Try Act[85] were enacted.

Following the introduction and passage of several state right-to-try laws, in

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78. *Id.*

79. *Id.*; *see also* Appendix II.

80. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018). The Federal Right to Try Act does not specifically include medical devices. Perhaps intentional or perhaps an oversight yet medical devices should have been included.

81. *Expanded Access (Sometimes Called "Compassionate Use"), supra* note 45.

82. Requirements for all Expanded Access Uses, 21 C.F.R. § 312.305 (2018).

83. *Expanded Access (Sometimes Called "Compassionate Use"), supra* note 45.

84. GOLDWATER INST., *supra* note 14.

85. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018).

February of 2015, the FDA released a proposed "streamlined" procedure for individual patient expanded access to experimental drugs.[86] The FDA published a final version on June 2, 2016, entitled "Individual Patient Expanded Access Applications – Form FDA 3962"[87], along with patient and physician information sheets and related guidances for all expanded access for experimental drugs and for charging for experimental drugs. Conditions include that the possible patient benefit validates the potential risks, the risk from the investigational drug is less than that from the disease or condition, the use will not interfere with drug development, and the patient cannot access an existing clinical trial.[88]

The aim of revised Form FDA 3926 procedures is for clarity purposes.[89] Form FDA 3926 was devised due to complaints about the prior forms, FDA 1571 and 1572, which were used for all IND submission, being both inappropriate and too burdensome for physicians to complete for individual patient access.[90] The revised procedure, which now also specifically applies to biologics, makes the application process easier for physicians; however, it relies on obtaining the agreement of the manufacturer/IND holder to provide the drug and to sign a Letter of Agreement (LOA) to allow access to its IND submission (and to contact the FDA when no LOA can be obtained or in a situation with an REMS), and retains the requirements for patient informed consent and IRB approval.[91] Further, Form 3926 is permitted for certain follow-up submissions.[92] Treatment may proceed 30 days after the FDA receives the completed Form FDA 3926 unless earlier notification is provided or under an emergency request.[93] There are follow-up responsibilities for the physician, now a sponsor-investigator.[94] These FDA procedures using Form FDA 3926 for individual expanded access are patient focused and streamlined.[95]

The state of Maryland Right-to-Try Act,[96] is intended to provide terminally

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86. U.S. DEP'T OF HEALTH & HUMAN SERVS., INDIVIDUAL PATIENT EXPANDED ACCESS APPLICATIONS: FORM FDA 3926 GUIDANCE FOR INDUSTRY (2017), https://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM432717.pdf [https://perma.cc/NWZ5-TAGC].

87. *Id.* Medical devices follow a separate pathway and are not included in this new guidance. The guidance provides that individual patient expanded access for drugs allows physicians to request access to investigational drugs outside of a clinical trial (or "for an approved drug where availability is limited by a risk evaluation and mitigation strategy (REMS)") for those individual patients who have serious or immediately life-threatening diseases or conditions where no other comparable or satisfactory treatment is available. *Id.*

88. *Id.*

89. *Id.*

90. *Id.* at 4.

91. *Id.* at 6.

92. *Id.* at 4.

93. *Id.* at 7.

94. *Id.* at 5.

95. *See id.*; *See also* app. III.

96. *See* Right to Try Act, MD. CODE ANN., HEALTH–GEN. § 21-2B-01 (LexisNexis 2017).

ill patients, who do not have the luxury of waiting for what may be a lengthy FDA approval process to be completed, with access to recommended experimental therapies, including drugs, biologics and medical devices, where a clinical trial is not available and currently approved and recognized treatment options are unlikely to prolong the patient's life. Like the FDA expanded access or compassionate use procedure, the Maryland Right-to-Try Act[97] relies on requests by physicians, patient informed consent, and the agreement of the manufacturer of the therapy in question to provide the therapy. Even prior to the enactment of the federal Right-to-Try Act, perhaps the related legislation in various states such as Maryland provided the impetus to the FDA to streamline its expanded access or compassionate use procedures for drugs and biologics.[98]

Does the Federal Right-to-Try Act provide anything further than what FDA has in place? Perhaps the Federal Right- to-Try Act is merely a step further toward a sense of right-to-request by the patient.[99] Optimistically, these laws increase the awareness of patients and physicians regarding state and federal procedures to obtain experimental therapies. It is important to remember though that both state and FDA procedures rely on the willingness of manufacturers to provide access to drugs, biologics, or perhaps devices upon request and insurers to pay.

Section 3 in the Federal Right-to-Try Act is of particular note.[100] Sense of the Senate of the Federal Right-to-Try Act which provides as follows:

> SEC. 3. SENSE OF THE SENATE.
> It is the sense of the Senate that section 561B of the Federal Food, Drug, and Cosmetic Act, as added by section 2—
> (1) *does not establish a new entitlement* or modify an existing entitlement, or otherwise establish a positive right to any party or individual (emphasis added);
> (2) does not establish any new mandates, directives, or additional regulations;
> (3) only expands the scope of individual liberty and agency among patients, in limited circumstances;
> (4) is consistent with, and will act as an *alternative pathway alongside, existing expanded access policies of the Food and Drug Administration*[101] (emphasis added);

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97. *Id.*; *see also* app. V.

98. *Id.*

99. Roseann B. Termini & Janet M. Lis, '*Right-to-Try' or 'Right-to-Ask'? Do State Right-to-Try Laws Offer an Answer for Terminally Ill Patients Seeking Access to Investigational Therapies?*, 38 PA. LAW. 45 (2016) (authors conclude "right-to-ask" provides autonomy).

100. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018).

101. *See, e.g.*, *Expanded Access (Sometimes Called "Compassionate Use")*, *supra* note 80.

> (5) will not, and cannot, create a cure or effective therapy where none exists;
> (6) recognizes that the eligible terminally ill patient population often consists of those patients with the highest risk of mortality, and use of experimental treatments under the criteria and procedure described in such section 561A involves an informed assumption of risk; and
> (7) establishes national standards and rules by which investigational drugs may be provided to terminally ill patients.[102]

The alternative pathway is that of expanded access discussed above. Again, a patient who is ineligible to obtain an experimental therapy through a clinical trial could request that his or her physician apply to the FDA to obtain an experimental therapy commonly known as compassionate use procedures. The FDA approval rate for expanded access or compassionate use therapy is 99 percent.[103] One question that remains unanswered is whether physicians and patients are aware of the federal expanded access or compassionate use mechanism already in place. Another issue is whether physicians are willing to expend the considerable time and effort needed to pursue expanded access.

VIII. PROACTIVE SOLUTIONS AND RECOMMENDATIONS

The intent of the Federal Right-to-Try Act is not to undermine FDA authority. Rather the intent is to provide an individual patient with the "liberty"[104] to request a potentially life-saving therapy.[105] However, the following are recommended actions to comport with the intent of the Federal Right-to-Try Act:

- The FDA and the health care industry should utilize a collaborative approach to ensure awareness of the expanded or "compassionate" use program in place at FDA.
- Congress should amend the Federal Right-to-Try Act to expressly address what was not clarified in the current Federal Right-to-Try Act such as medical devices,[106] medical foods[107] and perhaps dietary

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102. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018).

103. *See* Jennifer E. Miller, Joseph S. Ross, Kenneth I. Moch & Arthur L. Caplan, *Characterizing Expanded Access and Compassionate Use Programs for Experimental Drugs*, 10 BIOMED CENT. RES. NOTES 350 (2017) https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5534121/ [https://perma.cc/KJ3G-F5AQ]. *See also* app. I.

104. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018).

105. 164 CONG. REC. H43,55-66 (daily ed. May 22, 2018); Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018) ("[I]t is the sense of the Senate that section 561B of the Federal Food, Drug and Cosmetic Act, as added by section 2- . . . (4) is consistent with, and will act as an alternative pathway alongside, existing expanded access policies of the Food and Drug Administration.")

106. The prior legislative history details that medical devices were included; however, the law passed does not address medical devices. *See, e.g.*, Right to Try Act, H.R. 878, 115th Cong. (2017-

supplements for the desperately ill who have a life threatening condition(s).[108]

- Include Medical Devices through deeming or by amendment.[109]
- Congress should amend the Federal Right-to-Try Act or by delegation to FDA, should specify that an advocate be appointed for the patient.
- Congress could amend the Federal Right-to-Try Act or by delegation should provide clarity about those involved in the process so that a patient understands that the sponsor manufacturer, prescriber, dispenser or other individual entity[110] can deny access by not providing the requested therapy as well as understand the limitation as to liability and institute a legal action.[111]
- Terminology—The Federal-Right-to-Try Act[112] correctly references eligible patient yet should use the consistent terminology throughout for uniformity purposes such as life-threatening and terminally ill which

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2018) (as introduced in the House on Feb. 6, 2017) (This bill requires the "[T]he Federal Government shall not take any action to prohibit or restrict—(1) the production, manufacture, distribution, prescribing, or dispensing of an experimental drug, biological product, or device that—(A) is intended to treat a patient who has been diagnosed with a terminal illness; and (B) is authorized by, and in accordance with, State law . . .").

107. The term "medical food", as defined under the Federal, Food, Drug and Cosmetic Act, means "a food which is formulated to be consumed or administered enterally under the supervision of a physician and which is intended for the specific dietary management of a disease or condition for which distinctive nutritional requirements, based on recognized scientific principles, are established by medical evaluation." 21 U.S.C. § 360ee(b)(3) (2018).

108. *See* Dietary Supplement Health and Education Act of 1994, Pub. L. No. 103-417, 108 Stat. 4325 (FDA regulates dietary supplements post-market and unlike drugs and some medical devices there is no pre-approval of dietary supplements.).

109. *See* Deeming Tobacco Products To Be Subject to the Federal Food, Drug, and Cosmetic Act, as amended by the Family Smoking Prevention and Tobacco Control Act; Restrictions on the Sale and Distribution of Tobacco Products and Required Warning Statements for Tobacco Products, 81 Fed. Reg. 28973 (May 10, 2016) (to be codified at 21 CFR pt. 1100, 21 CFR pt. 1140, and 21 CFR pt. 1143) (As an analogy and example of deeming regulations, FDA issued deeming regulations which extends FDA's authority to include the regulation of electronic nicotine delivery systems (such as e-cigarettes and vape pens), all cigars, hookah (waterpipe) tobacco, pipe tobacco and nicotine gels. The final rule deems products "meeting the statutory definition of "tobacco product," except accessories of the newly deemed tobacco products, to be subject to the Federal Food, Drug, and Cosmetic Act (the FD&C Act), as amended by the Family Smoking Prevention and Tobacco Control Act (Tobacco Control Act)." *Id.*).

110. 164 CONG. REC. H43,56 (daily ed. May 22, 2018) (§ 561B(b)(2) Determination not to provide drug).

111. *Id.* at § 561B(b) No Liability.

112. The Federal-Right-to-Try Act, uses the term "terminal illness" and "eligible patient" in Sec. 2. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018).

could be accomplished by a technical amendment.[113]

- Informed Consent-perhaps Congress should review and expand on informed consent as addressed in the Maryland Right-to-Try Act as it is comprehensive and patient centered.[114]

IX. FINAL COMMENTARY-OVERALL BENEFIT OF RIGHT-TO-TRY

The Federal Right-to-Try Act, FDA's expanded access procedures known as compassionate use procedures as well as State Right-to-Try laws may more properly be seen as providing a right-to-request.[115] Both the Federal Right-to-Try Act and the state Right-to-Try laws do appear to be raising the awareness of the potential use of and empowering desperately ill patients to request experimental therapies.[116] For example, as detailed above, the FDA revised Form 3926 streamlines the process in obtaining FDA approval for compassionate use.[117]

What is most critical at this junction is to ensure a user-friendly approach to navigate the system to obtain an experimental therapy for an individual human being. It is uncertain, however, if the state Right-to-Try laws are effective in accessing experimental therapies or whether any eventual access is actually obtained through FDA procedures, and whether the state laws can stand in relation to the existing Federal Right-to-Try[118] authority and principles of preemption.[119] However, when patients do request experimental therapies they must be counseled on the process involved, all potentially available mechanisms, of the possibility that they may not receive the experimental therapy, the potential

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113. *See generally* 21 C.F.R. § 312.81 (2018). ("This section applies to new drug and biological products that are being studied for their safety and effectiveness in treating life-threatening or severely-debilitating diseases.(a) For purposes of this section, the term '*life-threatening*' (emphasis added) means: (1) Diseases or conditions where the likelihood of death is high unless the course of the disease is interrupted; and (2) Diseases or conditions with potentially fatal outcomes, where the end point of clinical trial analysis is survival (b) For purposes of this section, the term 'severely debilitating' means diseases or conditions that cause major irreversible morbidity. (c) Sponsors are encouraged to consult with FDA on the applicability of these procedures to specific products.").

114. MD. CODE ANN., HEALTH-GEN. § 21-2b (West)

115. Termini & Lis, *supra* note 97, at 45.

116. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018). *See also Expanded Access (Sometimes Called "Compassionate Use"), supra* note 45.

117. The revised Form FDA 3926 is less complicated and provides a streamlined process for submission to FDA. *See* U.S. DEP'T OF HEALTH & HUMAN SERVS., *supra* note 85. App. III for the timeline specifications.

118. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018).

119. *Id*. Yet, arguably, since the Federal Right-to-Try does not expressly cover medical devices, state Right-to-Try could prevail and provide a mechanism for experimental treatment in that regard.

risks, the costs involved, and the limitation of the right to institute a lawsuit.

Finally, as indicated, there is no simple definitive solution. Yet, perhaps the state Right-to-Try initiatives and now the Federal Right-to-Try Act,[120] albeit with realistic hope, may serve a valuable substantive purpose by providing a method for patients to assert their desires and needs, to foster autonomy, to further their self-determination for their health care , and above all to maintain their human dignity.

120. *Id.*

X. APPENDICES

A. Appendix I: Expanded Access INDs FY 2012-2017[121]

Expanded Access INDs

Expanded Access INDs		Individual (Single) Patient Non-Emergency IND		Individual (Single) Patient Emergency IND		Intermediate Size IND		Treatment IND	
		received	allowed to proceed	received	allowed to proceed	received	allowed to proceed	received	allowed to proceed
FY2017	CDER	1111	1107	461	461	36	35	0	0
	CBER	40	36	80	79	11	10	2	2
FY2016	CDER	997	992	473	473	37	33	0	0
	CBER	28	27	90	90	5	5	4	2
FY 2015	CDER	747	745	431	428	46	45	0	0
	CBER	32	29	68	66	2	1	2	2
FY 2014	CDER	696	692	1069	1066	52	50	0	0
	CBER	22	19	45	44	1	1	1	1
FY 2013	CDER	550	550	315	313	28	27	0	0
	CBER	37	31	112	112	10	8	0	0

121. *Expanded Access INDs and Protocols 2009-2017*, *supra* note 52; *IND Investigational New Drug Application*, U.S. FOOD & DRUG ADMIN., https://www.fda.gov/drugs/developmentapprovalprocess/howdrugsaredevelopedandapproved/approvalapplications/investigationalnewdrugindapplication/default.htm [https://perma.cc/GA7Y-74LZ] (last updated Oct. 5, 2017).

A. Appendix I: Expanded Access INDs FY 2012-2017 (cont'd)

Expanded Access INDs

Expanded Access INDs		Individual (Single) Patient Non-Emergency IND		Individual (Single) Patient Emergency IND		Intermediate Size IND		Treatment IND	
		received	allowed to proceed	received	allowed to proceed	received	allowed to proceed	received	allowed to proceed
FY 2012	CDER	498	496	289	287	14	14	0	0
	CBER	47	39	36	35	1	1	2	2
10/2010 to 10/2011 ***	CDER	652	652	443	442	0	0	1	1
	CBER	22	18	24	24	4	4	1	1
10/13/2009 to 10/12/2010 ***	CDER	484	484	516	500	2	2	0	0
	CBER	17	14	24	24	1	1	0	0

These reporting periods cover a one-year cohort starting the day the Final Rule for Expanded Access to Investigational Drugs for Treatment Use and Charging for Investigational Drugs went into effect. Starting with Fiscal Year 2012, the reporting period was changed to a fiscal year to match the reporting period for other IND activity reports.

B. Appendix II[122]

Compassionate Use IDE Supplements

Year	Total Submissions	Evaluable Submissions*	Percent Approved**
2012	135	123	99.19%
2013	181	175	98.86%
2014	228	216	99.54%
2015	215	208	99.04%

*Excludes those withdrawn or converted to Emergency Use while under review
**Based on Evaluable Submissions

Compassionate Use Requests Without an Ide

Year	Total Submissions	Evaluable Submissions*	Percent Approved**
2012	53	53	98.11%
2013	138	134	91.79%
2014	112	101	99.01%
2015	170	167	98.80%

*Excludes those withdrawn or converted to Emergency Use while under review
**Based on Evaluable Submission

122. *Expanded Access for Medical Devices*, *supra* note 69.

C. Appendix III[123]

Individual Patient Expanded Access IND Application for Emergency Use: Initial Submission

Time	Action	Supporting Documentation
Day 0-1	Contact sponsor/manufacturer to obtain their agreement to provide expanded access to the investigational drug	Letter of authorization from sponsor/manufacturer granting a right of reference to the information contained in their existing IND • Letter of Authorization (see online template) to be sent to FDA at the time of application submission by Day 15
Day 1	Call FDA to obtain FDA authorization for the expanded access use	Information will be requested by the FDA representative and can be provided via phone, fax, or e-mail
Day 1	Obtain informed consent from patient or their legally authorized representative prior to administering treatment	
Post-treatment by Day 5	Notify Institutional Review Board (IRB) of the emergency expanded access use	Supporting documentation as required by the respective applicable IRB

123. *Emergency IND Timeline,* U.S. DEP'T OF HEALTH & HUMAN SERVS., https://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/ucm597130.htm [https://perma.cc/D6QM-AYMH] (last updated Feb. 20, 2018).

By Day 15	Submit the expanded access IND application to the appropriate Review Division in the Center for Drug Evaluation and Research (CDER) at FDA Insert your IND number, provided to you by FDA staff, in the appropriate section of the application form (e.g., section titled, *Physician's IND Number* in section 3 of Form FDA 3926)	Form FDA 39261 Letter of Authorization2 from sponsor/manufacturer

[1] Form FDA 1571 and 1572 are also accepted, however, Form FDA 3926 is a streamlined form created specifically for individual patient IND submissions, including those for emergency use.

[2] In the absence of a Letter of Authorization from the sponsor/manufacturer, the expanded access IND application's sponsor is responsible for providing the following in the IND application submission:

- Description of the facility where the drug is manufactured;
- Chemistry, manufacturing, and controls information adequate to ensure the proper identification, quality, purity, and strength of the investigational drug;
- Pharmacology and toxicology information adequate to conclude that the drug is reasonably safe at the dose and duration proposed for the emergency use.

D. Appendix IV Federal Right to Try[124]

To authorize the use of unapproved medical products by patients diagnosed with a terminal illness in accordance with State law, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the "Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017".

SEC. 2. USE OF UNAPPROVED INVESTIGATIONAL DRUGS BY PATIENTS DIAGNOSED WITH A TERMINAL ILLNESS.

(a) In General.—Chapter V of the Federal Food, Drug, and Cosmetic Act is amended by inserting after section 561A (21 U.S.C. 360bbb–0) the following:

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124. Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, Pub. L. No. 115-176, 132 Stat. 1372 (2018).

"SEC. 561B. INVESTIGATIONAL DRUGS FOR USE BY ELIGIBLE PATIENTS.

"(a) Definitions.—For purposes of this section—

"(1) the term 'eligible patient' means a patient—

"(A) who has been diagnosed with a life-threatening disease or condition (as defined in section 312.81 of title 21, Code of Federal Regulations (or any successor regulations));

"(B) who has exhausted approved treatment options and is unable to participate in a clinical trial involving the eligible investigational drug, as certified by a physician, who—

"(i) is in good standing with the physician's licensing organization or board; and

"(ii) will not be compensated directly by the manufacturer for so certifying; and

"(C) who has provided to the treating physician written informed consent regarding the eligible investigational drug, or, as applicable, on whose behalf a legally authorized representative of the patient has provided such consent;

"(2) the term 'eligible investigational drug' means an investigational drug (as such term is used in section 561)—

"(A) for which a Phase 1 clinical trial has been completed;

"(B) that has not been approved or licensed for any use under section 505 of this Act or section 351 of the Public Health Service Act;

"(C) (i) for which an application has been filed under section 505(b) of this Act or section 351(a) of the Public Health Service Act; or

"(ii) that is under investigation in a clinical trial that—

"(I) is intended to form the primary basis of a claim of effectiveness in support of approval or licensure under section 505 of this Act or section 351 of the Public Health Service Act; and

"(II) is the subject of an active investigational new drug application under section 505(i) of this Act or section 351(a)(3) of the Public Health Service Act, as applicable; and

"(D) the active development or production of which is ongoing and has not been discontinued by the manufacturer or placed on clinical hold under section 505(i); and

"(3) the term 'phase 1 trial' means a phase 1 clinical investigation of a drug as described in section 312.21 of title 21, Code of Federal Regulations (or any successor regulations).

"(b) Exemptions.—Eligible investigational drugs provided to eligible patients in compliance with this section are exempt from sections 502(f), 503(b)(4), 505(a), and 505(i) of this Act, section 351(a) of the Public Health Service Act, and parts 50, 56, and 312 of title 21, Code of Federal Regulations (or any successor regulations), provided that the sponsor of such eligible investigational drug or any person who manufactures, distributes, prescribes, dispenses, introduces or delivers for introduction into interstate commerce, or provides to an eligible patient an eligible investigational drug pursuant to this section is in compliance with the applicable requirements set forth in sections 312.6, 312.7, and 312.8(d)(1) of title 21, Code of Federal Regulations (or any successor regulations) that apply to investigational drugs.

"(c) Use Of Clinical Outcomes.—

"(1) IN GENERAL.—Notwithstanding any other provision of this Act, the Public Health Service Act, or any other provision of Federal law, the Secretary may not use a clinical outcome associated with the use of an eligible investigational drug pursuant to this section to delay or adversely affect the review or approval of such drug under section 505 of this Act or section 351 of the Public Health Service Act unless—

"(A) the Secretary makes a determination, in accordance with paragraph (2), that use of such clinical outcome is critical to determining the safety of the eligible investigational drug; or

"(B) the sponsor requests use of such outcomes.

"(2) LIMITATION.—If the Secretary makes a determination under paragraph (1)(A), the Secretary shall provide written notice of such determination to the sponsor, including a public health justification for such determination, and such notice shall be made part of the administrative record. Such determination shall not be delegated below the director of the agency center that is charged with the premarket review of the eligible investigational drug.

"(d) Reporting.—

"(1) IN GENERAL.—The manufacturer or sponsor of an eligible investigational drug shall submit to the Secretary an annual summary of any use of such drug under this section. The summary shall include the number of doses supplied, the

number of patients treated, the uses for which the drug was made available, and any known serious adverse events. The Secretary shall specify by regulation the deadline of submission of such annual summary and may amend section 312.33 of title 21, Code of Federal Regulations (or any successor regulations) to require the submission of such annual summary in conjunction with the annual report for an applicable investigational new drug application for such drug.

"(2) POSTING OF INFORMATION.—The Secretary shall post an annual summary report of the use of this section on the internet website of the Food and Drug Administration, including the number of drugs for which clinical outcomes associated with the use of an eligible investigational drug pursuant to this section was—

"(A) used in accordance with subsection (c)(1)(A);

"(B) used in accordance with subsection (c)(1)(B); and

"(C) not used in the review of an application under section 505 of this Act or section 351 of the Public Health Service Act.".

(b) No Liability.—

(1) ALLEGED ACTS OR OMISSIONS.—With respect to any alleged act or omission with respect to an eligible investigational drug provided to an eligible patient pursuant to section 561B of the Federal Food, Drug, and Cosmetic Act and in compliance with such section, no liability in a cause of action shall lie against—

(A) a sponsor or manufacturer; or

(B) a prescriber, dispenser, or other individual entity (other than a sponsor or manufacturer), unless the relevant conduct constitutes reckless or willful misconduct, gross negligence, or an intentional tort under any applicable State law.

(2) DETERMINATION NOT TO PROVIDE DRUG.—No liability shall lie against a sponsor manufacturer, prescriber, dispenser or other individual entity for its determination not to provide access to an eligible investigational drug under section 561B of the Federal Food, Drug, and Cosmetic Act.

(3) LIMITATION.—Except as set forth in paragraphs (1) and (2), nothing in this section shall be construed to modify or otherwise affect the right of any person to bring a private action under any State or Federal product liability, tort, consumer protection, or warranty law.

SEC. 3. SENSE OF THE SENATE.
It is the sense of the Senate that section 561B of the Federal Food, Drug, and Cosmetic Act, as added by section 2—

(1) does not establish a new entitlement or modify an existing entitlement, or otherwise establish a positive right to any party or individual;

(2) does not establish any new mandates, directives, or additional regulations;

(3) only expands the scope of individual liberty and agency among patients, in limited circumstances;

(4) is consistent with, and will act as an alternative pathway alongside, existing expanded access policies of the Food and Drug Administration;

(5) will not, and cannot, create a cure or effective therapy where none exists;

(6) recognizes that the eligible terminally ill patient population often consists of those patients with the highest risk of mortality, and use of experimental treatments under the criteria and procedure described in such section 561A involves an informed assumption of risk; and

(7) establishes national standards and rules by which investigational drugs may be provided to terminally ill patients.

E. Appendix V: Maryland as an Example of State Right to Try Legislation[125]

TITLE 21. FOOD, DRUGS, AND COSMETICS > SUBTITLE 2B. RIGHT TO TRY ACT.

(a) In general. -- In this subtitle the following words have the meanings indicated.
(b) Carrier. -- "Carrier" has the meaning stated in § 15-10A-01(c) of the Insurance Article.
(c) Eligible patient. -- "Eligible patient" means an individual who:
(1) Has a terminal illness, attested to by the individual's treating physician;
(2) Has considered all other treatment options currently approved by the United States Food and Drug Administration;
(3) Has received a recommendation from the individual's treating physician for the use of an investigational drug, biological product, or device;
(4)
(i) Has given informed consent for the use of the investigational

125. MD. CODE ANN., HEALTH–GEN. § 21-2B01 (LexisNexis 2018)

drug, biological product, or device; or

(ii) If the individual is a minor or lacks the mental capacity to provide informed consent, has a parent or legal guardian who has given informed consent on the individual's behalf for the use of the investigational drug, biological product, or device;

(5) Is ineligible for or unable to participate in a clinical trial; and

(6) Has documentation from the individual's treating physician that the individual meets the requirements of items (1) through (5) of this subsection.

(d) Health occupations board. -- "Health occupations board" means a board established under the Health Occupations Article that issues licenses to practice a health occupation in the State.

(e) Informed consent. -- "Informed consent" means a written document prepared using the informed consent form developed by the Office of the Attorney General in accordance with § 21-2B-02(d)(1) of this subtitle that:

(1) Is signed by the patient or a parent or legal guardian of the patient;

(2) Is attested to by the patient's treating physician and a witness; and

(3) At a minimum:

(i) Explains the currently approved products and treatments for the disease or condition from which the patient suffers;

(ii) Attests to the fact that the patient concurs with the patient's treating physician in believing that all currently approved and conventionally recognized treatments are unlikely to prolong the patient's life;

(iii) Identifies clearly the specific proposed investigational drug, biological product, or device that the patient is seeking to use;

(iv) Informs the provider and eligible patient of any known or anticipated side effects, risks, or reported patient discomfort that is likely related to the treatment;

(v) Describes the best and worst potential outcomes of using the investigational drug, biological product, or device 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, based on the treating physician's knowledge of the proposed treatment in conjunction with an awareness of the patient's condition;

(vi) Makes clear that the patient's carrier and health care provider are not obligated to pay for any care or treatments that are necessary as a result of the use of the investigational drug, biological product, or device except as required by federal or State law or contract;

(vii) Makes clear that the patient's eligibility for hospice care may be withdrawn if the patient begins curative treatment with the investigational drug, biological product, or device and that hospice care may be reinstated if this treatment ends and the patient meets hospice eligibility requirements; and

(viii) States that the patient understands that the patient may be

liable for all expenses relating to the use of the investigational drug, biological product, or device and that this liability extends to the patient's estate, but not the heirs or legatees of the patient.

(f) Investigational drug, biological product, or device. -- "Investigational drug, biological product, or device" means a drug, biological product, or device that:

(1) Has successfully completed Phase I of a clinical trial but has not yet been approved for general use by the United States Food and Drug Administration; and

(2) Remains under investigation or in a clinical trial approved by the United States Food and Drug Administration.

(g) Terminal illness. -- "Terminal illness" means a disease or condition that, without life-sustaining procedures, will result in death or a state of permanent unconsciousness from which recovery is unlikely within 12 months.

F. Appendix VI[126]

Individual Patient Expanded Access IND Application for Emergency Use: Initial Submission

Time	Action	Supporting Documentation
Day 0-1	Contact sponsor/manufacturer to obtain their agreement to provide expanded access to the investigational drug	Letter of authorization from sponsor/manufacturer granting a right of reference to the information contained in their existing IND • Letter of Authorization (see online template) to be sent to FDA at the time of application submission by Day 15
Day 1	Call FDA to obtain FDA authorization for the expanded access use	Information will be requested by the FDA representative and can be provided via phone, fax, or e-mail
Day 1	Obtain informed consent from patient or their legally authorized representative prior to administering treatment	

126. *Emergency IND Timeline*, *supra* note 123.

Post-treatment by Day 5	Notify Institutional Review Board (IRB) of the emergency expanded access use	Supporting documentation as required by the respective applicable IRB
By Day 15	Submit the expanded access IND application to the appropriate Review Division in the Center for Drug Evaluation and Research (CDER) at FDA Insert your IND number, provided to you by FDA staff, in the appropriate section of the application form (e.g., section titled, *Physician's IND Number* in section 3 of Form FDA 3926)	Form FDA 39261 Letter of Authorization2 from sponsor/manufacturer

Form FDA 1571 and 1572 are also accepted, however, Form FDA 3926 is a streamlined form created specifically for individual patient IND submissions, including those for emergency use.

In the absence of a Letter of Authorization from the sponsor/manufacturer, the expanded access IND application's sponsor is responsible for providing the following in the IND application submission:

- Description of the facility where the drug is manufactured;
- Chemistry, manufacturing, and controls information adequate to ensure the proper identification, quality, purity, and strength of the investigational drug;
- Pharmacology and toxicology information adequate to conclude that the drug is reasonably safe at the dose and duration proposed for the emergency use.