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PATIENTS' RIGHT TO CHOOSE; THE RIGHT-TO-TRY LAW MOVEMENT

Implementation of right-to-try laws has swiftly increased among states all across the country. Currently, twenty-four states have signed right-to-try laws into effect, a number which is projected to grow in the near future.¹ Right-to-try laws purport to give terminally ill patients the right to receive an “experimental new drug treatment,” meaning that the drug has not received Food and Drug Administration (FDA) approval.² Proponents of right to try adamantly suggest patients and their physicians should be the one to decide whether or not a patient receives access to experimental drug treatment, not the government. But as many states begin to embrace right-try-laws, critics see it as a direct attack on the FDA regulations designed to protect patients, begging the question of whether the patient or the FDA should be the final decision maker when it comes to experimental drug treatments.³ And who is best suited to provide regulatory oversight to ensure patient safety.

Birth of the Right-to-Try Law Movement

Proponents of right-to-try laws claim access to investigational drugs through current FDA regulations is a time consuming process that requires approval through participation in a clinical trial or the expanded access provision.⁴ Time, however, is something these patients do not have. Each day without a viable course of treatment is another day detracted from their lives. In an effort to address public outcry for faster access to experimental drug treatment the state right-to-try law movement was born.

The rapid increase in these laws can be attributed to the series of publicized stories from terminally ill patients about their struggle to receive treatment. A perfect example of this is the

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story of Jenn McNary.⁵ Ms. McNary is a mother of two boys, both diagnosed with Duchenne Muscular Dystrophy, a debilitating disease that leads to muscle degeneration.⁶ One of Ms. McNary's sons was able to participate in an FDA approved clinical trial for a drug that could transform his disorder into a milder form of muscular dystrophy called Becker's.⁷ While one son's health significantly improved, she watched her other son's health deteriorate.⁸ Ms. McNary described the ordeal, saying, "having a child that is dying is the most painful thing in the world... [and] the only thing that is more painful is having a child that is dying and knowing there is a drug that could help them, and not being able to have access to it. It's a crime."⁹

Kianna Karnes, a 41-year-old mother of four, who was diagnosed with kidney cancer, has a similar story.¹⁰ The FDA denied her approval to participate in clinical trials for new investigational drugs that had potential to be a sustainable course of treatment for her disease, given the progression of her cancer.¹¹ Eager to save his daughter, Ms. Karnes's father sought approval through the FDA's compassionate use process.¹² Sadly, Ms. Karnes died the same day her compassionate use request was approved.¹³ The McNary and Karnes' stories are just two of several cited by supporters of right-to-try laws to push for statewide implementation. As social media platforms generate more public awareness of stories like Ms. McNary and Ms. Karnes, policy makers began to shift their focus to patient autonomy through state right-to-try laws.¹⁴

Power struggle: State vs. Federal Oversight

States do not have the power to bypass FDA regulations, as it would exceed the power granted to the states under federalism. But if states were allowed to bypass the FDA's decision-making power, states would have show they would be able to provide the necessary level of oversight that comes with monitoring patients decision to use experimental treatment. Currently, right-to-try laws emphasize patient autonomy, but fall short of patient protection; something

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much needed to protect this vulnerable group from harm. States ultimately fail to show that patients and their physicians can handle this type of power and instead highlight a new set of problems to follow if these laws were given legally effective power under state level authority. But, the right-to try law movement has rekindled the notion of patient autonomy and forced the FDA to revamp its expanded access application for faster patient access.

State Right-to-Try Laws promote patient autonomy, but fall short of patient protection

Critics of right-to-try laws argue that these laws would leave a number of unanswered legal questions.¹⁵ For example, if an insurer is not legally required to cover the cost of the investigational drug/product, patients may be expected to cover the cost themselves.¹⁶ Further, patients may need legal assistance when signing any potential additional consent forms required by the state.¹⁷ More importantly, the safety of patients who use these experimental drugs may be at risk if doctors and manufacturers are shielded from liability.¹⁸ Many of these issues are already presented in some state right-to-try laws. For instance, both the New Jersey bill and Minnesota law clearly focus on patient autonomy, yet they leave many unanswered questions.

Minnesota's right-to-try law defines who qualifies to receive the drug, what an investigational drug/biological product is, and what it means to be terminally ill under the provision.¹⁹ A patient qualifies to receive access to an investigational drug, biological product, or device once a physician has documented in writing that the patient has met all of the following requirements:²⁰

- (1) has a terminal illness; (2) has, in consultation with a physician considered all other treatment options currently approved by the FDA; (3) has been given a prescription or recommendation by a physician for an investigational drug, biological product, or device; and (4) has given

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informed consent, in writing, for the use of the investigational drug, biological product, or device, or if the patient is under the age of 18, or lacks the mental capacity to provide informed consent, a parent or legal guardian has given informed consent, in writing, on behalf of the patient.²¹

The Minnesota law gives the manufacturer the option of making its investigational drug, biological product, or device available to eligible patients under this section.²² A manufacturer can also provide the drug or biological product free of cost to the patient, or require the patient to pay the costs associated with the manufacturing of the product.²³ A patient's health insurance provider is not required to cover the cost associated with the investigation drug or biologic product, meaning patients may be left with high medical bills.²⁴ Although offering terminally ill patients an experimental drug free of charge is praiseworthy, this provision would also give manufacturers too much discretion in drug pricing. Without proper oversight, manufacturers have the potential to inflate the cost associated with the production of the drug and take advantage of this distressed group.

New Jersey's proposed right-to-try law, Bill No. 3474, includes a policy statement that asserts, it is a patient's fundamental right to have access to these investigational drugs.²⁵ The bill states:

the standards of the United States [FDA] for the use of investigational drugs, biological devices...may deny the benefits of potentially life-saving treatments to patients who have a terminal illness. Patients who are terminally ill have a fundamental right to attempt to pursue the preservation of their lives by accessing available investigational drugs, biological products, and devices.²⁶

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Similar to the Michigan right-to-try law, the New Jersey bill also contains some discrepancies. For example, in one section of the bill it states, “a government medical assistance program or private health insurer may, but is not required to, provide coverage for the cost of an investigational drug, biological product, or device.”²⁷ However, another section of the bill states “government medical assistance programs and private health insurers would not be required to... but private insurers would be permitted to provide coverage if they so choose.”²⁸ Therefore, the conflicting language makes it unclear if both a government assistance program and private insurer can cover the cost of the treatment, or only the private insurer.

These right-to-try laws also each have their own definition of certain terms that can leave a lot of the decision making up to interpretation. For instance, under the Minnesota statute, terminal illness is defined as “a condition or illness which, to a reasonable degree of medical probability, is not considered reversible and even with the administration of current FDA-approved and available treatments and the administration of life-sustaining procedures will soon result in death.”²⁹ The words “soon result in death” are not further clarified and given a physician’s expertise and discussion with a patient, each physician may have a different take on what is soon under the circumstances.

Although these laws often state that the new drug has to have successfully completed phase I of the FDA clinical trial investigational drug approval process as a requirement for access, they lack proper party accountability.³⁰ Under the Minnesota statute, healthcare providers are not liable for harm done to a patient as a result of using the experimental drug treatment, as long as the health care provider or entity has complied with the terms of the statute provisions.³¹ When physicians and manufacturers are not held liable for harm caused to patients, patient safety is at risk. Michigan and Colorado right to-try-laws explicitly state that a patient’s

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insurer does not have to cover cost associated with any complication that may arise as a result of receiving the investigational drug treatment.³² As seen under the Minnesota provision, these types of laws would allow manufacturers and treating physicians to escape harm caused to a patient as result of the investigational treatment. By also suggesting insurers are allowed to not cover cost associated with complications suffered by patients, individuals would be placed at a higher risk of harm, especially because patients are unlikely to be able to pay the cost to receive the necessary treatment out of pocket.

Some states, however, have chosen to take the opposite approach to right-to-try laws. California governor, Jerry Brown chose to go against the right-to-try law movement and vetoed California's right-to-try bill after it passed legislation.³³ Although, this is the less popular route, Governor Brown's justification for doing so is commendable. Instead of caving into the pressure of public scrutiny, he recognized that the FDA was the appropriate authority to grant access to experiment treatment. The governor has stated, "patients with life threatening conditions should be able to try experimental drugs, [but]... before authorizing an alternative state pathway... we should give this federal expedited process a chance to work."³⁴ Governor Brown is referring to the proposed changes to the FDA's expand access provision that would shorten the application process for experimental drug access for individual patients.³⁵

FDA Expanded Access Provision: Revamping "Compassionate Use" for Faster Access

The goal of the FDA's expand access provision, commonly known as "compassionate use," is to make drugs available to patients with serious diseases or conditions when there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the patient's disease or condition.³⁶ Therefore, the compassionate use provision is designed to accomplish the same goal as the one stated under right-to-try law, patient access. In fact, the FDA has granted over 99

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percent of requests for expanded access since 2009.³⁷ Further, as previously stated the FDA has the power to prevent states from using right-to-laws through its federal pre-emption authority.³⁸

The requirements stated under the compassionate use provision are not overly burdensome and the proper route to obtain access to experiment treatment, in order to preserve the safety of patients. Under expanded access, patients must either have an “immediately life-threatening disease or condition or a serious disease” to qualify for compassionate use.³⁹ Determining whether a patient falls under the serious or immediately life-threatening disease or condition definition is based upon the information provided by the treating physician to the FDA, and is the first step in the FDA approval process.⁴⁰ A patient with a life expectancy of no more than a few months, or who is likely to experience premature death is deemed to have a life threatening disease under the statute.⁴¹ A treating physician uses his or her clinical judgment to assess and determine the severity of a patient’s condition, and possible treatment options.⁴² Factors such as impact on day-to-day life, chances of survival, or probability of the disease or condition worsening if left untreated are all considered when determining whether a patient qualifies for compassionate use.⁴³

Next the FDA must determine whether the potential benefit of use of drug/treatment is warranted in light of the potential risk of harm to the patient under the given circumstances.⁴⁴ Finally, compassionate use of the drug cannot interfere with ongoing or the completion of clinical investigations to support marketing approval for expanded access use.⁴⁵

The requirements of compassionate use are similar to the requirements stated under many state right-to-try laws. In response to public criticism of the lengthy approval process, the FDA has proposed a streamlined application process for individual patients. Form 3926 is expected to reduce the application completion time from 8 hours to 45 minutes.⁴⁶ Draft Form 3926 is no

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more than two pages long and is filled out by the treating physician (also referred to as a sponsor-investigator) on behalf of the patient.⁴⁷ Once a complete 3926 Form has been submitted, the FDA will assign an individual patient investigational new drug (“IND”) number that either allows use of the investigational treatment or places the application on clinical hold.⁴⁸ A decision from the FDA is projected to take no longer than 30 days once a complete 3926 is received.⁴⁹ In cases of emergency, a treating physician may submit a request for access to investigational drug via telephone and, if approved, will also receive approval via telephone, allowing for an even quicker response time.⁵⁰ These changes offer patients faster access to experimental treatment, but still provide patient safety.

FDA regulations governing clinical trials for investigational new drug approval follow a rigorous set of requirements, which include three phases of clinical trials and legal informed consent from participants.⁵¹ The arduous process can be attributed to the legal response to past abuses suffered by research participants in early, unethical medical experiments such as the Tuskegee Syphilis Study and the Guatemala Experiment.⁵² The FDA’s goal is to ensure participant safety to prevent future abuses, while allowing the continuous follow of medical advancement.⁵³ Investigators and researchers are responsible for disclosing risk to participants as part of the informed consent process.⁵⁴ The compassionate use provision, in accord with the requirements under the IND clinical trial requirements, mandates that the physician authorizing use of the experiment drug “determine that the probable risk to the patient using the drug is not more than the probable risk from the disease.”⁵⁵ Furthermore, the FDA requires sufficient evidence of the “safety and effectiveness” of the investigational drug in order to be used under expanded access.⁵⁶

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The FDA compassionate use provision also contains safeguards that establish accountability for the sponsor (the individual or entity submitting application), investigator (treating physician), and sponsor-investigation (physician who submits application and treats patient).⁵⁷ Manufacturers are permitted to collect the cost of manufacturing the drug if they grant patient access to the investigational drug, however, they are still subject to FDA oversight, which ensures a level of accountability not found under state right to try laws.⁵⁸ Given, the FDA's resources and expertise it is clear they are better equipped to make the final determination for patient experimental drug access.

Although state level involvement has bolstered terminally ill patients' claims to speedier access to potentially lifesaving treatment, the FDA was and still is the best-suited regulatory authority to oversee the patient approval process to prevent harm to these patients in the future. While the right to try law movement has forced the FDA to revamp its current expand access provision, right to try laws are ultimately ineffective.

¹ Right to Try 2015, *In Your State Right to Try*, RIGHT TO TRY BLOG (Mar. 16, 2016, 9:30 PM), <http://righttotry.org/in-your-state/>.

² Julie Turkewitz, *Patients Seek 'Right to Try' New Drugs*, N.Y. TIMES (Jan. 10, 2015), http://www.nytimes.com/2015/01/11/us/patients-seek-right-to-try-new-drugs.html?_r=0.

³ Ed Silverman, *'Right to Try' laws wrong to skirt FDA*, BOSTON GLOBE (Oct. 12, 2015), <https://www.bostonglobe.com/business/2015/10/12/right-try-laws-are-wrong-usurp-fda/54tlCnBEQ4DHMIkYd1ZIdJ/story.html>.

⁴ Kimberly Leonard, *Seeking the Right to Try*, U.S. NEWS (Nov. 18, 2014), <http://www.usnews.com/news/articles/2014/11/18/right-to-try-laws-allowing-patients-to-try-experimental-drugs-bypass-fda>.

⁵ Darcy Olsen, *Dying Patients Should have 'Right to Try' New Treatments*, TIME (Nov. 10, 2015), <http://time.com/4091290/right-to-try/>.

⁶ *Id.*

⁷ *Id.* (Becker's is a chronic form of muscular dystrophy, but not fatal like Duchenne. Becker's unlike Duchenne allows those with the disease better mobility in voluntary muscles groups.) See MUSCULAR DYSTROPHY ASSOCIATION, <https://www.mda.org/disease/becker-muscular-dystrophy>.

⁸ *Id.*

⁹ *Id.*

¹⁰ Christine Corieri, *Everyone Deserves the Right to Try: Empowering the Terminally Ill to Take Control of their Treatment*, GOLDWATER INST. (2014), <http://goldwaterinstitute.org/en/work/topics/healthcare/right-to-try/everyone-deserves-right-try-empowering-terminally-/>. (An approved drug Ms. Karnes was taking called Interleukin-2 no longer stabilized her condition.)

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¹¹ *Id.* (The reason for the denial was that Ms. Karnes cancer had spread to her brain which made her ineligible for these clinical trials.)

¹² *Id.*

¹³ *Id.*

¹⁴ Michael Barone, *The success of Right to Try laws signals a major policy shift*, NEW YORK POST (Oct. 6, 2015), <http://nypost.com/2015/10/06/the-success-of-right-to-try-laws-signals-a-major-policy-shift/>.

¹⁵ *Id.*

¹⁶ *Id.*

¹⁷ *Id.*

¹⁸ David Siders, *Jerry Brown vetoes 'right to try' bill for experimental drugs*, THE SACRAMENTIO BEE (Oct. 11, 2015), <http://www.sacbee.com/news/politics-government/capitol-alert/article38756022.html>.

¹⁹ Minn. Stat. Ann. § 151.375 (West, Westlaw through Act chapter 84 of the 2016 Regular Session).

²⁰ *Id.* at Subd. 3.

²¹ *Id.*

²² Minn. Stat. Ann. § 151.375 at Subd. 4 (West, Westlaw through Act chapter 84 of the 2016 Regular Session).

²³ *Id.*

²⁴ Minn. Stat. Ann. § 151.375 at Subd. 5.

²⁵ Right to Try Act, 2014 Bill Text NJ A.B. 3474.

²⁶ *Id.* at 2(c)-(d).

²⁷ Right to Try Act, 2014 Bill Text NJ A.B. 3474.

²⁸ *Id.*

²⁹ Minn. Stat. Ann. § 151.375 (West, Westlaw through Act chapter 84 of the 2016 Regular Session).

³⁰ Darcy Olsen, *Can supporting 'Right to Try' drug laws save patients' lives?*, FOX NEWS (Jan. 13, 2016), <http://www.foxnews.com/opinion/2016/01/13/can-supporting-right-to-try-drug-laws-save-patients-lives.html>.

³¹ Minn. Stat. Ann. § 151.375 at Subd. 6 and 8 (West, Westlaw through Act chapter 84 of the 2016 Regular Session).

³² David Garski, *As in 2014, "right-to-try" laws continue to metastasize in 2015*, Science Based Medicine (May 18, 2015), <https://www.sciencebasedmedicine.org/the-con-game-that-is-right-to-try-legislation-continues-to-metastasize/>.

³³ David Siders, *Jerry Brown vetoes 'right to try' bill for experimental drugs*, THE SACRAMENTIO BEE (Oct. 11, 2015), <http://www.sacbee.com/news/politics-government/capitol-alert/article38756022.html>.

³⁴ *Id.*

³⁵ See *Individual Patient Expanded Access Applications: Form FDA 3926*, FDA (February 2015), available at <http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM432717.pdf>.

³⁶ See 21 CFR 312.300(a), available at

<https://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfcfr/CFRSearch.cfm?CFRPart=312&showFR=1&subpartNode=21:5.0.1.1.3.9>.

³⁷ *Id.*

³⁸ Carl Coleman, *J&J Leads the Way in Developing a Rational Process for Responding to Compassionate Use Requests*, HEALTH REFORM WATCH BLOG (Mar. 15, 2015), <http://www.healthreformwatch.com/2015/05/15/jj-leads-the-way-in-developing-a-rational-process-for-responding-to-compassionate-use-requests/>.

³⁹ 21 CFR 312.300(b), (“*Immediately life-threatening disease or condition* means a stage of disease in which there is reasonable likelihood that death will occur within a matter of months or in which premature death is likely without early treatment. *Serious disease or condition* means a disease or condition associated with morbidity that has substantial impact on day-to-day functioning.”).

⁴⁰ 21 § CFR 312.305(a)(1).

⁴¹ *Id.*

⁴² *Id.*

⁴³ *Id.*

⁴⁴ 21 § CFR 312.305(a)(2).

⁴⁵ 21 § CFR 312.305(a)(3).

⁴⁶ Carl Coleman, *J&J Leads the Way in Developing a Rational Process for Responding to Compassionate Use Requests*, HEALTH REFORM WATCH BLOG (Mar. 15, 2015), <http://www.healthreformwatch.com/2015/05/15/jj-leads-the-way-in-developing-a-rational-process-for-responding-to-compassionate-use-requests/>.

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⁴⁷ *Individual Patient Expanded Access Applications: Form FDA 3926*, FDA (February 2015), available at <http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM432717.pdf>. (Note: Treating Physician applying for expanded access to investigational drug needs to ask for permission from manufacturer for access to drug. If manufacturer allows access to drug, the manufacture needs to provide treating physician with a letter of authorization.)

⁴⁸ *Id.*

⁴⁹ *Id.*

⁵⁰ *Id.* (Physician is to submit a written submission for expand access use 15 days after emergency request.)

⁵¹ *IND Applications for Clinical Investigations: Clinical Protocols*, FDA, (last updated Oct. 9, 2015), available at <http://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/ucm362445.htm>.

⁵² See U.S. Dep't of Health & Human Services, *Fact Sheet on the 1946-48 U.S. Public Health Service Sexually Transmitted Diseases (STD) Inoculation Study*, HHS.GOV (last visited November 20, 2015), <http://www.hhs.gov/1946inoculationstudy/factsheet.html>.; See U.S. Public Health Syphilis Study at Tuskegee: *The Tuskegee Timeline*, CTR. DISEASE CONTROL AND PREVENTION (last updated September 24, 2013), <http://www.cdc.gov/tuskegee/timeline.htm> (“The study involved a total of 600 African-American men, 399 who were infected with syphilis and 201 without. The Tuskegee Institute agreed to support the study, so long as they can also receive credit”)

⁵³ Currently, human research subject protection for medical research is concurrently enforced by the Department of Health & Human Services (HHS) and the Federal Drug Administration (FDA). See FDA Dep't of Health and Human Services---Subchapter A-Protection of Human Subjects, 21 C.F.R. § 50 (2015).

⁵⁴ See 21 CFR § 50.27.

⁵⁵ 21 § CFR 312.310(a).

⁵⁶ *Expanded Access (Compassionate Use)*, FDA, <http://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/default.htm>.

⁵⁷ 21 § CFR 312.310(c).

⁵⁸ *Guidance for Industry: " Charging for Investigational Drugs Under an IND*, FDA, (May 2013), available at <http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm351264.pdf>.