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New Developments in Right to Try Legislation

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Almost exactly a decade after the first Right to Try laws (RTTs) were passed in state legislatures and over six years after the enactment of the federal Right to Try law,^[1] a new iteration of RTT is gaining momentum in state chambers. This legislation, known as the “Right to Try For Individualized Treatments”^[2] (RTT 2.0), is grounded in the same principles as the original law but focuses more narrowly on individualized therapies. While 41 states enacted RTT 1.0 prior to the passage of the federal Right To Try Act,^[3] it has rarely been utilized as an alternative pathway to access investigational products.^[4] Nevertheless, RTT 2.0 laws have been passed in six states and appear to be advancing in a number of additional states.^[5] This article discusses the federal RTT law in comparison to the U.S. Food and Drug Administration (FDA) expanded access pathway, compares RTT with RTT 2.0, and explores the challenges of each.

Right to Try vs. FDA Expanded Access Program

The federal RTT law creates a legal pathway by which a patient with a life-threatening disease or condition or their physician may request access to an experimental drug product that has not yet been granted marketing authorization by FDA. The law requires patients and physicians to have exhausted all other approved treatment options, to be ineligible to enroll in a clinical trial for the product that they seek, and to provide written informed consent.^[6] These requirements of the RTT pathway are similar to those of FDA's Expanded Access Program (EAP) for the use of unapproved drug products.^[7] The key differences between RTT and EAP lie in the regulatory oversight requirements for each. FDA oversees EAP approvals and has provided extensive guidance on when EAP is appropriate, how to request it, and how to safeguard patient safety.^[8] EAP also requires oversight by an Institutional Review Board (IRB), a multidisciplinary committee that provides ethical oversight of research involving human participants. By contrast, RTT is separate from and rests outside FDA's purview; further, while RTT, like EAP, does require written informed consent, the request does not require IRB review or approval, a written protocol, or process.

FDA review of expanded access requests helps to ensure that there is at least preliminary clinical evidence of both the safety and efficacy of the investigational product, as well as a review of the intended dose proposed for administration. Applicants for EAP submit an Investigational New Drug application (IND) that FDA reviews for its proposed use prior to approval. By contrast, the federal RTT law requires only the completion of a Phase I trial and no regulatory oversight. RTT also expressly prohibits FDA from using RTT-derived clinical data in the evaluation of future marketing applications for the investigational product, although manufacturers and sponsors who have provided investigational products through the RTT program are expected to submit annual reports to FDA. The purpose of these reports is solely for FDA to publish a consolidated yearly summary of RTT use.^[9] A summary of differences between EAP and RTT is provided in **Table 1** below.

Current data on the prevalence of RTT are scant, but the data that are available suggest that oncologists are considerably more likely to request access to an unapproved drug via EAP than they are via RTT.^[10] Given that the major difference between EAP and RTT is FDA oversight, a threshold question is whether FDA acts to deter or delay the approval of EAP requests. Evidence suggests that as many as 99% of all EAP requests are approved by FDA^[11] often within the first few days.^[12]

Despite its social media presence and visibility, demand for access via RTT has yet to materialize.^[13] Some experts have explicitly advised physicians to “steer patients away from [RTT] and toward [EAP]”^[14] while others claim they would never encourage a patient to avail themselves of RTT.^[15] The American Society of Clinical Oncology (ASCO) released a public statement condemning RTT, stating “ASCO supports access to investigational drugs outside of clinical trials when adequate patient protections are in

place . . . We don't support right to try legislation, however, because these laws ignore key patient protections without actually improving patient access to investigational drugs outside of clinical trials.”[\[16\]](#)

Reports have shown some confusion among requesting patients[\[17\]](#) and physicians[\[18\]](#) regarding RTT terminology, specifically whether RTT actually creates a legal “right to try” the drugs eligible under the program. To be clear, it does not.[\[19\]](#) Section 3 of the federal RTT law explicitly dispels this misconception by stating that RTT “does not establish a new entitlement or modify an existing entitlement, or otherwise establish a positive right to any party or individual.”[\[20\]](#) Additionally, federal courts have previously held “there is no fundamental right . . . of access to experimental drugs for the terminally ill.”[\[21\]](#) Assuming all legal requirements have been met, the final decision about access rests with the manufacturer of the eligible investigational drug, and the law insulates the manufacturer (and the prescriber, dispenser, and others) from liability for any decision not to provide access to the drug. Thus, RTT may be better characterized as a “right to ask.”

What is new in RTT 2.0?

Relative to the original RTT, the new RTT 2.0 iteration makes several key changes.[\[22\]](#) RTT 2.0 substantially alters the scope of the original RTT legislation to expand the eligible patient population, the diseases included, and when in the course of the disease the right to try becomes available.

Patient Eligibility

RTT 2.0 broadens the definition of “eligible patient” beyond those with a “life-threatening disease or condition” to include those with a “severely debilitating illness” (as defined by 21 C.F.R. § 312.81(b)), creating a substantially larger cohort of potential patients.[\[23\]](#) Further, an eligible patient must no longer have “exhausted approved treatment options,”[\[24\]](#) rather, under RTT 2.0, the eligible patient must have merely “considered” all other approved treatment options.[\[25\]](#)

Covered Health Needs

In addition to the changes in eligibility criteria and consideration of alternate therapies, the scope for the types of products covered by RTT 2.0 includes only individualized genetic treatments with specific, gene-based targets. RTT 2.0 requires that a physician “in good standing” who “will not be compensated by the manufacturer” of the investigational drug certify that the patient has exhausted treatment options as described above and is ineligible to participate in a clinical trial for the drug sought.[\[26\]](#) Patients who have “received a recommendation from his or her physician for an individualized investigational treatment, based on analysis of the patient’s genomic sequence, human chromosomes, deoxyribonucleic acid, ribonucleic acid, genes, gene

products (such as enzymes and other types of proteins), or metabolites”^[27] are eligible, where “individualized investigational treatment” is defined as “drugs, biological products, or devices that [are] unique to and produced exclusively for use for an individual patient, based on their own genetic profile. An ‘individualized investigational treatment’ includes, but is not limited to, individualized gene therapy antisense oligonucleotides (ASO) and individualized neoantigen vaccines.”^[28] RTT 2.0 is focused exclusively on bespoke treatments of genetic diseases.

Retained Similarities

Some key similarities remain between RTT and RTT 2.0. Notably, neither law requires the manufacturer of an investigational treatment to provide that treatment even if all eligibility criteria are satisfied. Both paradigms shield the manufacturer and physician from liability in case of adverse events, and neither paradigm requires payers to cover the costs associated with treatments or prevents manufacturers from charging for them. Like its predecessor, RTT 2.0 also does not require review or approval by FDA and/or an IRB.

According to the Goldwater Institute, a public policy think tank that advocates for Right to Try laws, six states have enacted RTT 2.0 statutes since 2022: Arizona in 2022, Nevada in 2023, and Louisiana, Maryland, Mississippi, and North Carolina in 2024.^[29] At present, several other states have bills progressing through their legislatures to enact new forms of RTT, amend or expand existing RTT, or enact new RTT 2.0. A summary of the key similarities and changes between RTT and RTT 2.0 is included in **Table 2** below.

Ethical Considerations

As the avenues toward preapproval access via RTT continue to expand, the ethical questions surrounding RTT grow more urgent. First, as courts have previously decided, the federal government has a long history of and a vested interest in “regulating the *safety* of drugs.”^[30] RTT legislation deliberately abrogates FDA’s role in that process, effectively leaving informed consent as the only mechanism in the RTT paradigm intended to inform patients about and protect them from undue safety risks. While informed consent satisfies the ethical imperative of patient autonomy, questions around the process, accessibility, and understandability of the information provided through the informed consent process remain. In any context, the informed consent process depends heavily on the quality of the information provided, and, by the nature of preapproval treatment, the information about the intervention proposed through RTT is incomplete. Similarly, the RTT paradigm is predicated on the supposition that individuals exist as actors in a free market and should, therefore, be permitted to make free decisions about the risks that they wish to assume. However, terminally ill patients often have few choices, and they approach RTT with hope and expectation, in the context of little available information about the risks. From a public health and individual

perspective, objective FDA oversight thus would serve as a guardrail between desperate patients and unproven treatments, but that oversight is not available in RTT.

Conclusion

With the abrogation of regulatory and ethical oversight, RTT is disfavored among physicians and manufacturers in favor of EAP.^[31] Nevertheless, both RTT and RTT 2.0 legislation continue to emerge at the state level. The dissonance between the apparent enthusiasm of the public to embrace these laws and the hesitancy of care providers and manufacturers to support or endorse these approaches requires consideration. On the one hand, desperate patients coping with life-limiting and debilitating illnesses may feel that it is within their own authority to decide whether or not to subject themselves to experimental and unproven therapies, whatever the risk. There may be a sense that the regulatory agencies that were created to protect the public are now too conservative, limiting access to potentially life-saving therapies unnecessarily and without due cause, but this perception is not reflected in the available data on the approval process for EAP. Perhaps there are elements of therapeutic misconception but without an appreciation of the adverse events that can result from the unproven intervention. It is interesting that the public appears willing to endorse Right to Try while clinical trial recruitment, enrollment, and retention remain so difficult to achieve. The fact that the regulatory pathway to approval for a drug or treatment is so long in duration and fraught with complexity is not helpful in this regard. While recognition of patient frustration and desperation, coupled with clear communication and empathy about turnaround times and success rates for EAP applications may help, they likely will not assuage the perceived need to which Right to Try legislation continues to respond.

Table 1: Eligibility requirements for the FDA Expanded Access Program vs. Federal Right to Try Legislation

	Expanded Access	Right to Try
Categories	• Individual patients, including emergency use • Intermediate-size patient populations • Treatment IND or Treatment protocol for widespread use	• Limited to individuals
Review	• FDA and IRB approval required for all, except advance IRB review not required for emergency use	• Not required
Informed consent	• Required for all	• Required for all
Factors	• Serious or life-threatening disease or condition • Potential benefits outweigh potential risks • Expanded access will not interfere with clinical trials or approval submissions	• Serious or life-threatening disease or condition • Exhaust all approved treatment options • Unable to participate in a clinical trial for the investigational drug
Requirements	• Formal submission required	• Completion of at least Phase 1 trial • Subject to NDA, BLA, or IND • Under active development by the manufacturer

Table 2: Key distinctions between Federal Right to Try and new state-level Right to Try 2.0 statutes

Requirement	Right to Try	Right to Try 2.0
Patient eligibility criteria	· Patient has a serious or life-threatening disease or condition · Patient has exhausted approved treatment options · Patient is unable to participate in a clinical trial involving the investigational drug in question · Patient has given informed consent	· Patient has a serious or life-threatening disease or condition OR has a severely debilitating illness · Patient has considered approved treatment options · Patient has received a physician recommendation for an individualized investigational treatment · Patient has given informed consent
Investigational medical product eligibility criteria	· Investigational medical product is a drug · Investigational drug has completed at least a Phase I clinical trial · Investigational drug is not yet approved for use in the US, but the manufacturer is actively developing the drug and/or seeking approval	· Investigational medical product may be a drug, biologic, or device · Individualized investigational treatment must be produced exclusively for use by an individual patient · Covered treatments include: - o Individualized gene therapy - o Antisense oligonucleotides - o Individualized neoantigen vaccines

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