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### RIGHT TO TRY LAWS

*An Opportunity for Physician, Public, and Private Sector  
Collaboration*

Presented to Representative Frank Edelblut (R-Dist. 38),  
Sponsor of HB 1138

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## **EXECUTIVE SUMMARY**

The state of New Hampshire recently introduced House Bill 1138 (NH HB 1138) which establishes the Terminal Patient's Right to Try Act, a piece of legislation which "allows a patient with a terminal illness access to investigational drugs, biological products, and devices." This bill seeks to allow terminally ill patients to try experimental treatments that have completed phase I testing and remain in clinical trials but have not yet been approved by the Food and Drug Administration (FDA). Right to Try (RTT) laws have spread across the country over the past two years. Colorado became the first state to pass an RTT law in May of 2014 and twenty-seven other states followed suit with their own RTT legislation. Advocates of such policies believe that RTT laws represent a way to bypass the slow FDA approval process, heighten awareness of investigational treatments, reduce deaths from terminal illnesses, and improve patient access to such treatments. Others oppose these laws because drug provision is not guaranteed and drug companies are reluctant to provide drugs outside the supervision of the FDA. Moreover, the laws can create false hope for patients due to the potentially prohibitive costs of treatment and the risk of a more painful, hastened death.

Currently, there is no way to know if RTT laws are helping or harming patients and pharmaceutical companies. Given that RTT laws continue to proliferate around the country, a potential policy option to address this issue consists of amending RTT laws to require some form of mandatory reporting. This reform would allow for the creation of a database tracking outcomes for RTT patients. Such a database would inform patients, physicians, pharmaceutical companies, and policymakers on the efficacy and potential consequences of RTT policies and guide future policy revisions.

### **1. INTRODUCTION**

The purpose of an RTT law is to increase access for terminally ill patients to potentially lifesaving treatments such as investigational new drugs (INDs), biological products, or medical devices that have passed phase I clinical trials but are not yet approved by the Food and Drug Administration (FDA). More than one million Americans die each year from terminal diseases and fewer than three percent of the terminally ill gain access to investigational drugs through clinical trials.<sup>1</sup> The FDA approval process for a drug can take up to 15 years, which is too long to wait for most dying patients.<sup>2</sup> While the FDA has an expanded access program, also known as 'compassionate use,' only approximately 1,200 patients are granted access each year.<sup>3</sup> The fact that such a small fraction of patients with terminal illnesses are able to utilize the expanded access program is often attributed to the lengthy application process which has a time burden of 100 hours on average per submission.<sup>4</sup> The aim of RTT legislation is to reduce this burden, return the control of treatment options back to patients and their doctors, and grant terminally ill patients a last chance at saving their lives.<sup>5</sup>

RTT laws are being promoted by the Goldwater Institute, a libertarian non-profit advocacy group named for late Arizona Senator Barry Goldwater, in an effort to bypass



part of the FDA's drug approval process for the benefit of patients.<sup>6</sup> In phase I clinical trials a drug is tested in small groups of patients for safety of consumption, toxicity levels, and side effects.<sup>7</sup> Because the drugs that would have increased access under the RTT laws have passed phase I trials, supporters of the policy say that it does not undermine the FDA.<sup>8</sup> Organizations that advocate for RTT, such as the Goldwater Institute, Fight to Live, and the Abigail Alliance for Better Access to Developmental Drugs, campaign for their cause by highlighting stories of patients who died or endured prolonged suffering because they could not get access to INDs. Presumably, these organizations believe these patients would have had access to life-saving drugs if RTT laws had been in place.

## **2. NEW HAMPSHIRE HB 1138**

With the rise of RTT laws across the nation, New Hampshire legislators followed suit by introducing similar legislation on February 10, 2016. Paralleling the success of RTT bills in other states, NH HB 1138 has already been passed by the New Hampshire House of Representatives and the Senate. If this bill is signed into law, the RTT policy will go into effect immediately.<sup>9</sup>

NH HB 1138 delineates the provisions of the Terminal Patient's Right to Try Act. First, the act describes which patients with terminal illnesses will be deemed eligible to request access to investigational drugs, biological products, and devices under the RTT policy. The bill then defines "investigational drug, biological product, or device," "physician," and "terminal illness" for the purposes of the policy.<sup>10</sup> Following this section, the bill lays out the terms for provision of the investigational drug, biological product, or device by the manufacturer. According to the policy, a manufacturer has the right to provide the investigational drug, biological product, or device without requiring compensation but also has the right to require the eligible patient to pay the costs associated with the manufacturing process. Additionally, the manufacturer can also require the eligible patient to "participate in data collection relating to the use of the investigational drug, biological product, or device."<sup>11</sup>

NH HB 1138 does not require state agencies or health care insurers to provide coverage for any investigational drug, biological product, or device. The bill also does not require the manufacturer to include an eligible patient in a particular clinical study or trial. Lastly, the bill protects both physicians and facilities from liability if they recommend or participate in the treatment of a patient with an investigational drug, biological product, or device.<sup>12</sup>

## **3. BENEFITS OF RIGHT TO TRY LAWS**

***Bypass the Slow, Expensive FDA Approval Process*** - The FDA approval process for investigational drugs, biological products, and devices in the United States takes an average of 10 years and drugs companies spend an average of 1.4 billion dollars to get new treatments approved.<sup>13</sup> Terminally ill patients do not have the luxury to wait for the



conclusion of this lengthy and costly process when their timelines are measured in days, weeks, and months, not decades.<sup>14</sup> RTT laws will allow eligible patients to bypass FDA approval and begin treatment sooner.

***Heighten Awareness for Investigational Treatments*** - The establishment of RTT laws across the country has created public and political pressure on pharmaceutical companies and the federal government to reform guidelines with respect to terminally ill patients seeking access to investigational treatments.<sup>15</sup> This awareness has extended to the federal government where the Right to Try Act of 2015 was introduced in the House of Representatives in July, 2015 and the Trickett Wendler Right to Try Act of 2016 was introduced in the Senate in May, 2016.<sup>16</sup>

***Reduce the High Death Rate from Terminal Illnesses*** - With over a million Americans dying from a terminal illness each year, access to INDs, biological products, and devices is often the last hope for dying patients to save their own lives or contribute to the study of a new drug to save the lives of others.<sup>17</sup> Terminally ill patients have the fundamental right to pursue the preservation of their own lives and, from the perspective of these patients, these investigational treatments are potentially lifesaving.<sup>18</sup>

***Improve Patient Access to Investigational Treatments*** - RTT laws were designed to improve access for the more than 97 percent of terminally ill patients who fail to gain access to INDs, biological products, and devices through clinical trials and compassionate use.<sup>19</sup> The use of these investigational treatments is a decision that should be made by a terminally ill patient in consultation with his or her physician. RTT laws returns control of these medical decisions to level of patients and doctors instead of leaving them in the hands of the government.<sup>20</sup>

#### **4. HAZARDS OF RIGHT TO TRY LAWS**

***Provision of Drugs Not Required by Law*** - RTT laws do not require pharmaceutical companies to provide the treatments being sought and pharmaceutical companies are reluctant to provide treatments outside the supervision of the FDA because of the potential for bad press, exposure to liability, and limited supplies of the treatment in question.<sup>21</sup>

***RTT Laws May Be Unconstitutional*** - RTT laws may violate the U.S. Constitution. The FDA has “exclusive jurisdiction over the regulation of drugs via Congress’s constitutional authority to regulate interstate commerce.”<sup>22</sup> Due to the Supremacy Clause, state laws that infringe on that exclusivity are pre-empted.<sup>23</sup> If challenged, RTT laws across the nation may be struck down.

***Widening Health Disparities*** - Under NH HB 1138, insurance companies are not required to cover the costs of an investigational treatment.<sup>24</sup> Laws in other states give insurance companies the right to deny coverage to patients for the time period they are taking an investigational treatment.<sup>25</sup> Due to these provisions, terminally ill patients



would likely need to be able to pay for expensive treatments. This limits the benefits of the law to those who are well-connected or well-resourced, thus widening health disparities between the poor and the rich.<sup>26</sup>

***False Hope for the Terminally Ill*** - After phase I trials, there is no safety or efficacy data on how the investigational drugs would affect the very sick patients seeking treatment. RTT laws may give patients false hope of good outcomes when none exist.

***Negative Impact on Pharmaceutical Research*** - RTT policies have limited contributions to the advancement of research since treatment provision takes place outside of strictly controlled, physician-monitored studies. These laws also may make it more difficult to recruit patients for clinical trials because many will want to bypass trials and the risk of receiving placebos in favor of direct access through RTT policies.<sup>27</sup>

***Low Chance of Improving Access beyond Current FDA Programs*** - Almost all expanded access requests submitted to the FDA are granted. In fiscal year 2015, the FDA rejected only six applicants out of a total of 1,262 applications.<sup>28</sup> Furthermore, under current law, the FDA is required to respond to any submissions within 30 days, though they can respond more rapidly due to the time-sensitive nature of these requests.<sup>29</sup> Overall, FDA approval of submissions does not seem to be a major obstacle for patients. More significant hurdles likely include the lengthy submission process and obtaining approval from pharmaceutical companies. To address the first issue, the FDA's individual patient expanded access process is currently in the process of being streamlined. Once the new application is finalized, physicians will be able to submit the form in only 45 minutes and will still retain the ability to petition for rapid approval of emergency access by phone if necessary.<sup>30</sup> However, RTT laws do not address the second issue and therefore may have little scope for improving access beyond any FDA programs that are already in place or currently in development.

## 5. POLICY OPTION

RTT laws have received widespread bipartisan support but the efficacy of the laws has yet to be tested. Extensive research by the authors of this report has revealed that the impact of RTT laws remains unclear. For instance, on one side, New Hampshire Public Radio reported that there have been no substantiated cases in which RTT laws helped someone gain access to investigational treatments as of April, 2016.<sup>31</sup> On the other side, as of May, 2016, a “spokeswoman for the Goldwater Institute claims that 40 patients have received medicines, but she could not provide the names of the physicians or companies involved.”<sup>32</sup> If such cases exist, some form of reporting would reveal the true impacts of RTT laws.

Unlike the FDA's expanded access program, there are no provisions in RTT laws requiring physicians or pharmaceutical companies to report data on patients treated under the policy.<sup>33</sup> Without some form of data collection, it is impossible to know the true outcomes resulting from RTT laws. Whether these laws are helping or harming





individuals is important information that would contribute to the debate on the value of RTT laws in improving health outcomes for the terminally ill. The only way to obtain this information is some form of data-tracking for patients receiving investigational treatments under the laws. To address this issue, a potential policy option consists of reforming RTT laws to require some form of mandatory reporting. This data collection would allow for the creation of a database to house detailed information on treatments received under RTT laws.

Because treatments given under RTT laws occur in the absence of FDA-controlled conditions and are negotiated only between physicians, patients, and pharmaceutical companies, any information gathered must be given by one of these three parties. Accordingly, states could amend their RTT laws to make reporting mandatory for some combination of these three parties. This reporting mechanism would allow data from RTT patients to contribute to ongoing trials and investigations of the drug in question which would benefit both pharmaceutical companies and other patients. This would also allow for some measure of oversight and data-tracking without requiring full FDA approval.

This policy reform would result in the creation of a database containing the following information: treatment received, rationale for use, dosage, length of treatment, name of pharmaceutical company, treatment cost, patient history, side effects experienced, and location of treatment. This RTT data could be analyzed in conjunction with analogous data from the FDA's expanded access program in order to develop a comprehensive comparison of the two policies. Data analysis may also yield further information on which patients benefit the most from these policies and which drugs have the most successful outcomes. The results of this process would inform patients, physicians, drug companies, and policymakers on the efficacy and potential consequences of RTT policies and guide future policy revisions.

In the past, in response to public and patient demand, the FDA created accelerated approval processes to hasten the availability of new treatments that fulfill an unmet need or demonstrate a substantial improvement over current treatments.<sup>34</sup> Based on the data obtained from the proposed database, similar measures could be taken with regards to the expanded access program. For example, if further study finds that drugs which have been approved overseas, but are still in clinical trials in the U.S., tend to have the best outcomes, these drugs could be given first priority for access. On the other hand, if the study finds that patients suffering from a particular disease are much less likely to benefit from investigational treatments, publicizing the results would allow those patients to avoid unnecessary pain and expense.

One concern for pharmaceutical companies may be that reporting on drug provision under RTT laws may allow negative patient outcomes to disrupt ongoing trials. However, this concern also applies to the current expanded access program. Furthermore, an internal review of 10,000 expanded access IND requests found only two trials that were halted due to adverse events experienced by patients receiving treatment under expanded



access. In addition, at least one drug was approved based solely on use in patients under expanded access, indicating that pharmaceutical companies may benefit from reporting data on drug provision under RTT laws.<sup>35</sup>

Overall, this policy option is designed to allow for a comparison of the outcomes of RTT laws and the expanded access program so that terminally ill patients can better navigate both policies and make the best decisions regarding their health

## **6. CONCLUSION**

RTT laws have proliferated across the country over the past two years. Proponents of such policies believe that RTT laws represent a way to bypass the slow FDA approval process, heighten awareness for terminally ill patients to seek investigational treatments, reduce deaths from terminal illnesses, and improve patient access to INDs, biologics, and medical devices. Critics oppose these laws because drug provision is not guaranteed and the laws may be unconstitutional. In addition, opponents believe that the policies create health disparities, provide false hope for terminally ill patients, hinder pharmaceutical research, and have little scope for impact. However, both supporters and critics can agree that it is frustrating for patients, families, and physicians to know that there are treatments being developed that might help patients, but there is no way to access to them. It is the goal of this paper to illustrate that the first step to improving health outcomes for terminally ill patients is to reform RTT laws to allow for the systematic study and analysis of both RTT and expanded access policies.





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