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Legal Sidebar

Federal "Right-to-Try" Legislation: Legal Considerations

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Congress has been engaged in a vigorous debate over how patients with life-threatening conditions may access investigational therapies that have not been approved under the Federal Food, Drug, and Cosmetic Act (FD&C Act). At the heart of this debate is the balance between permitting sufficient access to experimental medications for patients who lack other treatment options and obtaining adequate evidence of safety and effectiveness for these products. This debate is nothing new, and state and federal lawmakers are currently addressing this issue through so-called "right-to-try" laws. The general intent behind right-to-try laws is to create a pathway for such patients to obtain speedy access to experimental treatments, without clinical trial participation or the Food and Drug Administration's (FDA's) permission. Thirty-eight states have passed right-to-try laws, and Congress is now considering a similar mechanism at the federal level.

The merits of right-to-try laws are contested. Supporters claim that these laws are a beneficial means of empowering terminally ill, desperate patients who are unable to participate in clinical trials and are willing to take a risk on an unproven medication. On the other hand, critics assert that the products are more likely to be harmful than helpful to patients and that these laws potentially threaten the integrity of clinical trials and are unnecessary in light of FDA's existing expanded access program. This Legal Sidebar post provides an overview of the FD&C Act provisions regarding access to experimental drugs for treatment use, as well as state right-to-try laws. The post also addresses recent legislation, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Acts (H.R. 5247 and S. 204) that, if enacted, could alter the legal landscape with respect to a patient's ability to access unapproved drugs and other medical products.

FFDCA and Patient Access to Investigational Drugs

The FD&C Act establishes a comprehensive federal system of premarket approval for drugs in the United States. The Act generally prohibits introducing or delivering new drugs in interstate commerce unless the drug is approved by FDA. Under current law, in order to market a new brand-name drug, a manufacturer must file a new drug application (NDA) with FDA, which must include, among other things, "full reports of investigations which have been made to show whether or not such drug is safe for use and whether such drug is effective in use." FDA may approve an NDA only if the sponsor of the application (e.g., a

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drug manufacturer or marketer) demonstrates, among other things, that the drug is safe and effective for the conditions prescribed, recommended, or suggested in the product's labeling. Proof of safety and effectiveness must consist, at least in part, of adequate and well-controlled clinical investigations, including studies involving humans, which are conducted by qualified experts.

In general, in order to exempt drug sponsors from the prohibition on the dissemination of unapproved new drugs and permit them to perform the clinical testing necessary to secure FDA approval of an NDA, the sponsor must submit an investigational new drug (IND) application that contains detailed information demonstrating, among other things, adequate safeguards for the safety and rights of human subjects. Once an IND application has been approved, clinical trials may begin, and these trials may last several years. After this testing is completed, the sponsor may present results of the trials as part of its NDA as evidence of the product's safety and effectiveness. FDA reviews these results as well as other information, and once the NDA is approved, the drug may be commercially marketed for its intended use.

Over the past few decades, in order to address unmet medical needs of patients with life-threatening or seriously debilitating diseases, Congress and FDA have permitted "expanded access" to investigational drugs outside of clinical trials. Under FDA's current expanded access program, individual patients may request and potentially access unapproved new drugs for therapeutic reasons, rather than for clinical investigations, if certain specified criteria are met. Among these criteria, FDA must determine that there is sufficient evidence of safety and effectiveness to support the use of the drug, and that provision of the drug will not interfere with the initiation, conduct, or completion of clinical investigations to support NDA approval. Following complaints that obtaining products through the expanded access program was complicated and burdensome, FDA recently took action to streamline the process. Under this new process, FDA estimates that it takes approximately 45 minutes to complete the requisite application form (compared to 100 hours for the previous form). According to the FDA Commissioner's recent congressional testimony, emergency access requests for individual patients are typically approved immediately over the phone, and non-emergency requests are usually processed "within a few days." In recent years, the agency has allowed 99 percent of these requests to proceed. Additionally, other entities besides FDA play pivotal roles in the expanded access program. Manufacturers, for example, must decide whether or not to allow a patient to obtain the experimental treatment.

In contrast to the FD&C Act's new drug approval requirements, and regardless of FDA's expanded access program, all states have either considered or enacted right-to-try laws, which generally provide that manufacturers and other entities may make certain investigational drugs and other products available to eligible patients. In general, under these laws (see a proposed model here), patients must have a terminal illness, have considered other FDA-approved therapies, have a prescription or recommendation from their physician to take the drug, and must give written informed consent. Right-to-try laws generally prohibit state medical licensing boards from taking disciplinary action against physicians prescribing or recommending investigational drugs, and some of the laws also aim to restrict the liability of manufacturers that provide the drugs to patients. In addition, it appears most, if not all, right-to-try laws expressly indicate that manufacturers are not required to supply experimental drugs to patients, and insurers are not required to provide coverage for the costs of the drugs.

Some question whether state right-to-try laws meaningfully change the legal framework governing patient access to experimental medications. These state laws do not alter federal requirements that restrict access to unapproved drugs. Additionally, because of the Supremacy Clause of the Constitution and the primacy of federal statutes, it seems unlikely that this type of state law could be construed so as to override federal drug regulation. It is possible that state right-to-try laws may be viewed as state provisions that exempt access to experimental medical products from any applicable state penalties under state law. However, it does not appear that these state laws could function to exempt manufacturers and others from compliance with the FD&C Act and other federal laws, nor could they immunize these persons from any legal

consequences of violating such federal requirements. To date, there are no reported court cases examining the scope of any state right-to-try laws or their potential interplay with federal law.

Federal Right-to-Try Legislation

Members of Congress have introduced legislation that, similar to state right-to-try laws, aims to allow broader access to investigational drugs and biologics for patients with life-threatening illnesses. Unlike state right-to-try laws, federal proposals passed by the House (H.R. 5247) and the Senate (S. 204) would amend the FD&C Act and potentially limit FDA's role in the oversight of these unapproved medicines. While the House and Senate bills are not identical, both would exempt an "eligible drug" provided to an "eligible patient" from compliance with the requirements of the FD&C Act related to new drug and biologic approval, certain labeling violations, and the IND process, assuming certain conditions are met. An eligible drug, among other things, must have completed the first phase of clinical trials and must be actively making its way through the development process.

While the House and Senate bills differ as to who may be considered an eligible patient, both proposals generally would allow individuals to obtain eligible drugs if the individual has provided informed consent, and a physician certifies that the individual has exhausted other treatment options and is unable to participate in a clinical trial. While the federal legislation does not expressly remove FDA's existing authority to operate its expanded access program, the legislation would appear to permit an alternative means of access, under which FDA's assessment of preliminary safety and efficacy evidence, as well as the potential for interference with clinical trials, would not be needed to provide or obtain an investigational therapy.

Another notable feature is that similar to state right-to-try laws and the current expanded access program, the federal right-to-try legislation would not compel manufacturers or other entities to provide eligible drugs to patients. This is a frequently recognized, key obstacle to investigational therapy access—for a variety of reasons, manufacturers may be unwilling to provide products to patients who are not participating in clinical trials. For example, drug companies and their representatives have voiced concerns that any adverse event associated with early access may jeopardize the subsequent approval of these products. In order to address manufacturers' apprehension, the federal legislation restricts the Secretary of Health and Human Services from using clinical outcome data associated with the use of an eligible drug to "delay or adversely affect the review or approval of such drug" unless, among other things, the Secretary determines that it is "critical" to ascertaining the safety of the investigational drug. The legislation does not define the term critical, and presumably the Secretary would have some degree of discretion in making this determination.

Additionally, in an effort to eliminate concerns over potential liability for injuries to patients, the bills would curb the ability of patients to sue manufacturers, prescribers, dispensers and others in cases where patients are hurt by the experimental medication. While the Senate bill would limit liability for acts related to the provision of eligible drugs under the right-to-try legislation, the House bill would restrict liability against entities that provide drugs under the legislation, as well as through the current expanded access program. Accordingly, House bill's liability protections may potentially incentivize access to eligible drugs both with and without the FDA's permission. However, despite these measures, there may be other reasons why manufacturers may be reluctant or unable to provide drugs to patients outside of clinical trials. For example, FDA's Commissioner has stated that "the biggest obstacle ... is the availability of supply for patients who want to get access to unproven therapies." Should federal right-to-try legislation be enacted, it remains to be seen how manufacturers will respond.

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