Expanding access to unapproved treatments through right-to-try

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State-level right-to-try laws have given patients and their families hope that they might gain access to breakthrough investigational treatments. But traditionally, the FDA and pharmaceutical industry have been cautious about providing access to these treatments, at least without robust clinical study data providing preliminary evidence that the unapproved drug is effective.

As states have continued passing new right-to-try laws, it has helped make the case for expanding access at the federal level. Rob Church, partner in Hogan Lovells' Pharmaceutical and Biotechnology Practice Group, discusses the opportunities – and challenges – these regulatory changes might bring.

Q: When looking at right–to–try, how have things changed over the past few years?

"More than 30 right-to-try laws have been passed at the state level, and at the US congressional level there is interest in a federal law. Even so, it should be noted that FDA already has in place 'Expanded Access' regulations that make it possible for patients and doctors to obtain access to unapproved drugs. FDA has made a number of changes to its Expanded Access program over the last several years to help streamline the process and respond to requests from patients and doctors more quickly."

"There's a lot of interest within the patient and legislative communities in continuing to make it easier for patients to gain access to early-stage treatments. Progress toward a federal right-to-try law that increases access to investigational drugs is a key area to watch in the coming year."

Q: What are some of the challenges that might impact growth of right-to-try?

"One challenge is that both the FDA and the pharmaceutical industry remain cautious about providing access to these early-stage medicines. And although many of the state level right-to-try laws are very well intended, they haven't yet had much of an impact. If a federal right-to-try law is enacted, this will pose a number of significant challenges for FDA, including how to assess the data generated from right to try programs and whether that data can be used to support new drug approvals."

Q: How might the 21st Century Cures Act help with expanded access?

“There’s language in the 21st Century Cures Act (legislation designed in part to speed approval of new drugs and devices) intended to enhance how the FDA currently reviews new drug applications. The Cures Act directs the FDA to take greater steps in accepting real-world evidence and patient reported data, which could potentially be generated in right-to-try or expanded access programs.”

Q: What will this expanded access to investigational therapies mean for your clients?

“I think everybody wants to accelerate the rate of development of novel products so that they are more accessible to patients. But at the same time, the FDA has very legitimate interests around ensuring that development and testing of those drugs on a trial basis is handled in a scientifically defensible and ethically permissible way.”

“Companies working in this space will need to stay attuned to how the FDA might change, expand, or ease its regulations for investigational medicines. There is enormous potential, but there are still a lot of questions to answer as we move into the future.”

For additional insights from Rob Church on right-to-try laws, watch the video above.

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