On May 30, 2018, President Trump signed S204, the “Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017,” into law. This law allows patients in the United States with life-threatening diseases or conditions to use investigational drugs outside of clinical trials without their physicians having to secure approval from the Food and Drug Administration (FDA).

Prior to the enactment of the new law, patients, through their doctors, could request investigational medical products from companies via the FDA’s Expanded Access pathway. Under Expanded Access, after a company agrees to provide an investigational product, the FDA and an institutional review board (IRB) must approve the request before the product is given to a patient.

The Expanded Access (EA) and Right to Try (RTT) pathways now co-exist. Neither pathway requires companies to grant requests for investigational drugs. Companies may choose to grant access via EA, RTT, both, or neither.

We know that the new federal RTT law:

• does not require IRB approval of requests, although healthcare institutions or companies can require IRB approval;
• requires patients to give informed consent, but does not delineate an informed consent process or stipulate what elements the consent document must include; nor does the law require independent review/oversight of the consent process;
• requires eligible drugs to have completed Phase 1 testing, be under ongoing investigation, and not be subject to a clinical hold, although “ongoing investigation” is not precisely defined;
• has limited manufacturer reporting requirements (numbers of patients treated and doses, purposes of drugs requested, and adverse events);
• shields companies/sponsors from all liability, and physicians and other non-sponsor individuals/entities from liability except in cases of gross negligence, reckless or willful misconduct, or an intentional tort under applicable state law.

The FDA’s EA program:

• requires IRB approval of requests, except in emergencies; the approval process was streamlined in October 2017 to allow approval by the IRB chair or a designee instead of by a full board;
• requires informed consent in compliance with federal regulations;
• allows access to investigational medical products at any stage of development, based on a favorable benefit/risk ratio for the specific patient;
• does not provide liability protection.
What the RTT law will mean for patients and patient groups, healthcare providers and institutions, and drug companies and other sponsors is unclear. We know that some companies have seen an uptick in requests for their investigational drugs after the bill’s signing. Additionally, CUPA members have been fielding calls asking for information and guidance; however, there is little information we can provide at this point. The law’s provisions need to be turned into rules in order for everyone to understand what the federal RTT law does and does not allow. Soon after the bill’s signing, Janet Woodcock, director of the FDA’s Center for Drug Evaluation and Research, recommended that questions be directed to the drug companies from which drugs are being requested.

We have reached out to companies and health law experts, but we have not yet been able to answer several key questions, including:

- How many companies or healthcare institutions will permit use of the RTT pathway?
- S.204 amends the Federal Federal Food, Drug, and Cosmetic Act. Who will oversee the implementation of the law (write rules/guidance) and compliance with it?
- Although the statute references a federal regulation that stipulates that sponsors can charge only direct costs for investigational products, what will this restriction mean in practice: How will companies interpret it? How will it be enforced, and by whom?
- Who will pay for drugs obtained via RTT and the costs of administering them? What about related medical, travel, childcare, and other expenses?
- Are companies’ RTT policies subject to the 21st Century Cures Act’s transparency provisions that require companies with investigational products in Phase 2 or later to make their expanded access policies publicly available?
- In the absence of federally mandated FDA and IRB oversight, will healthcare institutions or companies require in-house or other third-party oversight of non-trial use of investigational drugs?
- Now that there is a federal RTT law, what is the status of the 40 state RTT laws and their various provisions, particularly those that could be used to deny insurance coverage for hospice care, home healthcare assistance, and/or health insurance altogether?
- Who has jurisdiction to enforce the law’s limited reporting requirements?

We are closely watching how the new bill affects these and other issues. We will keep you informed in our monthly newsletters and via the CUPA website, which will be updated to reflect details of the new law and its ramifications.

Meanwhile, CUPA will continue to study all facets of pre-approval access, including the roles of real world evidence in both easing access to and generating data about the use of investigational medical products outside of clinical trials; the ethical obligations of companies to provide post-trial access to investigational agents for those who participated in their trials; the role of IRBs vis-à-vis pre-approval access; ethical issues in crowdsourcing funding to pay for treatments; pre-approval access for gene therapies, and more.