FACILITATING PATIENT ACCESS TO PROMISING INVESTIGATIONAL DRUGS (PROJECT FACILITATE)

The U.S. Food and Drug Administration (FDA) has helped provide access to investigational drugs for patients with few or no treatment options through its Expanded Access (EA) program since the 1980s. Between 2011 and 2016, the FDA received about 9,000 EA, or compassionate use, requests and approved 99 percent of them.

Launched in June 2019, Project Facilitate is a pilot project through which the FDA is seeking to streamline and increase access to investigational anticancer therapeutics. Through Project Facilitate, the FDA established a single point of contact, a call center, through which physicians can initiate and/or get help completing single-patient investigational new drug (SPI) requests. SPI requests are the way in which physicians secure the FDA's approval to treat individual patients with unapproved investigational therapeutics. Trained FDA staff will guide callers through the request process, assisting with the necessary paperwork and identifying contacts at pharmaceutical companies and institutional review boards (IRBs).

Because Project Facilitate staff will be copied on all requests to the companies, the FDA will, for the first time, be able to collect data on demand for and outcomes from the use of investigational (unapproved) anticancer therapeutics. Currently, the FDA only becomes aware of expanded access requests if they are accepted by the companies who developed the treatment. Companies will still be able to decide whether to provide requested therapeutics, but for requests initiated through Project Facilitate, they will be required to provide the rationale for denying access. Project Facilitate staff will follow up with physicians whose patients received investigational therapeutics to learn about how the patients did with the treatment.

Project Facilitate and Expanded Access operate in the same space as, but separate from, federal Right-to-Try legislation that was signed into law in 2018. Like Project Facilitate and EA, Right-to-Try is a mechanism by which terminally ill patients can request access to investigational therapeutics. However, unlike the FDA programs, the federal law circumvents involvement by the FDA and IRBs.