ASCO Releases Position Statement on Access to Investigational Drugs

Supports Strengthening FDA Expanded Access Program, Opposes Right-to-Try Measures as Ineffective and Potentially Harmful to Patients

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Alexandria, VA — The American Society of Clinical Oncology (ASCO) strongly supports increasing access to investigational new treatment options for patients with cancer, while raising serious concerns about recently proposed federal "right-to-try" (RTT) legislation as well as enacted state RTT laws. In a position statement released today, ASCO said these measures lack adequate patient protections and do not remove any of the major barriers patients face in accessing investigational drugs outside of clinical trials.

"ASCO supports access to investigational drugs outside of clinical trials when adequate patient protections are in place," said ASCO Chief Medical Officer Richard L. Schilsky, MD, FACP, FASCO. "We don't support right-to-try legislation, however, because these laws ignore key patient protections without actually improving patient access to investigational drugs outside of clinical trials."

ASCO's position statement, "American Society of Clinical Oncology Position Statement on Access to Investigational Drugs," asserts that most RTT laws, while well intentioned, are "not an effective mechanism for improving access to investigational drugs for terminally ill patients and may cause unintended harms," for the following reasons:

- Independent review of the potential safety and efficacy of investigational drugs is important for patient safety. Such review is by-passed in RTT laws. Under the current expanded access program provided by the Food and Drug Administration (FDA), the FDA conducts a prompt review of the available data and makes an independent assessment of the risks and potential benefits of the proposed treatment on behalf of the patient.
- Right-to-try laws do not include an enforcement mechanism to provide access, since they do not require or compel drug manufacturers to provide investigational products. As such, these laws do not remove a frequent barrier to access.
- RTT laws place no legal obligations on insurers to pay for the routine care costs associated
with delivery of treatment, particularly in the case of complications caused by these drugs — unlike coverage requirements that exist for patients who participate in clinical trials. Thus, RTT laws establish no new rights or protections for patients.

In addition to their lack of patient protections, RTT laws do not mitigate delays that can occur in the process of seeking access to an investigational drug. For example, under both RTT and the expanded access process, applicants must determine a manufacturer's willingness to provide the investigational drug, and providers and patients consistently report difficulties in locating contact information for such requests, significant delays in responses from manufacturer's, and denied requests.

The FDA offers access to investigational drugs outside of clinical trials — known as "expanded access"— for individual patients with terminal disease, on a case-by-case basis. FDA recently streamlined its expanded access application process, and last year it approved approximately 99.5% of expanded access requests, with a median approval time of four days for non-emergency cases. Nevertheless, 33 states have passed RTT laws, which allow patients to request access to investigational treatments from drug manufacturers without the requirement of FDA review. Indeed, recently proposed legislation in Congress would prohibit the FDA from restricting access to unapproved products when a patient has a terminal illness and lives in a state with a RTT law.

**Improving Access to Investigational Drugs: Next Steps**

ASCO supports further improvements to existing mechanisms for expanded access, a number of which are included in the *21st Century Cures Act*. These provisions will help simplify access to investigational drugs by requiring drug manufacturers to make information about their expanded access policies and processes readily available to patients and providers.

"New policy initiatives should focus on increasing transparency among pharmaceutical manufacturers' expanded access policies," said Dr. Schilsky, "while ensuring that existing expanded access programs are timely and efficient for patients and their physicians."

To address delays in the expanded access process, ASCO supports development of a navigation service to assist patients in submitting requests to manufacturers for investigational drugs. Ideally, an online portal or tool could provide patients and physicians seeking drug access with a universal point-of-entry to facilitate the step-by-step process for obtaining access to investigational agents from drug manufacturers.

ASCO urges all stakeholders to work together to support expanded access programs that include FDA oversight and to promote the optimal functioning of these programs.
The full statement is available now.

About ASCO:

Founded in 1964, the American Society of Clinical Oncology, Inc. (ASCO®) is committed to making a world of difference in cancer care. As the world’s leading organization of its kind, ASCO represents more than 40,000 oncology professionals who care for people living with cancer. Through research, education, and promotion of the highest-quality patient care, ASCO works to conquer cancer and create a world where cancer is prevented or cured, and every survivor is healthy. ASCO is supported by its affiliate organization, the Conquer Cancer Foundation. Learn more at www.ASCO.org, explore patient education resources at www.Cancer.Net, and follow us on Facebook, Twitter, LinkedIn, and YouTube. Visit ascoaction.asco.org for the latest cancer policy developments. For an overview of current policy issues, read ASCO’s cancer policy issue briefs.