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**Via Electronic Submission**

September 22, 2020

Stephen M. Hahn, MD  
Commissioner  
U.S. Food and Drug Administration  
5630 Fishers Lane Room 1061  
Rockville, MD 20852

Subject: Proposed Rule – Annual Summary Reporting Requirements Under  
the Right to Try Act (Docket No. FDA-2019-N-5553)

Dear Commissioner Hahn:

The Association for Clinical Oncology (ASCO) appreciates the opportunity to provide comments on the U.S. Food and Drug Administration (FDA) proposal to implement the statutory requirement under the Right to Try Act that sponsors and manufacturers provide an annual summary of any investigational drugs supplied for use to an eligible patient.

ASCO is a national organization representing nearly 45,000 physicians and other professionals specializing in cancer treatment, diagnosis, and prevention. We are also dedicated to conducting research that leads to improved patient outcomes, and we are committed to ensuring that evidence-based practices for the prevention, diagnosis, and treatment of cancer are available to all Americans.

The Association thanks the Agency for facilitating the implementation of this provision of the Right to Try Act through its proposed rule, including the establishment of an annual deadline and the required components of industry's annual summaries. As noted in the proposed rule, the Right to Try Law established a new pathway for patients diagnosed with life-threatening diseases or conditions to directly petition manufacturers for access to unapproved, investigational drugs and for manufacturers to choose to provide access. FDA is proposing that the manufacturers include in the annual summary the following: the number of doses supplied of an eligible investigational drug; the number of patients treated; the use for which the drug was made available for each disease or condition; and any known serious adverse events. Because the law excludes the FDA from the decision-process regarding drug access, the annual summary will provide

important information for the Agency to track the use of investigational therapies provided under this mechanism.

We offer the following comments regarding the annual summary content areas the FDA is proposing for inclusion in the manufacturers' reports.

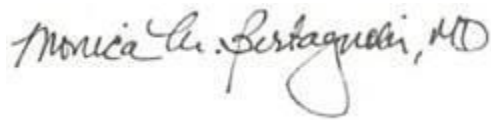
- **Number of Doses Supplied** – The rule proposes that manufacturers or sponsors submit the aggregate number of doses supplied across all patients. However, we believe that such information is much less useful than the number of doses per patient, therefore we suggest that the agency require the reporting of this more detailed information. While we support the Agency in its efforts to reduce reporting requirement burdens, we believe that the number of doses per patient will be tracked by the manufacturers' investigational drug use or compassionate use programs, so it should not pose an undue burden for this more granular level of data to be included in the annual summaries.
- **Number of Patients Treated** – The Right to Try Act mandated that the annual summaries include information on the number of patients treated. However, we encourage the FDA to also require information on patient key demographics, such as age, disease, and comorbidities. Demographic information is necessary to understand the use of therapies outside the usual clinical trial population. Collection and analysis of this information could potentially identify patterns related to eligibility criteria and exclusion from clinical trials. The Right to Try Law requires as a condition of eligibility that the patient exhaust approved treatment options and is unable to participate in a clinical trial involving the eligible investigational drug. Information on why patients are ineligible for trials will help trial sponsors to improve clinical trial access and representativeness of clinical trial participants.
- **Any Known Serious Adverse Events and Outcomes** - FDA proposes that the manufacturer submit a summary of any known serious adverse events, including outcomes of such events, experienced by patients treated with an eligible investigational drug under the Right to Try Act. We commend the agency for proposing to require outcomes as a reporting requirement because reporting of an adverse event alone would be less useful to the Agency, clinicians, and other stakeholders. We also support the Agency's proposal for manufacturers to use common medical terminology included in the Medical Dictionary for Regulatory Activities (medDRA). ASCO continues its efforts to create a set of standard data elements through our collaborative Minimal Common Oncology Data Elements (mCODE) project to eliminate the use of various terms to describe the same type of cancer and the collection of data in varying formats.

Additionally, ASCO supports the FDA's efforts to ensure patient privacy in the annual reports. ASCO agrees that any patient-identifying information should not be included in annual reports but suggests that the manufacturers use methods such as assigning patient ID numbers to ensure the manufacturers can track individual patients and their outcomes with regard to both safety and efficacy.

The transparency these reports will provide about the use of investigational drugs is critical because the FDA would not otherwise be aware of the use of investigational drugs under this new pathway and any known serious adverse events. ASCO agrees that with these reports there may be increased awareness of investigational drugs; the diseases or conditions for which patients are seeking access; and patient safety concerns that may result from such use.

We thank you again for this issuing this Proposed Rule to implement reporting requirements and for the opportunity to comment. We look forward to the dissemination of this important information on the use of investigational therapies upon finalization of these requirements. Please contact Shimere Sherwood ([Shimere.Sherwood@asco.org](mailto:Shimere.Sherwood@asco.org)) with any questions or for further information.

Sincerely,

A handwritten signature in black ink that reads "Monica M. Bertagnoli, MD". The signature is written in a cursive style with a large, stylized 'M' and 'B'.

Monica M. Bertagnoli, MD, FACS, FASCO  
Chair of the Board, ASCO Association for Clinical Oncology