September 22nd, 2020

Dockets Management Staff (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

Re: Docket No. FDA-2019-N-5553: Annual Summary Reporting Requirements Under the Right to Try Act, Proposed Rule

Dear Sir/Madam:

The Biotechnology Innovation Organization (BIO) thanks the Food and Drug Administration (FDA or Agency) for the opportunity to submit comments regarding FDA's Proposed Rule *Annual Summary Reporting Requirements Under the Right to Try Act* (Proposed Rule).

BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. BIO's members develop medical products and technologies to treat patients afflicted with serious diseases, to delay the onset of these diseases, or to prevent them in the first place.

BIO believes that the Proposed Rule provides important information for Sponsors to follow regarding the reporting requirements under the Right to Try Act.

We appreciate FDA's consideration of implementing the concept of "least burdensome" in its approach. An alternate approach to the separate reporting process as articulated in the Proposed Rule is to align the Right to Try Act reporting with annual reporting for Expanded Access. Creating two separate annual reporting pathways increases the burden on Sponsors and manufacturers responding to these requests. We suggest that in the aligned annual reporting, there can be two sections within the annual report, one for Right to Try and one for Expanded Access for FDA to be able to differentiate between the two. In addition, we suggest that serious adverse event (SAE) reporting should also be aligned with the standard SAE reporting that Sponsors and manufacturers already perform, again, to create a least burdensome approach to these reporting requirements.

Further, we believe that the example given in the Proposed Rule of a tabular summary goes beyond the level of information required by the legislation which cites "the number of doses supplied, the number of patients treated, the uses for which the drug was made available, and any known serious adverse events". We encourage the FDA to work with Sponsors or manufacturers to tailor the needed information in a manageable and least burdensome way.

Within the Proposed Rule when discussing this least burdensome approach for reporting, FDA differentiates between "sponsors who provide drugs under the Right to Try Act and sponsors who do not provide drugs under the Right to Try Act (*for whom there will be no obligation to review any changes with respect to the process for annual summaries*)" (emphasis added). Taken with the rest of the Proposed Rule, BIO reads this as stating there are no reporting requirements if a Sponsor or manufacturer chooses not to provide a drug under the Right To Try Act (e.g., a Sponsor or manufacturer would not have to report on

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the number of requests they receive if they do not provide the drug under this pathway). BIO strongly supports this approach and requests that FDA revise the Proposed Rule to explicitly state that there are no reporting requirements for Sponsors or manufacturers who choose not to grant such a request and do not provide products under the Act. It would be beneficial to also include this clear statement in the Executive Summary of the Proposed Rule.

While we understand that this Proposed Rule is specific to the reporting requirements for a Sponsor or manufacturer, BIO believes it will be equally important to understand additional detail on FDA's intent to "post online an annual summary report in accordance with section 561B(d)(2) of the FD&C Act". BIO is particularly interested in how FDA will report and assess "the number of drugs for which clinical outcomes associated with the use of an eligible investigational drug" was used in accordance with the statute or not used in the review of an application[1]. BIO is also interested in how FDA's posting of their annual summary report "may increase awareness about the availability of investigational drugs" as noted in the Proposed Rule. It will be important that the information posted in the FDA annual summary report does not convey or imply any conclusions regarding the safety or efficacy of the products provided under the Right to Try Act. It may also be helpful for the website with the FDA annual summary report to link to additional information regarding Expanded Access.

Section E of the Proposed Rule states "[w]e expect the designated point of contact would be an email contact or electronic portal." For purposes of data management, BIO strongly supports FDA utilize an electronic portal, rather than emailing a contact. This also fits more broadly into recent and ongoing FDA conversations regarding modernization of data strategy and could provide additional benefit to both the Agency and Sponsors.

Finally, the Agency proposes that "the first annual summary submitted by a manufacturer or sponsor under this section must cover the period from enactment of section 561B of the FD&C Act, May 30, 2018, through the date the final rule becomes effective." Rather than a retrospective application of reporting requirements, BIO suggests that the first annual summary cover the period beginning from the finalization of the Proposed Rule onward (prospective rather than retrospective reporting).

BIO appreciates this opportunity to submit comments regarding FDA's Proposed Rule *Annual Summary Reporting Requirements Under the Right to Try Act*. We would be pleased to provide further input or clarification of our comments, as needed.

Sincerely,

/S/
Victoria A. Dohnal, RAC
Director, Science and Regulatory Affairs
Biotechnology Innovation Organization

[1] Section 561B(d)(2) of the FD&C Act: The Secretary shall post an annual summary report of the use of this section on the internet website of the Food and Drug Administration, including the number of drugs for which clinical outcomes associated with the use of an eligible investigational drug pursuant to this section was— (A) used in accordance with subsection (c)(1)(A); (B) used in accordance with subsection (c)(1)(B); and (C) not used in the review of an application under section 505 of this Act or section 351 of the Public Health Service Act.