June 8, 2018

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

Re: Docket No. FDA–2018-N-0663: Tissue Agnostic Therapies in Oncology: Regulatory Considerations for Orphan Drug Designation.

Dear Sir/Madam:

The Biotechnology Innovation Organization (BIO) thanks the Food and Drug Administration (FDA or Agency) for the opportunity to submit comments regarding Tissue Agnostic Therapies in Oncology: Regulatory Considerations for Orphan Drug Designation.

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 members are involved in the research and development of innovative healthcare, agricultural, industrial, and environmental biotechnology products.

General Comments:
While advances in genomics and precision medicine have significantly improved the ability to develop medicines aimed at specific molecular targets that may be present in multiple diseases and/or cancers, there is much to be learned through continued research and development. BIO appreciates the FDA taking a forward-leaning approach to have early discussions regarding how these advances may impact other aspects of drug development and review, such as orphan drug designations. As the Agency moves forward with these discussions, BIO has included several items below for the Agency's consideration.

BIO requests, in order to ensure that all stakeholders participating in the tissue agnostic discussion understand what the FDA considers a "tissue agnostic" therapy or approach, the Agency define the terms "tissue agnostic". It is BIO's interpretation that "tissue agnostic" refers to the ability to develop therapies based upon biomarkers or other molecular targets to treat a disease, irrespective of the anatomical location in which the diseases is identified.

BIO also strongly believes that incentives provided by the Orphan Drug Act have been effective in encouraging investments and research and development for therapies to treat rare diseases or conditions that affect fewer than 200,000 individuals each year in the United States, and since the Orphan Drug Act was passed in 1983 there has been tremendous growth in the development of products for treating rare diseases. However, of the over 7,000 identified rare diseases, only approximately 350 have approved treatments. Additionally, it is estimated that approximately 30 new rare diseases are identified each year.[1] BIO strongly believes that continued support of the current state of the Orphan Drug Act and associated incentives is essential for ensuring that therapies continue to be developed for patients suffering from rare diseases.

[1] Quintiles IMS: Orphan Drugs in the United States: Providing Context for Use and Cost, 2017.

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In order to continue to incentivize the development of therapies for rare diseases, BIO recommends that Sponsors have the opportunity to determine, based on current scientific understanding, whether to apply for a designation based upon a tissue agnostic approach, or an anatomically-defined approach. Additionally, in order to support consistency and transparency and to encourage innovation we ask that once the FDA grants an orphan designation for a tissue agnostic therapy, based upon the criteria that the disease being treated impacts fewer than 200,000 individuals in the United States per year, if, at a later time, additional scientific evidence is collected, indicating that the particular target plays an important role in other diseases that impact more than 200,000 individuals, the FDA should not retract the previously granted orphan designation. BIO also asks that because tissue agnostic approaches create new ways in which healthcare professionals and patients can treat and target cancer, the FDA's policy in this area should promote the availability of tissue agnostic approaches as orphan diseases, should they qualify as outlined by the current statute. As such a biomarker defined, tissue agnostic disease should be considered a different disease or condition from an anatomically-defined disease as it relates to orphan drug designation and associated exclusivity. BIO believes that these approaches to tissue agnostic drug development will encourage continued innovation for novel therapies to treat rare diseases.

Building upon discussions of tissue agnostic approaches in the context of oncology, BIO also encourages the FDA to consider how similar approaches may be used, and how it may affect product development and Sponsor investment for therapeutic areas outside oncology and for other patient populations, including pediatrics. At the public workshop, FDA acknowledged that tissue agnostic development for rare diseases has already advanced into other therapeutic areas. Commissioner Gottlieb and Debra Lewis, Acting Director of the Office of Orphan Products Development, mentioned a tissue agnostic development approach being sought for an ophthalmology product which has received orphan drug designation. While we believe that it is important for the FDA to consider under what other circumstances a tissue agnostic approach might be appropriate, further conversations regarding the application of tissue agnostic approaches outside oncology are needed. We request the Agency consider developing guidance for tissue agnostic therapies beyond oncology, as knowledge in these diseases is further developed and understood.

Finally, in order to implement appropriate policy for tissue agnostic therapies that supports innovation, we ask the FDA to continue to partner with various stakeholders to discuss the impacts of tissue agnostic therapies on drug development and review. We also encourage the Agency to consider precedential effects in other disease states and impacts on incentives for innovation in the rare disease space more broadly when crafting this policy.

BIO appreciates this opportunity to submit comments regarding FDA's docket on Tissue Agnostic Therapies in Oncology: Regulatory Considerations for Orphan Drug Designation. We would be pleased to provide further input or clarification of our comments, as needed.

Sincerely,

/S/
Danielle Friend, Ph.D.
Director, Science and Regulatory Affairs
Biotechnology Innovation Organization