September 14th, 2022

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

Re: Docket No. FDA-2022-D-745 Voluntary Consensus Standards Recognition Program for Regenerative Medicine Therapies; Draft Guidance for Industry

Dear Sir/Madam:

The Biotechnology Innovation Organization (BIO) thanks the Food and Drug Administration (FDA or Agency) for the opportunity to submit comments regarding the *Voluntary Consensus Standards Recognition Program for Regenerative Medicine Therapies; Draft Guidance for Industry* (Draft Guidance or Guidance).

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 is supportive of this guidance and the use of voluntary consensus standards (VCS) for regenerative medicine therapies, as they are a necessity to ensure product quality and patient safety. BIO believes that VCS will accelerate the development of regenerative medicine, which encompasses a set of complex rapidly evolving technologies, by making it easier for developers to comply with FDA regulations and by enabling developers to better compare different strategies for manufacturing cell and gene therapy (CGT) products. Importantly, because this program is voluntary, developer participation is a choice and not a requirement.

BIO views VCS as essential for developing the types of platform approaches sought by the regenerative medicine community that would streamline the development of CGT products and create opportunities for sustainable commercialization of these therapies, including therapies for ultra-rare diseases where development would otherwise be infeasible. Further, a VCS program will enable members of the regenerative medicine community to provide technical and scientific recommendations to FDA. We note that CDRH's long-running VCS program is widely considered a success and demonstrates the feasibility and advantages of this approach.

While VCS are important, BIO recommends that FDA consider the following:

- Continue to promote the need for product quality and patient safety while encouraging innovation (i.e., we do not want standards to stifle research and development or impede development of new modalities)
- Ensure standards evolve with emerging science, manufacturing, and clinical experience (in the RMT field)
- Ensure modality-specific considerations (e.g., AAV vectors versus CAR-T cells) when developing VCS that apply across all RMTs
- Ensure standards are developed after sufficient evidence has been generated
- Ensure standards are in line with benefit/risk assessment e.g., if product will be an N of 1 or is developed under a perpetual IND, FDA should carefully evaluate which standard should be applied or may not be needed

BIO also offers the following specific comments:

- We note that the following language in this draft guidance -

  > *"In addition, the appropriate use of VCS in a regulatory submission may reduce the amount of documentation that a submitter needs to provide to CBER."*

  is similar to language in the CDRH/CBER final guidance titled "Appropriate Use of Voluntary Consensus Standards in Premarket Submissions for Medical Devices."[1] The language in this final guidance is reproduced here -

  > *"When used appropriately, consensus standards will typically reduce the amount of documentation that a submitter needs to provide and may reduce FDA review time."*

  We request conforming edits to this draft guidance to reflect the language in the "Appropriate Use of Voluntary Consensus Standards in Premarket Submissions for Medical Devices" final guidance.

- In 'Appendix 2: Acronyms,' of this draft guidance, 'DOC: Declaration of Conformity' is listed as an acronym, but the use of this term is not mentioned elsewhere in the draft guidance. Since "DOC" is used in S-CAP for medical devices, please clarify whether CBER intends on adopting the use of declaration of conformance to consensus standards that FDA has recognized or decided to recognize as part of the SRP-RMT.

[1] https://www.fda.gov/regulatory-information/search-fda-guidance-documents/appropriate-use-voluntary-consensus-standards-premarket-submissions-medical-devices

BIO appreciates this opportunity to submit comments regarding the Draft Guidance *Voluntary Consensus Standards Recognition Program for Regenerative Medicine Therapies*. We would be pleased to provide further input or clarification of our comments, as needed and we look forward to future opportunities to collaborate with the Agency on this important topic.

Sincerely,

/s/
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