

Regulatory: Judy Arcidiacono, Regulatory Scientist, U.S. Food and Drug Administration

Hello, my name is Judy Arcidiacono, and I am a regulatory scientist at the U.S. FDA Center for Biologics Evaluation and Research Office of Tissues and Advanced Therapies. I am the lead for the standards program for regenerative medicine therapies. The purpose of our program is to support the development and use of standards for these products. I would like to present FDA's views on the use of standards for regenerative medicine therapies.

What are the benefits of standards?

There are many benefits to the use of standards. From the product development perspective, standards can help facilitate consistent and predictable product manufacturing and assessments. They can also assist in product labeling, as well as facilitate the exchange of clinical data. From FDA's perspective, the standards used can help streamline premarket review and facilitate market entry for safe and effective products, especially innovative products like regenerative medicine therapies.

How do standards help developers of regenerative medicine therapies?

The use of standards can help developers overcome challenges for these novel products with respect to product testing, development of performance characteristics, testing methodologies, scientific protocols, and compliance criteria.

Does CBER have guidance on how to use regenerative medicine standards?

In 2019, CBER published guidance for industry titled Standards Development and the Use of Standards in Regulatory Submissions [Reviewed in] the Center for Biologics Evaluation and Research. The purpose of this guidance is to describe CBER's recommendations on the use of standards in product development and control, as well as the use of standards in CBER's managed review process. In 2022, CBER announced that they intend to publish a draft guidance that will describe a standards program for regenerative medicine therapies at FDA Center for Biologics Evaluation, designed to identify voluntary consensus standards to facilitate the development and assessment of regenerative medicine therapy products regulated in CBER.

How do sponsors cite standards in regulatory submissions to CBER?

When a standard is cited in a regulatory submission, the sponsor must identify the standards development organization, the designated number assigned by the standards development organization, and the title of the standard. The sponsor may use the standard as written, and they would cite that they used it as written in the submission, or if they have not used the standard as written, they must provide rationale for not following the standard exactly. So, for example, if you have a standard that is a guide to measure the differentiation potential of regenerative stem cells, the sponsor could say, "We followed that standard as written," or they can say, "We followed that standard as written, except that we extended the culture period to 24 hours to more accurately mimic the actual manufacturing process used to develop this product." So, the sponsor may have to provide additional

information in their submission describing the actual protocol that they used, even though they cited the standard.

What role does FDA play in the standards development process?

FDA staff participate as liaisons in standards development organizations, and they also participate in research projects that lead to the development of standards with our colleagues from the National Institute for Standards and Technology. It’s important for stakeholders to participate in the standard development process to make sure their needs are met.

Audio	Visual
<p>It’s important for stakeholders to participate in the standard development process to make sure their needs are met.</p>	<p>Standards can help facilitate consistent and predictable product manufacturing and testing</p> <p>Standards can streamline premarket review by FDA</p> <p>Collaboration between U.S. government agencies and stakeholders help ensure needed standards are developed</p>

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