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Using Standards in the Development of Regenerative Medicine Products

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Types of Standards

FDA

ISO

ASTM INTERNATIONAL
Standards Worldwide

Documentary standards

Reference materials

Documentary Standards

- Performance characteristics
- Testing methodology
- Manufacturing practices
- Scientific protocols
- Ingredient specifications
- Data standards
- Terminology/Nomenclature

Physical Standards/Reference Materials

- Highly characterized reagents that are distributed to assure consistency, quality and safety.
- *ISO Guide 35 Reference Materials:* Material, sufficiently homogeneous and stable with reference to specified properties, which has been established to be fit for its intended use in measurement

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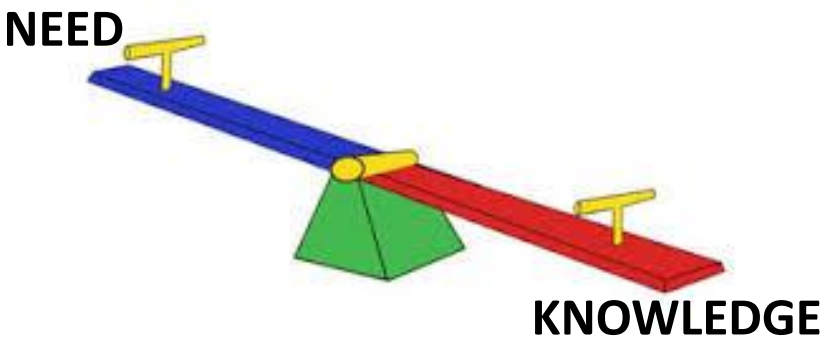
Standards Development Process

Key Principles in Consensus Standards Development

1. Standards respond to a need in the market
2. Standards are based on global expert opinion
3. Standards are developed through a multi-stakeholder process
4. Standards are based on a consensus

When should a standard be developed?

- Does the base of scientific knowledge on the subject support the development of standardized approaches to methods, testing, etc. ?
- Is there consensus among the scientific community that the proposed approaches are appropriate to address the need for standardization?



Other Options When Standardization is not Achievable

- Standard guides
- Terminology
- Practices
- Guidelines
- Publically Available Specifications (PAS)

Considerations When Drafting a Standard

- What is the scope of the standard?
- What is the purpose of the standard?
- Who will likely use the standard?
- What are the possible intended/unintended consequences of the standard?
- How does the proposed standard affect existing work?
- Are there similar efforts to develop a specific standard in other standards venues?

Considerations for Developing Reference Materials

- What is the intended use of the material?
- What is the state of the methodology for which the RM will be used? (standardized/validated?)
- Who will be likely use the standard?
- How will inter-laboratory testing be conducted?
- What are the possible intended/unintended consequences of the reference material?
- How does the proposed reference material affect existing work?
- Are there similar efforts to develop a specific standard in other standards venues?

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FDA Standards Activities for Regenerative Medicine Products

Standards Activities for Regenerative Medicine Products

- Staff act as liaisons to Standards Development Organization (SDO) technical committees
  - FDA researchers, reviewers, and policy makers
- FDA/OCTGT Standards Working Group
- Workshop sponsorship and participation
- Laboratory Programs
  - Inter-lab round-robin testing (e.g., ISO cell counting, ASTM Demineralized Bone Matrix standard)
  - Collaborations with NIST (e.g., flow cytometry, viability)

FDA Standards Workshop

Synergizing Efforts in Standards Development for Cellular Therapies and Regenerative Medicine Products (March 31, 2014) sponsored by FDA/CBER/OCTGT

http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/WorkshopsMeetingsConferences/ucm364114.htm

- Inform stakeholders on:
  1) The role that Federal Agencies play in standards development;
  2) the different types of standards that can be useful in these types of products;
  3) identify organizations that are developing standards for such products.

Workshop Agenda

1. Government Overview
   - FDA/CBER, FDA/CDRH, NIH, NIST, NSF
2. Standards Development Organizations
   - ATCC/ANSI, ASTM, ISO
3. Professional Societies
   - AABB, ISCT/ARM, TERMIS, USP
4. Case Studies on standards development and standards use in product development
5. Panel Discussion

Outcome: Factors Important for Standards Development in the RM Space

- Awareness of existing standards- (e.g., mechanism to identify existing standards, a mechanism to allow widespread commenting on draft standards under development by various Standards Development Organizations (SDOs))
- A mechanism to identify the need for specific standards

Factors (Continued)

- Education on standards development and standards use at scientific conferences, society meetings and universities
- Coordination between groups interested in standards development to prevent duplication of efforts
- Funding support and scientific interest in developing the needed standards

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Standards Use to Support Regulatory Applications Submitted to the U.S. FDA

Types of Standards that Can be used to Support a Regulatory Application

- Voluntary Consensus Standards (ISO, ASTMi, etc.)
- Harmonization Standards
  - WHO:
    - Requirements, Recommendations, and Guidelines
    - Physical Standards for manufacture and control of biological products
  - ICH
    - Standards on Quality, Safety and Efficacy
- Pharmacopeial Standards (USP, Eur. Ph., JP)

OTAT Contact Information

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OCTGT Learn Webinar Series:
http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/ucm232821.htm


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