FDA/SCB Webinar: CBER's Consensus Standards Recognition Program

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February 21, 2024
Standards Coordinating Body

STANDARDS COORDINATING BODY
REGENERATIVE MEDICINE

Webinar Agenda

Time	Discussion Topic
11:05 am – 11:10 am	Welcome
11:10 am – 11:40 am	Standards basics/ resources
11:40 am – 12:00 pm	CBER VCS recognition program
12:00 pm – 12:10 pm	Initial list of recognized standards
12:10 pm – 12:55 pm	Discussion and Questions
12:55 pm – 1:00 pm	Wrap-up

Welcome

Welcome and thank you for your involvement and participation in this webinar. All participation and views are welcome and appreciated. This an exciting opportunity for us to share about the new CBER standards recognition program and allow for questions and discussions.

Goals of the Webinar

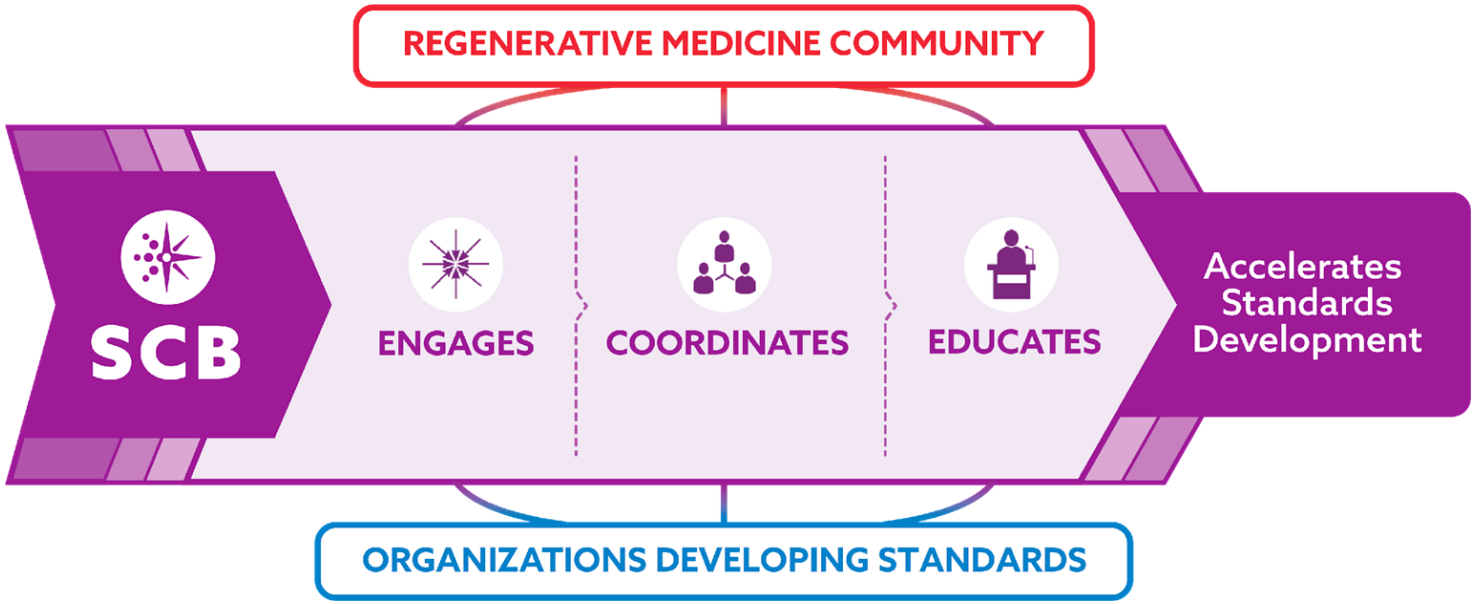
The goal of the Standards Development Forum is to bring together organizations involved in regenerative medicine in order to educate about the new VCS recognition program for regenerative medicine.

This forum is intended to facilitate conversation around this topic and allow for a better understanding of how to use standards in your approval process and awareness of resources for the public to find and understand standards.

Webinar Expectations

- Everyone has the opportunity to ask questions in the second half of the webinar of both SCB and FDA
- Please send questions to Dawn Henke in the chat
- Please raise your hand if you would like to speak
- Please mute yourself when you are not speaking

Connecting the regenerative medicine community to standards development



Established in 2016 and launched in January 2017, SCB is an **independent 501(c)(3)** organization

Occupies unique niche within field with **no vested interests in specific scientific, commercial, clinical or policy approaches**

SCB is **not an SDO**, but rather **coordinates** the standards development process

Serves as **communication vehicle** among all stakeholders, including government agencies, critical to the development of standards

Bringing value to patients and the community

MISSION: Coordinate the accelerated advancement and improved awareness of standards and best practices that address the rapidly evolving needs of the global regenerative medicine advanced therapy community

VISION: Improve patient lives through the widespread use of standards that enhance the consistency, availability, efficacy, quality, and safety of regenerative medicine therapies

Standards Recognition Program

Regulations, Guidances, and Standards

Regulations:
Have the force and effect of law and are usually mandatory, setting out specific requirements that regulated products and organizations must meet. In the United States, regulations are written in the Code of Federal Regulations and published in the Federal Register.

Guidances:
Formal documents issued by a government agency **to clarify** the agency's **thinking on existing laws or regulations** and offer guidelines for how industry **can comply with these regulations**.

Standards:
Voluntary rules, conditions, characteristics, or physical materials that an organization can adopt to make a process safer, more efficient, or better aligned with the practices of other organizations in their industry.

Different standards types include:

- **Documentary Standards**
- Standard Reference Material
- Standard Reference Data

Regulatory perspective on standards

- Regulatory have clearly expressed the preference for the use of consensus based standards in the approval process when applicable

Voluntary Consensus Standards Recognition Program for Regenerative Medicine Therapies

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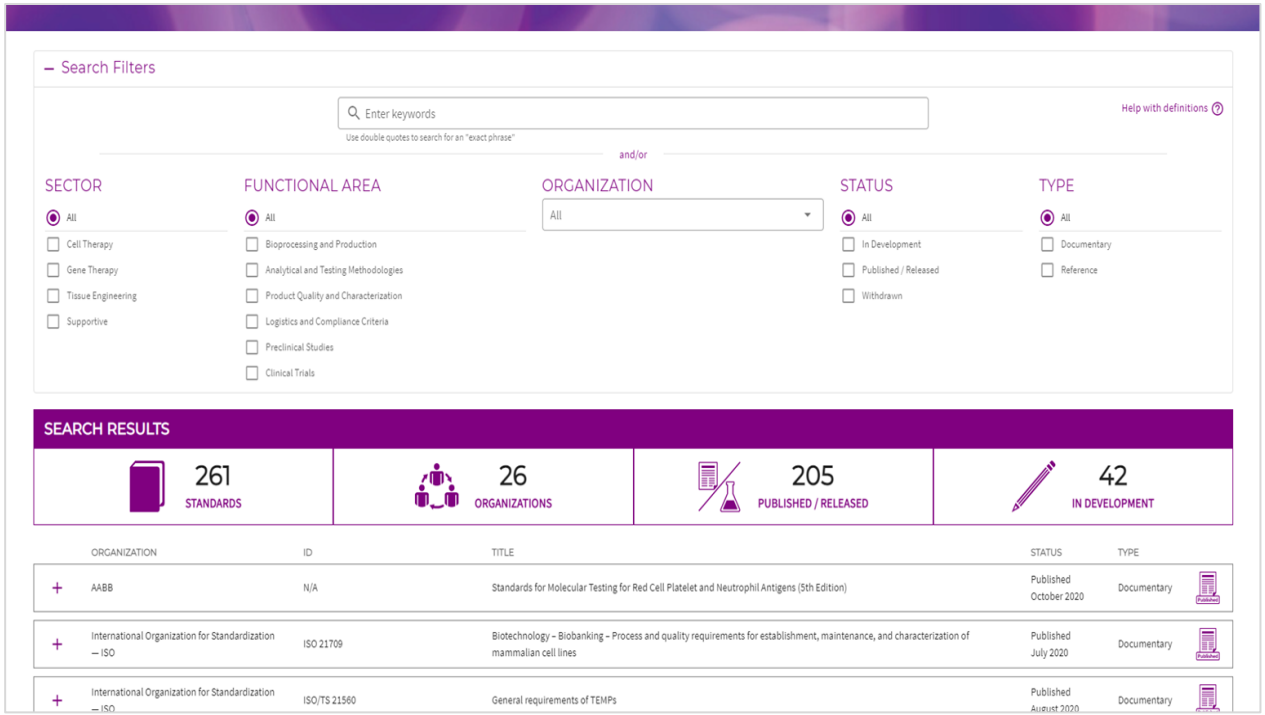
Guidance for Industry

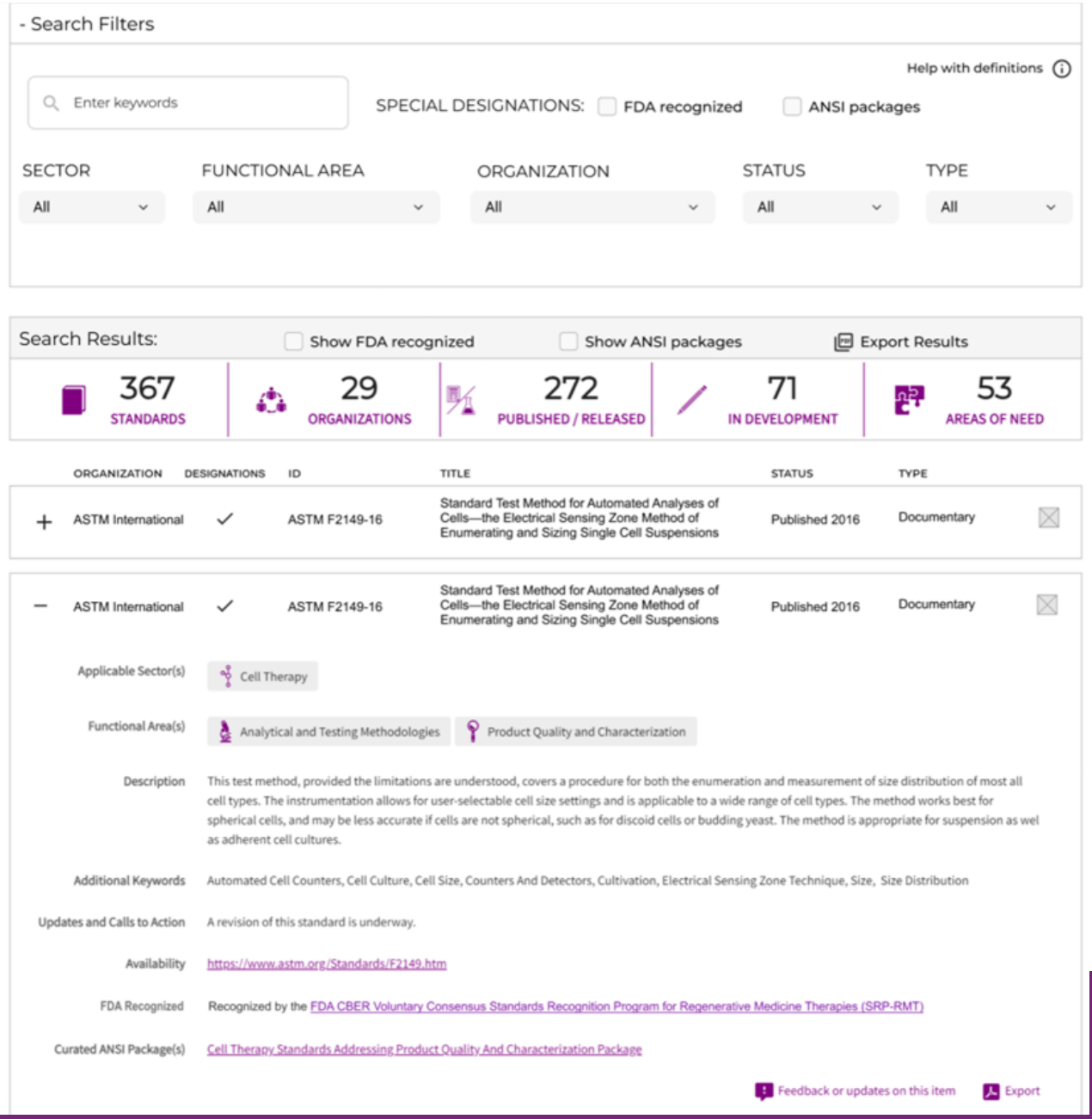
Standards Resources

Regenerative medicine standards portal

- Portal.standardscoordinatingbody.org
- Launched publicly in March 2021
- Interactive database of easily searchable information on published standards, in-development standards, and areas of need for the regenerative medicine community



Regenerative medicine standards portal



STANDARDS COORDINATING BODY
REGENERATIVE MEDICINE

Needed regenerative medicine standards

To provide feedback for the next update, please fill out the needed standards survey.

In order to create standards that are impactful and needed by the field we need to coordinate and devote resources to getting these standards developed

It is important to identify gaps in standards that can be filled so that we can ensure these standards that will advance the field are created

Some of these standards that will be created based off these needs could become part of the SRP-RMT

The needed standards survey can be found at: https://www.standardscoordinatingbody.org/needsurvey

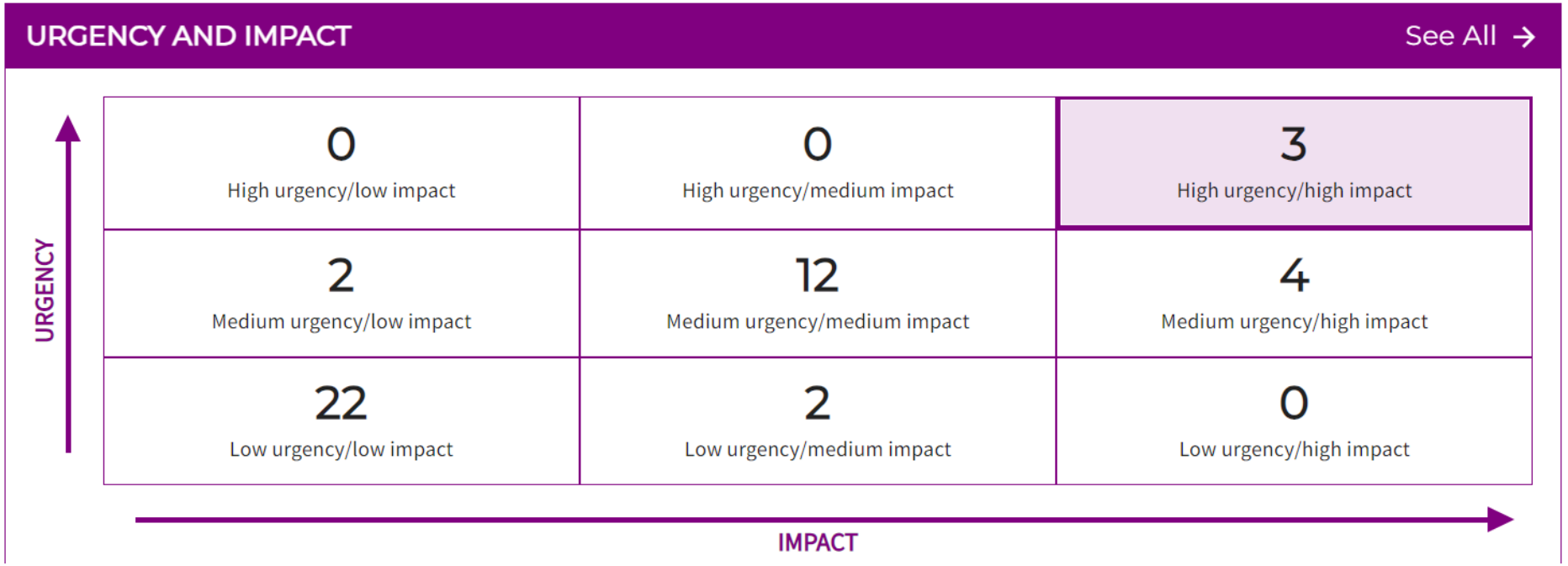
Needed regenerative medicine standards

To provide feedback for the next update, please fill out the needed standards survey.

Currently we are collecting responses for the update as part of the FDA contract. The most recent updates were reflected in 11/23

Based on the feedback, the chart below is updated semi-annually to reflect the community's prioritization perspectives.

TAKE THE SURVEY



The needed standards survey can be found at: https://www.standardscoordinatingbody.org/needsurvey

ANSI Packages for Recognized Standards

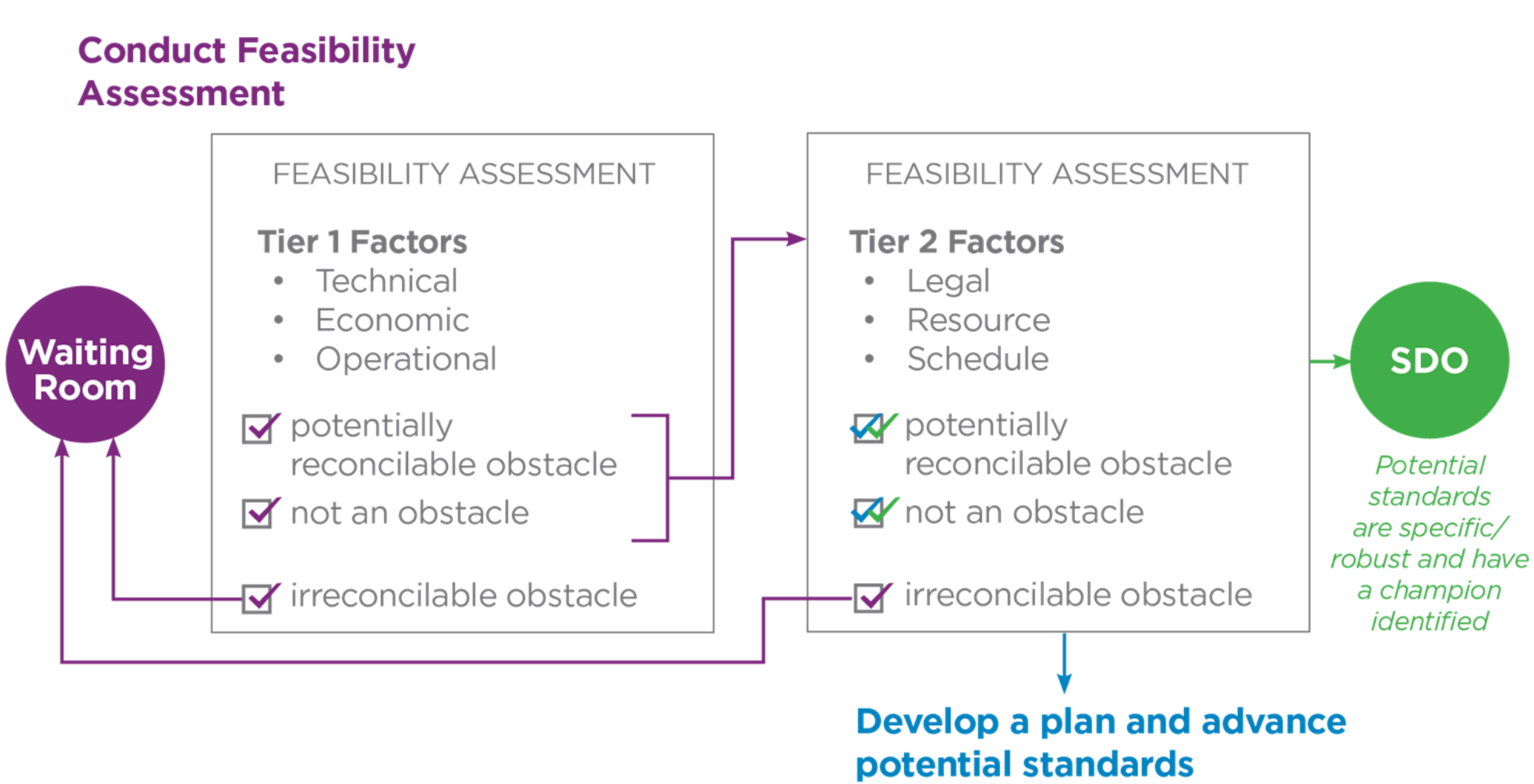
SCB has curated five new ANSI packages:

1. CBER Voluntary Consensus Standards Recognized: Cell Characterization

2. CBER Voluntary Consensus Standards Recognized: Sequencing
3. CBER Voluntary Consensus Standards Recognized: Cryropreservation and Storage
4. CBER Voluntary Consensus Standards Recognized: Scaffolds
5. CBER Voluntary Consensus Standards Recognized: Full List

Once the packages are published, they will be available here: https://www.standardscoordinatingbody.org/regen-med-standards-packages

Feasibility Assessment Plan



https://www.cellandgene.com/doc/the-importance-of-feasibility-assessments-in-the-standards-development-process-0001

Opportunities to Impact Standards Development and Implementation

New Working Groups

SCB, FDA, and USP co-organized a 2-day workshop to build on the results of two December 2022 feasibility assessments to identify specific topics that are feasible to standardize and would make a significant positive impact on the regenerative medicine field. More information can be found here, as well as a full workshop report: https://www.standardscoordinatingbody.org/release-standards-needs-identification-workshop

Two new working groups will be formed based on this workshop and feasibility assessment

- Empty, Full, Partial capsid characterization
- Killing assays for CAR-T products

To learn more or sign up for the upcoming working groups please fill out this survey: https://www.surveymonkey.com/r/Feb2024workinggroups

Open Ballots and Updates

1. ISO certificate of analysis: currently drafting. Submitting initial comment period in early February
2. Framework for cryopreservation: currently drafting. Submitting initial comment period in early February
3. PDA Cell Collection: PDA and the Task Force for this effort are actively drafting the standard
4. Patient Data Management: Working group has started developing standard
5. ISO Cell Viability: Current draft is available for review
6. ISO Minimum Requirements for Cellular Morphological Analysis — Image capture, image processing, and morphometry. At stage for final technical comments before publication.
7. ISO Packaging: Published
8. ISO cell line authentication: Published
9. ISO Nucleic Acid Synthesis — Part 2: General definitions and requirements for the production and quality control of synthesized gene fragment, gene, and genomes: Final stages before publication
10. ISO Gene delivery systems — Part 1, 2, and 3 – Passed NP Ballot, Looking for experts to engage for comment

Education Updates

Workforce Development Course Update

Pilot training program for standards:

SCB is designing and implementing a pilot training program (with ARMI | BioFab USA) to help manufacturers to avoid/minimize many of the common front-end issues of the manufacturing process.

ISO Cell Counting Part 1&2: The course has been launched through ISCTs platform.

ISO Ancillary Materials: Recruiting additional SMEs to assist with the creation of course content.

If interested in serving as a subject matter expert or to contribute case studies, please contact Katie at CZander@regenmedscb.org.

ISO Cell Counting Implementation Course

ISCT International Society for Cell & Gene Therapy

Contact Us | ISCT in the News | Media | Career Center | Donate | ISCT Community | LOGIN

ANNUAL & REGIONAL MEETINGS | MEMBERSHIP | WORKFORCE DEVELOPMENT | PUBLICATIONS | EDUCATION | ABOUT ISCT

THE IMPLEMENTATION OF CELL COUNTING STANDARDS

A Partnership between ISCT & SCB

ISCT INSTITUTE OF TRAINING & DEVELOPMENT

ABOUT THE COURSE

The Implementation of the ISO Cell Counting Standards course is a partnership between ISCT and the Standard Coordinating Body (SCB). This course was developed with input from the Subject Matter Experts (SMEs), including **NIST, FDA, and device manufacturers,** who created the ISO 20391-1:2018 (Part 1) and ISO 20391-2:2019 (Part 2) Standards.

This course will give you a fundamental understanding of cell counting terminology, methods, reporting, processes for cell counts, sample selection, and data analysis. You will learn from experts how to apply the principles of quality indicators for evaluating the quality of a cell counting measurement process. **As part of the course, you will receive an electronic copy of ISO 20391-1:2018 (Part 1) and ISO 20391-2:2019 (Part 2) Standards.**

At the end of the course, you will be confident in implementing ISO

Focus Areas

Since our inception in 2017, SCB has accelerated the advancement of 34+ standards. But we need additional community support to respond to the interrelated challenges facing the regenerative medicine field. To address this need we have developed SCB Focus Areas.

The first two Focus Areas are:

1. Standards Implementation Education and Workforce Development
2. Data Management

Donor Benefits Include:

- SCB's formal recognition of your contribution in meetings and media
- A seat on the Focus Area's Steering Committee
- Discounted course registration for your employees

Focus Areas

If interested in learning more about SCB Focus Areas, please contact Justin at JBarch@regenmedscb.org.

Standards Implementation Education and Workforce Development

KEY CHALLENGE:
Standards can only benefit the community if there is broad understanding of how to implement them to meet regulatory expectations.

FOCUS AREA SOLUTIONS:
Develop courses on how to use specific regenerative medicine standards to demonstrate regulatory compliance and improve the efficiency and repeatability of internal processes.

Data Management

KEY CHALLENGE:
Variability in data management practices and manufacturer requirements are common pain points for collection centers and can even overburden staff to the point of limiting their capacity to support clinical trials of potentially lifesaving therapies.

FOCUS AREA SOLUTIONS:
Identify common data needs and approaches to streamline data management practices.

Stay up to date on standards

Follow us on social media to stay up to date on news surrounding regenerative medicine standards, including webinars, FDA guidance documents, NIST consortium opportunities, open ballots, and new working groups.

Linkedin:https://www.linkedin.com/company/standards-coordinating-body

Twitter: https://twitter.com/SCBRegenMed

FOR MORE INFORMATION VISIT
www.standardscoordinatingbody.org

OR CONTACT dhenke@regenmedscb.org

Thank you!

CBER Standards Recognition Program for Regenerative Medicine Therapies (SRP-RMT)

Judith Arcidiacono, M.S.

Special Projects and Policy Staff

FDA/CBER/Office of Therapeutic Products

FDA/SCB Webinar: Introduction to CBER's Consensus Standards Recognition Program
February 21, 2024
11:00 AM – 1:00 PM

Voluntary Consensus Standards Recognition Program for Regenerative Medicine Therapies

Guidance for Industry

Additional copies of this guidance are available from the Office of Communication, Outreach and Development (OCOD), 10903 New Hampshire Ave., Bldg. 71, Rm. 3128, Silver Spring, MD 20993-0002, or by calling 1-800-835-4709 or 240-402-8010, or email ocod@fda.hhs.gov, or from the Internet at https://www.fda.gov/vaccines-blood-biologics/guidance-compliance-regulatory-information-biologics/biologics-guidances.

For questions on the content of this guidance, contact OCOD at the phone numbers or email address listed above.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Biologics Evaluation and Research
October 2023

OMB control number: 0910-0338
Current expiration date available at https://www.reginfo.gov.
See additional PRA statement in Section VIII of this guidance.

Contains Nonbinding Recommendations

Table of Contents

https://www.fda.gov/media/159237/download

Purpose of the SRP-RMT

Promote	Promote the development of standards that facilitate product development and streamline the review of RMT products
Identify	Identify standards that have been reviewed by FDA for scientific soundness and consistency with FDA regulations and policies.
Assist	Assist stakeholders and FDA reviewers in evaluating the proper use of a standard (fit-for-purpose) in a regulatory submission.

Types of Standards Reviewed for Recognition

- Only standards produced by Voluntary Consensus Bodies (VCSB) will be reviewed for recognition
- Processes that follow openness, balance, consensus, and due process
- Examples:
  - ATCC - American Type Culture Collection
  - ASTM - American Society for Testing Materials
  - IEEE- Institute of Electrical and Electronics Engineers
  - ISO - International Organization for Standardization
  - PDA - Parenteral Drug Association
  - CLSI - Clinical and Laboratory Standards Institute
  - Others

Standards Not Reviewed in the SRP-RMT but may be used in Regulatory Submissions for RMTs

Pharmacopeial standards

- Examples: US Pharmacopeia, Japanese Pharmacopeia, European Pharmacopeia

Accreditation standards

- Standards set forth by accreditation organizations to ensure that certain criteria are met for a specified process or system.
- Examples: Foundation for the Accreditation of Cellular Therapy (FACT), Association for the Advancement of Blood & Biotherapies (AABB)

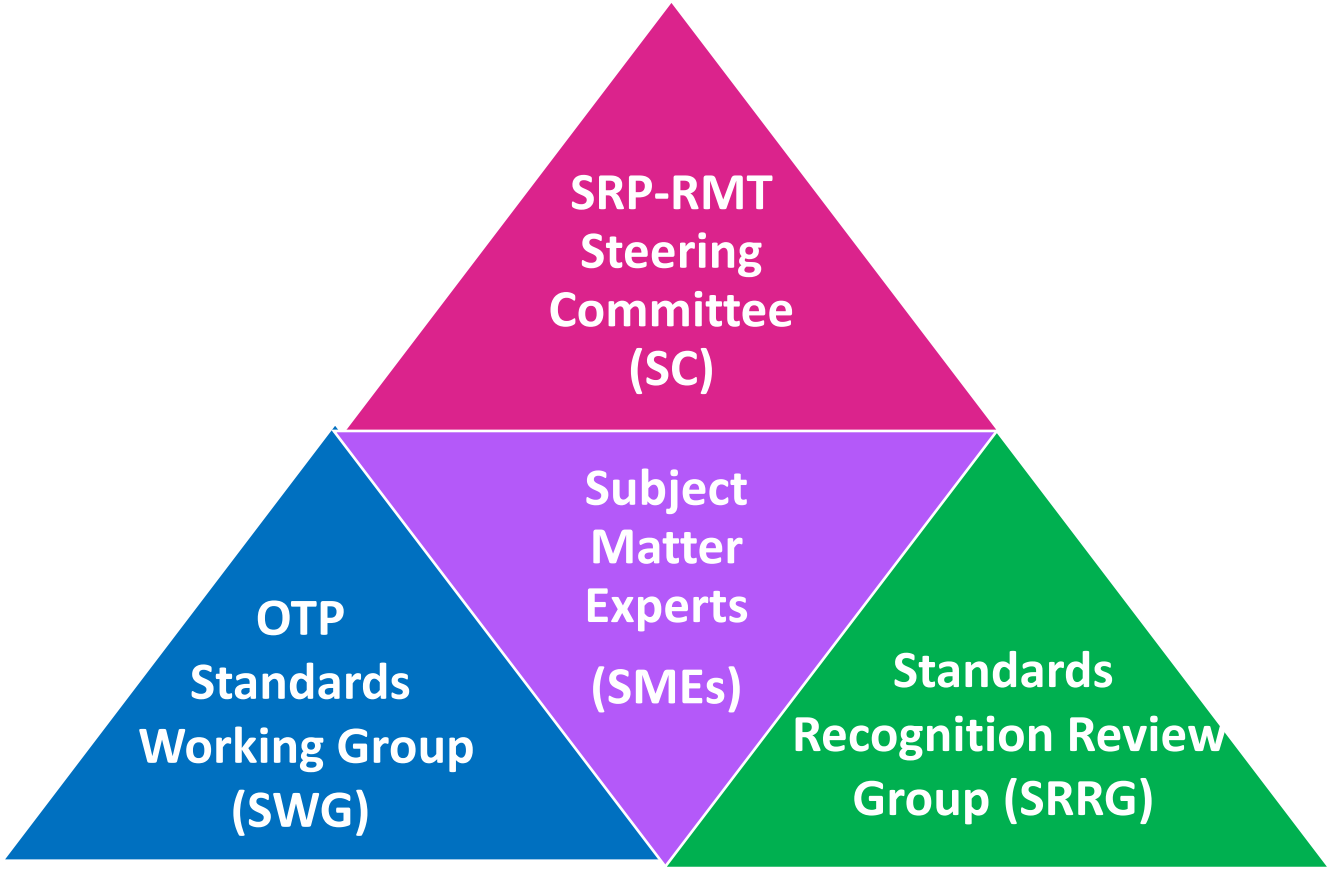
Standards created by institutions or societies

- Examples: International Society for Stem Cell Research (ISSCR), International Society for Cell & Gene Therapy (ISCT)

CBER Office of Therapeutic Products (OTP) SRP-RMT Teams

OTP is responsible for management of the SRP-RMT

- SC: Develop policy and processes
- SWG: Identify standards to be reviewed
- SMEs: Evaluate standards for recognition based on specified criteria
- SRRG: Discuss SME recommendations, vote on level of recognition



SRP-RMT Standards Review Process

Levels of Standard Recognition

FDA subject matter experts will evaluate standards for:

- Complete Recognition - the entire content of the standard is recognized
- Partial Recognition - only portions of the standard are recognized
  - FDA will identify the section(s) of the standard that are recognized.
- Non-recognition – the standard does not meet the criteria for recognition

Criteria for Evaluating Standards for Recognition

THE STANDARD WAS DEVELOPED BY A VCSB

THE STANDARD DOES NOT CONFLICT WITH CURRENT FDA STATUTE, REGULATIONS, OR POLICY

THE STANDARD IS SCIENTIFICALLY SOUND

THE STANDARD MAY FACILITATE THE ABILITY OF A SPONSOR TO MEET REGULATORY EXPECTATIONS

THE STANDARD CAN ASSIST FDA IN THE ASSESSMENT OF A REGULATORY SUBMISSION FOR RMT PRODUCTS

How will stakeholders know if a standard has been recognized?

- Recognized standards will be posted on the Standards Development for Regenerative Medicine Therapies page on the FDA website twice/year https://www.fda.gov/vaccines-blood-biologics/standards-development-regenerative-medicine-therapies
- Recognized standards will be accompanied by a Standards Recognition Sheet (SRS) that defines the terms of recognition

CBER Voluntary Consensus Standards Recognition Program for Regenerative Medicine Therapies

Last updated on November 28, 2023

Recognition Number	SDO	Designation ID	Year	Title	Recognition Status	Standard Recognition Summary (SRS)
001	ASTM	F2944	2020	Standard Practice for Automated Colony Forming Unit (CFU) Assays—Image Acquisition and Analysis Method for Enumerating and Characterizing Cells and Colonies in Culture	Complete Recognition	Rec 001
002	ANSI/ PDA	02	2021	Cryopreservation of Cells for Use in Cell Therapies, Gene Therapies, and Regenerative Medicine Manufacturing: An Introduction and Best Practices Approach on How to Prepare, Cryopreserve, and Recover Cells, Cell Lines, and Cell-Based Tissue Products	Complete Recognition	Rec 002
003	ASTM	F2212	2020	Standard Guide for Characterization of Type 1 Collagen as Starting Material for Surgical Implants and Substrates for Tissue Engineered Medical Products (TEMPs)	Complete Recognition	Rec 003
004	ASTM	F2739	2019	Standard Guide for Quantifying Cell Viability and Related Attributes within Biomaterial Scaffolds	Partial Recognition	Rec 004
005	ASTM	F3206	2017	Standard guide for assessing medical device	Complete	Rec 005

CBER Standards Recognition Program for Regenerative Medicine
Standards Recognition Summary

Recognition Number: 001

Date of Recognition: 03/17/2023

SDO Name/Designation: ASTM F2944

Year of Publication: 2020

Title: Standard Practice for Automated Colony Forming Unit (CFU) Assays – Image Acquisition and Analysis Method for Enumerating and Characterizing Cells and Colonies in Culture

Scope: 1.1 This practice, provided its limitations are understood, describes a procedure for quantitative measurement of the number and biological characteristics of colonies derived from a stem cell progenitor population using image analysis.
(19 additional clauses are in the scope of this document.)

Extent of Recognition: Complete Recognition

Rationale for Recognition: This standard is relevant to regenerative medicine therapies and is recognized because it is scientifically and technically valid and does not conflict with existing regulations and policy.

Standards Development Organization: https://www.astm.org

Please note that this standard may also be recognized under the Center for Devices and Radiological Health's *Recognized Consensus Standards Database for Medical Devices*, found here *https://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfStandards/search.cfm*

Citation of a Standard in a Regulatory Submission (Example)

SDO Name/Designation: ASTM F2944 **Year of Publication:** 2020

Title: Standard Practice for Automated Colony Forming Unit (CFU) Assays – Image Acquisition and Analysis Method for Enumerating and Characterizing Cells and Colonies in Culture

Possible sponsor statements in a submission:

The standard was used as written to count cell colonies derived from Hematopoietic Stem Cells (HSC).

OR

The method described in the standard was optimized for 2D cultures. The optimization protocol is provided in this submission.

SRP-RMT Guiding Principles

- The use of standards in a regulatory submission is not required.
- Most standards, including those that do not meet the definition of voluntary consensus standard can be used if fit-for-purpose.
- Use of a standard does not preclude FDA from asking for additional information to support the regulatory evaluation of a product.
- Stakeholders may request a standard be reviewed for recognition by email: SRP-RMT@fda.hhs.gov
  - Name and electronic or mailing address of the requester;
  - Name of the SDO;
  - Title of the VCS;
  - The VCS reference or SDO designation number and publication date (e.g., Q1234-2019);
  - Proposed list of products for which the standard could apply routinely;
  - Rationale for request; and
  - A brief description of the testing, performance, or other characteristics of the RMT product(s) or process(es) that would be addressed by the proposed standard.

Final Remarks

- Stakeholders are encouraged to participate in standards development activities.
  - Identifying needed standards
  - Contributing content to standards
  - Commenting on draft standards
- FDA, NIST, and SCB collaborative activities support the development of standards for the SRP-RMT.
- SCB Standards Portal is an important tool for stakeholders
  - Knowledge of existing standards, standards under development, and needed standards.

https://portal.standardscoordinatingbody.org/

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OTP Learn Webinar Series
https://www.fda.gov/vaccines-blood-biologics/news-events-biologics/otp-learn

CBER Website: https://www.fda.gov/vaccines-blood-biologics
Phone: 1-800-835-4709 or 240-402-8010
Consumer Affairs: OCOD@fda.hhs.gov
Manufacturers Assistance and Technical Training Branch:
industry.biologics@fda.hhs.gov

Questions and Answers

Discussion Questions

- If a standard isn't accepted the first time it is submitted can the decision be appealed?
- Are there plans to coordinate with the CDER recognized standards for medical devices?
- Can standards that aren't aren't on RMT specific topics but that might have relevance for RMT manufacturing be submitted?
- Is there any special way in your approval process that you have to denote or recognize that you are using a recognized standard?
- Can an international VCS be submitted?

Thank you!