Standards Coordinating Body

Catherine Zander, Ph.D.
Director of Standards Education & Outreach

Voluntary Consensus Standards

Ecosystem Building

In-Space Biomanufacturing and Standards

STANDARDS COORDINATING BODY
REGENERATIVE MEDICINE

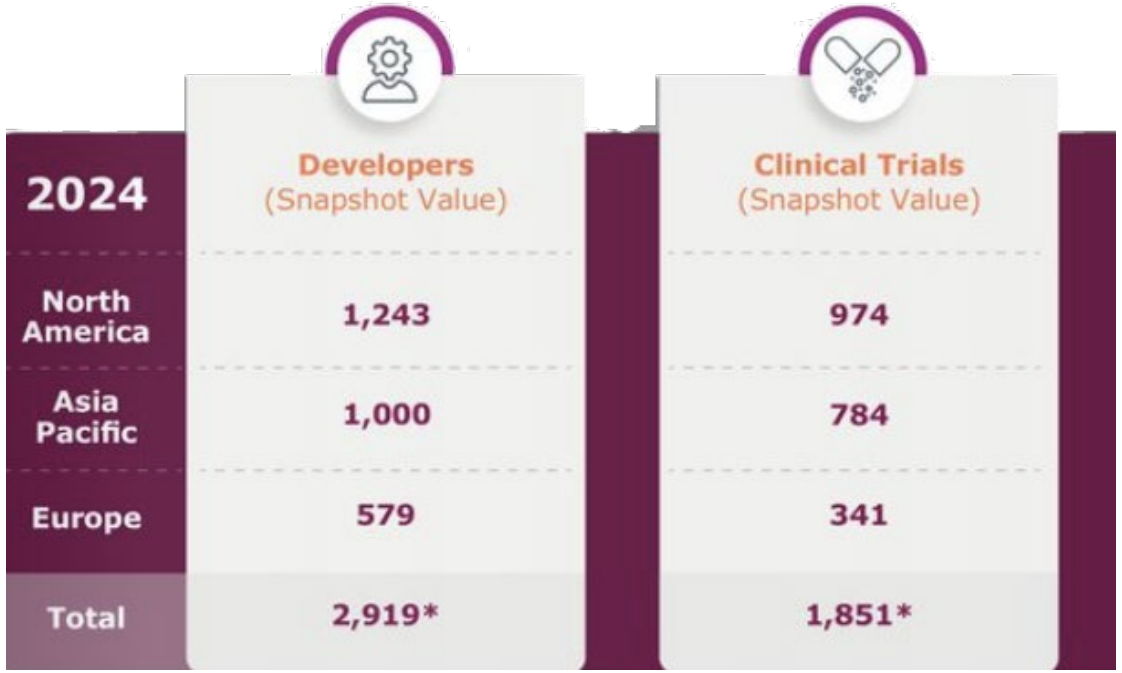
The Standards Coordinating Body (SCB)
&
Voluntary Consensus Standards (VCS)

Solving a Mission-critical CGT Challenge

Replacing confusion with consistency

Thousands of disparate clinical trials, processes, preferred practices, and other splintering factors result in a fractured sector that struggles to scale and and cannot efficiently create the systems needed to serve the millions of patients who would benefit from CGTs.

About 3,000 therapeutic developers operating almost 2,000 clinical trials worldwide. [1]



2024	Developers (Snapshot Value)	Clinical Trials (Snapshot Value)
North America	1,243	974
Asia Pacific	1,000	784
Europe	579	341
Total	2,919*	1,851*

"Instead of asking 200 transplant centers to conform, maybe these companies can join hands together… and start to standardize and harmonize." [2]

1. Alliance for Regenerative Medicine ,2024
2. Cell and Gene Therapy Insights, 2022

The Need for Regenerative Medicine Standards

Regenerative medicine therapies present unique challenges related to product testing, scientific protocols, product quality and specifications, performance characteristics, and compliance criteria.

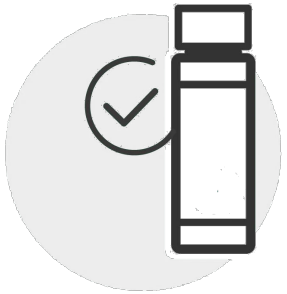
What is a Standard?

Common and repeated use of rules, conditions, guidelines or characteristics for products or related processes and production methods, and related management systems practices.

Documentary & Reference Standards

Documentary Standards

- Written document containing technical specifications or other criteria
- Written rules, guidelines, or definitions of characteristics



Reference Standards

- Homogenous and stable material; highly characterized reagents that are distributed to assure consistency, quality, and safety
- Established to be fit for intended use in a measurement process

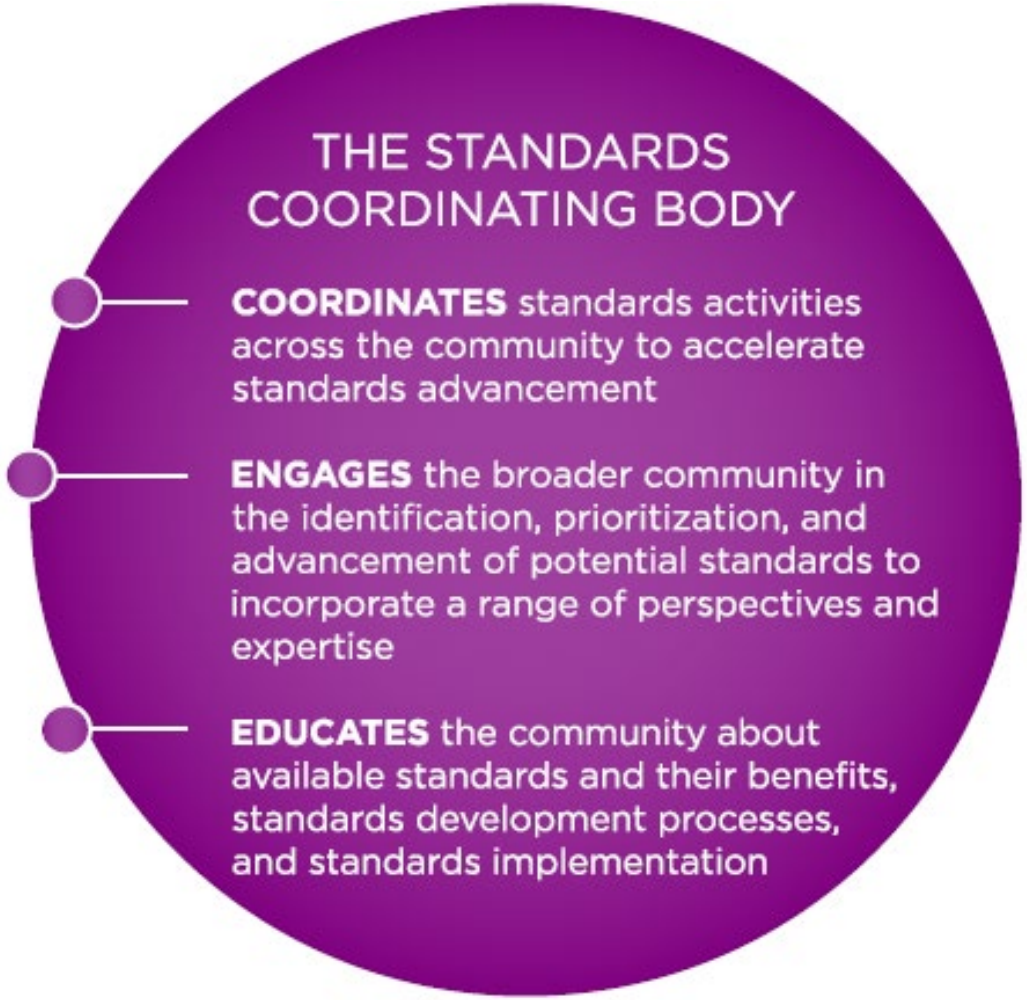
Voluntary Consensus Standards (VCS)

Requirements for the creation process for a voluntary consensus standard are:

- Openness
- Balance of Interest
- Due Process
- Appeals Process
- Consensus

ANSI
American National Standards Institute

Founding of the Standards Coordinating Body (SCB)



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 Standards Development Organization (SDO)**, but rather **coordinates** the standards development process

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

How SCB Works

The most robust, effective organization in CGT standards

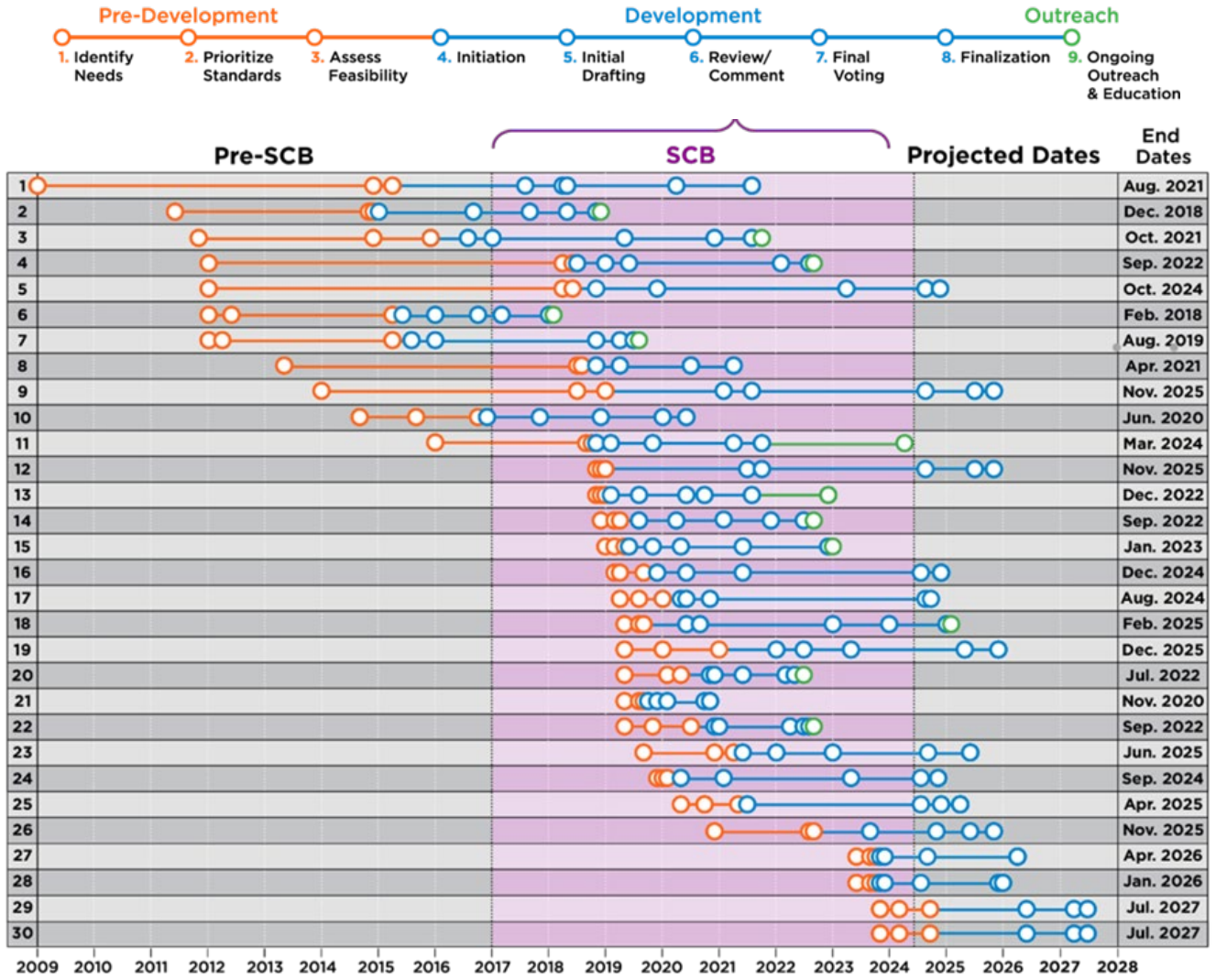
We work with thousands of experts and stakeholders in independent, non-biased operational models to facilitate discussion, identify needs, and advance impactful standards development.

- Before standards are developed: educate, identify needs, prioritize, assess feasibility
- During standards development: initiate, develop drafts, gather reviews and comments, voting
- Across the lifecycle: facilitate sector-wide discussion and education, conduct workshops, conduct surveys and gather feedback, manage Focus Areas of critical sector importance, ensure that regional voices are surfaced in global standards forums
- Substantial impact across CGT: workforce development training, ongoing education, ongoing neutral forum for standards discussion and vetting, online learning platform and standards library, curated standards packages, and more

1. Alliance for Regenerative Medicine ,2024
2. Cell and Gene Therapy Insights, 2022

Delivering Dramatic Standards Acceleration

With a full-time team of no more than three standards professionals, SCB has increased the efficiency of working with the regenerative medicine community to **identify needed standards**, **prioritize those needs** that will have the greatest impact on the field, and **assess the feasibility** of developing and **implementing standards** in these areas.

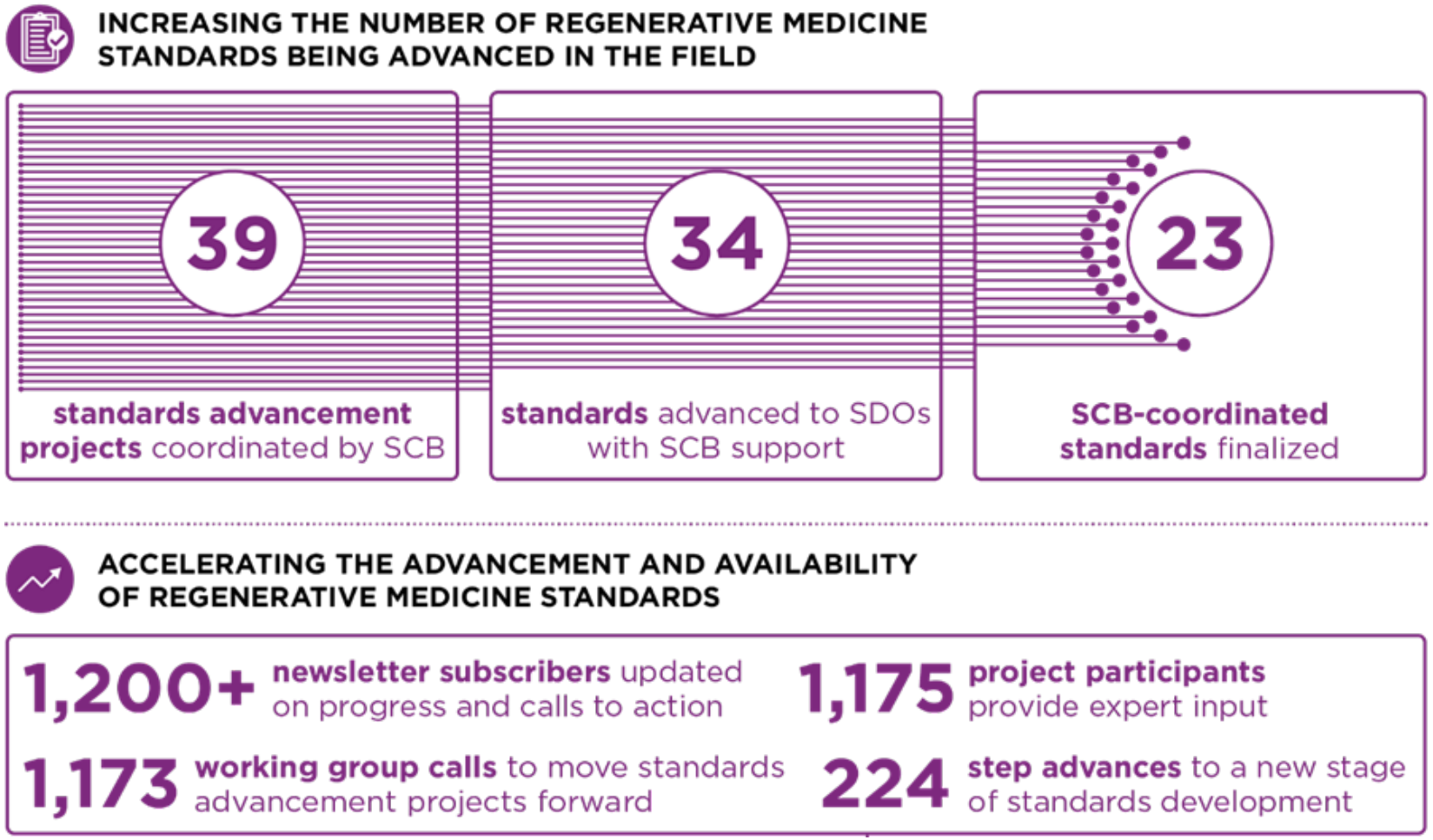


A Proven Track Record Across our Sector

SCB has increased the average rate of a standard's development by 3 FOLD.

With SCB, time from the establishment of need to a completed consensus standard has been shortened **to less than 4 years**.

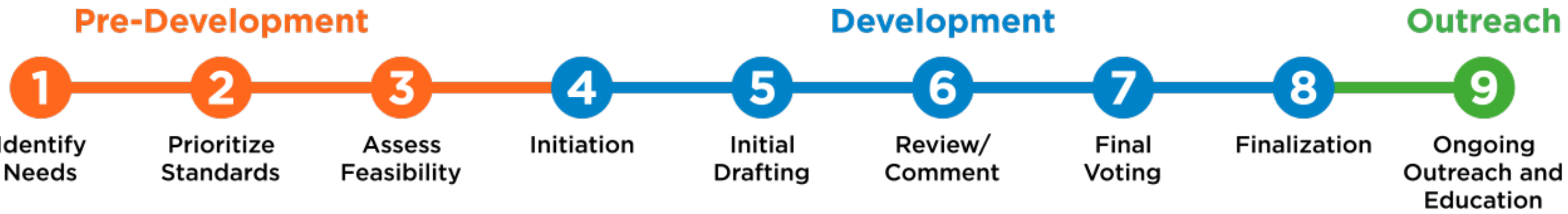
Additionally, **more than half** of the standards recognized by the CBER Voluntary Consensus Standards Recognition Program were facilitated by SCB.



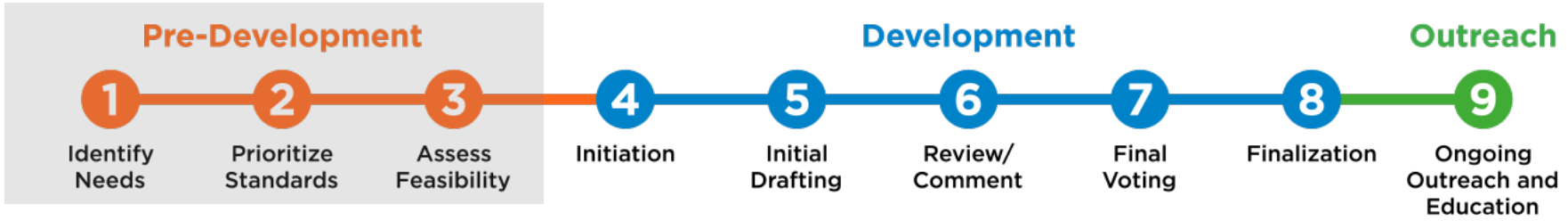
STANDARDS COORDINATING BODY
REGENERATIVE MEDICINE

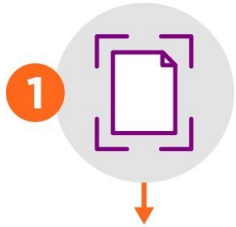
Voluntary Consensus Standards (VCS) Development Process

Standard Development Process



Standard Development Process



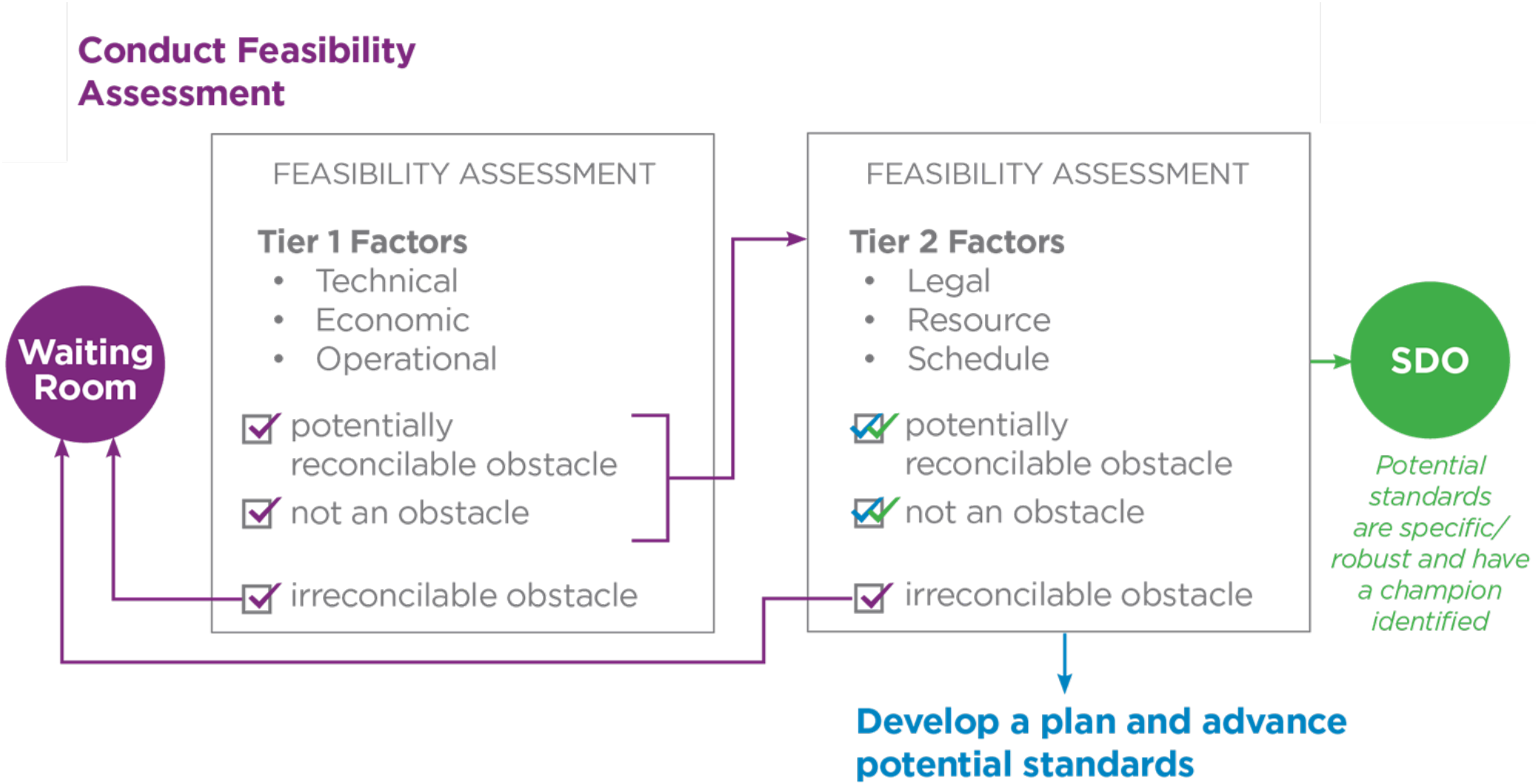


Identify Needs
Identify the need for a written/documentary standard or reference material/physical standard

Prioritize Standards
Solicit input from the community to determine which areas of need would have the greatest benefit for the field if standardized

Assess Feasibility
Gather experts to discuss the feasibility of developing the standard and confirm whether a standard has the necessary technical foundation and community support to move forward

Feasibility Assessment Plan



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

Needed Regenerative Medicine Standards

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

To share your standardization needs click here:
https://www.standardscoordinatingbody.org/needsurvey

Needed Regenerative Medicine Standards

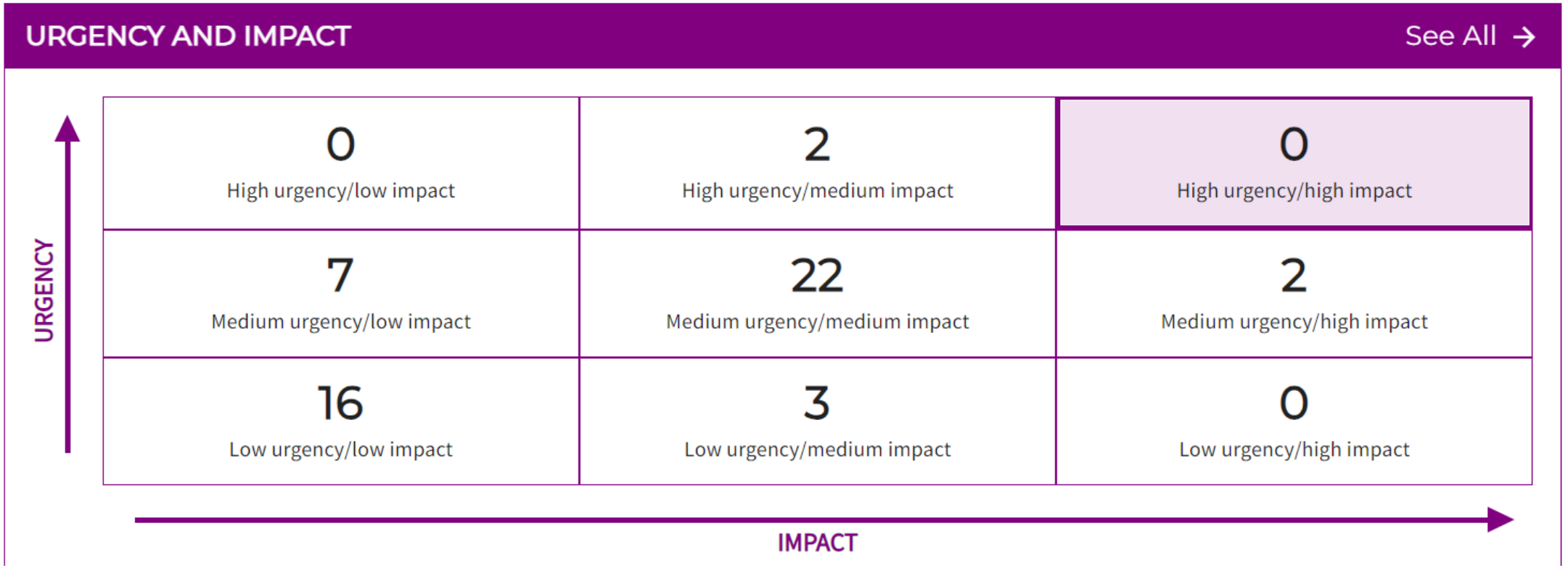
Areas of Need

SCB regularly solicits input from the regenerative medicine community to help identify and prioritize areas of need. Take our survey to provide feedback on:

- The **impact** that a standard could have on the availability of safe and high-quality regenerative medicine therapies
- The level of **urgency** with which a needed standard should to be pursued to mitigate risk and realize opportunities

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

TAKE THE SURVEY



STANDARDS COORDINATING BODY
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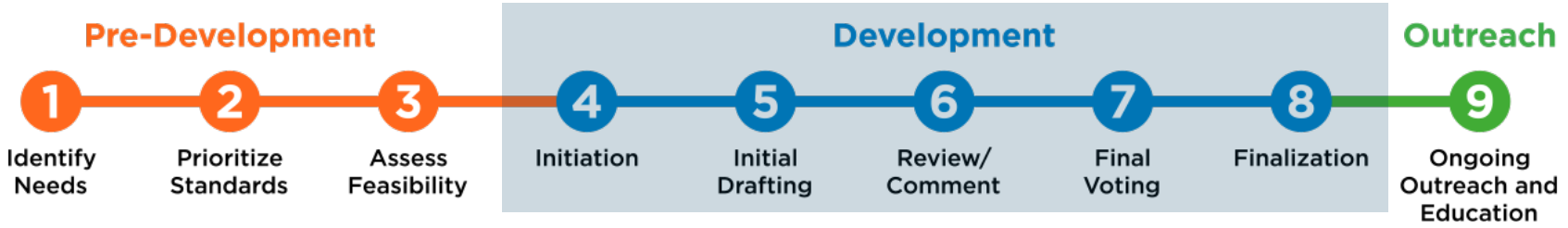
https://www.standardscoordinatingbody.org/needsurvey

Standard Development Process



Initiation: Present the standard concept to an SDO or other standards developing body and receive acceptance for standard development

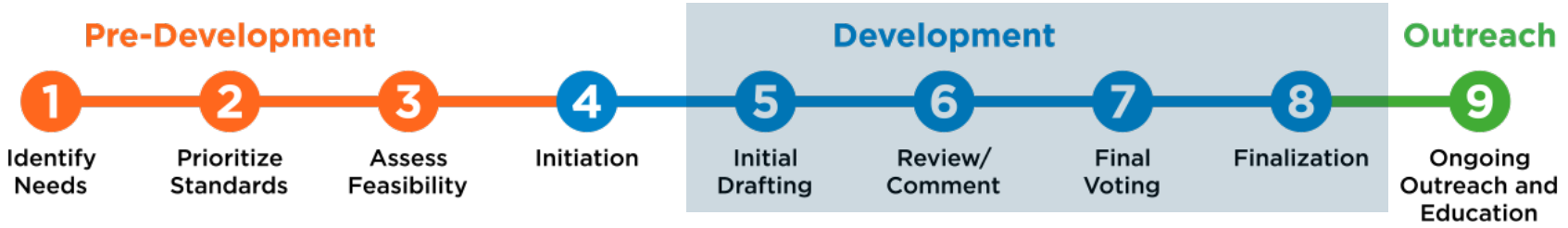
Initial Drafting: Experts within a working group begin to draft the standard

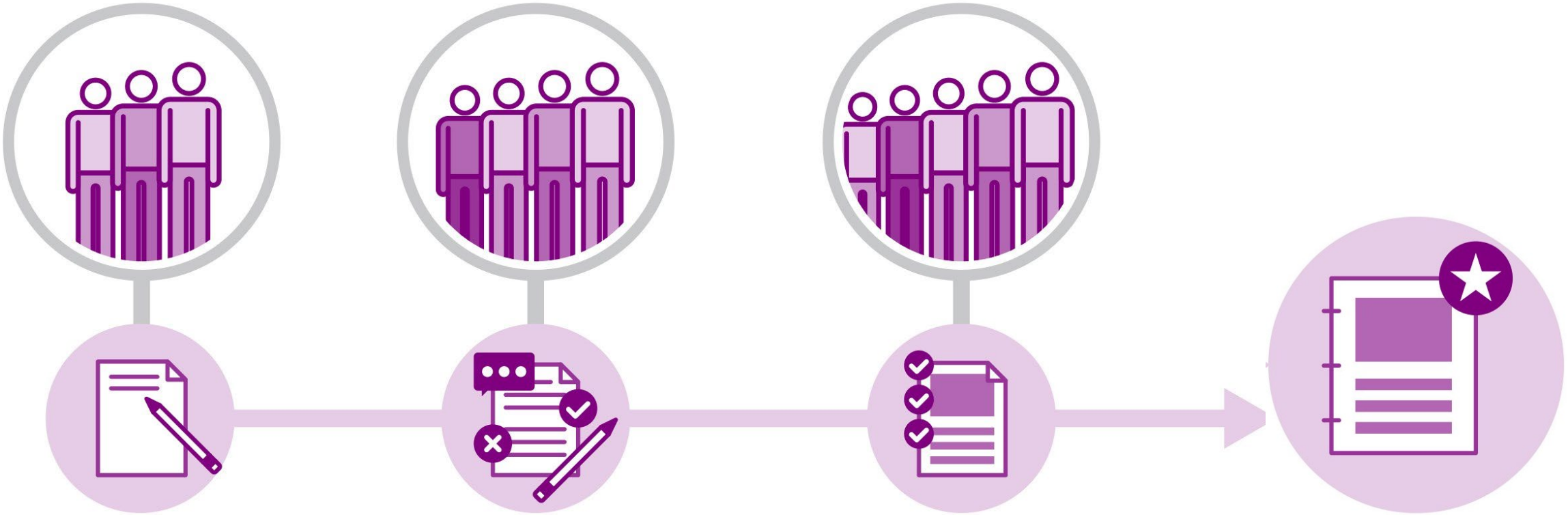
Review/Comment: Drafts of the written standard are circulated for comment, voting, and editing.

Final Voting: A final round of voting is conducted and the standard is approved for publication

Finalization: Final changes are incorporated into the standard and it is prepared for publication

Standard Development Process





Working Group Survey

SCB has established a simple and direct way to sign up and share SCB working groups by creating a short survey.

Any working group discussed during this call can be signed up for here: https://www.surveymonkey.com/r/SCBWorkinggroups

VCS & the Regulatory Process

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

Legal Basis for the Use of Standards in the Regulatory Process

The legal basis of the federal use of consensus standards is found in the:

1. National Technology Transfer and Advancement Act (NTTAA)
2. Federal Food, Drug, and Cosmetic Act (FD&C)

It is further clarified in two FDA guidances,

1. *Standards Development and the Use of Standards in Regulatory Submissions Reviewed in the Center for Biologics Evaluation and Research*
2. *Appropriate Use of Voluntary Consensus Standards in Premarket Submissions for Medical Devices*

This stance across the federal government is well summarized by the Office of Management and Budget (OMB) as follows:

> *"Agencies shall use existing voluntary consensus standards, both domestic and international, in their regulatory and procurement activities as a means of carrying out policy objectives or activities determined by the agencies, unless use of such standards would be inconsistent with applicable law or otherwise impractical.* ***Agencies shall use such voluntary consensus standards for test methods, procurement guidelines, management systems, sampling procedures, or protocols to determine whether established regulatory limits or targets have been met."***

Regulatory perspective on standards

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

A. The National Technology Transfer and Advancement Act (NTTAA)

In 1996, Congress passed the National Technology Transfer and Advancement Act (NTTAA) (Pub. L. No. 104-113), codifying an Office of Management and Budget (OMB) directive, Circular A-119, Federal Participation in the Development and Use of Voluntary Consensus Standards and in Conformity Assessment Activities, that had previously been issued several times, dating back to the late 1970s.[1] The NTTAA and OMB Circular A-119 established Federal government policies to improve the internal management of the Executive Branch by directing agencies to use voluntary consensus standards in lieu of government-unique standards except where voluntary consensus standards are inconsistent with law or otherwise impractical.[2]

Regulatory perspective on standards

B. The Food and Drug Administration Modernization Act and 21st Century Cures Act

Congress also enacted the Food and Drug Administration Modernization Act of 1997 (FDAMA) (Pub. L. No. 105-115) and the 21st Century Cures Act (Pub. L. 114-255), which amended section 514(c) of the Federal Food, Drug, and Cosmetic Act (FD&C Act). Section 514(c) states, in part, that FDA "shall, by publication in the Federal Register . . . recognize all or part of an appropriate standard established by a nationally or internationally recognized standard development organization for which a person may submit a declaration of conformity in order to meet a premarket submission requirement or other requirement," 21 U.S.C. 360d(c)(1)(A).

The term "recognize" in section 514(c) of the FD&C Act refers to FDA's formal identification of a standard after a determination that it is appropriate for manufacturers of products to declare conformance to meet relevant requirements in the FD&C Act, including premarket submission requirements.

This guidance refers to voluntary consensus standards recognized by FDA in the *Federal Register* in accordance with section 514(c) of the FD&C Act as "FDA-recognized consensus standards." A list of consensus standards that FDA has recognized or decided to recognize is available on the FDA Recognized Consensus Standards Database Web site.[3] See section IV for more information about standards that FDA has decided to recognize but for which recognition is still pending.

Voluntary Consensus Standards Recognition Program for Regenerative Medicine Therapies

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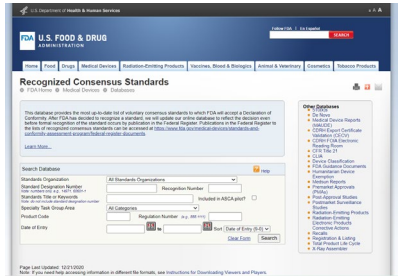
Draft Guidance for Industry

Overall purpose/scope of guidance



This program is modeled after the formal standards and conformity assessment program or S-CAP for medical devices

This guidance describes a program at CBER for **recognition of VCS relevant to RMT products regulated in CBER**. This guidance also describes how CBER intends to review VCS for recognition in the SRP-RMT. This program will not apply to: statutory and regulatory standards that are legally binding, such as certain provisions of the FD&C Act (21 U.S.C. 301-et seq.) and PHS Act (42 U.S.C.); to standards developed by SDOs that do not follow consensus mechanisms; or to electronic data exchange standards for submissions to CBER.

The SRP-RMT is expected to facilitate product development by:

- Using Agency expertise to evaluate and recognize voluntary consensus standards related to RMT products that are potentially useful to industry and CBER staff. Specifically, this process will allow CBER to:
  - **Receive a candidate VCS**, with relevant information (e.g., the scope of the standard and the purpose), from internal or external parties for informal recognition.
  - Determine whether to recognize a standard in **whole or in part** following an internal scientific evaluation.
  - **List the recognized standards** in a publicly searchable database on CBER's website, accompanied by an information sheet describing the scope and the extent of CBER's recognition of that standard and any other relevant information about the standard.
- Providing **transparency to industry and other stakeholders regarding CBER's thinking** about a particular method or approach, thereby increasing regulatory predictability.
- **Promoting the visibility and use of standards** applicable to its public health mission.

FDA evaluation of standards

- The standard does not conflict with the FD&C Act, the PHS Act, applicable regulations, or current policies;
- The standard is scientifically sound;
- The standard can assist in the assessment of a regulatory submission for RMT products; and;
- Use of the standard may facilitate the ability of a sponsor to meet regulatory requirements for RMT products reviewed in CBER.

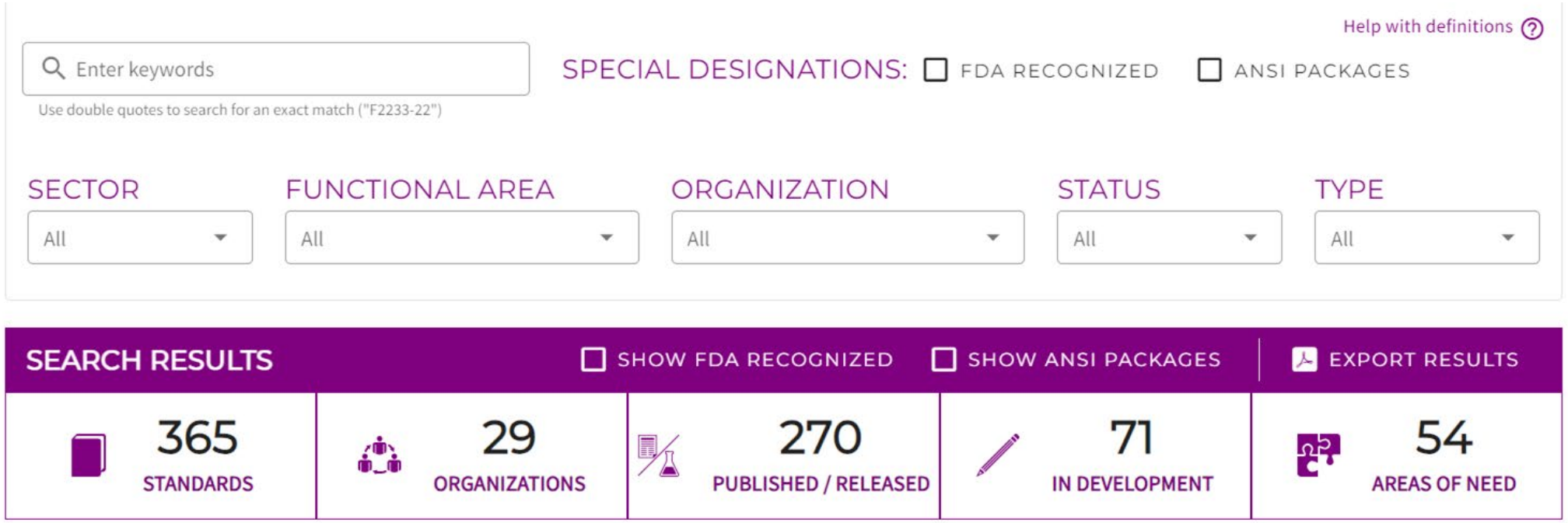
Voluntary Consensus Standards Recognition Program for Regenerative Medicine Therapies

Guidance for Industry

- Guidance outlines a program for the FDA to vet and formally recognize standards that are applicable to regulatory approval of regenerative medicine products
- SCB hosted a webinar on 2/21 information. The recording is posted on our website: https://www.standardscoordinatingbody.org/release-scb-webinar-cber-consensus-std-recog
- Link to FDA Guidance: https://www.fda.gov/regulatory-information/search-fda-guidance-documents/voluntary-consensus-standards-recognition-program-regenerative-medicine-therapies
- CBER Recognized Standards List: https://www.fda.gov/vaccines-blood-biologics/standards-development-regenerative-medicine-therapies

Standards Resources

SCB's 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

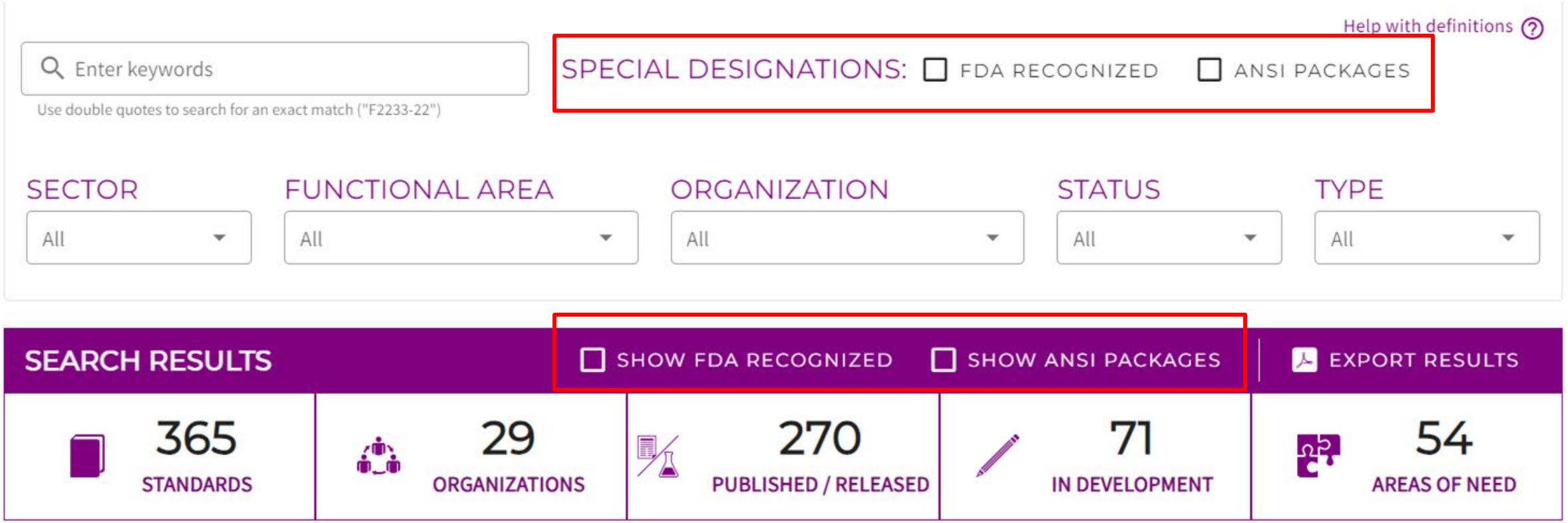
ANSI Packages for Recognized Standards

SCB has curated four new ANSI packages:

1. CBER Voluntary Consensus Standards Recognized: Cell Characterization
2. CBER Voluntary Consensus Standards Recognized: Sequencing
3. CBER Voluntary Consensus Standards Recognized: Cryopreservation and Storage
4. CBER Voluntary Consensus Standards Recognized: Scaffolds

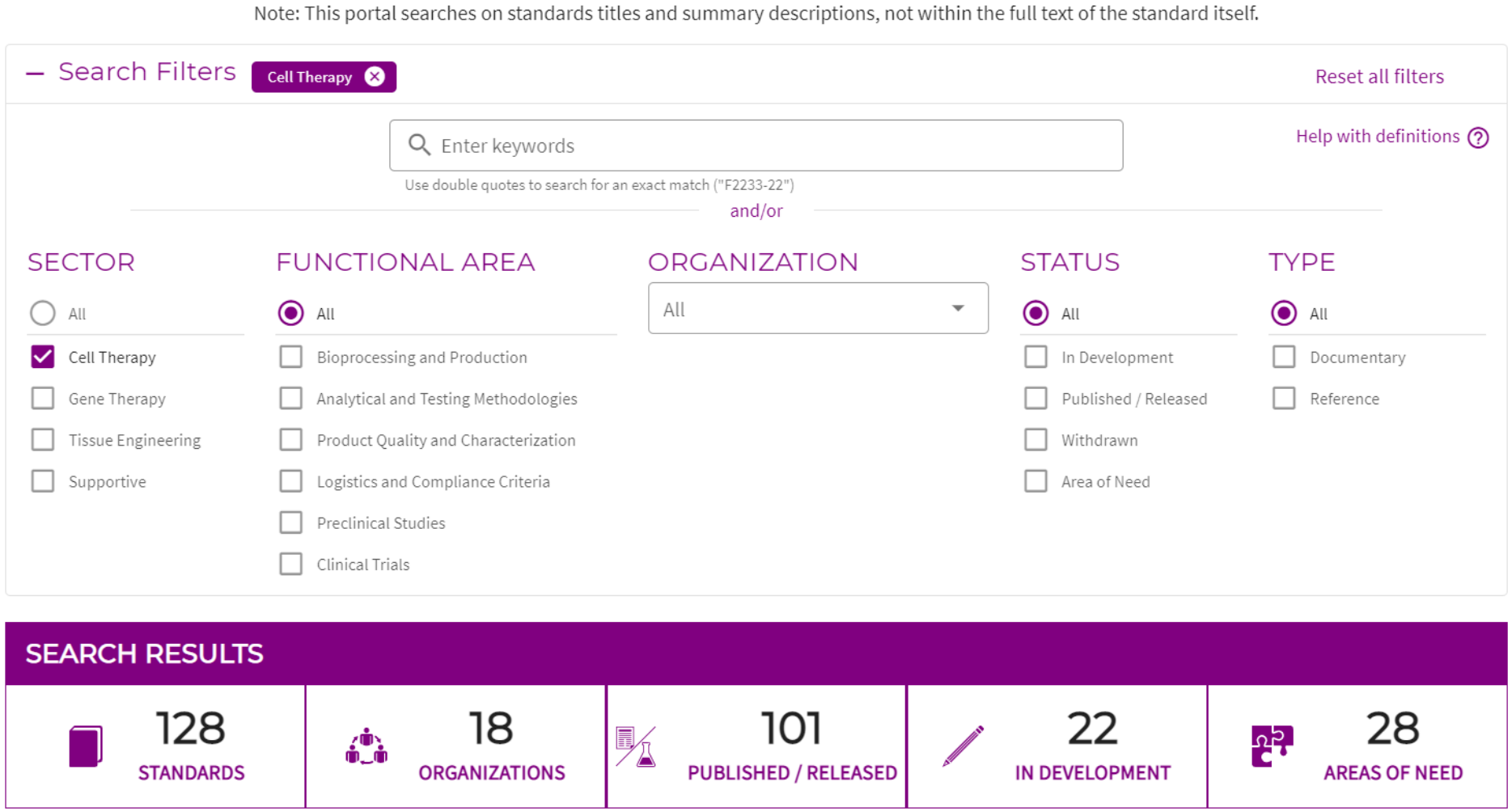
These packages are available here:
https://www.standardscoordinatingbody.org/regen-med-standards-packages

SCB's Standards Portal



- Search features for special designations were added to select standards recognized by the CBER Standards Recognition Program and/or those available as part of an SCB Curated ANSI Package.

SCB's Standards Portal



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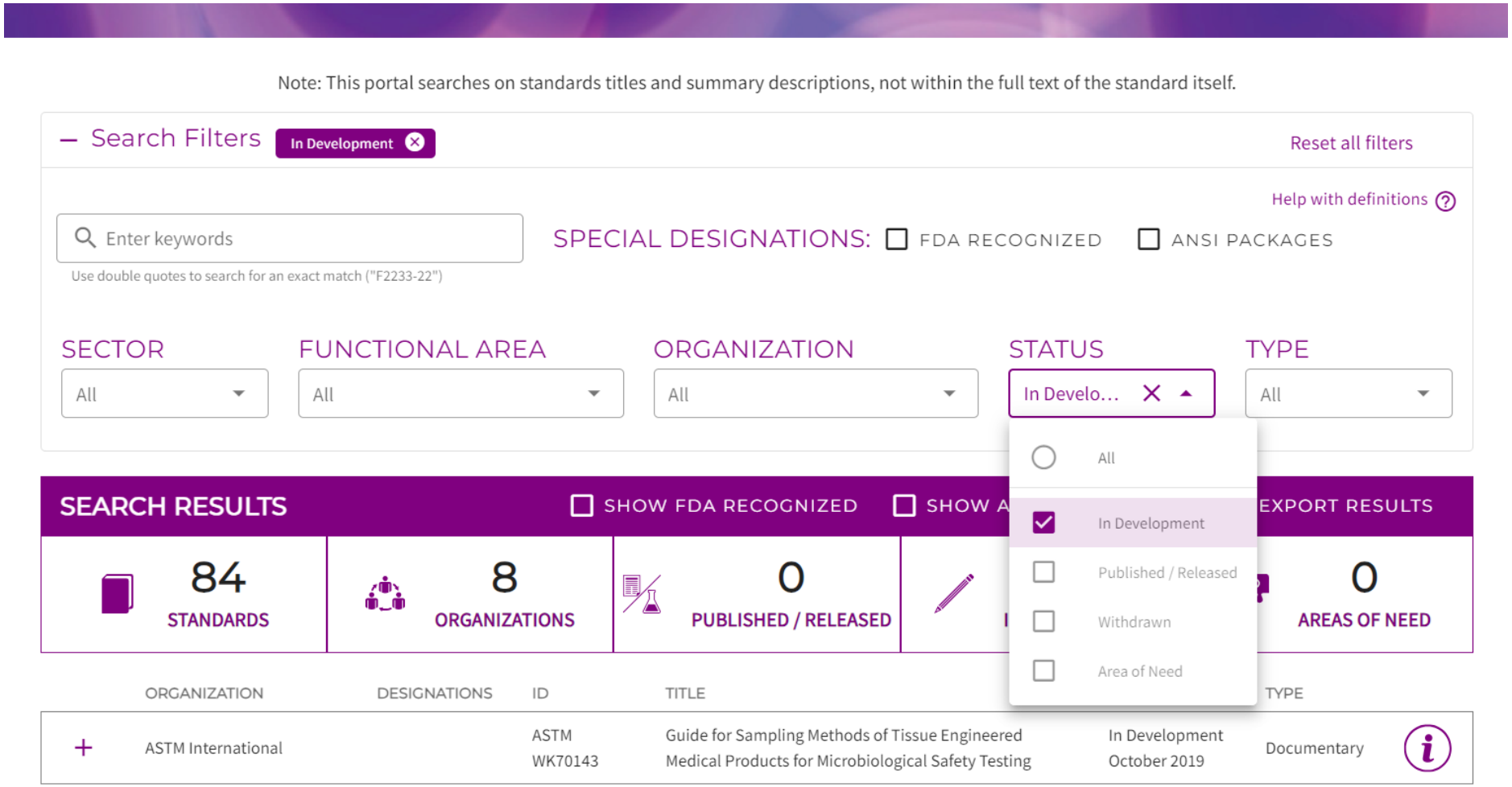
https://portal.standardscoordinatingbody.org/

Standards in Development



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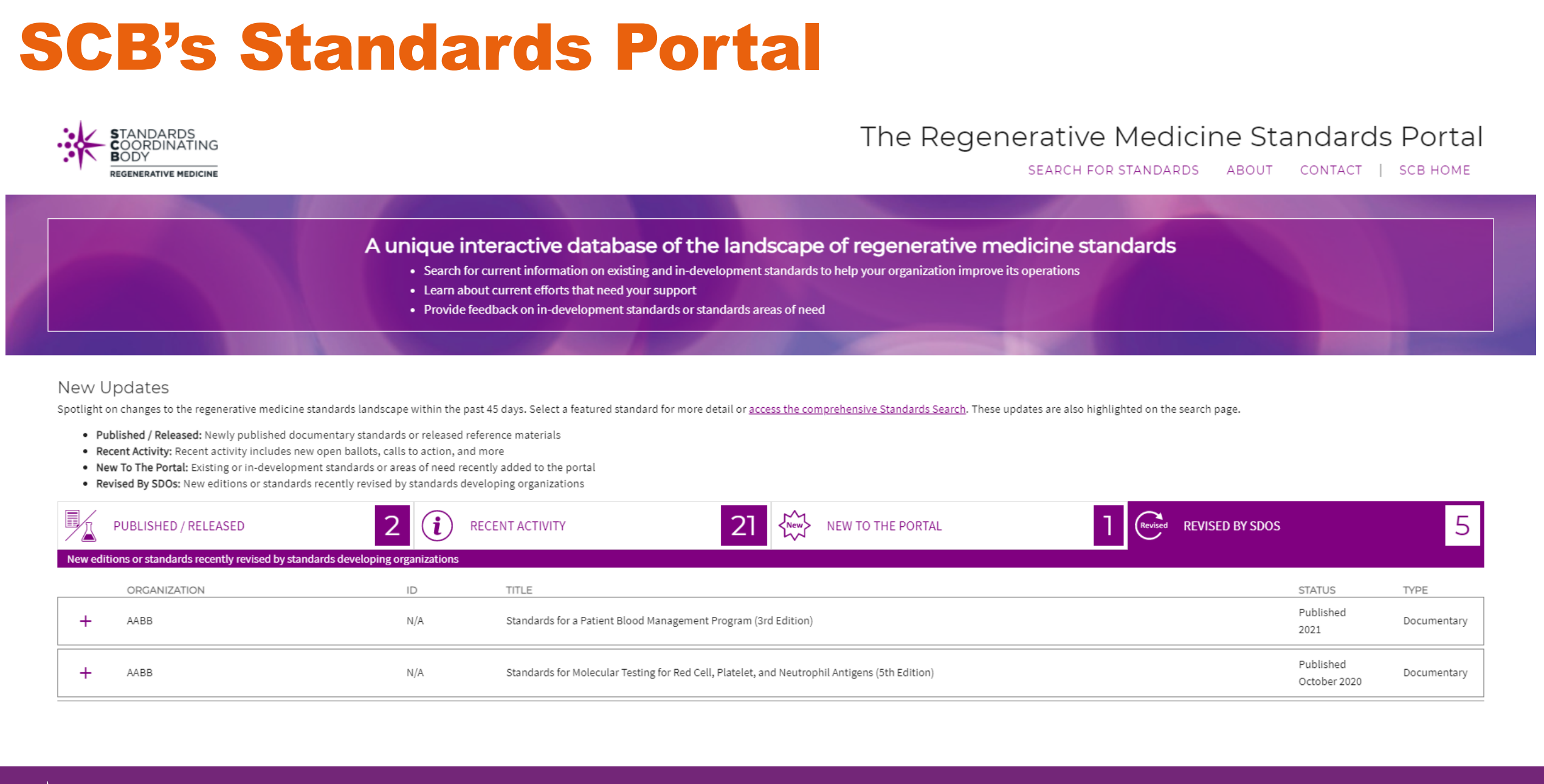

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SCB's Standards Portal

+	International Organization for Standardization — ISO	ISO/AWI TS 23494-1	Biotechnology — Provenance information model for biological material and data — Part 1: Design concepts and general requirements	In Development May 2020	Documentary	
−	International Organization for Standardization — ISO	ISO 20391-1:2018	Biotechnology - Cell Counting - Part 1: General guidance on cell counting methods	Published 2018	Documentary	

Applicable Sector(s) Cell Therapy

Functional Area(s) Analytical and Testing Methodologies

Description ISO 20391-1:2018 defines terms related to cell counting for biotechnology. It describes counting of cells in suspension (generally cell concentration) and cells adhered to a substrate (generally area density of cells). It provides key considerations for general counting methods (including total and differential counting, and direct and indirect counting) as well as for method selection, measurement process, and data analysis and reporting.

Additional Keywords advanced therapies, front end, upstream, product development, regenerative medicine, hemocytometer, dyes, stains

Availability https://www.iso.org/standard/68879.html

Report errors or needed 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 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: SMEs working on 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

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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

https://www.isctglobal.org/resources/training-programs/iso-cell-counting

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

Questions?

FOR MORE INFORMATION VISIT

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OR CONTACT

CZander@regenmedscb.org

Thank you!