STANDARDS DEVELOPMENT FOR
REGENERATIVE MEDICINE THERAPIES

Thank you for joining the webinar on **Regenerative Medicine Standards Development Processes.** The webinar will begin shortly.

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Claudia Zylberberg

CEO, Akron Biotech

Judith Arcidiacono

International Regulatory Expert and Standards Liaison, Office of Tissue and Advanced Therapies, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration

Sumona Sarkar

Biomedical Engineer, National Institute of Standards and Technology

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STANDARDS DEVELOPMENT FOR
REGENERATIVE MEDICINE THERAPIES

Regenerative Medicine Standards Development Processes

June 26, 2018

Webinar Purpose

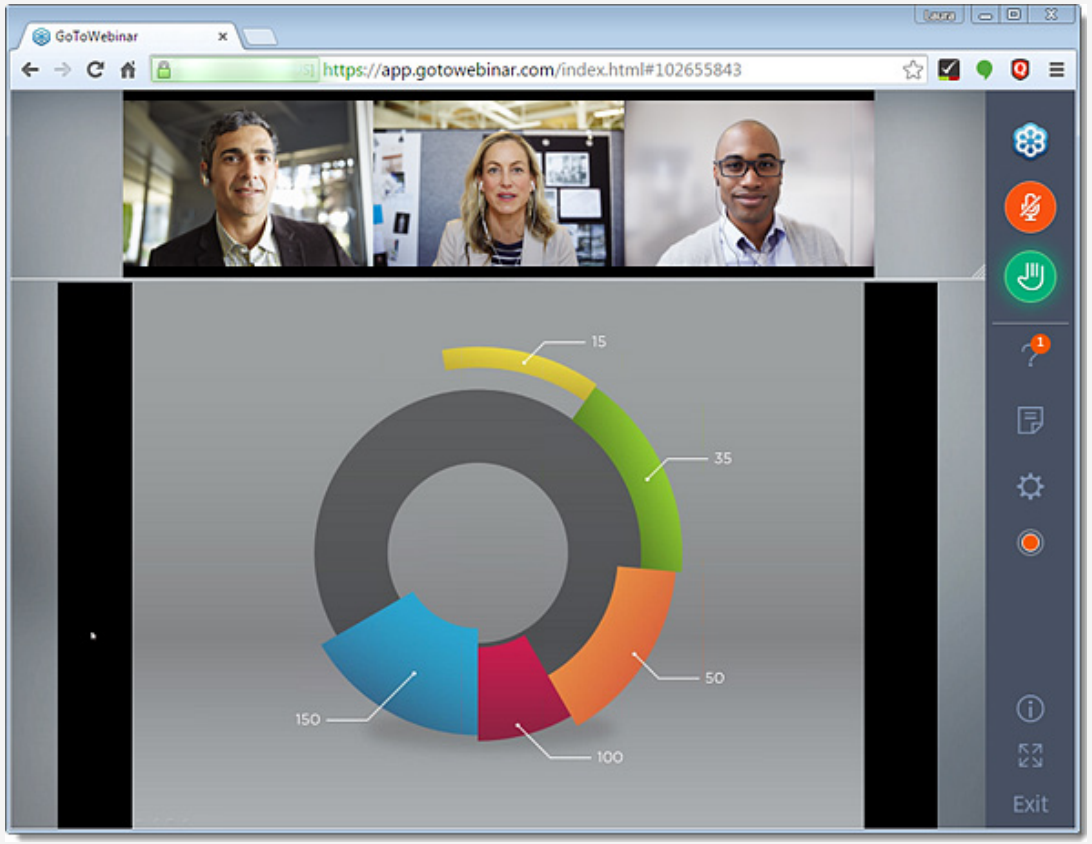
- Learn about how standards can benefit you or your organization.
- Understand how the Standards Coordinating Body is supporting coordination of, engagement in, and awareness of standards development.
- Understand how you can learn more about identifying or advancing needed standards.
- Identify specific ways you can become engaged in standards development.

Webinar Outline

- Webinar logistics
- The benefits of standards for regenerative medicine
- Potential improvements to and engagement opportunities in standards development
- Wrap-up and next steps

Logistics

- The webinar is being recorded
- Please mute your microphones/phones unless you are speaking
- If you are having trouble with the GoToWebinar tool, please use the chat function at the question mark icon
- If you would like to ask a question, please use the chat function at the question mark icon



The Benefit of Standards for Regenerative Medicine

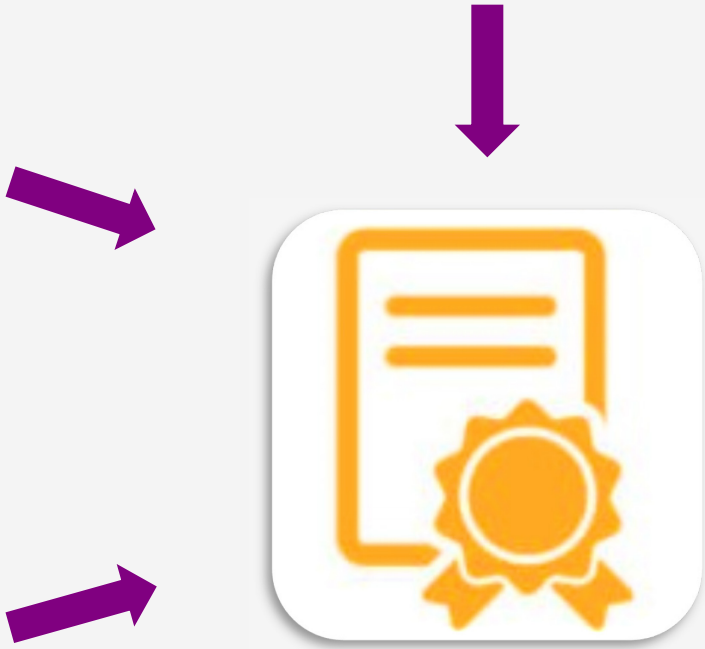
Allison Getz
Operations Program Manager,
The Standards Coordinating Body

What is a Standard?

Requirements, specifications, guidelines, or characteristics
to ensure something is fit for its purpose

Tools to drive innovation and increase productivity

An agreed way of doing something

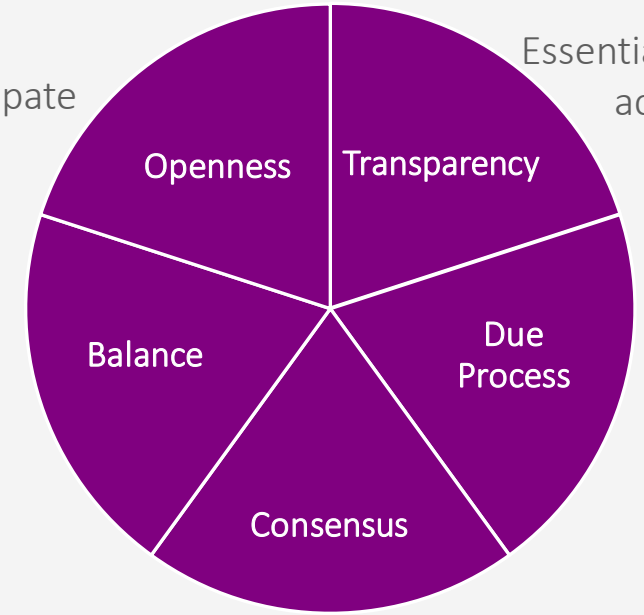


Knowledge

Distilled wisdom of people with expertise in their subject matter

Standards Development Principles

Documentary Standards (Voluntary Consensus)



Anyone can participate

Essential information accessible to all

No single interest may dominate

Fairness & equity

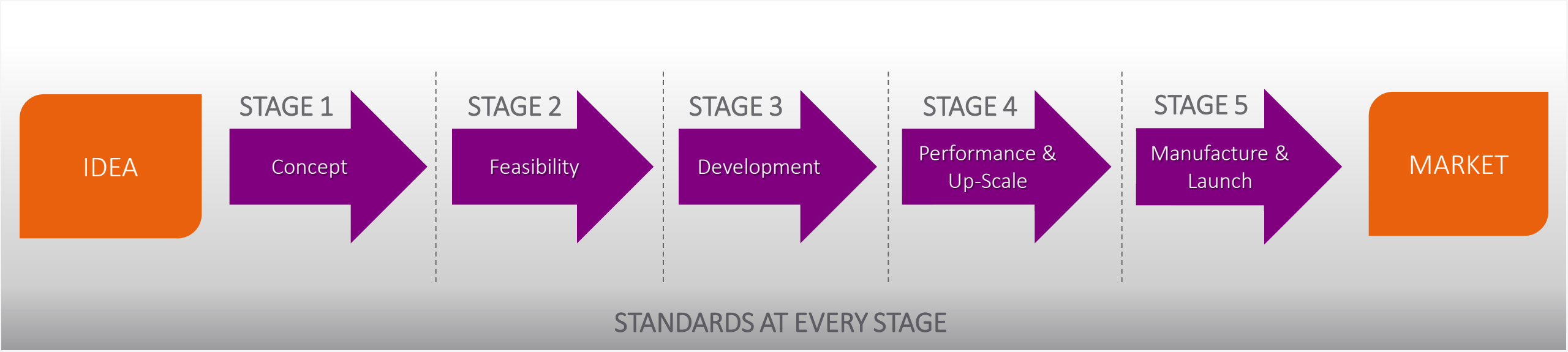
General agreement; not unanimity

Standard Reference Materials

- Fitness for Intended Use
- Reference to Specified Properties
- Homogeneity
- Stability

Standards Contribution to Product Development

Product Development Quality System



Benefits of Regenerative Medicine Therapy Standards

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

STANDARDS CAN:

- Reduce barriers to innovation
- Increase safety and reliability of therapies
- Facilitate more efficient regulatory review processes
- Decrease the cost of therapies
- Mitigate the risks to business or programs

Benefits of Standards: Industry Perspective

Claudia Zylberberg
CEO, Akron Biotech

"Solid standards are a sign of industry maturity. They will be instrumental in creating safe, efficacious, and consistent products."

- Ensure technical relevance of content
- Improve efficiency by eliminating redundancy
- Lower R&D costs by building upon existing technologies
- Increase trade by making cross-border interoperability possible
- Shorten time between concept and global availability
- Support other infrastructure (e.g., data interoperability, chain of custody, management systems)

Benefits of Standards: Regulatory Perspective

Judith Arcidiacono
International Regulatory Expert and Standards Liaison, Office of Tissue and Advanced Therapies, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration

"The use of standards by manufacturers and sponsors of regulatory applications for regenerative medicine therapies can contribute to more efficient evaluations of regulatory submissions to FDA."

- Sponsor's use of existing consensus standards can facilitate product development by reducing the need to develop unique methods or reference materials for individual products
- Establishes a common language for therapeutic areas through information models, concepts, and controlled terminologies
- Enhances the ability to perform complex analyses
- Builds a foundation for broader benefits to clinical research, premarket analysis, and safety signal detection

Benefits of Standards: Translational Researcher Perspective

Sumona Sarkar

Biomedical Engineer, National Institute of Standards and Technology

"Standards are pre-competitive resources that build upon the collective best practices and science of the field to help drive innovation efficiently to the consumer."

- Reduce the time to market by providing guidelines and considerations for critical aspects of product manufacturing and quality control
- Facilitate communications between operating units as well as with regulators and stakeholders by establishing common understanding of analytical methods and product characterization
- Lower barriers for interacting with key service providers, including ancillary materials providers, transportation providers, tissue/cell source providers, and equipment providers

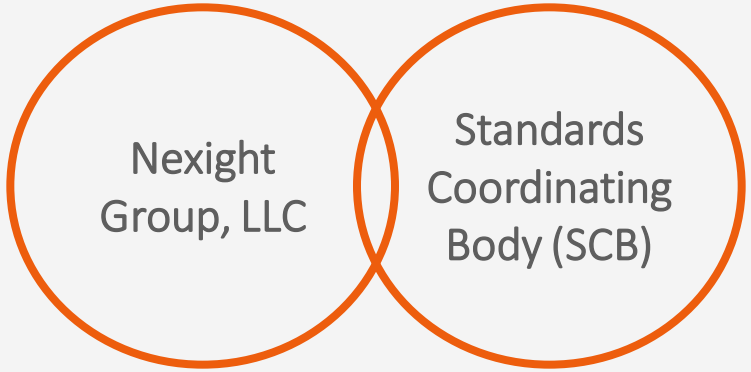
Supporting the Standards Development Process

Dawn Henke
Technical Program Manager, The Standards Coordinating Body

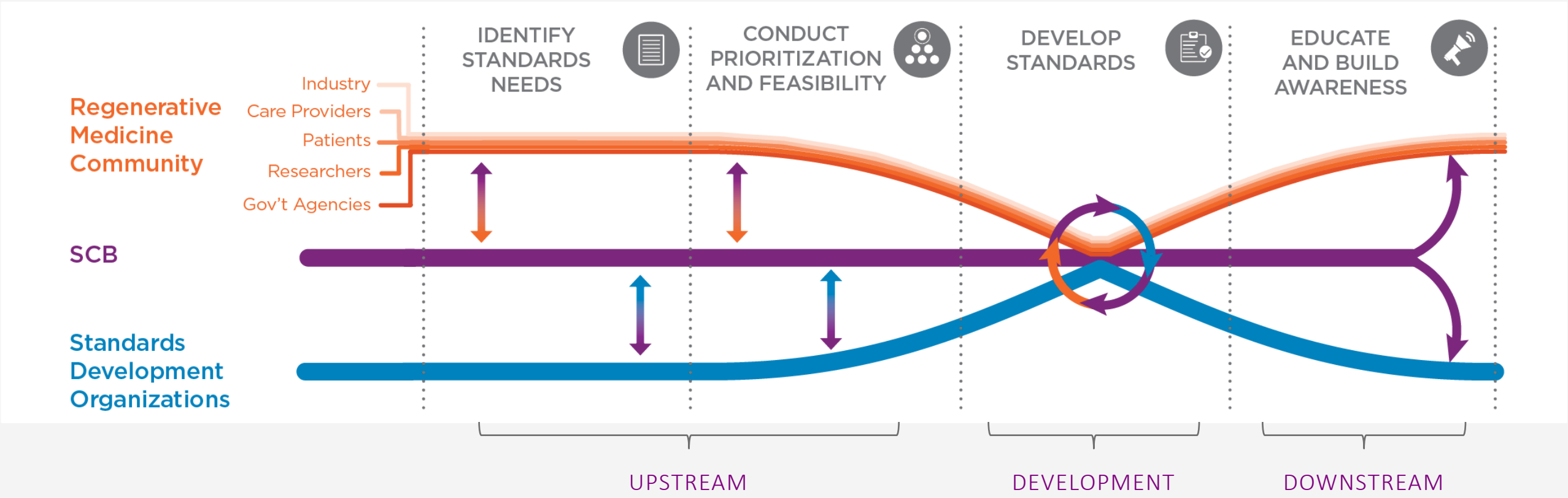
21st Century Cures Act

FDA, in consultation with NIST and stakeholders, will facilitate an effort to coordinate and prioritize the development of standards for regenerative medicine therapies.

SEP 2017 ◆——————————◆ SEP 2018



Connecting the Regenerative Medicine Community to the Standards Development Process



Current State: Standards Development Processes

Documentary Standards Development Process Steps	Reference Materials Development Process Steps
STEP 1: Identify needed standards	STEP 1: Identify needed reference material
STEP 2: Submit concept to SDO	STEP 2: Submit concept to SDO
STEP 3: SDO reviews concept and drafts initial standard	STEP 3: SDO reviews concept and prepares initial material
STEP 4: Obtain additional stakeholder inputs and supporting data	STEP 4: Conduct scientific studies and review final reference material
STEP 5: Publish finalized standard	STEP 5: Develop and distribute reference materials
STEP 6: Revise standards as needed	STEP 6: Withdraw reference materials

Proposed Support from SCB to Complement Existing Processes

Upstream	STEP 1.	Identify needed standards
	STEP 2.	Prioritize needed standards
	STEP 3.	Conduct feasibility assessment
	STEP 4.	Develop a plan and advance potential standards
Standards Development	STEP 5.	Support standards development and coordination
Downstream	STEP 6.	Educate and build awareness of standards

Step 1: Identify Needed Standards

WHAT IS THE PURPOSE OF THIS STEP?

- Identify existing standards and gaps across current standards
- Minimize redundancy across community efforts and those of SDOs
- Help assess the maturity of regenerative medicine therapies space
- Inform needs for science and technology development or policy decisions

Step 1: Identify Needed Standards

WHAT RESOURCES ARE/WILL BE AVAILABLE?

- *Regenerative Medicine Standards Landscape Report* (available today!)
- Real-time, web-accessible, and searchable landscape of existing standards, broken down by sector and application area
- Case studies of current standards use or development that demonstrate the value of standards in the field of regenerative medicine
- Annual report on areas of needed standards

HOW CAN YOU ENGAGE?

- Provide feedback on the *Regenerative Medicine Standards Landscape Report*
- Participate in an annual survey on existing standards and needs
- Attend SCB-hosted workshops and teleconferences with SCB sector groups
- Provide ongoing feedback on what standards you are using or what gaps are affecting your work

Step 2: Prioritize Needed Standards

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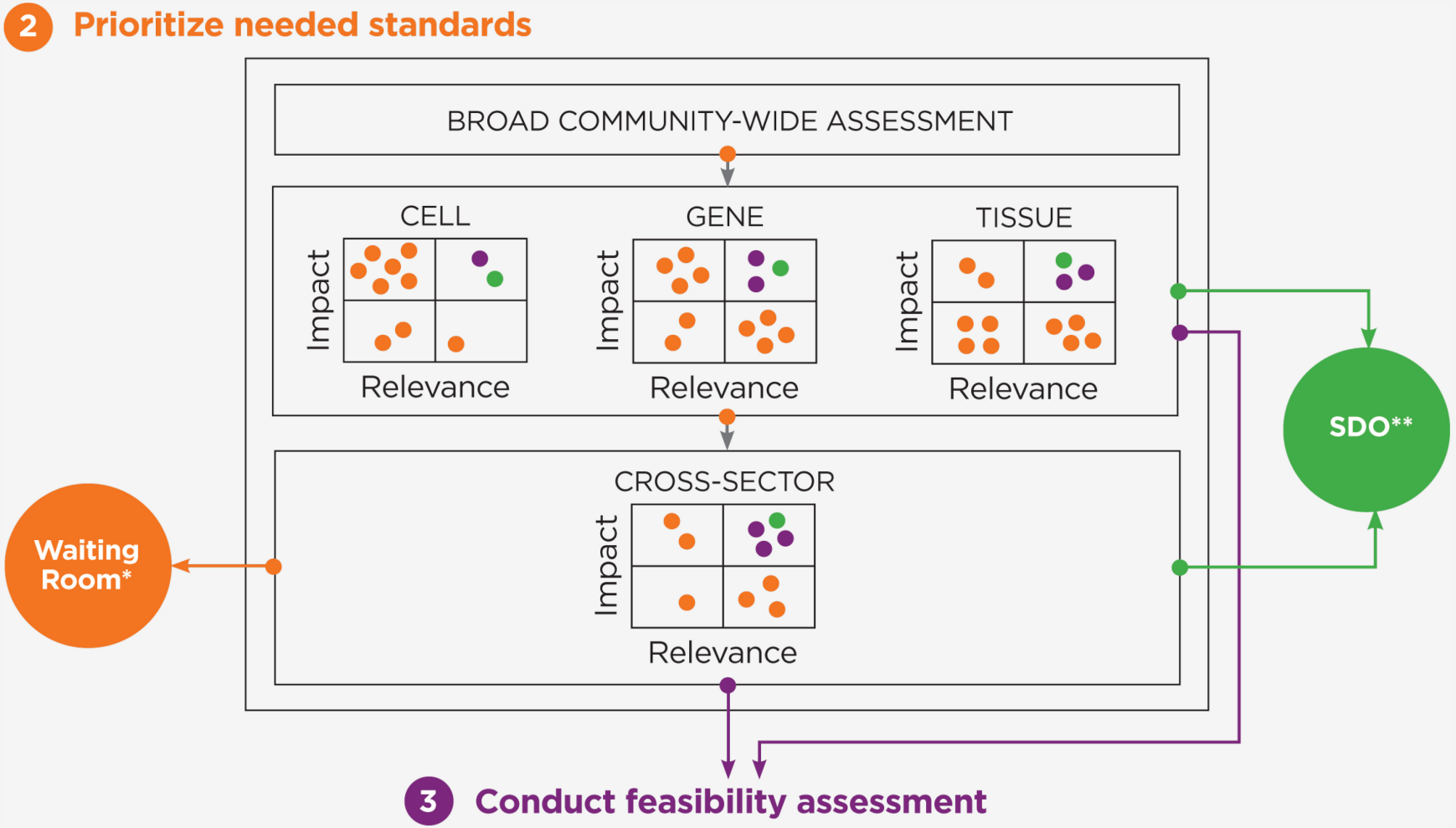
WHAT IS THE PURPOSE OF THIS STEP?

- Prioritize the advancement of potential standards that are most relevant, timely, and can have the greatest impact
- Facilitate early identification of potential barriers or challenges
- Inform decisions on the use of time and resources to advance potential standards
- Identify potential standards that can immediately begin standards development

Step 2: Prioritize Needed Standards



***Waiting Room**: Concept is not yet ready for standards development; it is kept in a tracking system and evaluated periodically for reconsideration.

****SDO**: Potential standards are specific/robust and have a champion identified; can go directly to an SDO.

Step 2: Prioritize Needed Standards

1

WHAT RESOURCES ARE/WILL BE AVAILABLE?

- Report, made publicly available, on Needed Standards in Regenerative Medicine
- Short summary of each prioritized needed standard

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HOW CAN YOU ENGAGE?

- Participate in SCB-hosted workshops and teleconferences with SCB sector groups
- Participate in an annual survey to prioritize needed standards

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Step 3: Conduct Feasibility Assessment

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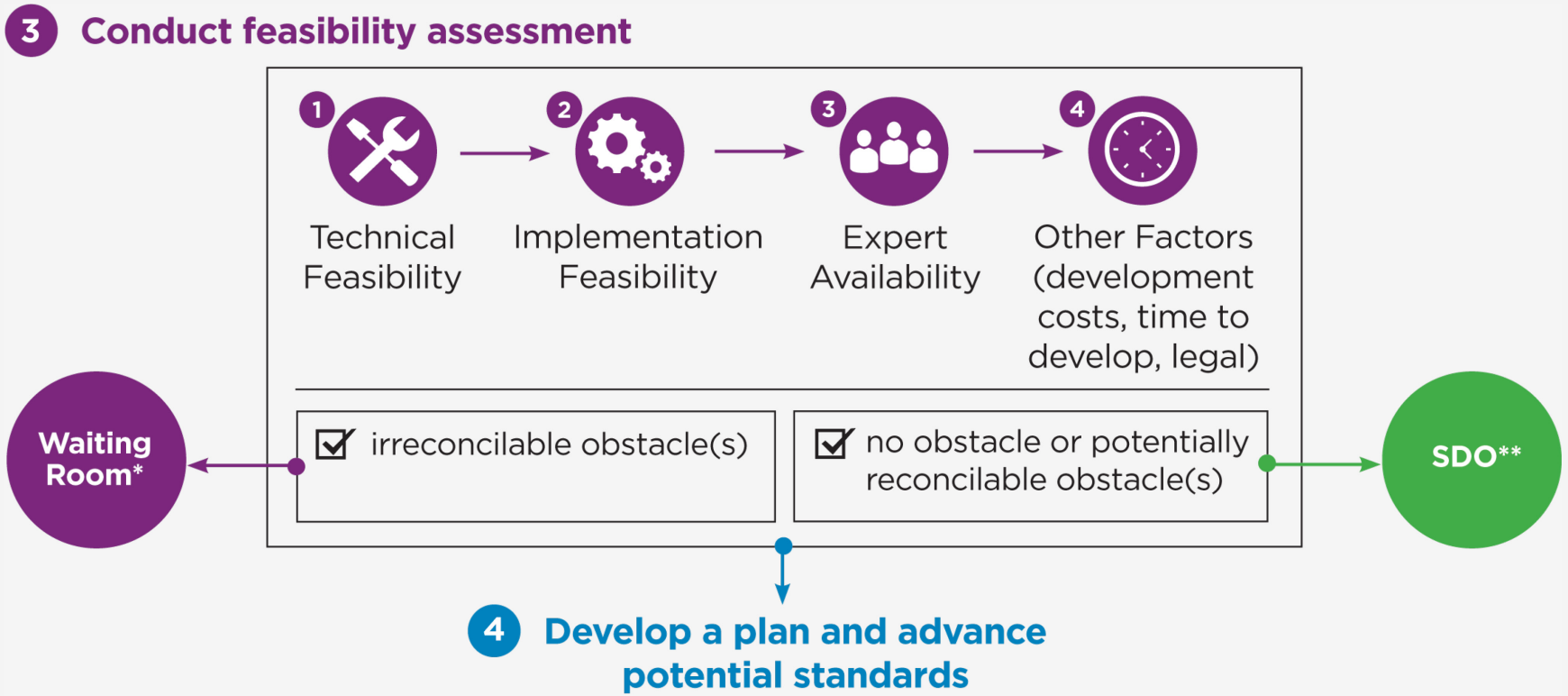
WHAT IS THE PURPOSE OF THIS STEP?

- Evaluate the practical viability of a potential standard based on its scientific maturity, projected economic impact, and other relevant factors that affect its development or eventual adoption
- Determine whether the standard represents a worthwhile endeavor despite potential barriers or impediments
- Facilitate more efficient use of resources, support identification of experts and champions
- Identify potential standards that can immediately begin standards development

Step 3: Conduct Feasibility Assessment



*Waiting Room: Concept is not yet ready for standards development; it is kept in a tracking system and evaluated periodically for reconsideration.

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Step 3: Conduct Feasibility Assessment

1

WHAT RESOURCES ARE/WILL BE AVAILABLE?

- 1-10 page summary of each of the prioritized needed standards
- Summary descriptions of the value proposition for the prioritized needed standards

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HOW CAN YOU ENGAGE?

- Participate in electronic surveys
- Participate in SCB work group meetings and workshops
- Review newsletters that include results of feasibility assessments

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Step 4: Develop a Plan and Advance Potential Standards

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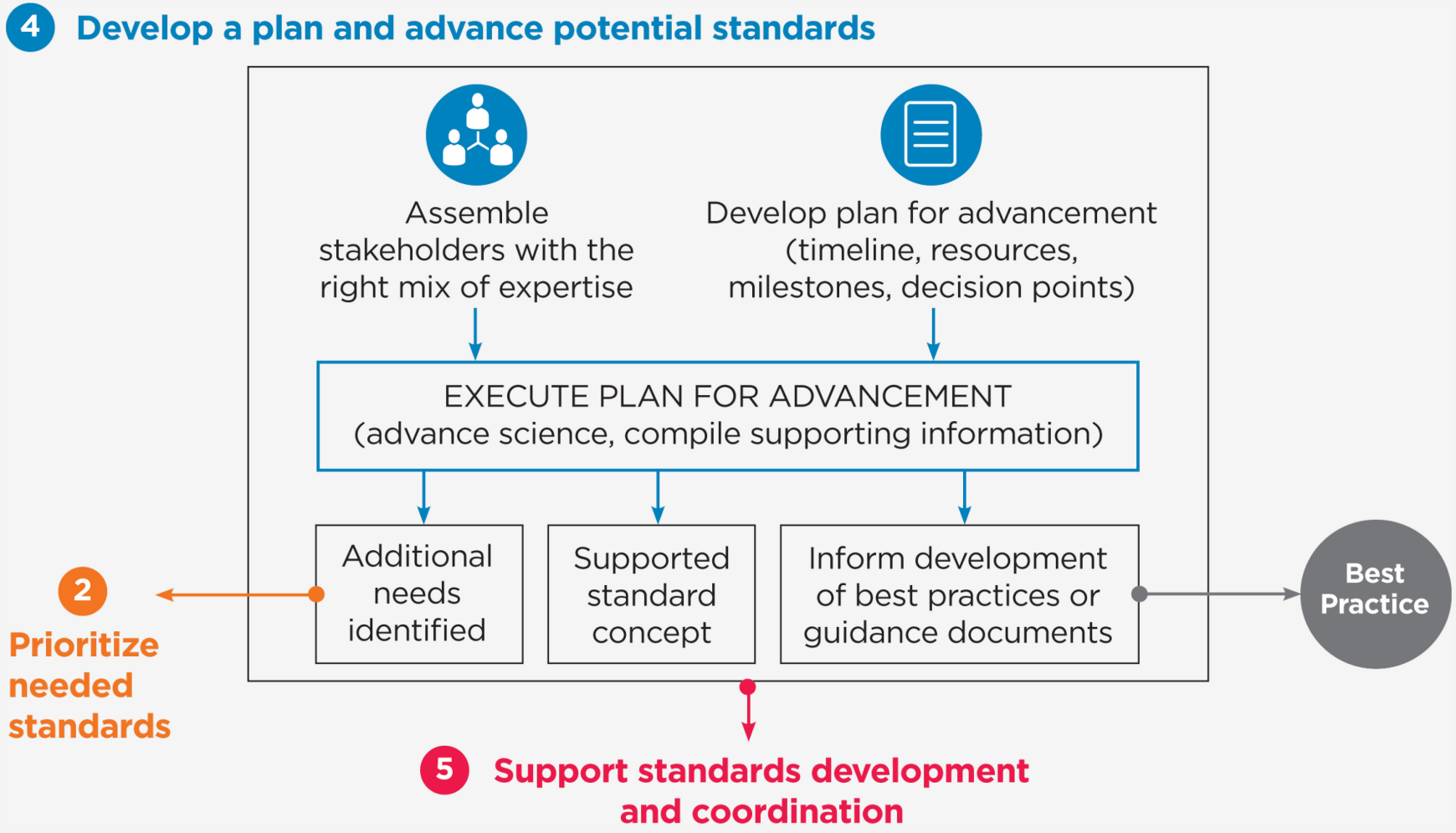
WHAT IS THE PURPOSE OF THIS STEP?

- Clearly articulate the relevance and impact of the potential standard, developing additional supporting evidence and information as needed
- Establish a plan and timeline for obtaining needed supporting information to advance a potential standard
- Leverage the expertise and funding available to drive progress in advancing a potential standard

Step 4: Develop a Plan and Advance Potential Standards



Step 4: Develop a Plan and Advance Potential Standards

1 2 3 4 5 6

WHAT RESOURCES ARE/WILL BE AVAILABLE?

- 1-10 page summary of each potential standard being advanced
- White papers, best practices, or guidance documents
- Documented potential standard advancement plan for each existing potential standard

HOW CAN YOU ENGAGE?

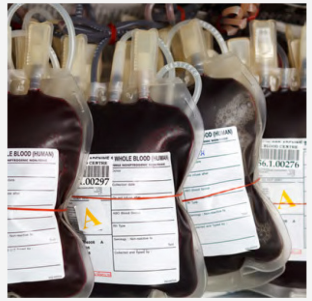
- Participate in SCB facilitated teleconferences with working group
- Join and participate in SDO technical committees/working groups (e.g., ASTM, ISO)
- Contribute to the ongoing SCB projects listed on the SCB website

Standards Development In Action

RAPID MICROBIAL TEST METHODS

- SCB, NIST, BioFabUSA, and NIIMBL hosted an RMTM workshop on April 10, 2018
- From this workshop, SCB is currently leading the development of a white paper (expected mid 2018) that will drive RMTM standards development
- SCB is also leading efforts toward the development of two standards (ISO and ASTM) based of the output of the workshop

CELL TRANSPORTATION



- SCB is working with U.S. and international experts to address cell transportation challenges from the perspectives of both cell therapy product suppliers and transportation/ logistics companies
- These efforts are ongoing in the ISO/TC276 group

Step 5: Support Standards Development and Coordination

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WHAT IS THE PURPOSE OF THIS STEP?

- Assemble the required expertise, stakeholders, and support
- Link efforts across SDOs, reducing duplication
- Communicate standards development progress to the broad regenerative medicine community

Step 5: Support Standards Development and Coordination

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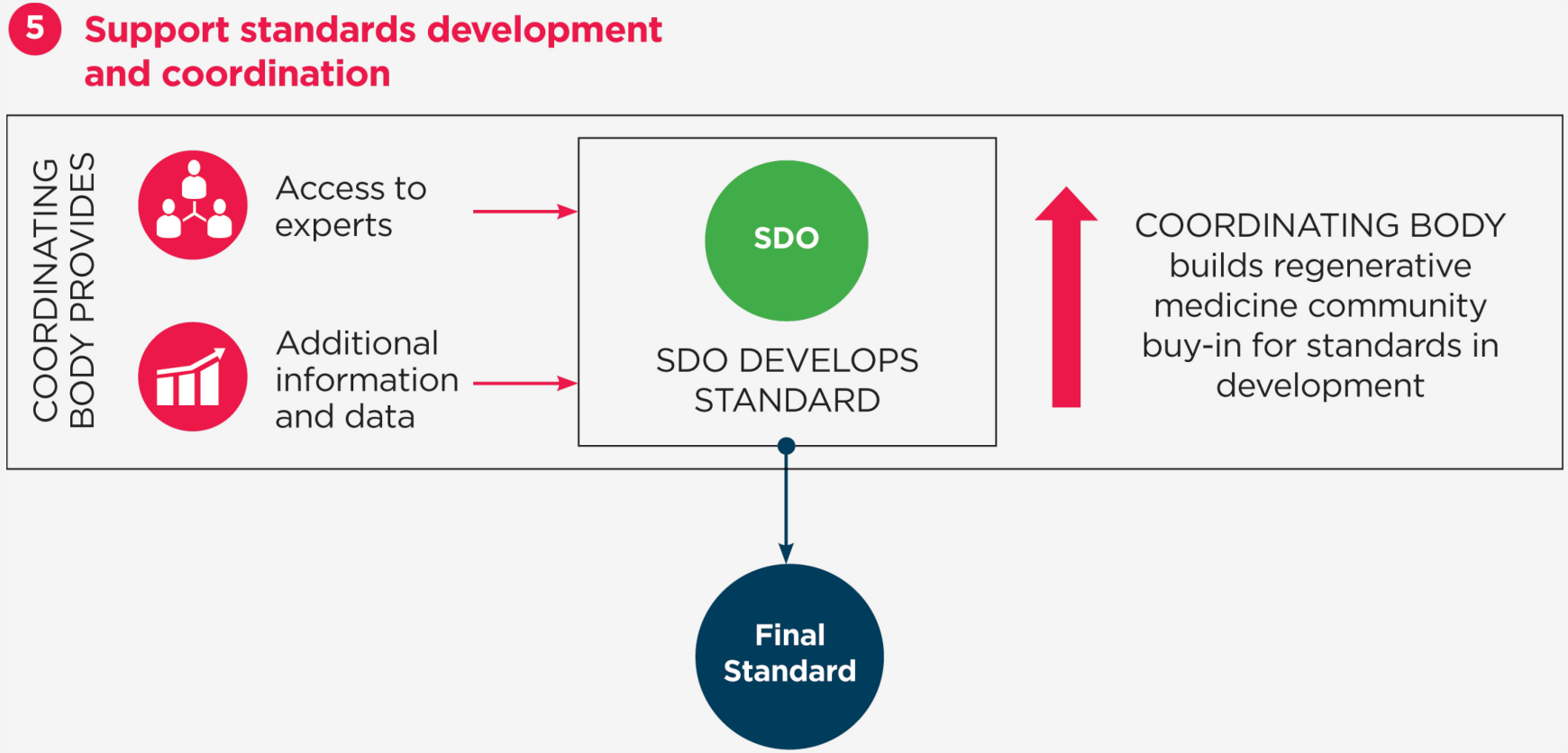


Step 5: Support Standards Development and Coordination

1 2 3 4 5 6

WHAT RESOURCES ARE/WILL BE AVAILABLE:

- Monthly newsletter
- Updates to *Landscape of Regenerative Medicine Standards*
- Published standards available to regenerative medicine community

HOW CAN YOU ENGAGE?

- Attend regular meetings with SDOs to stay updated on standards development progress and identify gaps in expertise or stakeholder engagement
- Participate in SCB work group meetings and workshops
- Join and participate in SDO technical committees/working groups (e.g., ASTM, ISO)

Step 6: Educate and Build Awareness of Standards

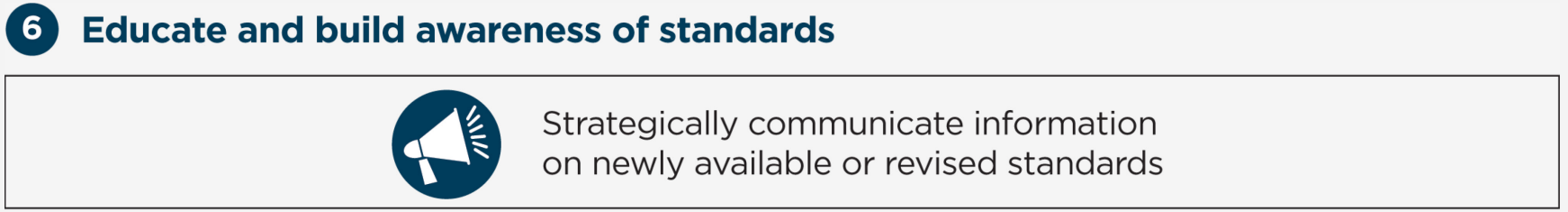
1 2 3 4 5 6

WHAT IS THE PURPOSE OF THIS STEP?

- Adoption of standards spurs innovation, enables efficient use of resources, and accelerates development and availability of therapies
- Inform training, certification, and curricula

HOW CAN YOU ENGAGE? / WHAT RESOURCES ARE AVAILABLE?

- Participate in webinars
- Contribute to blog posts and website content
- Develop case studies
- Participate in annual conference
- Present at regenerative medicine community events



Questions and Answers

Webinar Conclusion

- You learned about how standards can benefit you or your organization.
- You are aware of the Standards Coordinating Body's efforts to support standards development.
- You are aware of resources to learn more about identifying or advancing needed standards.
- You know how you can engage in advancing and developing standards.

How to Stay Engaged?

View/Attend our
WEBINARS

Sign up at
SCB Webinars

Get Involved in the
PROCESS

Check out the ongoing project list on the SCB website

Sign up with
Dawn Henke

Provide input on
NEEDED STANDARDS

View at
SCB Publications

Attend a 2018
WORKSHOP

July 11 CASSS CGTP Symposium
August 10 Characterization of Fiber-Based Scaffolds

Sign up with
Allison Getz

Sign up for regular communications about this work: agetz@regenmedscb.org

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Thank you!