USP Today: Standards for Biopharmaceutical Innovation

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Biologics Stakeholders Forum
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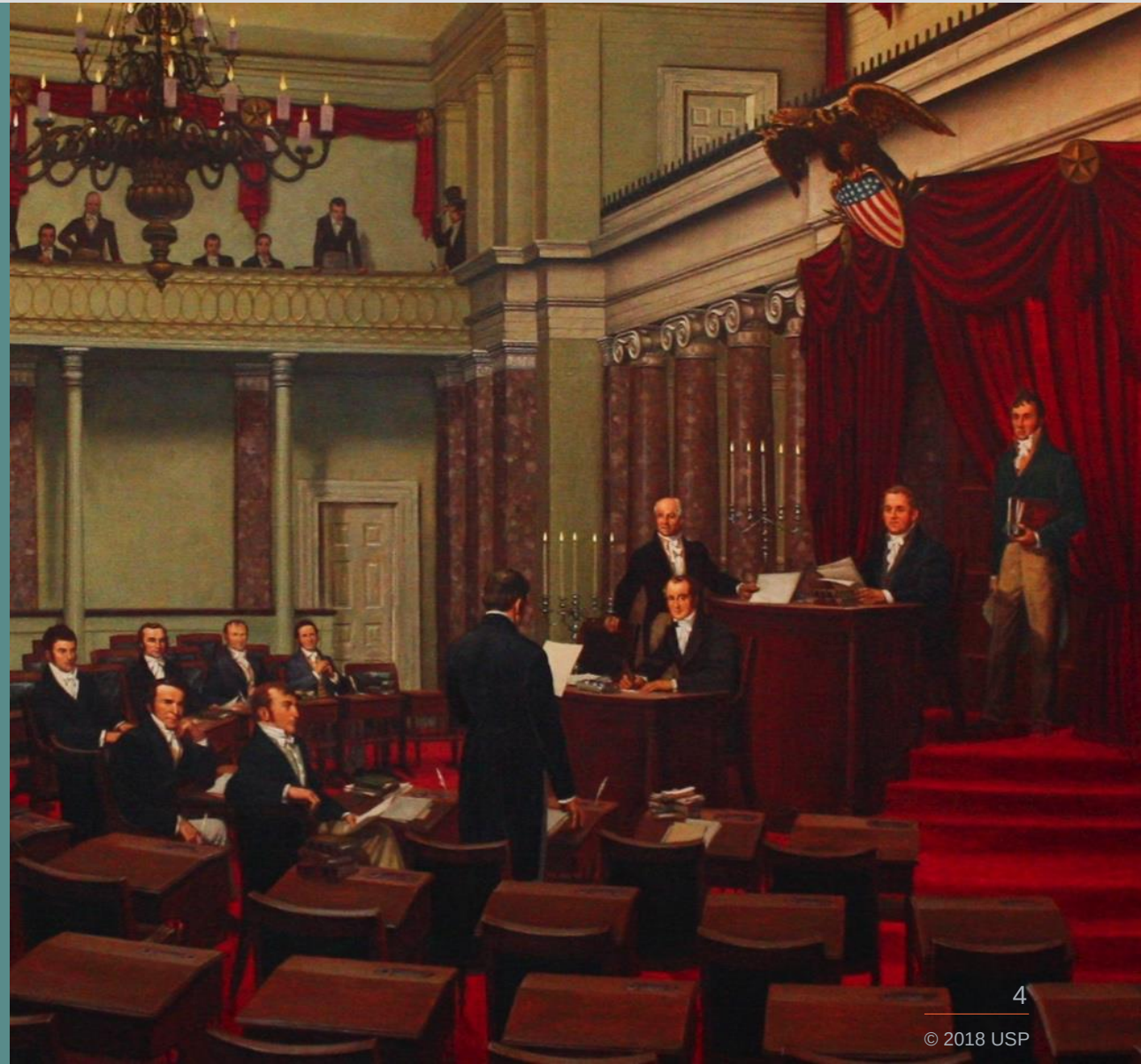
Overview

- Celebrating 200 years of USP
- Engagement with regulators worldwide
- Accelerating adoption of new innovations in cell and gene therapy

1 Celebrating 200 years of USP

Celebrating 200 years since our founding in 1820

“The value of the Pharmacopoeia depends upon the fidelity with which it conforms to the best state of medical knowledge of the day.”
- *Jacob Bigelow, MD, 1808*

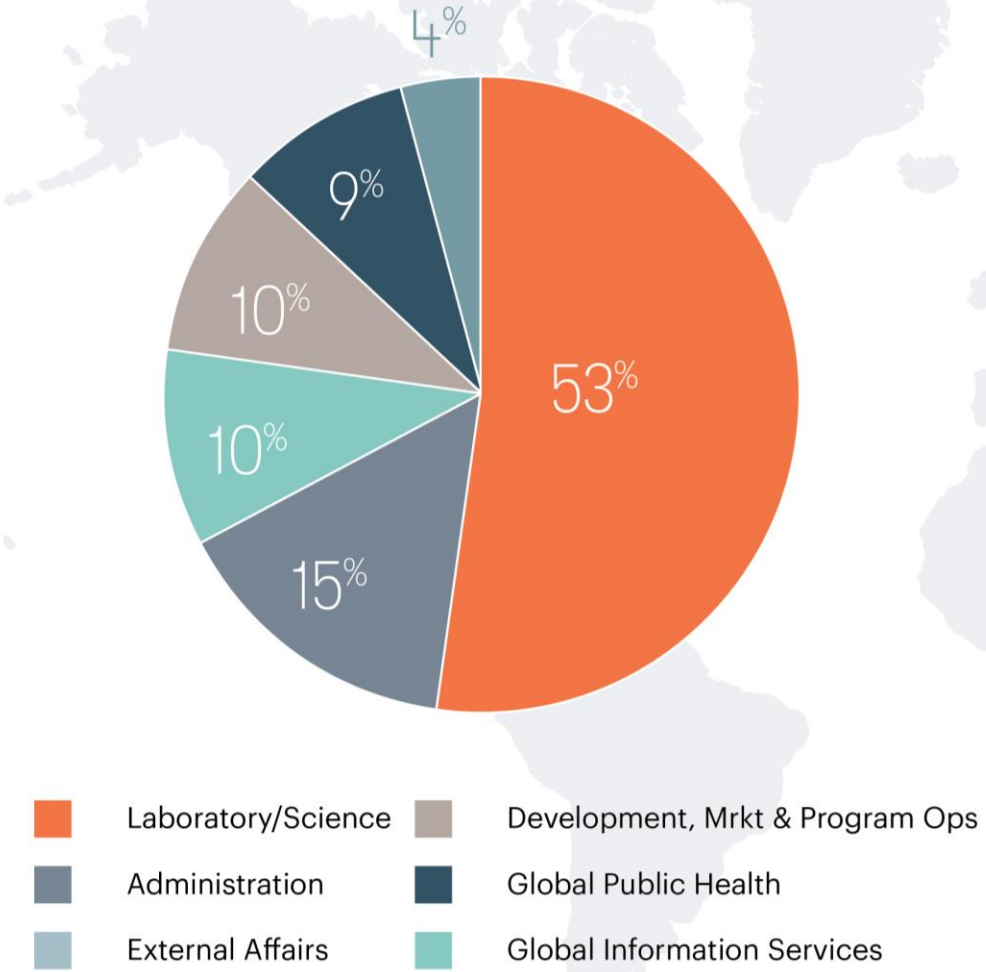


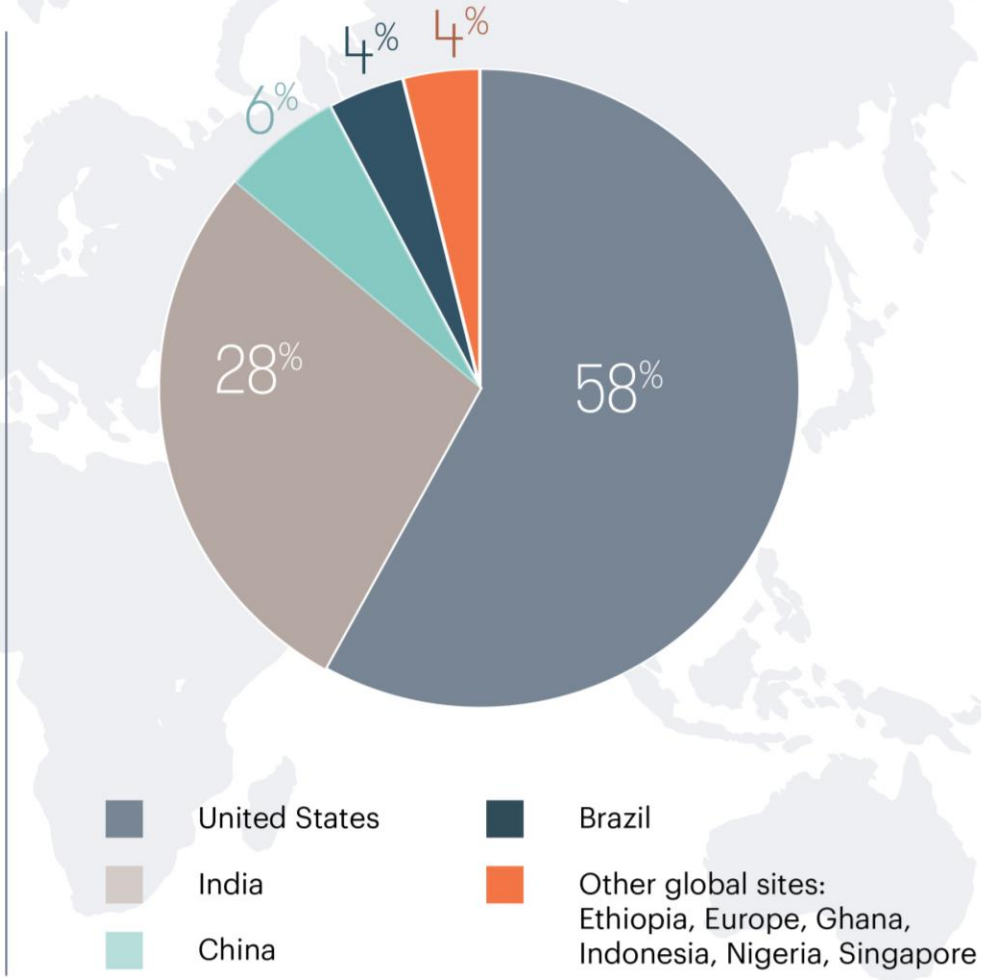
Mission

To improve global health through public standards and related programs that help ensure the quality, safety and benefit of medicines and foods

Global staff presence

1200+ Staff





Public quality standards benefit public health

Industry

Reduce risk to manufacturers in entering the marketplace

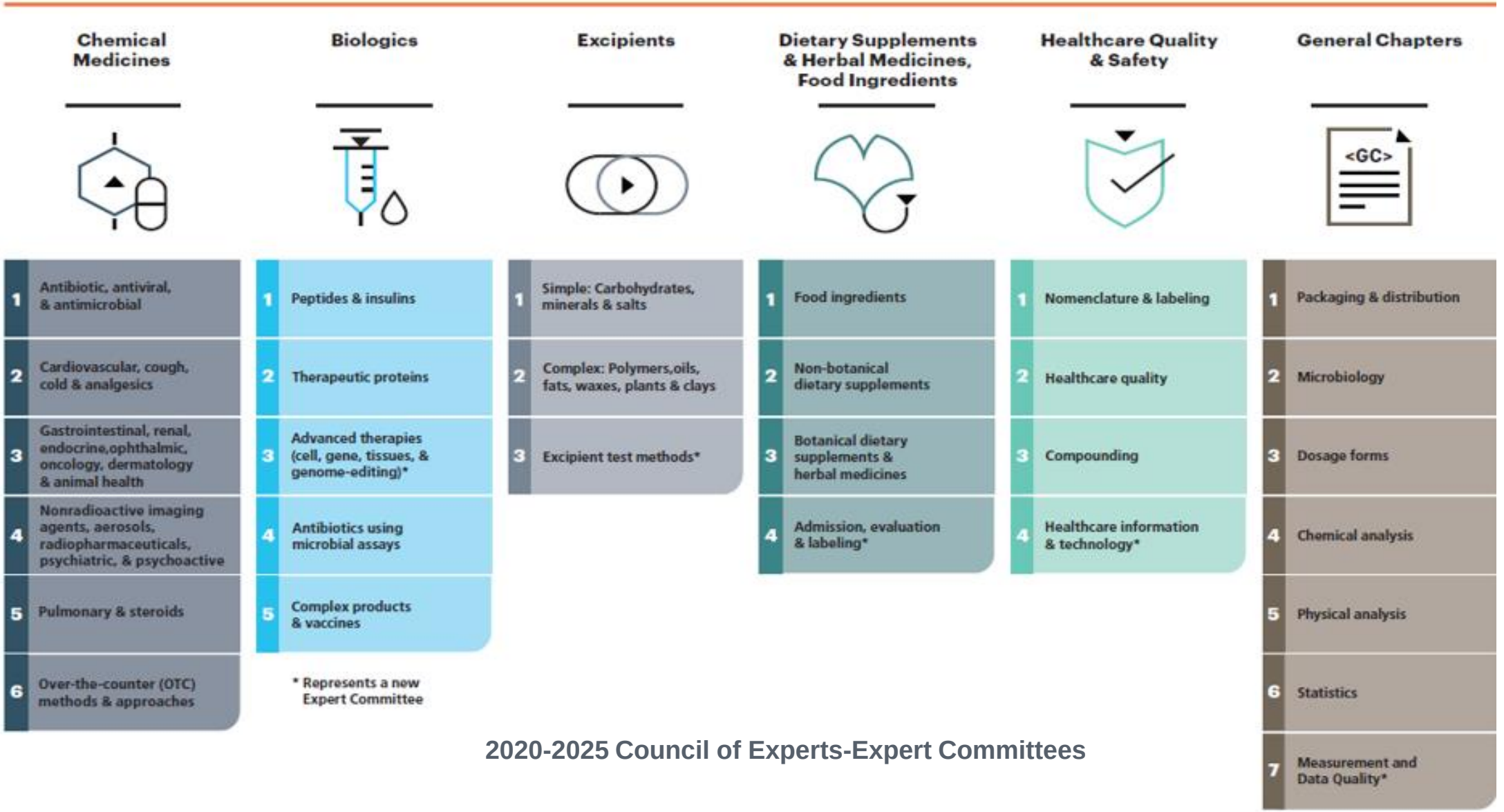
Practitioner/Patient

Uphold practitioner and patient confidence in medication therapy

Government

Regulators ensure quality medicines reach patients

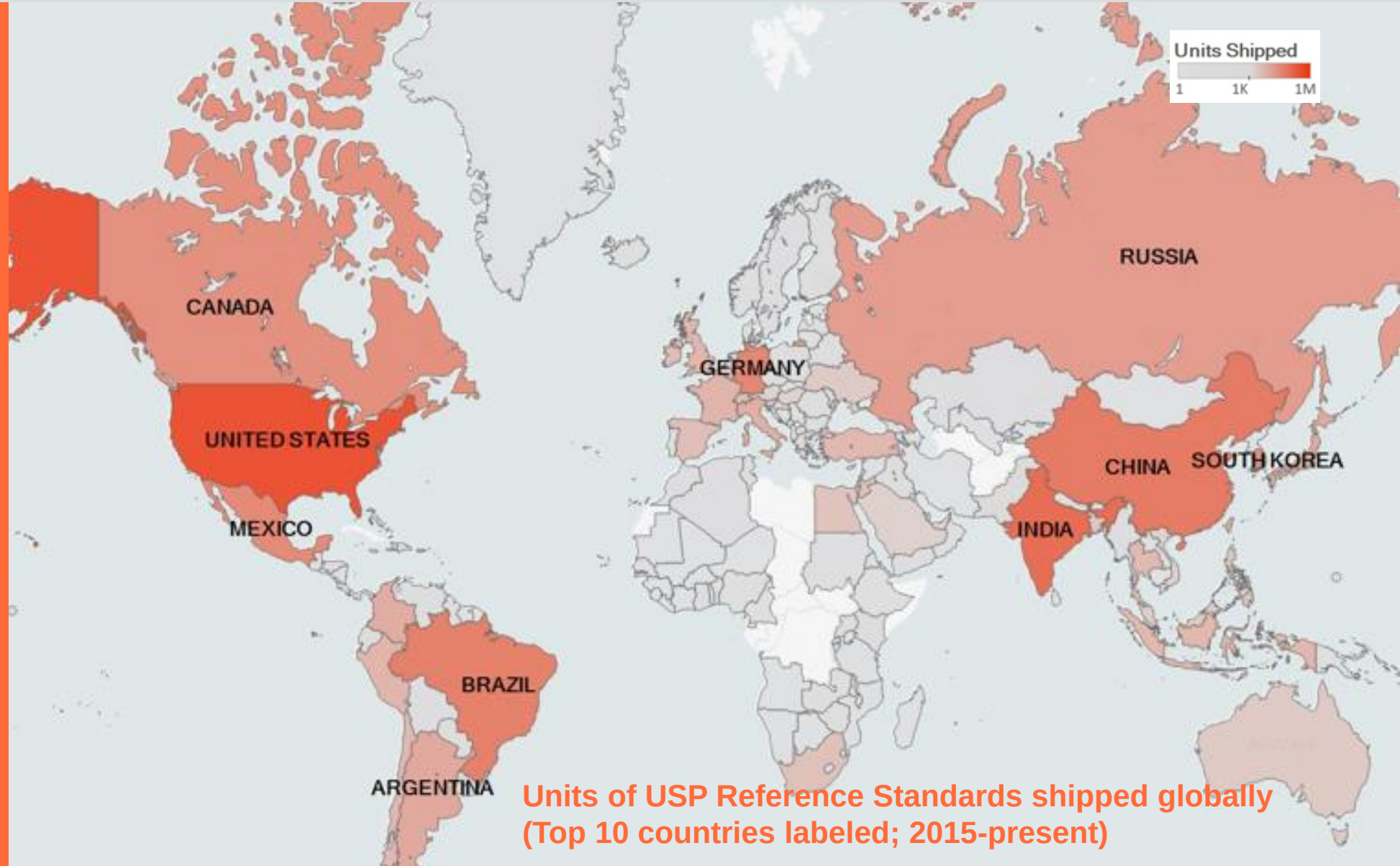
The breadth of USP's work can be seen through the scope of our expert committees



2020-2025 Council of Experts-Expert Committees

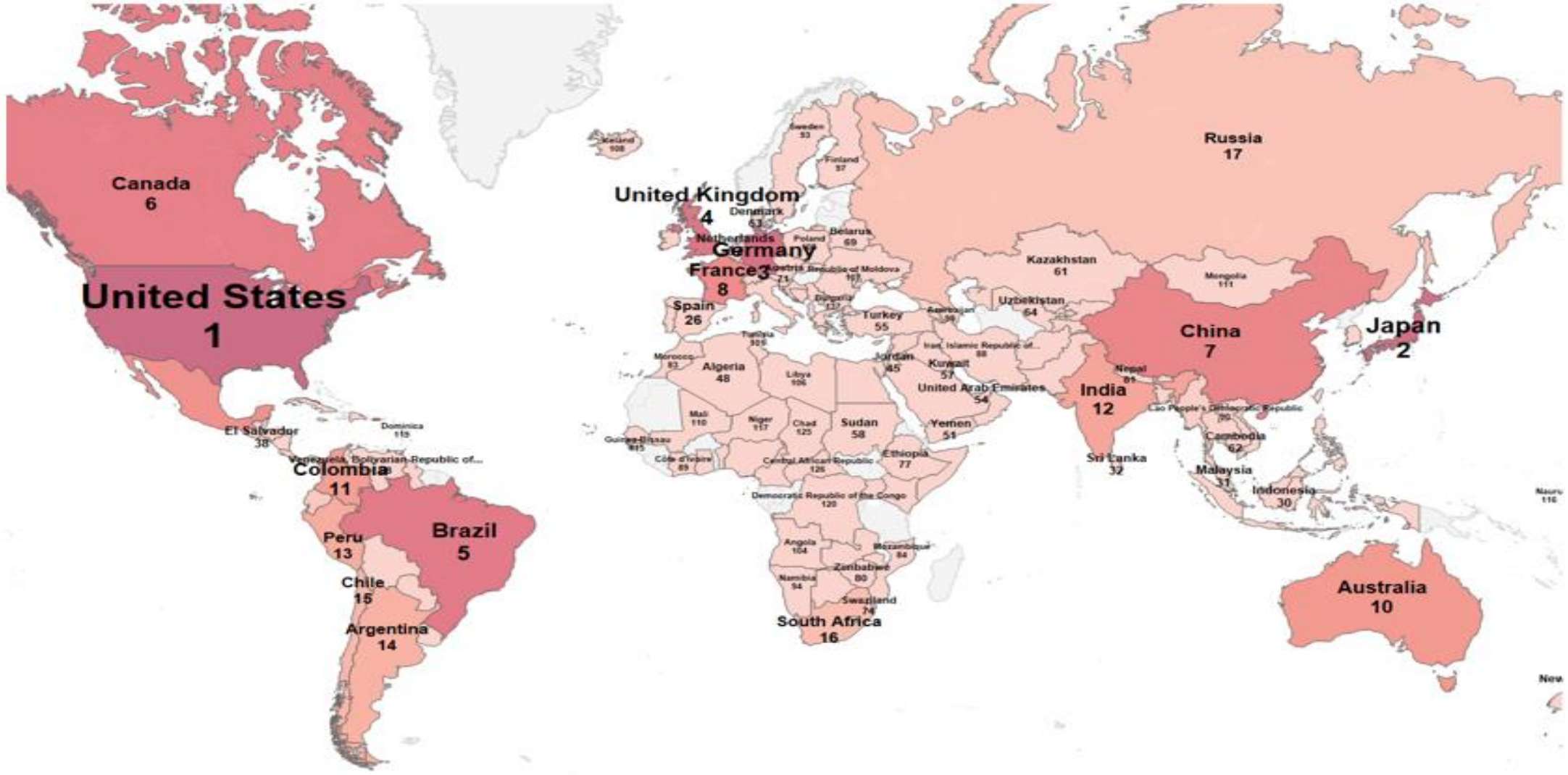
Global usage of USP reference standards

USP Standards were shipped to over 22,000 manufacturers in FY19



Units of USP Reference Standards shipped globally
(Top 10 countries labeled; 2015-present)

Export markets for manufacturers that use USP's reference standards



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T15 You noted that you produce products for export markets. Please indicate your top 3 export markets. (n-2019=1,108)

2 Engagement with regulators worldwide

Engaging with regulators to ensure quality of medicines worldwide

- Usage of USP standards in over 150 countries
- Our partnership with U.S. FDA
  - Dating back to 1906 Pure Food and Drug Act
- Meeting with regulators through direct engagement and regional platforms

Regulatory partners around the world

BRAZIL

National Health Surveillance Agency (ANVISA) *Signed June 2016*

CHINA

Chinese Pharmacopoeia Commission (ChP) *Renewed October 2016*

INDIA

Indian Pharmacopoeia Commission (IPC) *Renewed March 2017*

INDIA

National Institute of Pharmaceutical Education and Research – Hyderabad (NIPER) *Signed October 2016*

JAPAN

Ministry of Health, Labour & Welfare, Pharmaceuticals & Medical Devices Agency (MHLW/PMDA) *Signed September 2016*

MEXICO

Permanent Commission of the Pharmacopeia of the United Mexican States, Fed. Commission for the Protection Against Sanitary Risks (FEUM/COFEPRIS) *Renewing March 2018*

LATIN AMERICA & CARIBBEAN

Pan American Health Organization (PAHO) *Renewed June 2017*

RUSSIA

Federal Service on Surveillance in Healthcare (ROSZDRAVNADZOR) *Renewed June 2015*

SAUDI ARABIA

Saudi Food & Drug Authority (SFDA) *Signed September 2015*

SOUTH KOREA

National Institute of Food & Drug Safety Evaluation (NFIDS) *Renewed April 2015*

WHO WORLD MEETING OF PHARMACOPEIAS

Global & regional platforms driving harmonization

Official Relations Framework for Engagement of Non-State Actors (FENSA)

NGO Consultative Status
United Nations Economic & Social Council

Observer Status
International Council for Harmonisation

APEC Life Sciences Innovation Forum
Board Member; Center of Regulatory Excellence

African Medicines Quality Forum
Reference Center of Regulatory Excellence (RefCORE)

Official Observer Status
Non-State Actor

Advocate for medicines quality issues in global policies
Consultations at different levels of governments

Regional platform: APEC

- 19 out of 21 APEC economies use USP standards
- USP-APEC *Center of Excellence* designation in 2017- focus on supply chain
- New *Center of Excellence* in 2020 with focus on advanced therapies
- Regional approach to train and implement quality standards
  - Convening US and international regulators, and industry on training to GMP, GDP, and screening technology best practices identified by APEC community

Regulatory activities in China through USP-China's 11-year history

- Regular dialogue with senior officials from NMPA, ChP and NIFDC, and key industry stakeholders
- Advocate for strengthened global collaborations to advance medicine quality
- Held closed-door meeting with ChP, discussing future strategic collaboration direction
- Continued dialogue with ChP, NMPA

Examples of our regulatory engagement

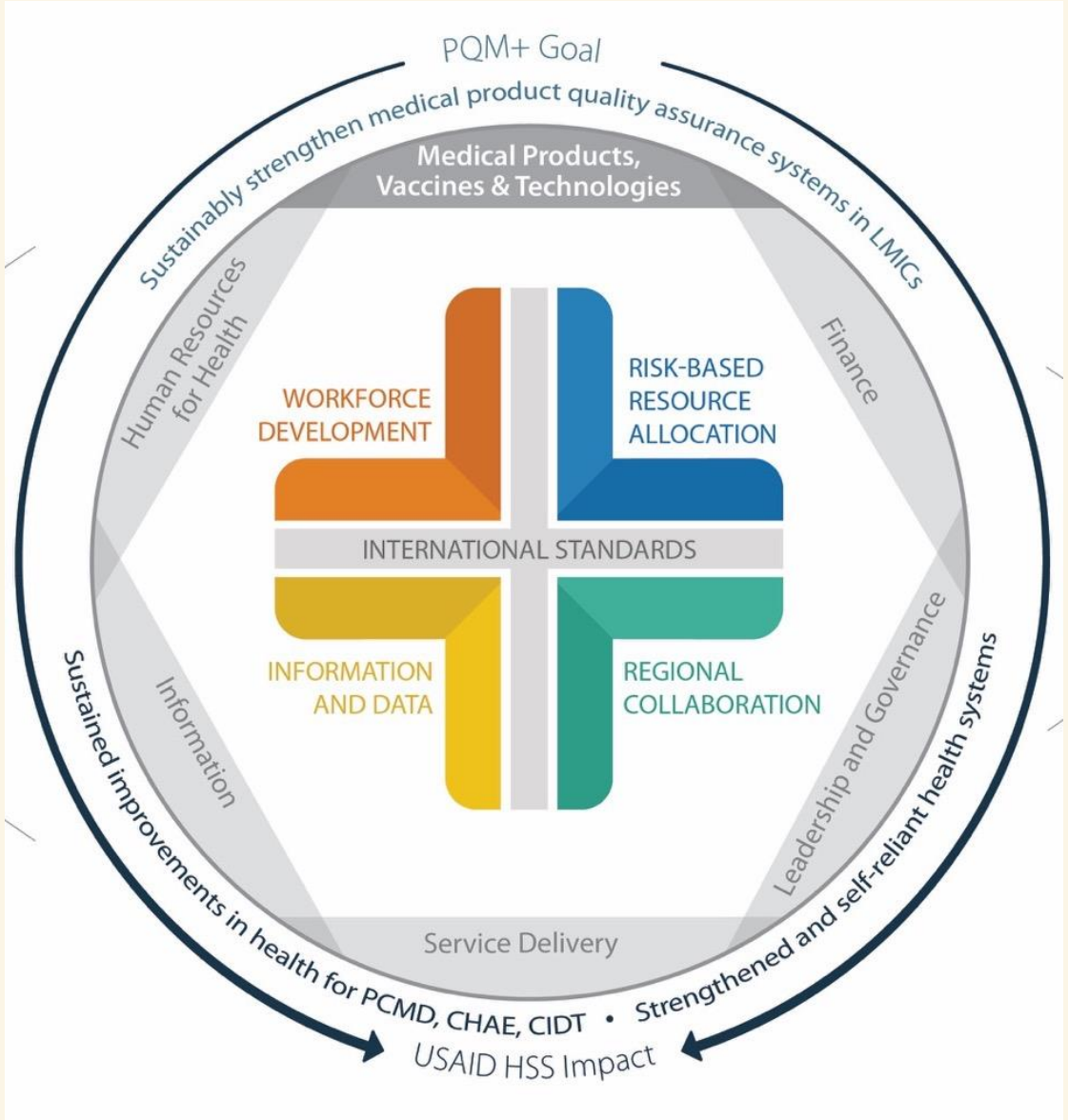
- Brazil
  - Since 2016, strengthened regulatory cooperation with ANVISA under ongoing MoU
  - Contributed to ANVISA's proposal of DS regulation
  - Deeper engagement with Brazilian Pharmacopoeia through roundtables, stakeholder forums, and joint education
- Korea
  - Renewed collaboration between USP and Korea's drug regulator NIFDS with special focus on improving quality standards for biologic medicines for Korea and internationally
    - Joint roundtable discussions on quality of biologics
    - Scientific visitor exchange program
- Mexico
  - Signed an MoU with Mexico Pharmacopeia to strengthen quality standards and patient safety

USP | COFEPRIS Meeting, Mexico

USP | ANVISA Meeting

Strengthening medical product quality assurance regulatory systems in low-middle income countries

- 25+ year history with USAID
  - 10 year Promoting the Quality of Medicines (PQM) program
  - PQM+ work commenced October 2019
    - $160 Million cooperative agreement over five years
- Ensuring robust quality medicine through
  - Risk-based resource allocation
  - Information and data
  - Regional collaboration
  - Workforce development



3

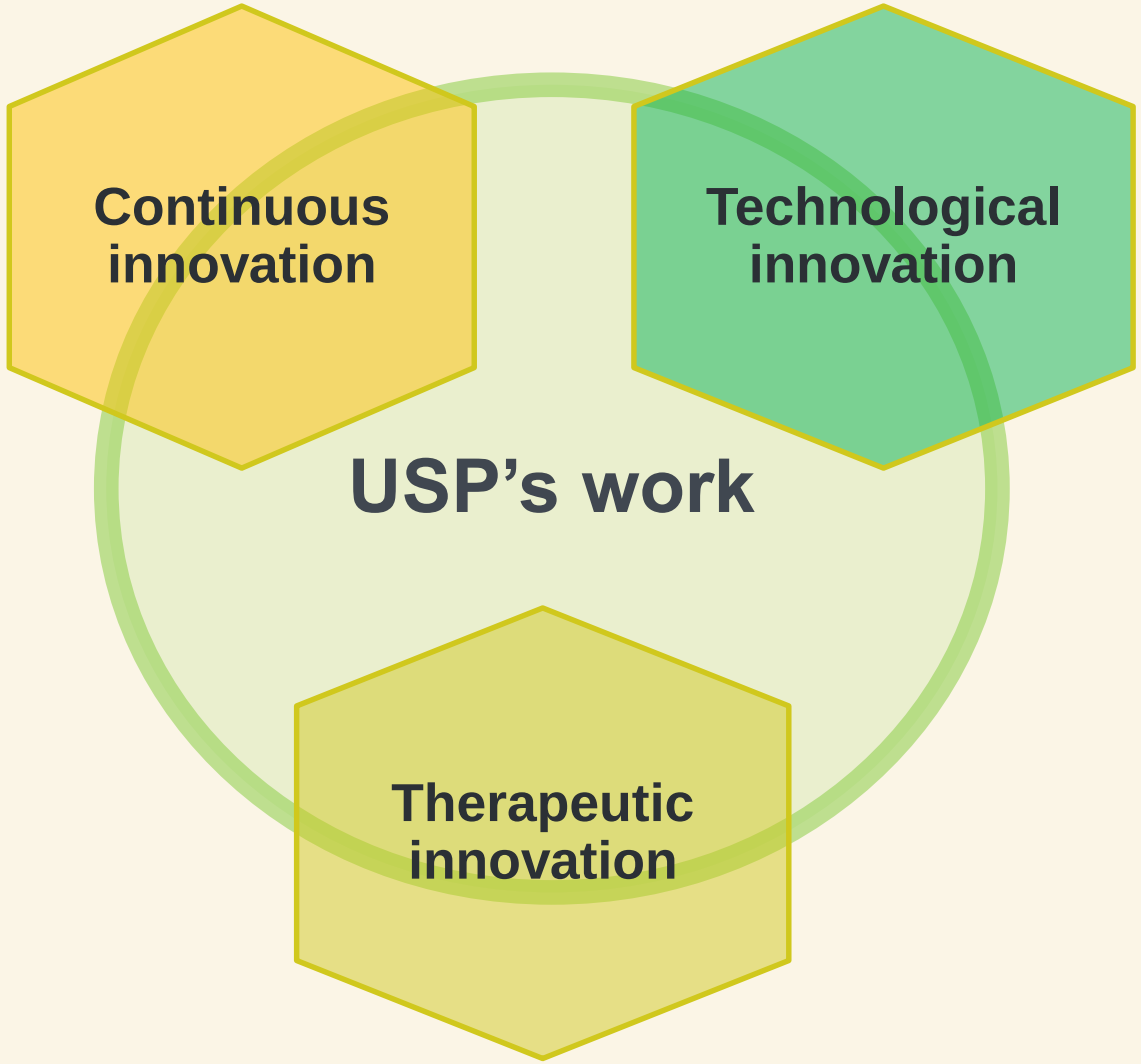
Accelerating adoption of new innovations in cell and gene therapy

Two centuries of accelerating innovation

- **Reduce barriers to innovation** with Up-To-Date trusted, tested public standards and methods
- **Accelerate the uptake of innovative technologies** via partnerships and collaboration for their timely adoption
- **Facilitate the spread of innovation** by engaging internationally through programs and trainings to grow capabilities on standards, quality, and regulatory topics
  - USP's standards are used in over 150 countries
  - Drug/medical device combinations
  - Biologics performance standards

How USP supports innovation

- Continuous innovation
  - Evaluating state of the industry practices to update our standards and respond to emerging needs
- Technological innovation
  - We assess innovative technologies for suitability and readiness for use by stakeholders, reducing risk to industry.
- Therapeutic innovation
  - We proactively work with the dynamic pace of healthcare by facilitating continued modernization of standards for legacy products



Pharmaceutical Continuous Manufacturing

USP's role in promoting quality

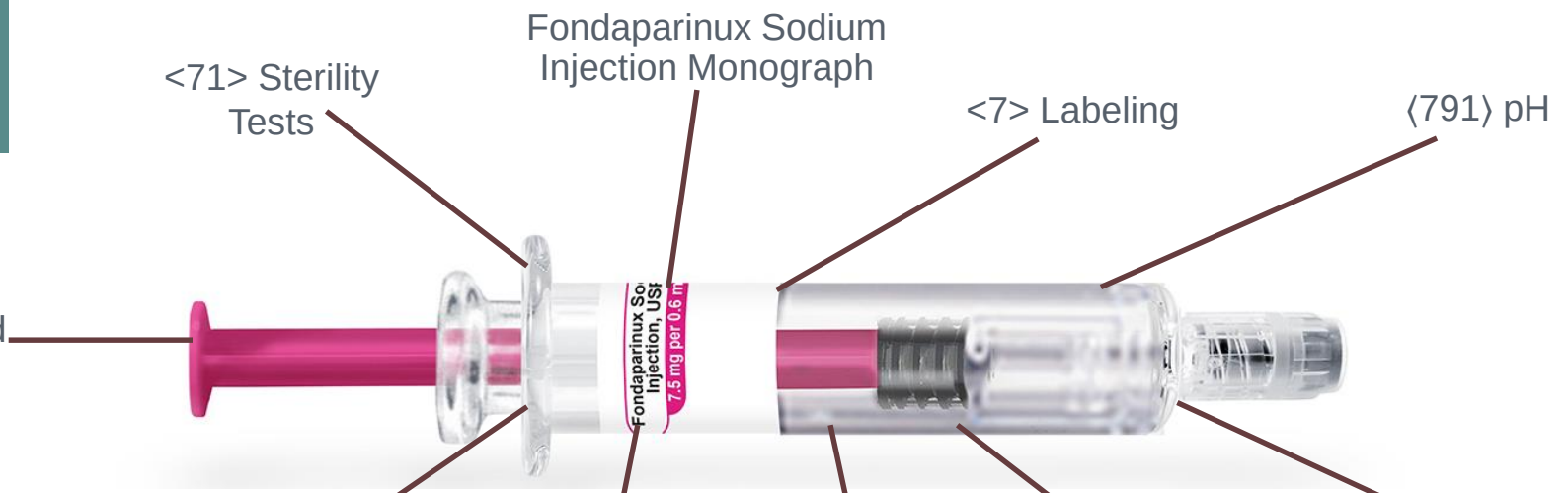
- Helping formulate harmonized guidelines (ICH Q13)
- Developing curriculum to train workforce
- Sponsoring scientific research comparing Batch-CM routes
- Published stimuli article in *USP-NF*
- Exploring application of PCM in biologics

Convergence supporting innovation

- **Efficiency and regulatory predictability in manufacturing and innovation processes**
  - Standards that verify and codify processes and components of product development and manufacturing to enable innovators to focus on discovery
- **Science-based expectations for medicine quality**
  - A fundamental tool for regulatory oversight, and pharmaceutical manufacturing

Prioritizing public quality standards for devices that are critical to a medicine having its intended therapeutic effect

NOT EXHAUSTIVE
Prefilled syringe example



Sources: USP-NF Research; Chapter Chart 4b Chemical Medicines Drug Products—Specific Tests
Note that the monograph mentions prefilled syringes in context of being a container; syringes are defined as primary packaging components, being in direct contact with the article. USP does not have a monograph for the performance of the specific drug-device product. Not pictured: USP Fondaparinux Sodium for Assay RS, USP Fondaparinux Sodium System Suitability Mixture B RS

How USP drives impact in biologics

Develop new standards for biologics based on broad understanding of public health as well as regulatory and technology impact

- Modernization of standards for legacy biological products
- Prioritization of performance standards with broad applicability to classes and families of products
- Development of standards to support emerging therapies based on novel technologies
- Engagement and scientific connectivity with biologics stakeholders across industry, government, regulators, and academia

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Empowering a healthy tomorrow