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NIST-FDA Cell Counting Workshop: Sharing practices in cell counting measurements

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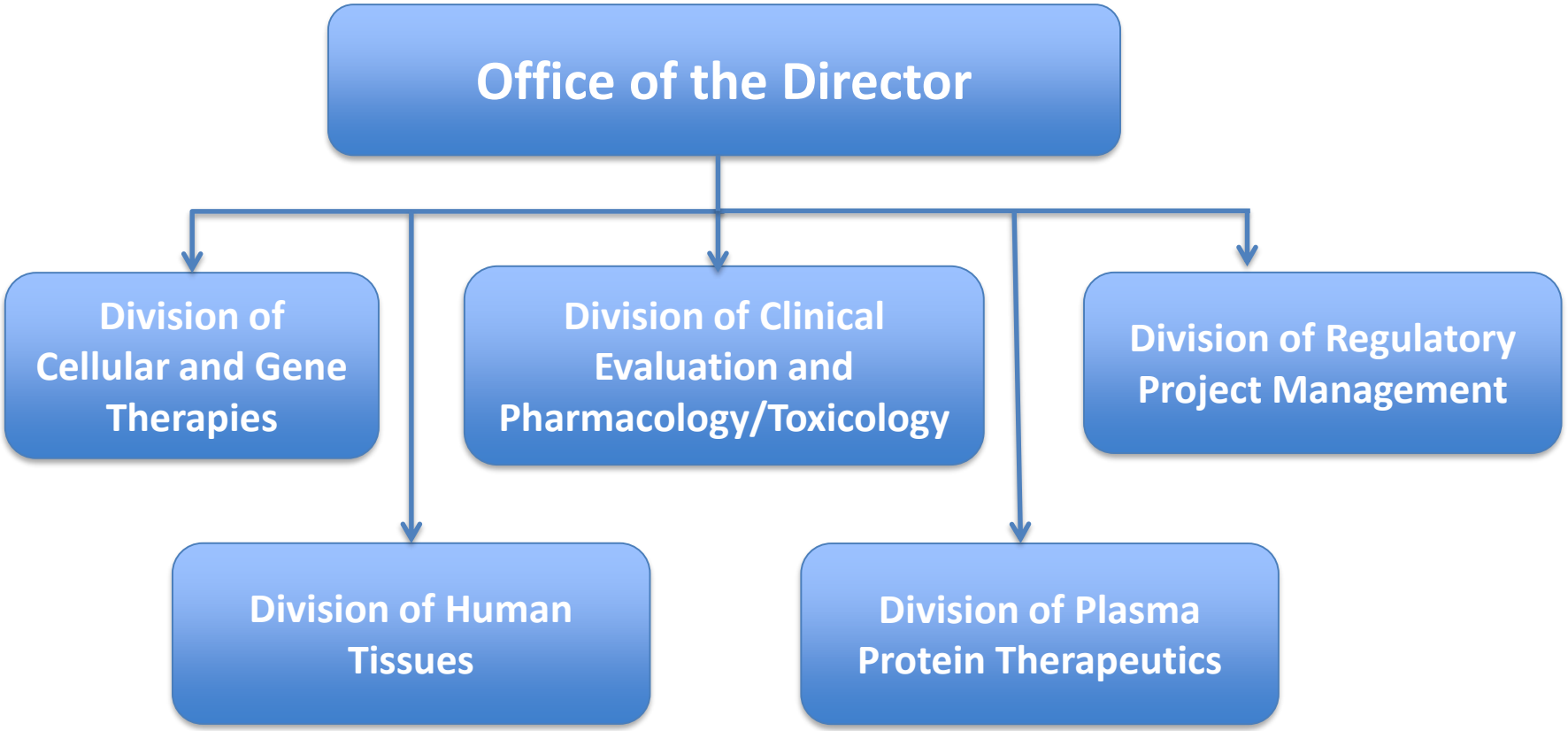

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The 21st Century Cures Act: Title III, 3033-3036: Regenerative Medicine Provisions

21st Century Cures Act: Title III, Sections 3033-3036

- Regenerative medicine provisions:
  - Section 3033: Creates pathway for designation as a regenerative medicine advanced therapy
  - Section 3034: Mandates that FDA develop guidance regarding devices used in the recovery, isolation, or delivery of regenerative medicine advanced therapies
  - Section 3035: Mandates that FDA report yearly to Congress on regenerative advanced therapies
  - **Section 3036: Directs HHS, in consultation with NIST and stakeholders, to facilitate efforts around development of standards for regenerative medicine therapies and regenerative medicine advanced therapies**

Definition of Regenerative Medicine Therapies

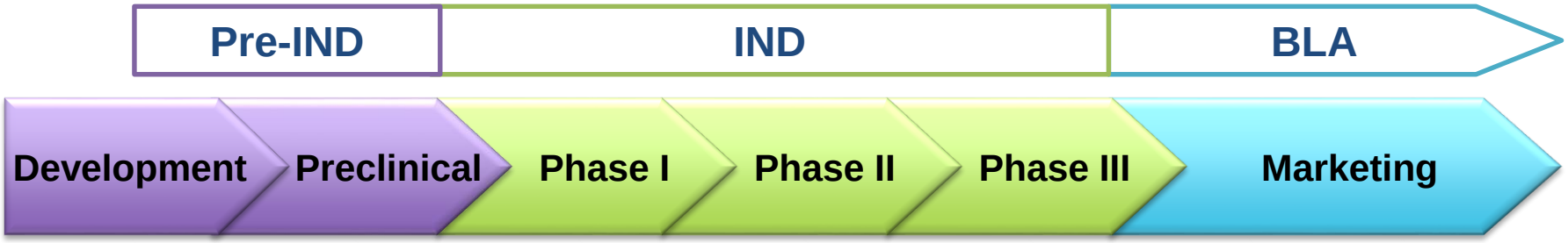
Cell therapies, therapeutic tissue engineering products, human cell and tissue products, or combination products with any such therapies or products, except for those regulated solely under section 361 of the Public Health Service Act (PHS Act) (42 U.S.C. 264) and Title 21 of the Code of Federal Regulations, Part 1271 (21 CFR Part 1271).

Regulatory Challenges for Assessing RM Therapies

- Product Testing
- Development of performance characteristics
- Testing methodologies
- Scientific protocols
- Compliance criteria

Cell Counting

- Crucial for measurements of purity, identity, potency, and dosing
- Methods vary and depend on the product
- Validation of cell counting methods required for licensure
  - Expected by Phase III, required for BLA



FDA Efforts in Regulatory Science

- International harmonization discussions with foreign regulatory authorities
- Standards development activities with ISO, ASTMi, ATCC, etc
- Regulatory focused research programs
  - New approaches for product manufacturing and quality
- Laboratory collaborations for standards development
  - Inter-lab round-robin testing
  - Collaborations with NIST

Why is FDA interested in standards development for regenerative medicine therapies?

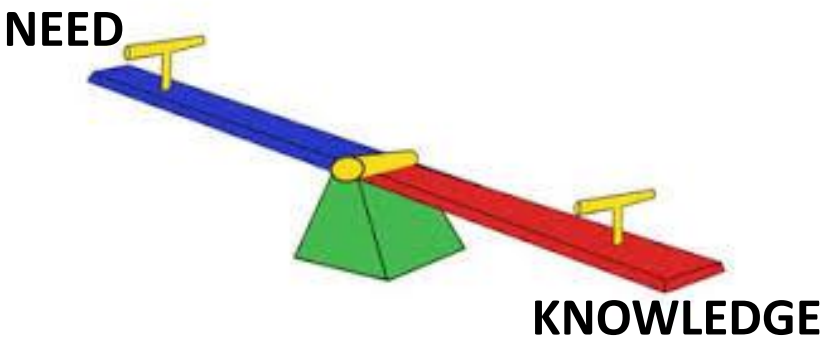
- National Technology Transfer and Advancement Act of 1995
- Standards can help developers of regenerative medicine therapies meet regulatory expectations
  - Product characterization
  - Potency Assessments
  - Identity Testing
  - Purity
  - Etc.

Benefits of Standards Use

- Help a sponsor of a regulatory submission meet regulatory requirements
- Facilitate product characterization
- Reduce time to market by leveraging industry and government efforts

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 approaches proposed are appropriate to address the need for standardization?



Outlook for Cell Counting

Identifying appropriate methods for cell counting and understanding the instrumentation available could lead the development of documentary standards and reference materials that can help to address cell counting challenges for regenerative medicine therapies.

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