NIST-FDA Genome Editing Workshop

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NIST – Who we are today

The National Metrology Institute
Global harmonization of measurement and traceability to the SI

“Industry’s National Laboratory”
Non-regulatory agency partnering/serving industry to help maintain US leadership in science and technology products

Department of Commerce
developing standards to support international trade and commerce
Bio at NIST

Develop measurement science, standards, data & technology to support development, manufacturing, & approval of product enabled by emerging biotechnology

• Works closely with stakeholders to identify key measurement problems and solutions
• Draws from a broad array of unique, cutting-edge expertise, resources, and facilities available at NIST
• Is a scientifically trusted “3rd” party that works to promote collaboration & sharing
NIST Practices

Measurements and Technology
• Develop advanced measurement capabilities
• Improve measurement quality and assurance

Reference Materials /Standards
• Develop and certify NIST SRMs and RMs
• Generate reference data (e.g., chemical spectra)

Standards
• Lead and contribute to documentary standards development through SDOs (e.g., ISO, IEC, ASTM, etc.)
• Conformity assessment
• Standards education
FDA-NIST Collaborations to leveraging unique expertise: Standards for Cell and Gene Therapy

NIST engages in discussions and collaborates with industry and others on pre-competitive technologies. NIST expertise in measurement sciences address specific analytical challenges.

FDA scientific and regulatory expertise ensure that standards:
- do not conflict with FDA regulation and policy
- address significant regulatory challenges that recur across the field.
### Selected recent workshops

**Bold represents Joint NIST-FDA Workshops**

<table>
<thead>
<tr>
<th>Month</th>
<th>Workshop Description</th>
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<tbody>
<tr>
<td>May 2015</td>
<td>NIST Workshop: Strategies to Achieve Measurement Assurance for Cell Therapies Workshop</td>
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<tr>
<td>Oct 2015</td>
<td>NIST-FDA Standards for Pathogen Detection via NGS workshop</td>
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<tr>
<td>Feb 2016</td>
<td>CAR-T Biomanufacturing Symposium</td>
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<td>May 2016</td>
<td>NIST Genome Editing Standards Workshop</td>
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<td>Aug 2016</td>
<td>NIST Standards for Microbiome Measurements</td>
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<td>Apr 2017</td>
<td>NIST-FDA Cell Counting Workshop: Sharing practices in cell counting measurements</td>
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<td>Aug 2017</td>
<td>NIST-DHS-FDA Workshop on Standards for Pathogen Detection</td>
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<td>Oct 2017</td>
<td>NIST-FDA Flow Cytometry Workshop</td>
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Workshop outcomes inform the development of documentary standards and reference materials.
Important roles of standards

Many types of standards to accelerate R&D, product translation and commercialization

- Common understanding (term, data interpretation)
- Common practices
- Traceable materials
- Common requirements
- Common operational and management systems
Standards Development Principles

Documentary Standards (Voluntary Consensus)

- **Openness**
  - Anyone can participate

- **Transparency**
  - Essential information accessible to all

- **Balance**
  - No single interest may dominate

- **Consensus**
  - General agreement; not unanimity

- **Due Process**
  - Fairness & equity

Reference Materials

- Fitness for intended use
- Reference to specified properties
- Homogeneity
- Stability
Ongoing ISO/TC 276 Projects Relevant to Genome Editing and related Therapeutic applications

Biobanking and Bioresources for R&D
- Global overarching standard for ALL biobanks
- Explanatory Document
- Validation & Verification

Cell therapy Bioprocessing
- 3-Part Ancillary Materials
- Cell Transportation
- Cell Manufacturing Equipment

Analytical Methods
- Quality to oligo
- Genome synthesis
- Nucleic acid quantification
- NGS
- Cell counting
- Cell characterization
- Cell authentication/identity

Data Processing & Interoperability
- Genome Compression
- Requirements for data formatting and description
Developers of documentary standards

- ISO TC/276: Biotechnology
- ASTM
- American National Standards Institute (ANSI) accredited SDOs: CLSI, ATCC
- Professional organizations
- Accreditation bodies
- Industry consortia

Coordination is required to avoid conflict and duplication