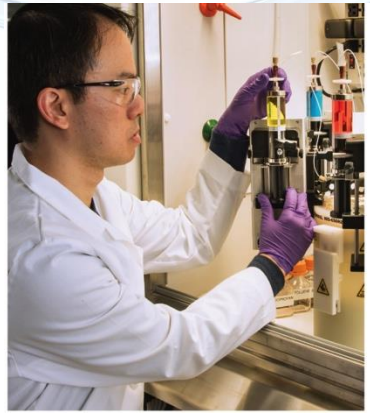
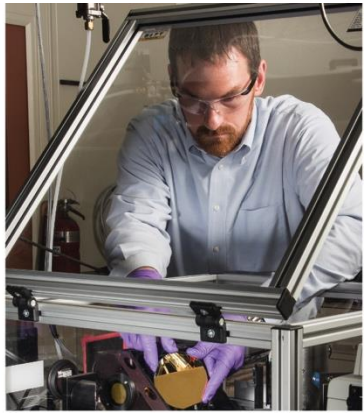




NIST Genome Editing Consortium Overview

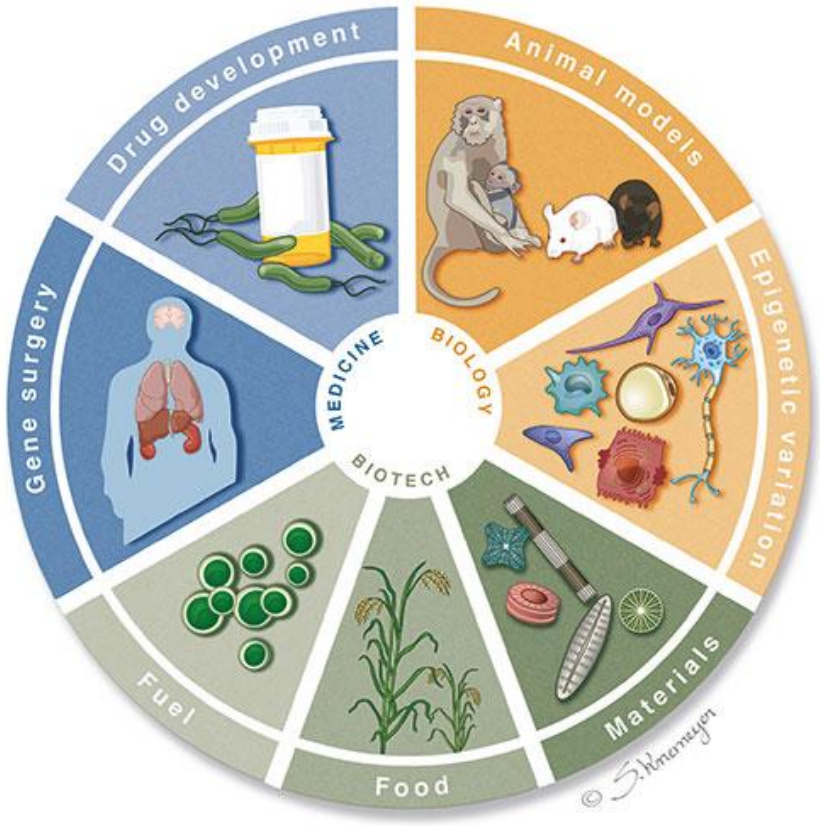
Samantha Maragh
Leader, Genome Editing Program
Samantha@nist.gov

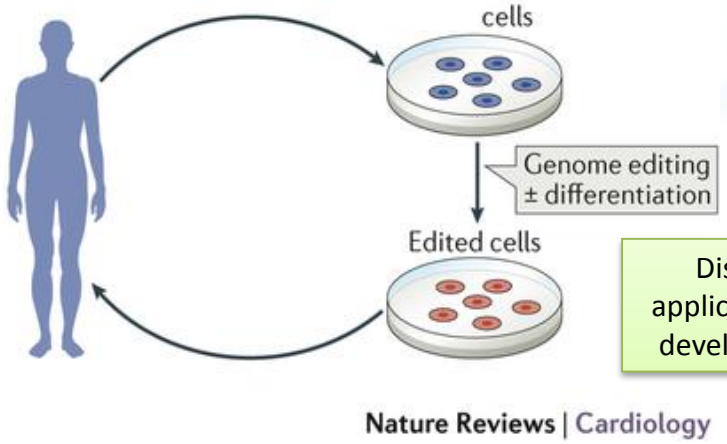
National Institute of Standards and Technology
U.S. Department of Commerce

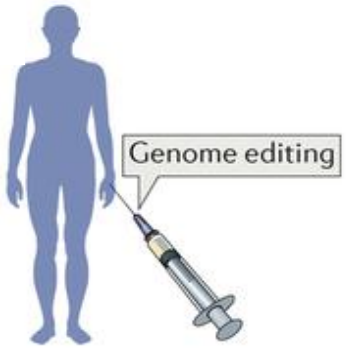
MATERIAL MEASUREMENT LABORATORY

How did we get here?

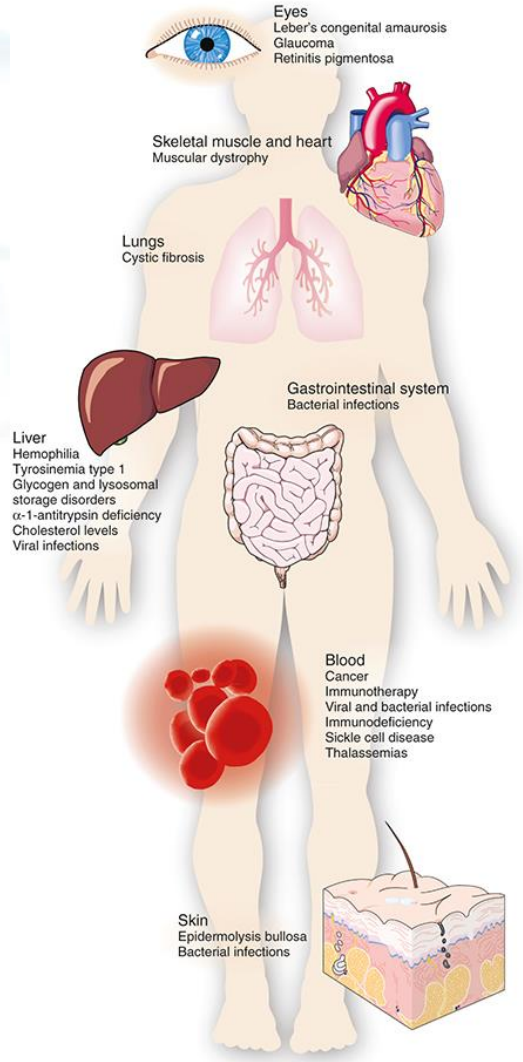
REGENERATIVE MEDICINE/CELL/GENE THERAPY EXAMPLE







Disease applications in development



How did we get here?

NIST National Institute of Standards and Technology U.S. Department of Commerce

AMERICAN SOCIETY of GENE & CELL THERAPY

Sponsors: Microsoft, editas MEDICINE

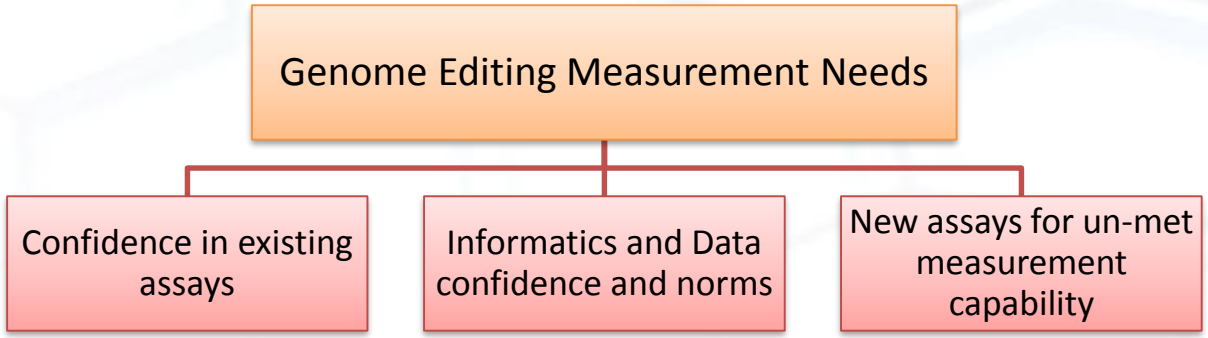
Genome Editing Standards Workshop

May 2, 2016 NIST- hosted

Where is confidence needed?

What is needed to achieve this confidence in this context?

65 participants at the 2016 NIST Genome Editing Standards Workshop identified pre-competitive standards and measurements needed to establish greater confidence in characterization of genome editing outputs.



Leading recurring needs shared from the community

- How to quantify and reliably detect on-target and off-target genome editing?
- How to compare existing off-target location discovery assays?
- How to determine reliability of validating sequence variants?
- How to compare different genome editing reagents?
- Agreement on data reporting and informatics data formats
- A standard data set to test and understand comparability of informatics pipelines

How did we get here?

NIST National Institute of Standards and Technology U.S. Department of Commerce

NIST Genome Editing Measurements and Standards Discussion at the Keystone Precision Genome Engineering Conference Jan 11th 2017

Sponsor illumina

Focus group of experts supported a path forward of a NIST led Genome Editing Consortium with 3 focus areas:

1. Specificity Measurements
2. Data & Metadata
3. Lexicon

Some of the participants in the process:

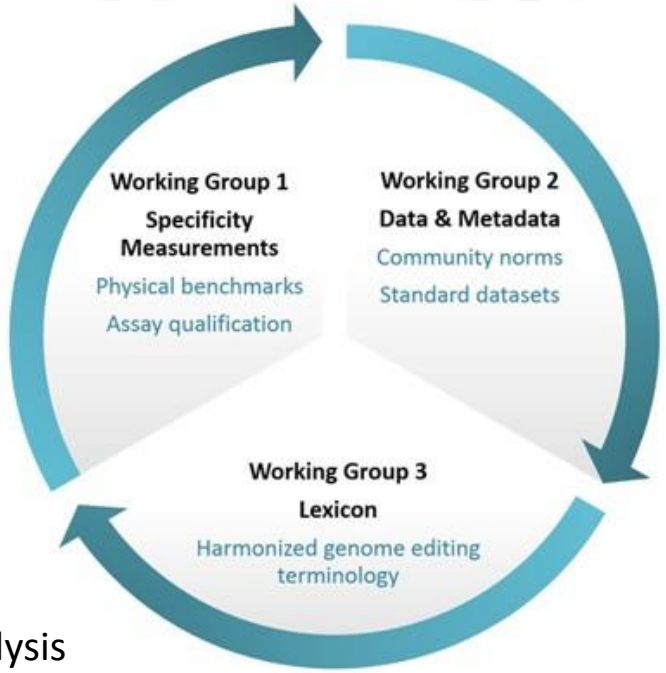
NIST Genome Editing Consortium (now accepting members)

MISSION

Convene experts across academia, industry, non-profit & government to addresses the measurements and standards needed to increase confidence and lower the risk of utilizing genome editing technologies in research and commercial products.

CONSORTIUM GOALS

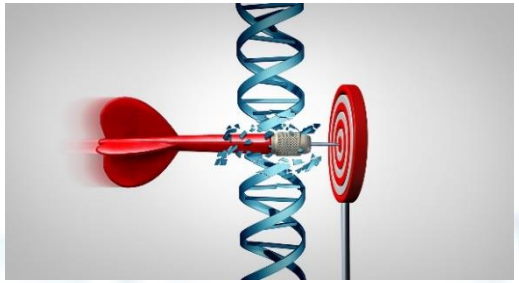
- Qualify genomic assays used to evaluate genome editing outputs
- Develop reference materials
- Generate reference data and standard data formats
- Develop suggested minimal information reporting for public studies
- Generate a common lexicon for genome editing studies



CONSORTIUM IMPACT

- Shared genome editing relevant reference materials
- Community norms for minimum data reporting and tools for supporting data analysis
- Improved understanding of and confidence in detecting and quantifying on- and off-target genome editing
- Harmonized genome editing lexicon

NIST Genome Editing Consortium Working Groups



Working Group 1: Specificity Measurements

- NIST qualification of existing genomic assays used to identify on-target and off-target genome editing genomic locations and sequence variants
- Develop & qualify reference materials

Working Group 2: Data and Metadata

- Determine community norms for minimum information reporting
- Identify community norms for data formats and tools for benchmarking data analysis (e.g. in silico data sets, experimental data sets)

Working Group 3: Lexicon

- Identify terms and related definitions to form a common genome editing community lexicon

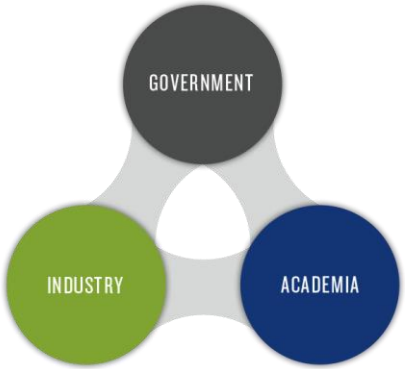
NIST Genome Editing Consortium (now accepting members)

WHY NIST?

- Cross-disciplinary expertise in engineering, and the physical, information, chemical, and biological sciences
- As a non-regulatory agency of the U.S. Department of Commerce, NIST does not impose standards; standards are accepted by consensus
- Neutral convener for industry consortia, standards development organizations, federal labs, universities, public workshops, and interlaboratory comparability testing

MODEL

- Convenes industry, academia, and government to identify and address measurement and standards needs across the genome editing field
- Enables members to work with NIST to develop measurement solutions and standards
- Leverages NIST expertise in measurement science, standards development, reference materials, technology development, and basic research
- Collaborates with related programs at other federal agencies



NIST Genome Editing Consortium

***MEMBER BENEFITS**

- Access to a neutral forum for addressing pre-competitive needs
- Participation in the development of experimental benchmarks, guidelines and terminology
- Access to tools developed by the consortium ahead of public release
- Institutional representation on consortium steering committee

Membership Process:

- complete a Letter of Interest.
- NIST will reach out to interested parties to initiate signing a Cooperative Research and Development Agreement (CRADA). Consortium CRADA is identical for all participants.
- *Membership fee of $20K or in-kind support of equivalent value to join. These contributions will supplement the NIST resources to enable us to generate and qualify the pre-competitive standards, norms and data the consortium members determine are needed to push the field forward and support innovation.

website: https://go.usa.gov/xnV3 | email: samantha@nist.gov (Samantha Maragh)

NIST Genome Editing Consortium + public workshops

At these workshops, consortium members will:

- Discuss the progress of the consortium work
- Continue to get broad input to update or refine the consortium work plan
- Continue to broadly solicit consortium membership
- Invite members to participate in work plan implementation, planned future experiments, and analysis

NIST-FDA Genome Editing Workshop April 23-24, 2018

Purpose: convene key stakeholders from industry, academia and government, to define a path forward for developing pre-competitive solutions to address the wet lab, dry lab and documentary benchmarks, norms and standards needed for genome editing- particularly the needs to support development of gene therapies.

Format: general session talks with discussion + breakout sessions to get detailed feedback and engagement.

Goals:

1. clarify regulatory perspectives
2. Inform on the path forward for the NIST Genome Editing consortium-
3. Get public input on path forward
    - consortium members working with NIST will determine how to proceed and execute to develop the needed products.

NIST-FDA Genome Editing Workshop April 23-24, 2018
Agenda Overview

DAY 1

Morning session 1: NIST, FDA, NIH SCGE

Break

Morning session 2: Expert perspectives & panel discussion

Lunch

Afternoon session 1: Plan for path forward presentations

Break

Afternoon session 2: Breakout sessions & end of Day 1

DAY 2

Morning session 1: Technology Talks (approaches for DNA off-target and genomic rearrangement detection)

Break

Morning session 2: Reports from breakout sessions, next steps, and wrap-up

Adjourn