

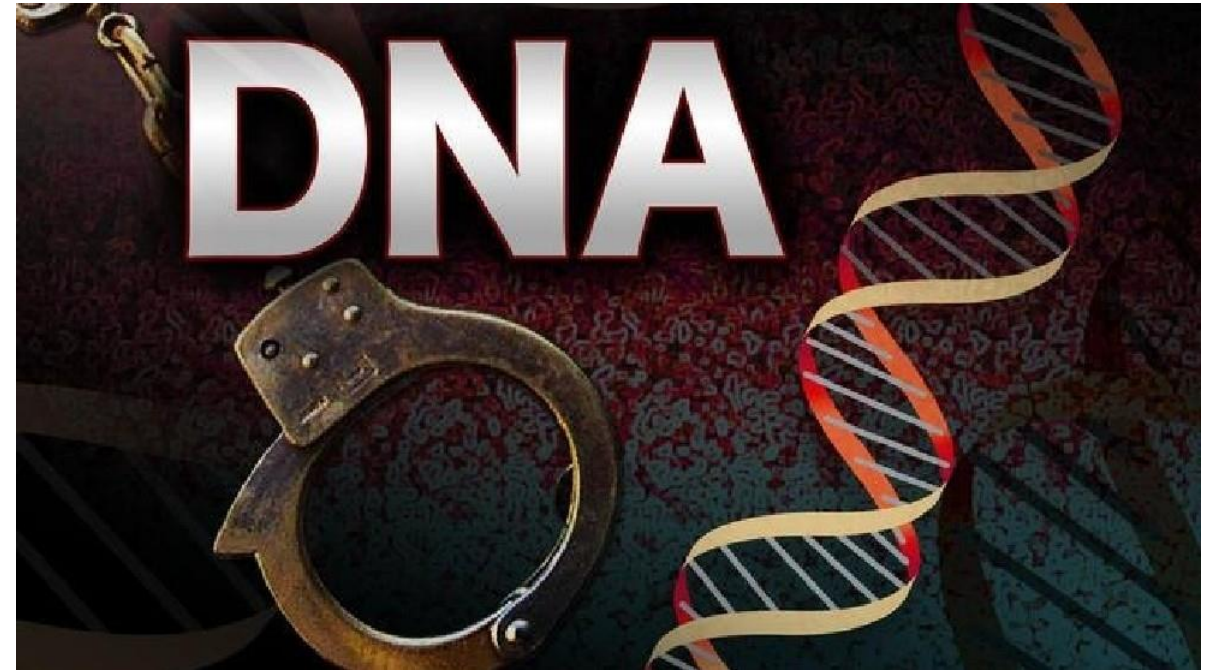
Empowering Genome Editing Through Standards

Samantha Maragh, Ph.D.

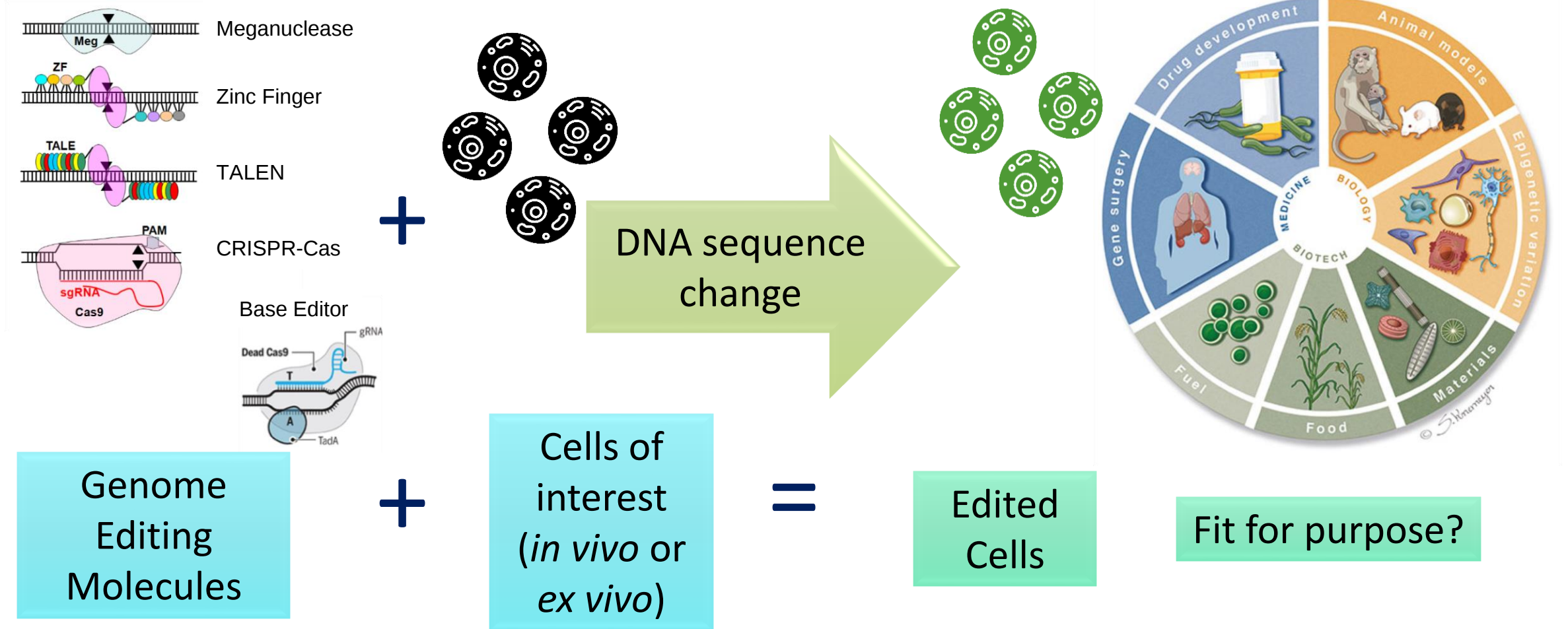


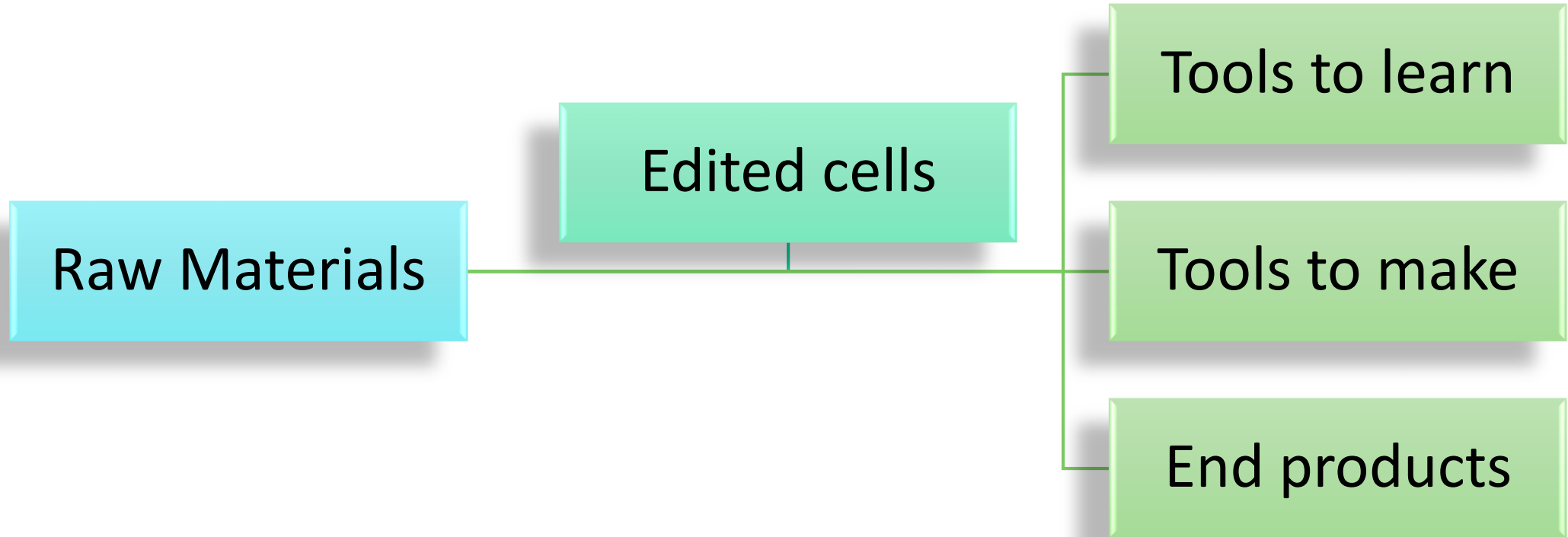
How confident are you that the answer is correct?

What is the consequence if it is wrong?



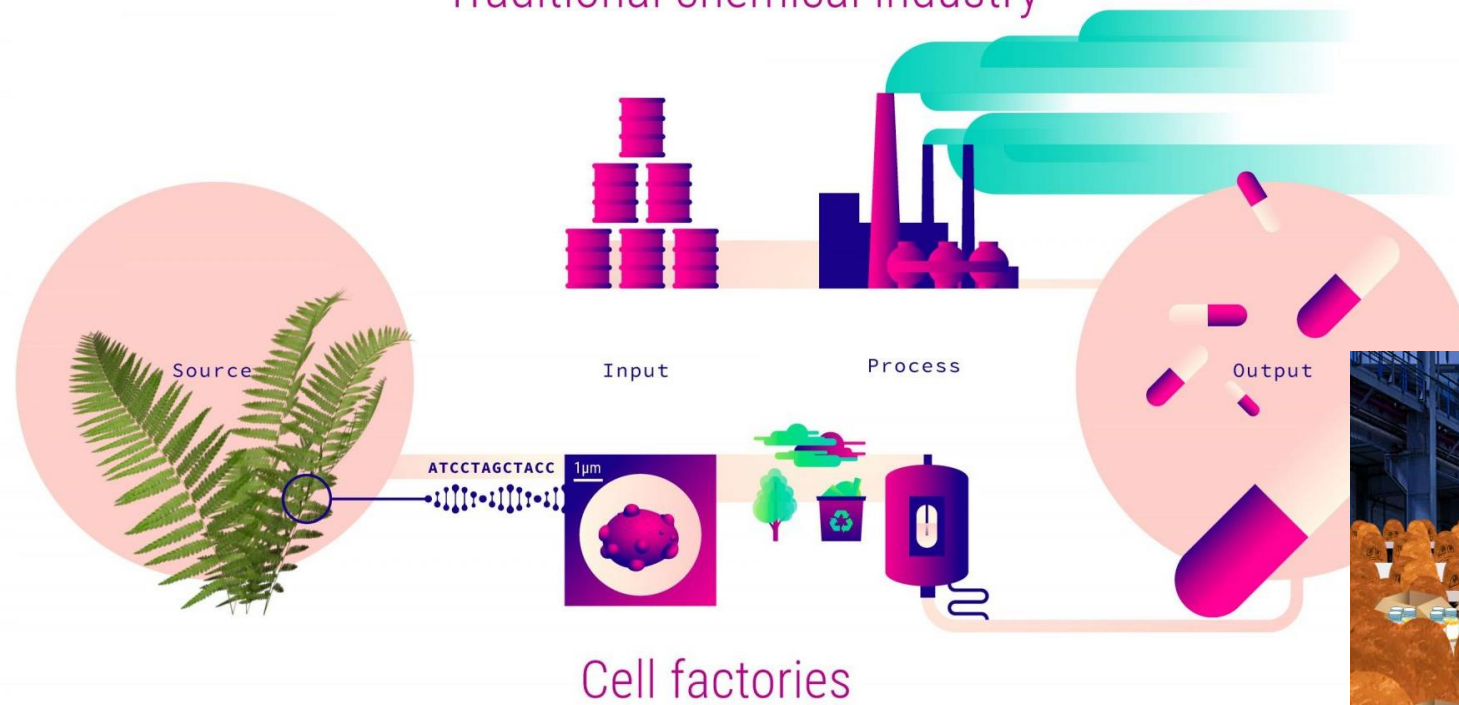
Genome Editing





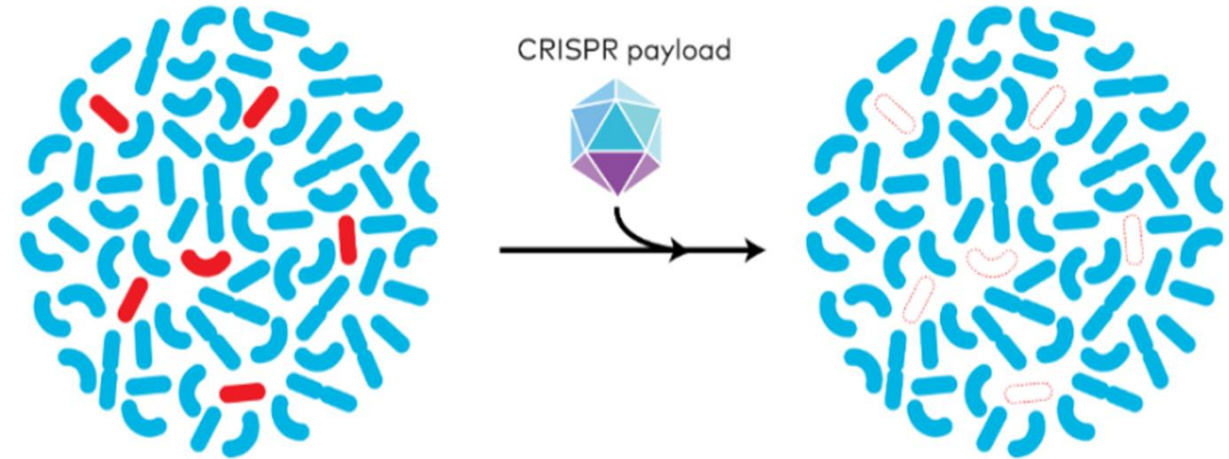
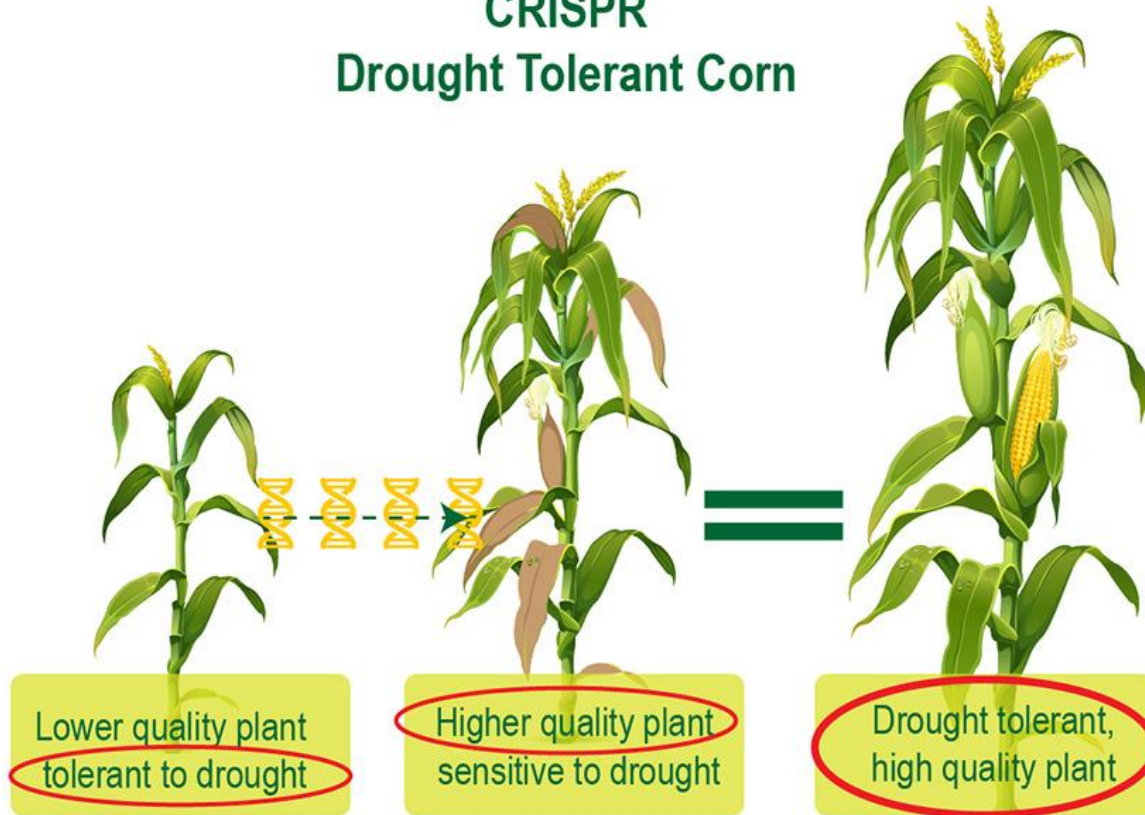
Traditional chemical industry

Tools to make

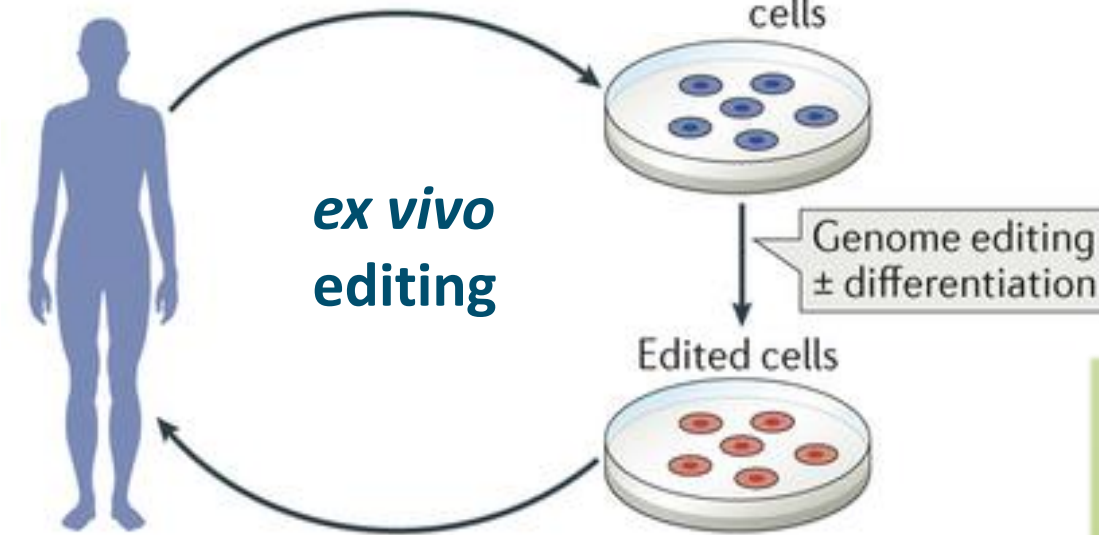


End Products

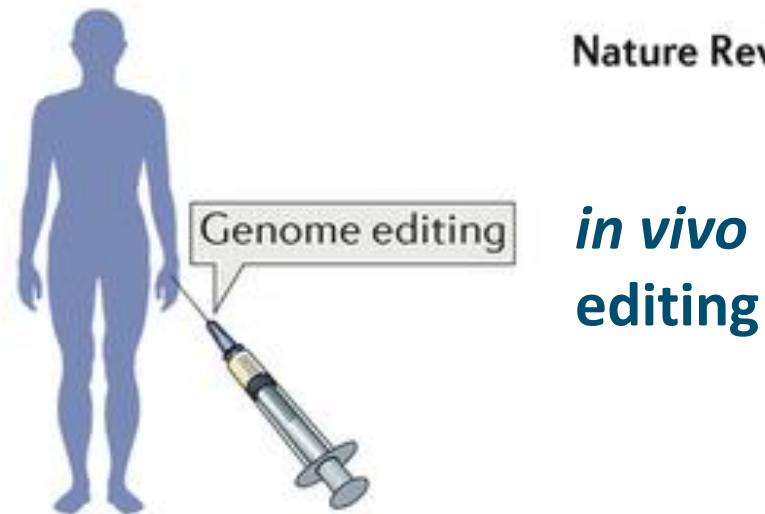
CRISPR Drought Tolerant Corn



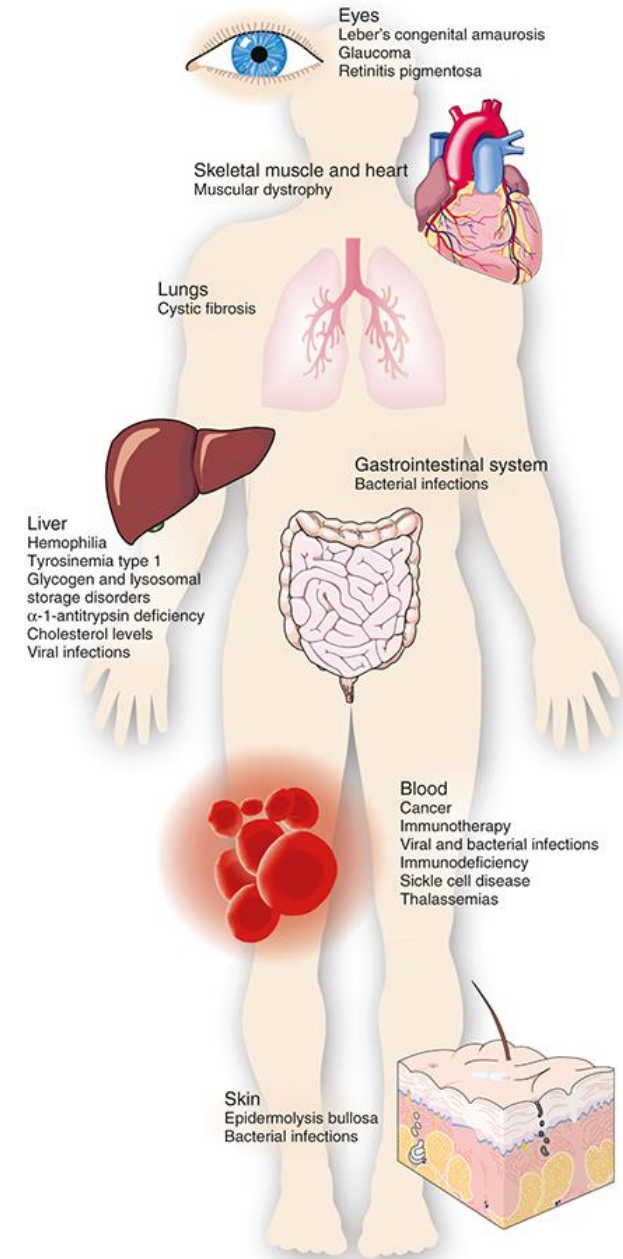
End Products



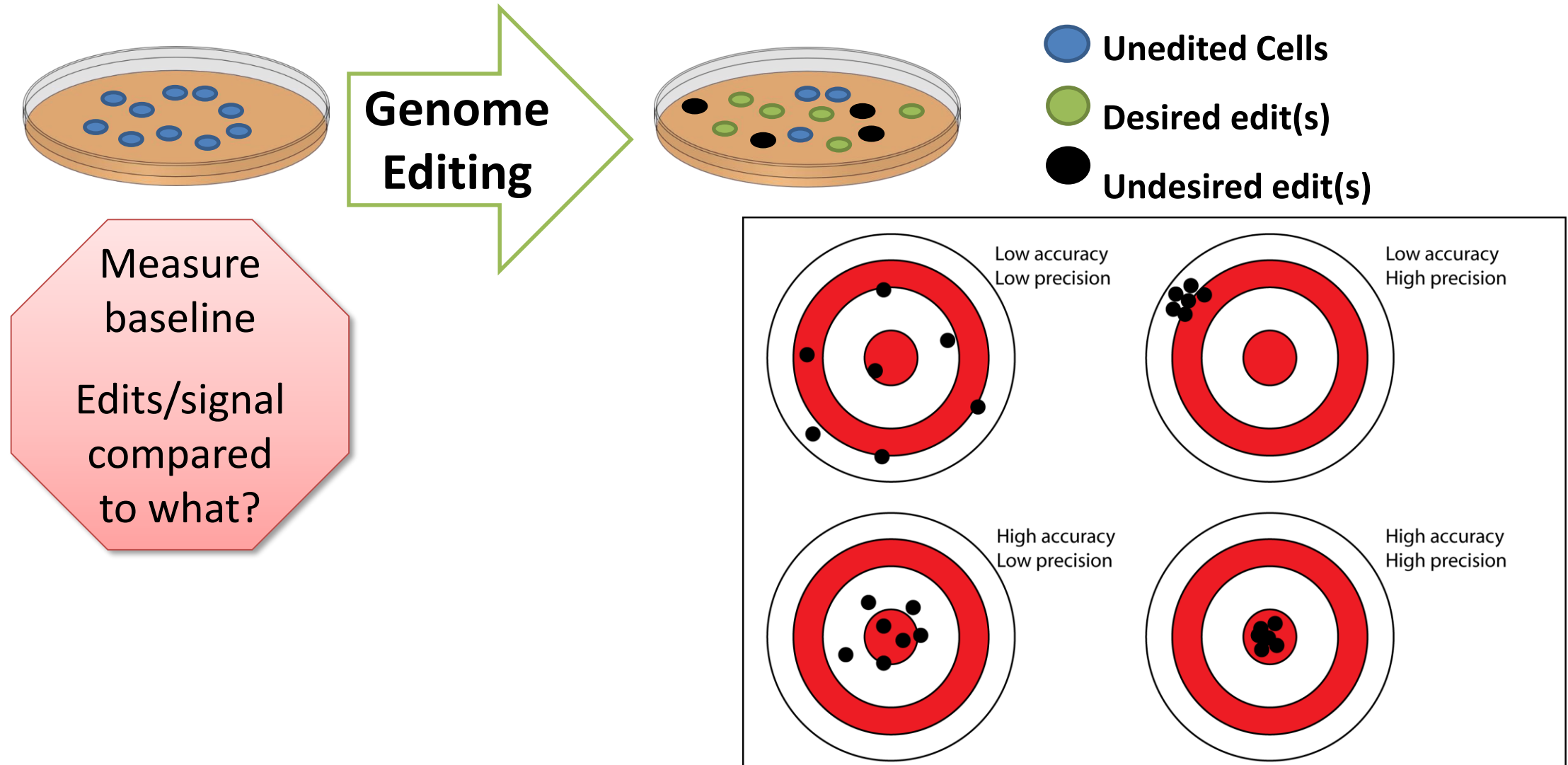
Nature Reviews | Cardiology



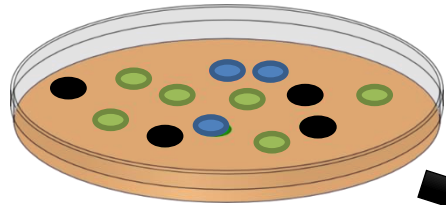
Disease
applications in
development



Genome Editing Outcomes are Complex!



Genome Editing Outcomes are Complex!



- Unedited Cells
- Desired edit(s)
- Undesired edit(s)

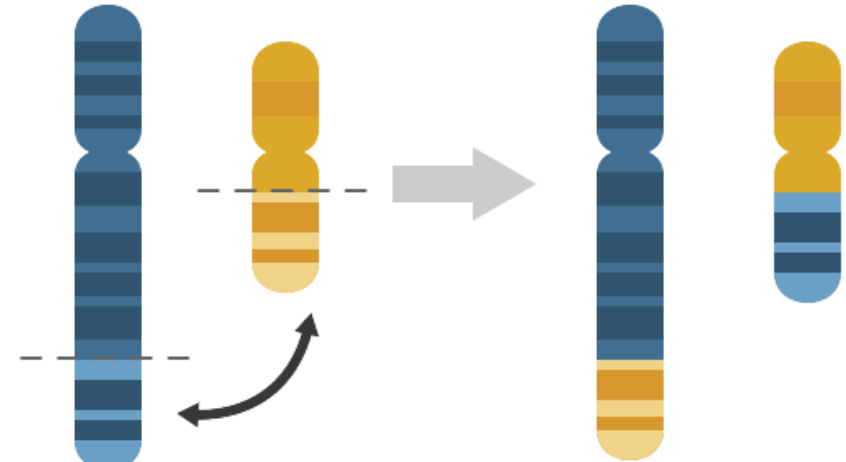


```
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ACAGCGTGAACCACATCAACCAGACGTACGAGTTTG--CAGCGGATGCG
ACAGCGTGAACCACATCAACCAGACGTACGAGT-----AGCGGATGCG
ACAGCGTGAACCACATCAACCAGACGTACG-----CAGCGGATGCG
ACAGCGTGAACCACATCAACCAGACGTA-----CAGCGGATGCG
ACAGCGTGAACCACAT-----GCGGATGCG
CAGCGTGAACCACATCAACCAGACGTACGAGTTTGTGACAGCGGATGCG
TGAACCACATCAACCAGACGTACGAGTTTGTGGCTTTAAAGCGGATGCG
AACCACATCAACCAGACGTACGAGTTTGTGCAAGGAACTGCGGATGCG
```

Wild type

Deletion

Insertion



I N N O V A T I O N

Where?

Who?

How?

What?

Fit?

Determine
location to
edit

Design
editing
system

Formulate
& Deliver

Confirm
sequence
change

Decide if
fit for
purpose

Measurements

Data

Metadata

How confident are you that the answer is correct?

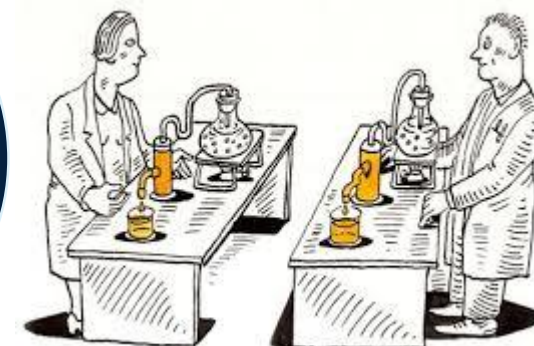
What is the consequence if it is wrong?

...What can be done to understand if the answer is correct?

Common
Understanding
(terms, data
interpretation)



Common
Practices



Traceable
Materials



STANDARDS

Common
Requirements



Common
Operational &
Management
Systems

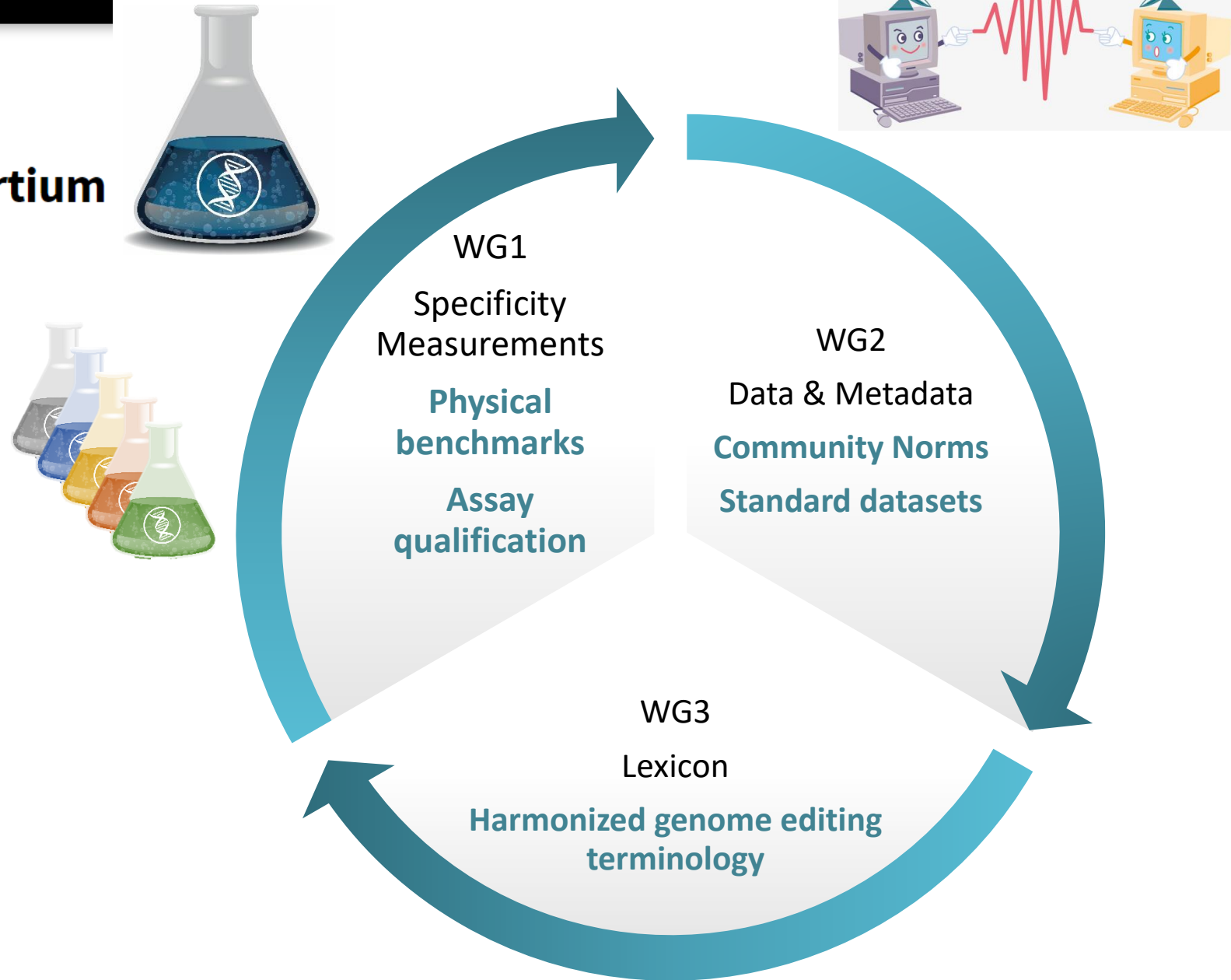




NIST Genome Editing Consortium

MISSION

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



UPDATES

NIST Seeks Comments on Genome Editing Terms

September 18, 2019

[The NIST Genome Editing Consortium](#) seeks feedback on terms and definitions specific to genome editing. The purpose of this project is to encourage the use of a unified standard set of terms and definitions that serve the needs of and act as a reference for the genome editing community. We welcome comments on the terms and definitions in [version 1 of the NIST Genome Editing Consortium Lexicon](#).



TECHNICAL CONTACT

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(301) 975-4947



ORGANIZATIONS

Material Measurement Laboratory

Biosystems and Biomaterials Division

Bioassay Methods Group

Genome Editing

Data sets to train design algorithms

Innovative delivery methods and models

Qualification of off-target location discovery

Technology to track cells over time *in vivo*

Assays & norms to qualify input reagents

DNA sequence change

Standard data sets to benchmark algorithms

Genome Editing Molecules

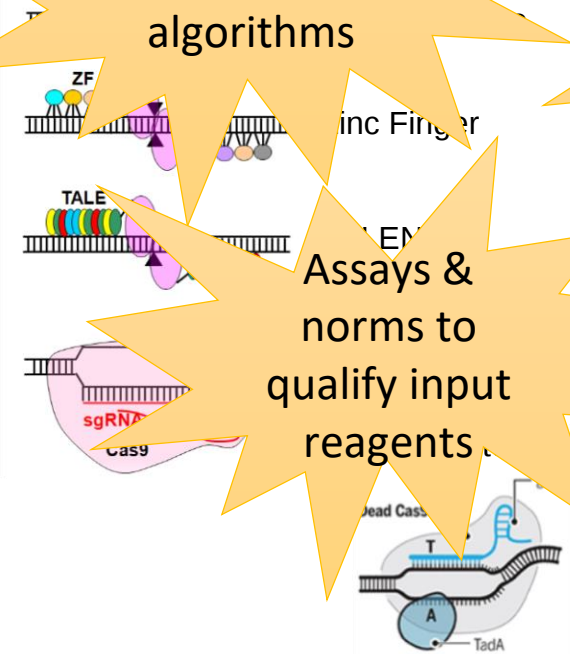
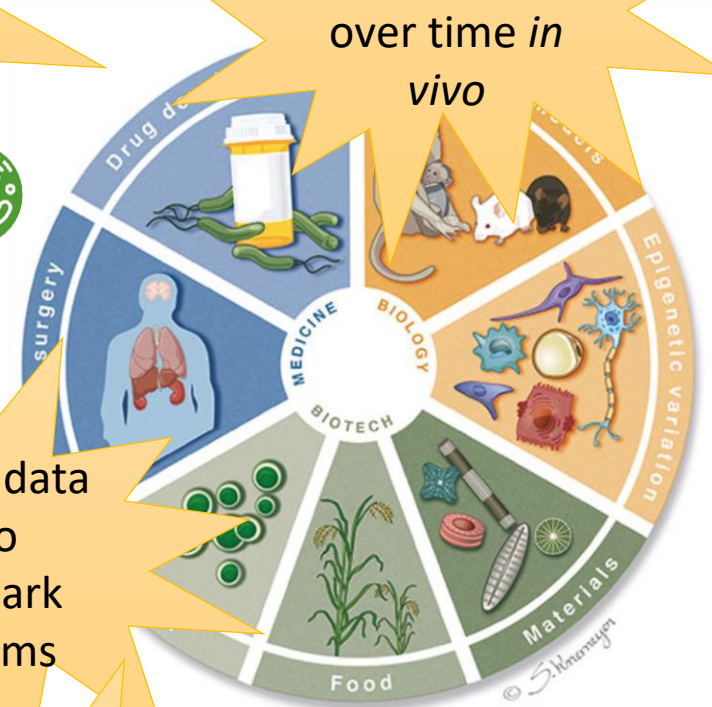
+

Cells of interest (*in vivo* or *ex vivo*)

Physical benchmarks

Data format norms & shared data repositories

Fit for purpose?



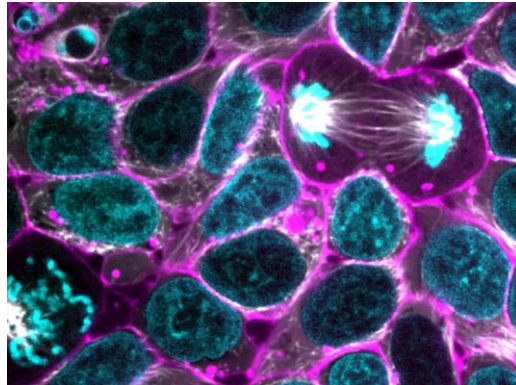
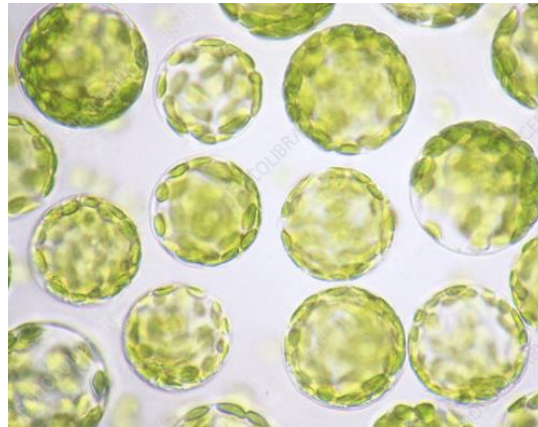


Raw Materials

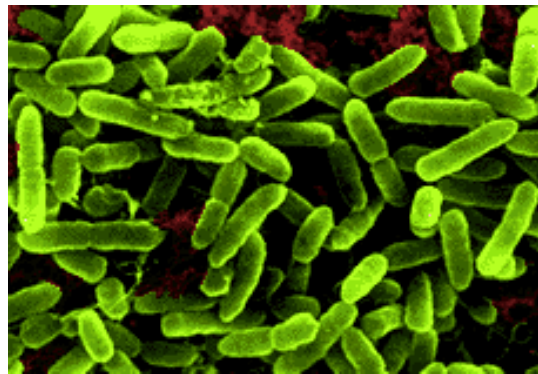
People



Plant



Human



Microbial

Formulation
and delivery

DNA



RNA



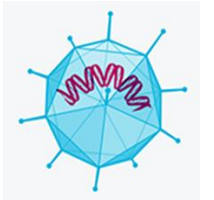
Protein



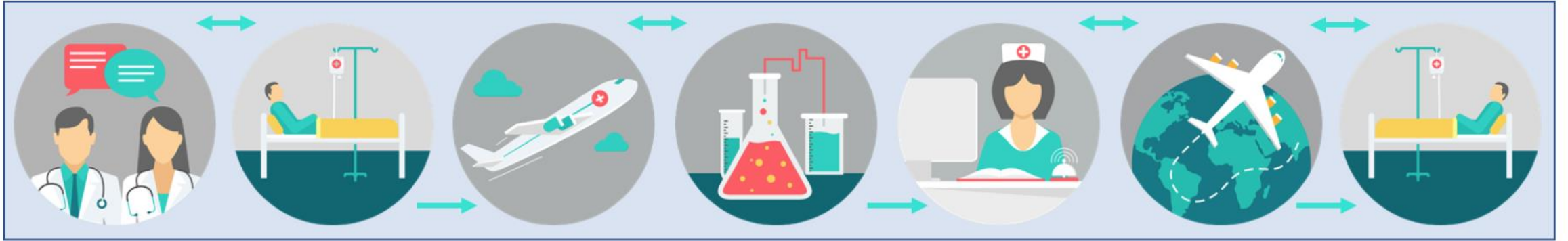
Lentivirus

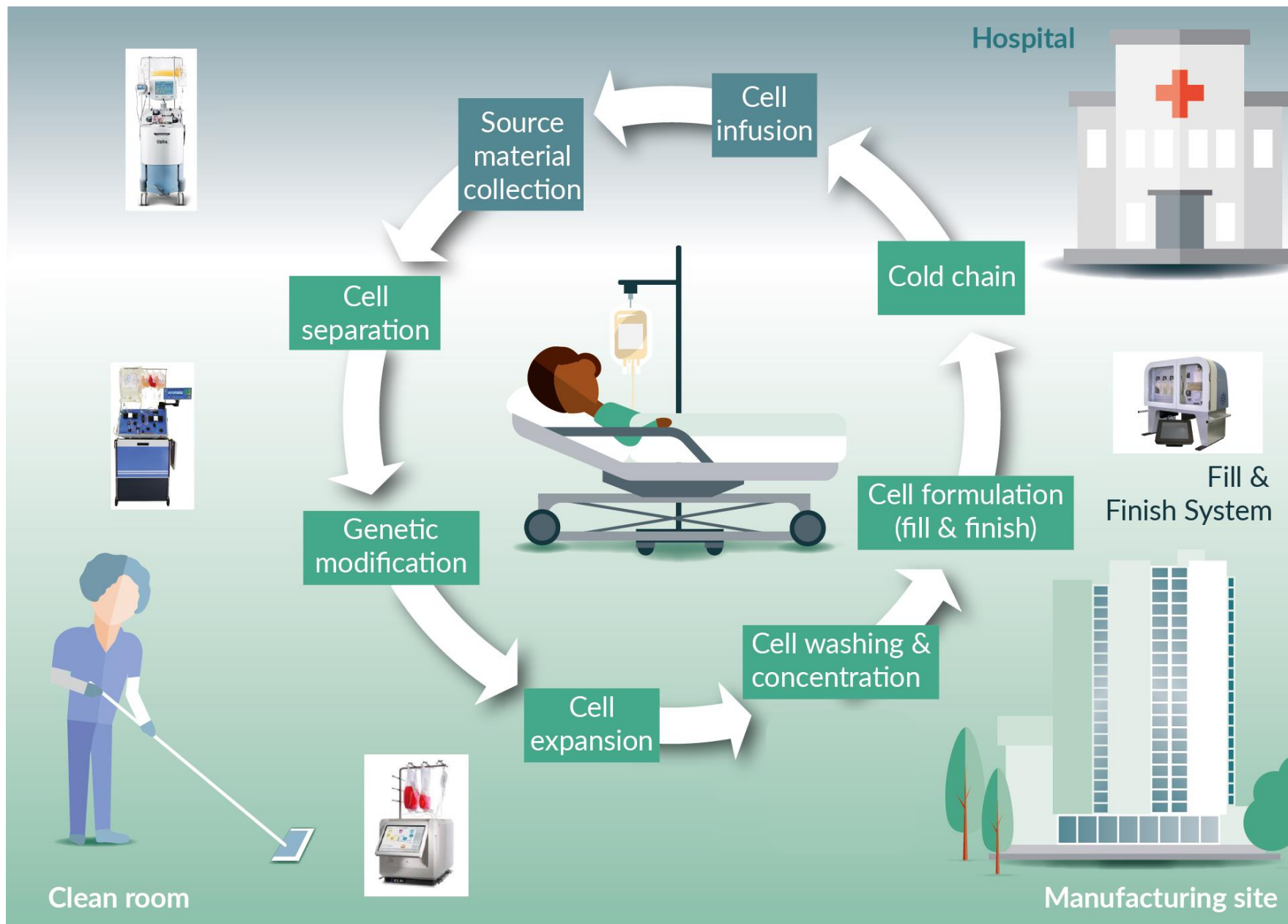


Nanoparticle



Adeno
Associated
Virus (AAV)





QUALITY



Ethics



Rare diseases



Clinical follow-up

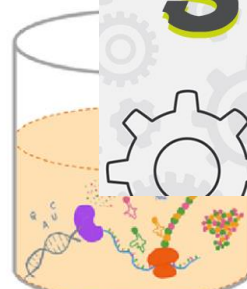
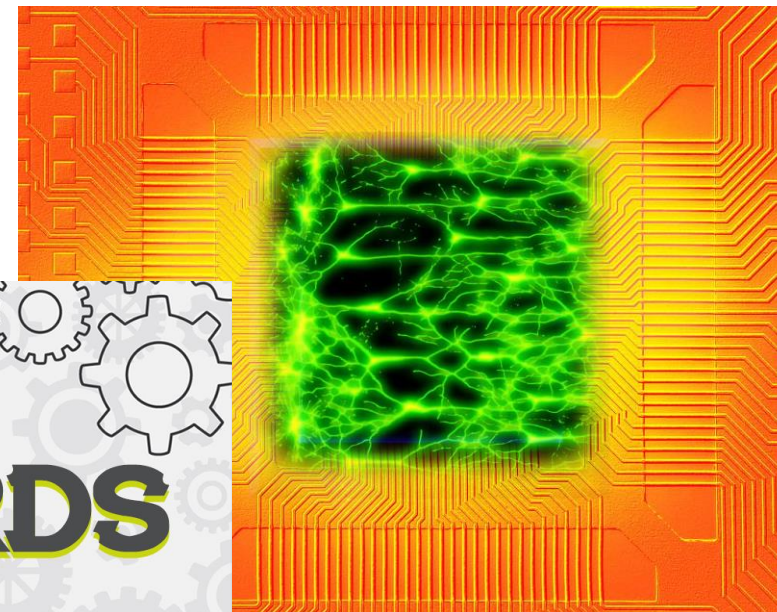


Therapy access



Consent

Purpose
driven
engineering?



Cell-free system

