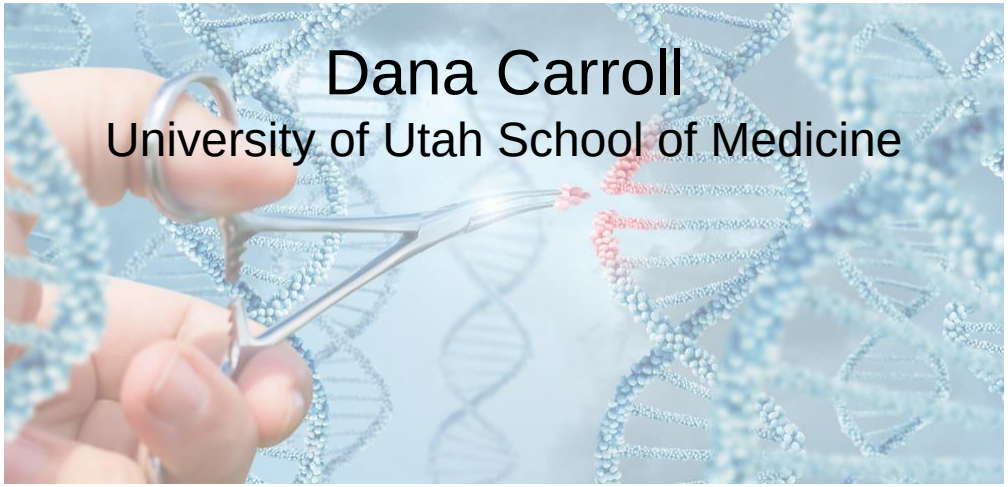
Human Genome Editing

Emerging Areas of Science, Engineering and Medicine for the Courts

February 25, 2021



Dana Carroll

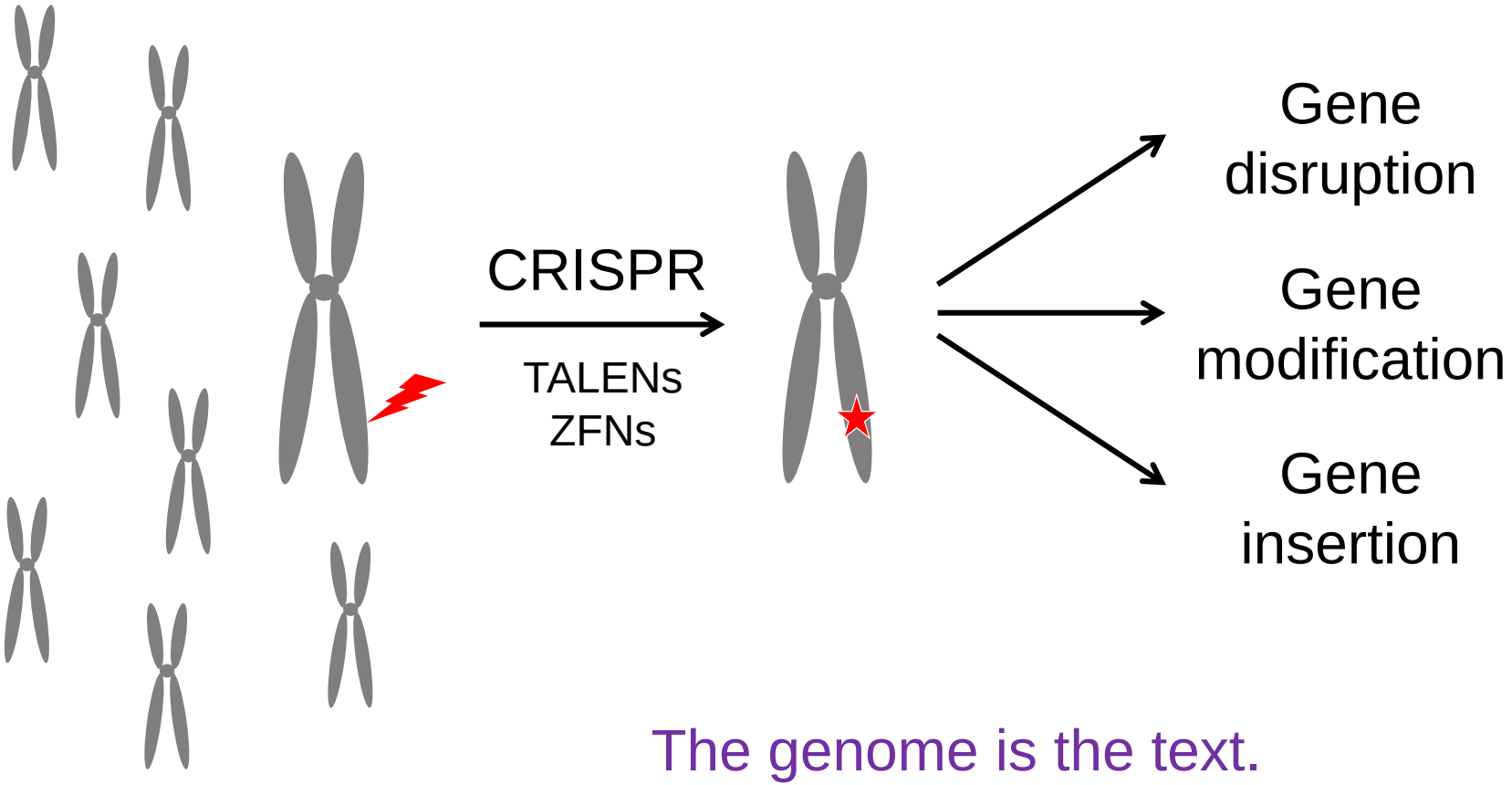
University of Utah School of Medicine

Financial relationships: Sangamo Therapeutics; Recombinetics, Inc.

2020 Nobel Prize in Chemistry for CRISPR

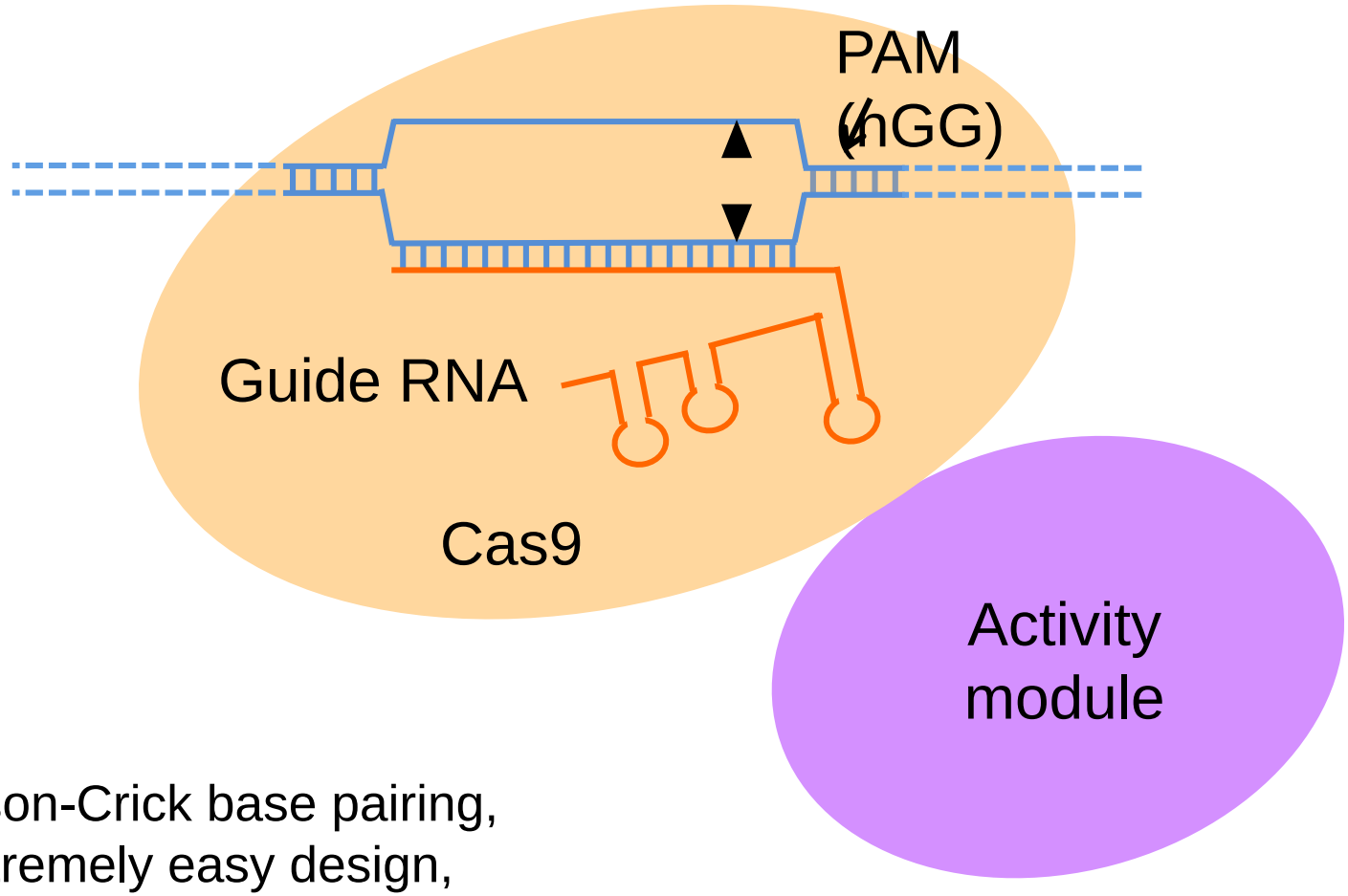
Jennifer Doudna Emmanuelle Charpentier

What CRISPR does – genome editing



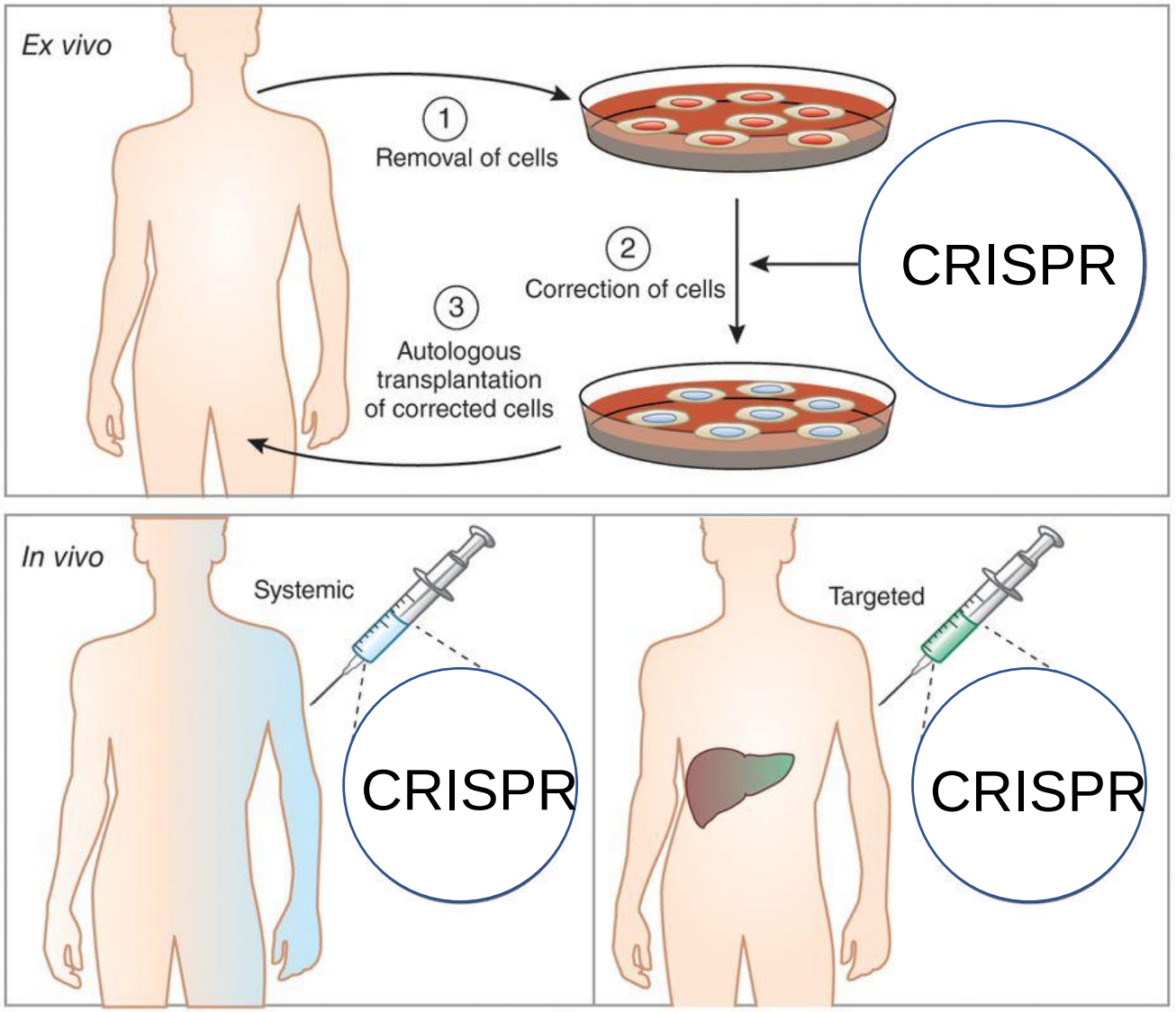
The genome is the text.
CRISPR places the cursor.

CRISPR/Cas system



Watson-Crick base pairing,
extremely easy design,
only one, constant protein

Types of human somatic genome editing



Soma = body

Cox et al. (2015) Nature Medicine 21: 121

After A Life Of Painful Sickle Cell Disease, A Patient Hopes Gene-Editing Can Help

October 10, 2019 · 5:07 AM ET
Heard on Morning Edition

ROB STEIN

Victoria Gray

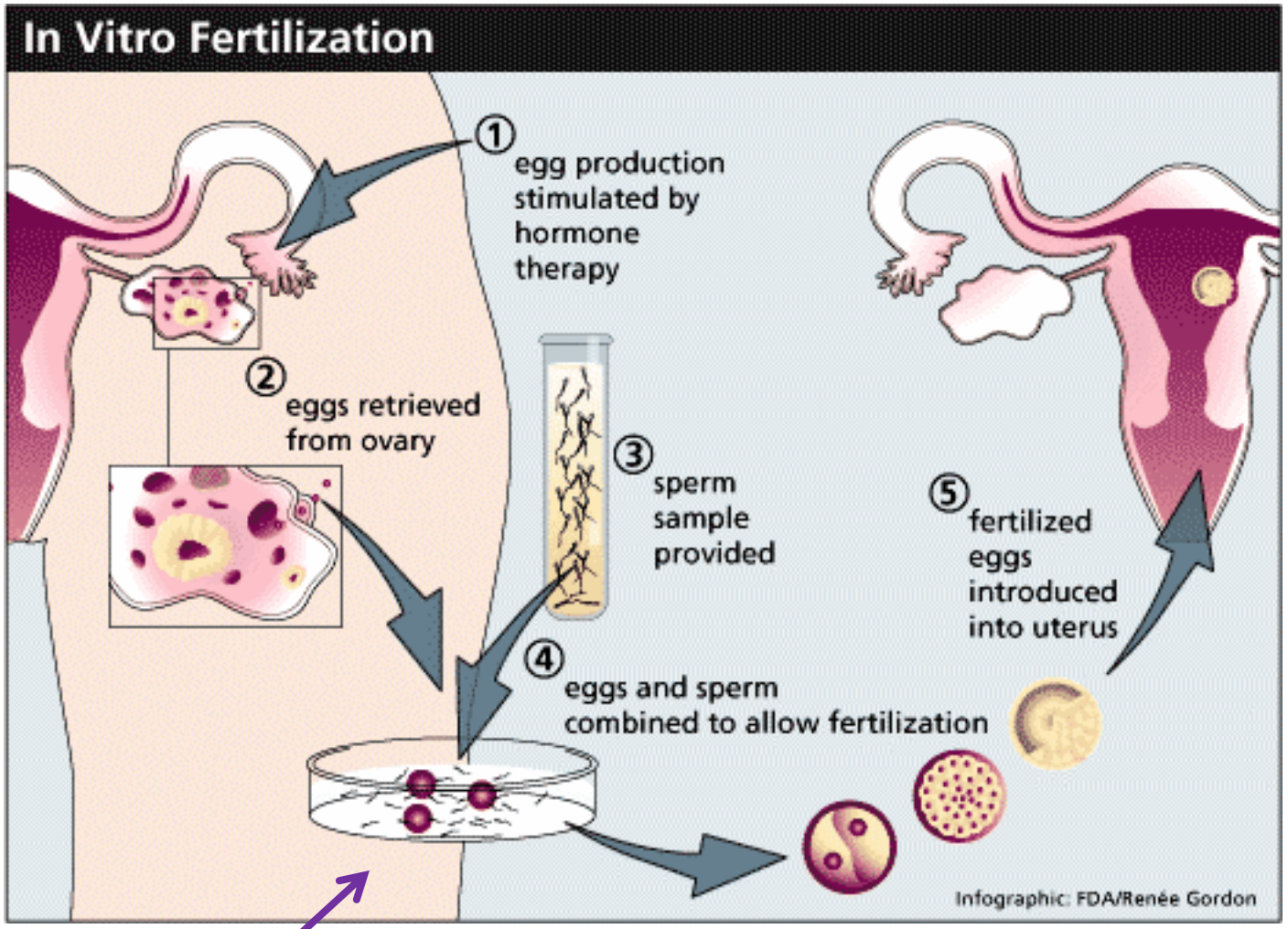
https://www.npr.org/sections/health-shots/2019/10/10/766765780/after-a-life-of-painful-sickle-cell-disease-a-patient-hopes-gene-editing-can-hel

CRISPR Baby Talk Shrouds Human Genome Editing Summit

Opening Day of the Highly Anticipated Human Genome Editing Conference in Hong Kong Reacts to Stunning News of a Chinese Group's Claims of Delivering Twins via Germline Editing

November 28, 2018

Heritable (germline) genome editing



CRISPR

https://infofru.com/health/pregnancy/infertility-management-recent-advancement/in-vitro-fertilization-and-embryo-transfer-ivf-et/

Agricultural genome editing

Healthier soy oil

Non-browning mushrooms

Disease-resistant bananas

Healthier cassava

Heat-tolerant “slick” cattle

Disease-resistant cattle and pigs

Pig models of human diseases

How should genome edited organisms be regulated?

Teosinte Maize

Genome editing by selective breeding

Are they really any different from organisms produced by standard selective breeding?

USDA currently does not regulate edited crops, unless they contain foreign DNA

Patents in Genome Editing

Anticipated income is huge

What constitutes an invention?

Limited licenses

Inventor protection vs. constraints on use

What's in the future?

Gene drives for disease vector control (malaria)

In utero genome editing

Generating edited human eggs and sperm

Military uses (IR vision?)

Bioterrorism potential

New, more specific laws?

Specificity vs. overkill and suppression


Questions?

What genome editing can do

- Modify essentially any DNA sequence in any organism
- Change the expression of single genes
- Make human disease mutations in other organisms
- Reverse individual disease mutations

What genome editing cannot do

- Make exactly the change you want every time
- Avoid making other changes
- Change many genes all at once
- Cure diseases that don’t have a genetic solution
- Make your smarter

Legal issues with somatic therapies

- Safety: risks vs. benefits
  - Unanticipated adverse effects
  - Limits of scientific knowledge
- Exacerbating health inequities – cost and access
- DIY biohackers

Current regulation

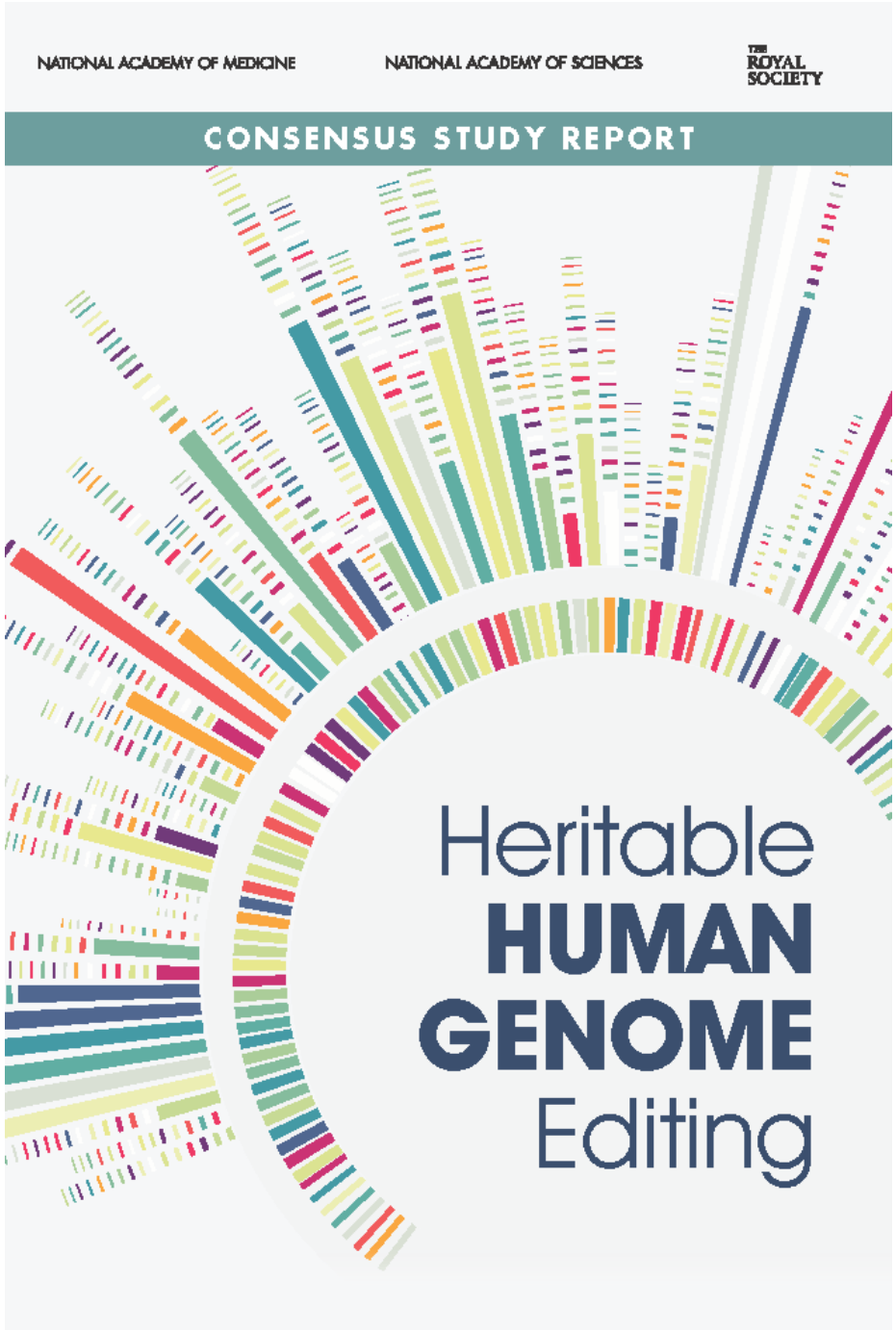
- FDA treats as gene therapy; multiple trials approved
- Multiple review steps, including IRBs
- Legal force of government guidelines, professional standards?
- Patents
- State and local laws

Legal issues with germline therapies

- Safety: risks vs. benefits
  - Unanticipated adverse effects
  - Limits of scientific knowledge
- All stages of development affected
- Exacerbating health inequities – cost and access
- Illegal practice of medicine
- DIY biohackers
- Enhancements

Current regulation

- FDA may not review human embryo proposals
- NIH will not consider grant proposals to edit human embryos
- No federal funding can be used for human embryo research
- Professional standards
- State and local laws may prohibit explicitly



September 3, 2020

https://www.nap.edu/read/25665/chapter/1