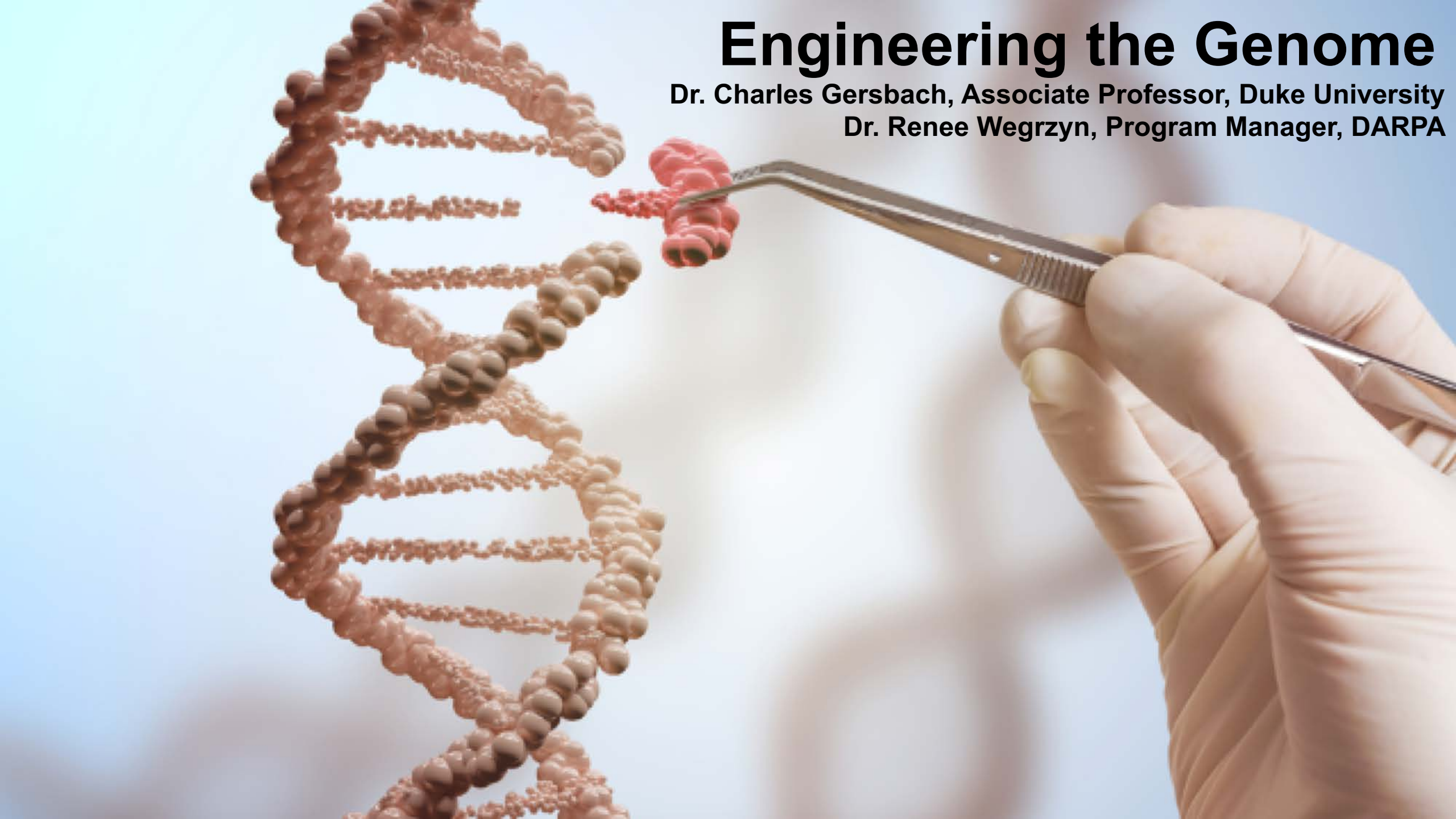
Engineering the Genome

Dr. Charles Gersbach, Associate Professor, Duke University
Dr. Renee Wegrzyn, Program Manager, DARPA



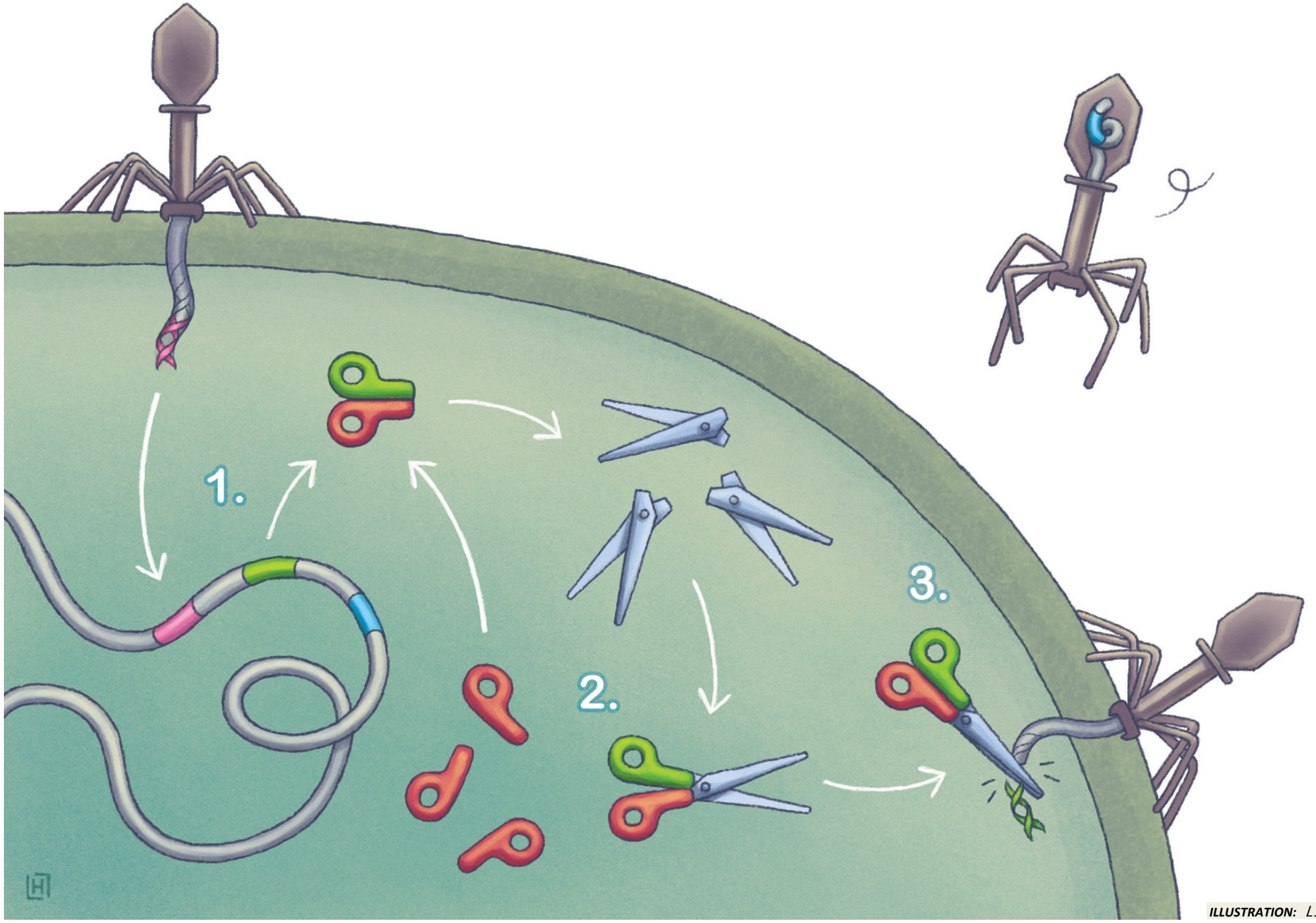
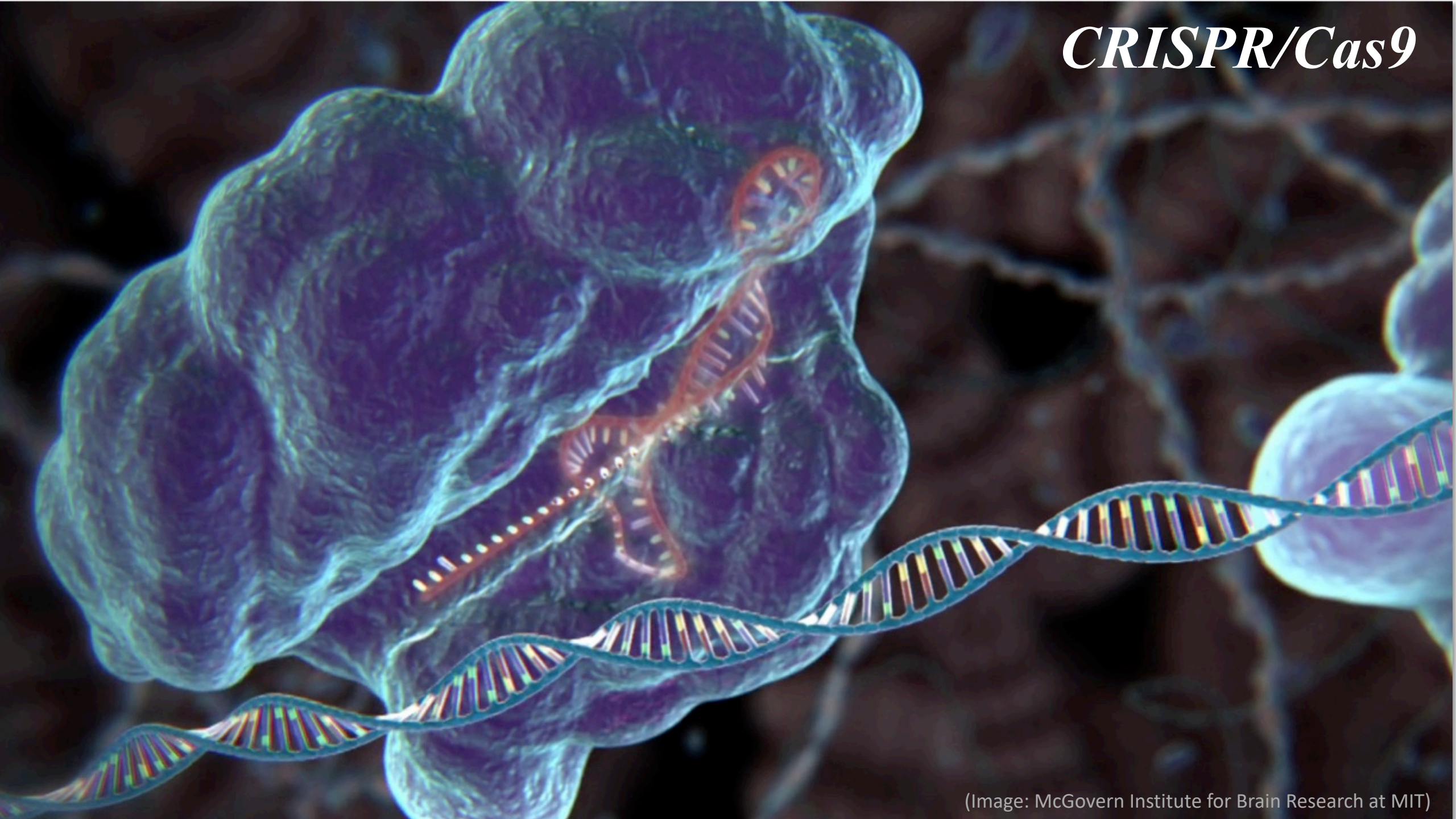


ILLUSTRATION: *LINNEA HOLMSTRÖM LJUNG*

CRISPR/Cas9



(Image: McGovern Institute for Brain Research at MIT)

The New York Times | http://nyti.ms/1NXrTiF

SCIENCE

Gene Editing Offers Hope for Treat Duchenne Muscular Dystrophy, Studies Find

By NICHOLAS WADE DEC. 31, 2015

THE NEW YORKER

ANNALS OF SCIENCE | NOVEMBER 16, 2015 ISSUE

THE GENE HACKERS

A powerful new technology enables us to manipulate our DNA more easily than ever before.

BY MICHAEL SPECTER

At thirty-four, Feng Zhang is the youngest member of the core faculty at the Broad Institute of Harvard and M.I.T. He is also among the most accomplished. In 1999, while still a

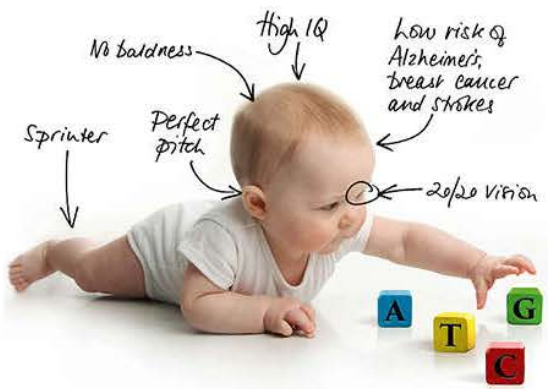
The Economist | World politics | Business & finance | Economics | Science & technology | Cultur

Genetic engineering

Editing humanity

A new technique for manipulating genes holds great promise—but rules are needed to govern its use

Aug 22nd 2015 | From the print edition | Timekeeper | Like 8K | Tweet



The New York Times | http://nyti.ms/1fCYbTG

HEALTH

A Powerful New Way to Edit DNA

THE WALL STREET JOURNAL.

LIFE | HEALTH | JOURNAL REPORTS: HEALTH CARE

Why Gene-Editing Technology Has Scientists Excited

Researchers explore the idea of treating disease by replacing defective genes

Economist | World politics | Business & finance | Economics | Science & technology | Cultu

Genome editing

The age of the red pen

It is now easy to edit the genomes of plants, animals and humans

Aug 22nd 2015 | From the print edition | Timekeeper | Like 2.2K | Tweet

The Economist | World politics | Business & finance | Economics | Science & technology | Cultur

Gene editing

Even CRISPR

A new way to edit DNA may speed the advance of genetic engineering

Oct 3rd 2015 | From the print edition | Timekeeper | Like 384 | Tweet

Gene Drives

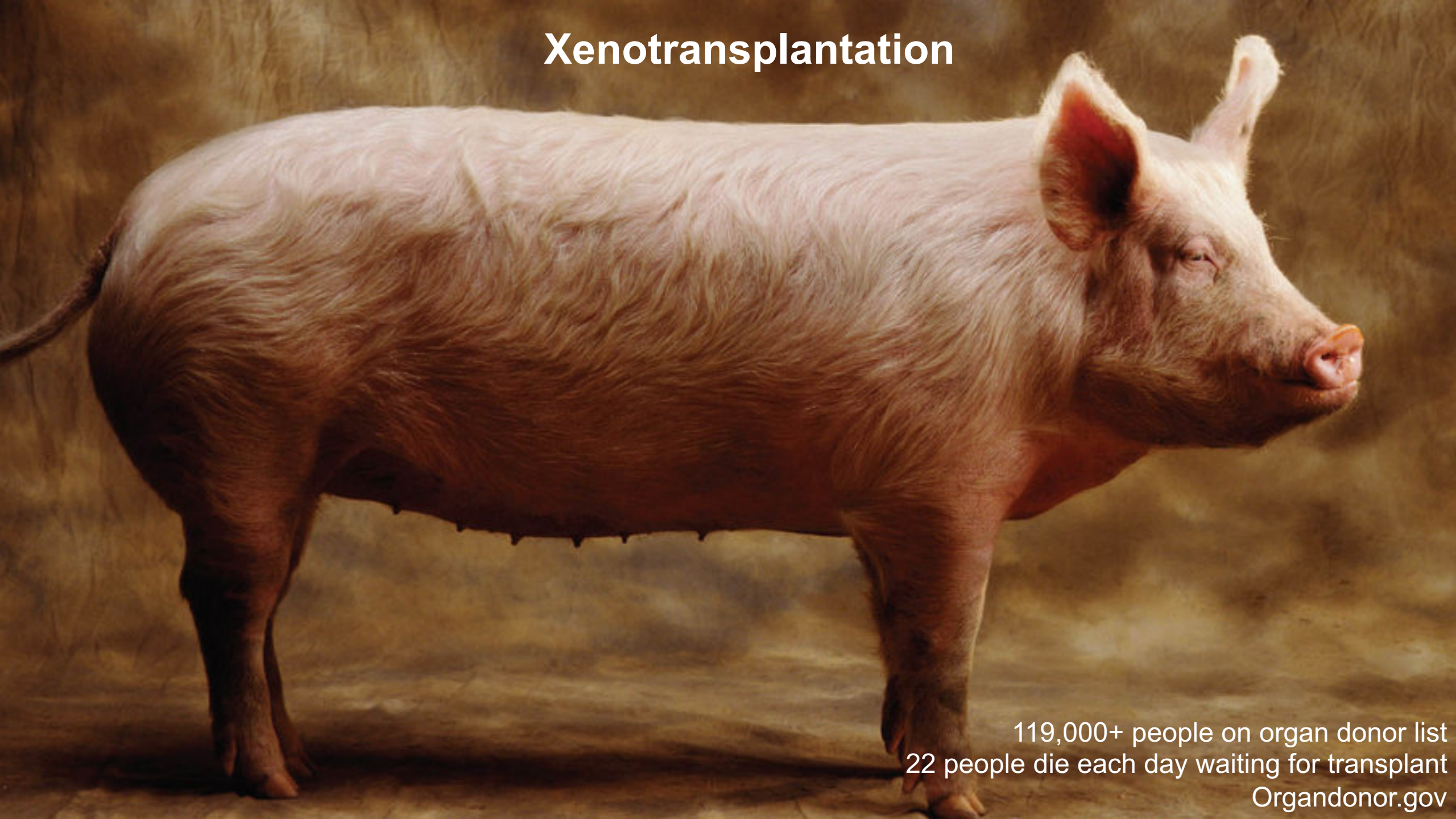
Malaria in 2015 - 212,000,000 cases, 429,000 deaths (WHO)
Zika virus – 1,845 infected pregnant women in US (CDC)

World Hunger & Genetically Modified Organisms (GMOs)

795 million people worldwide suffering from chronic undernourishment (1:9)

161 million with growth defects

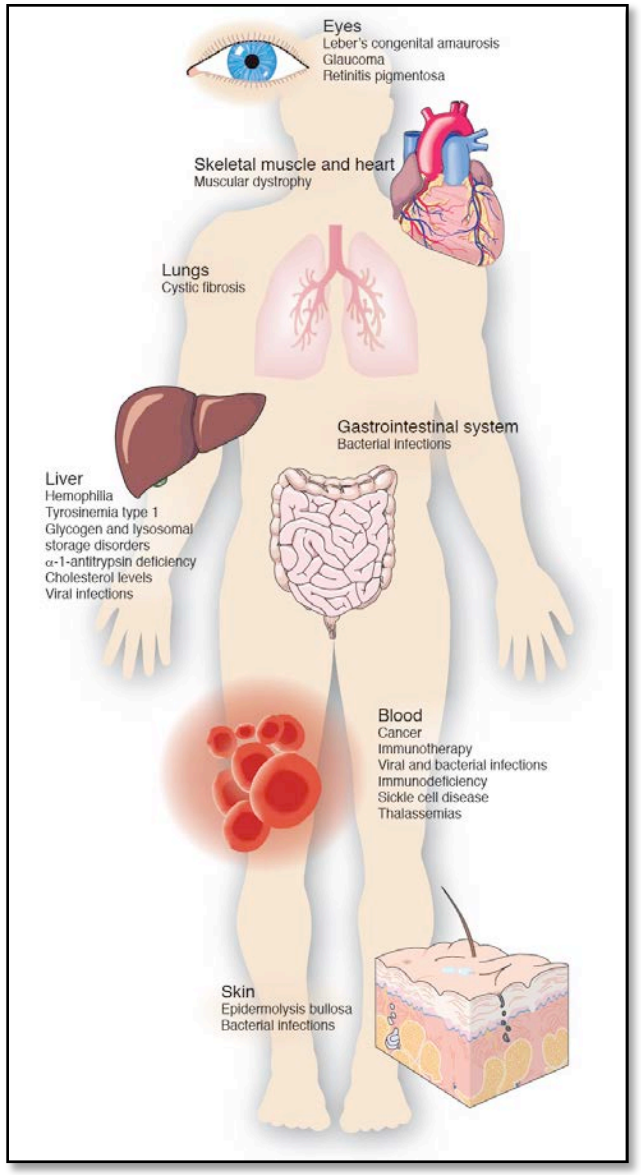
Xenotransplantation



119,000+ people on organ donor list
22 people die each day waiting for transplant
Organdonor.gov


GTAACGCCATTGAATGCCCATCGGATG

Therapeutic Gene Editing



- Cancer immunotherapy
- Viral infection
- Cystic fibrosis
- Blindness
- Glaucoma
- Hemophilia
- Enzyme deficiencies
- Bacterial infection
- Immunodeficiences
- Sickle cell disease
- Thalessemias
- Epidermolysis bullosa
- Muscular dystrophies

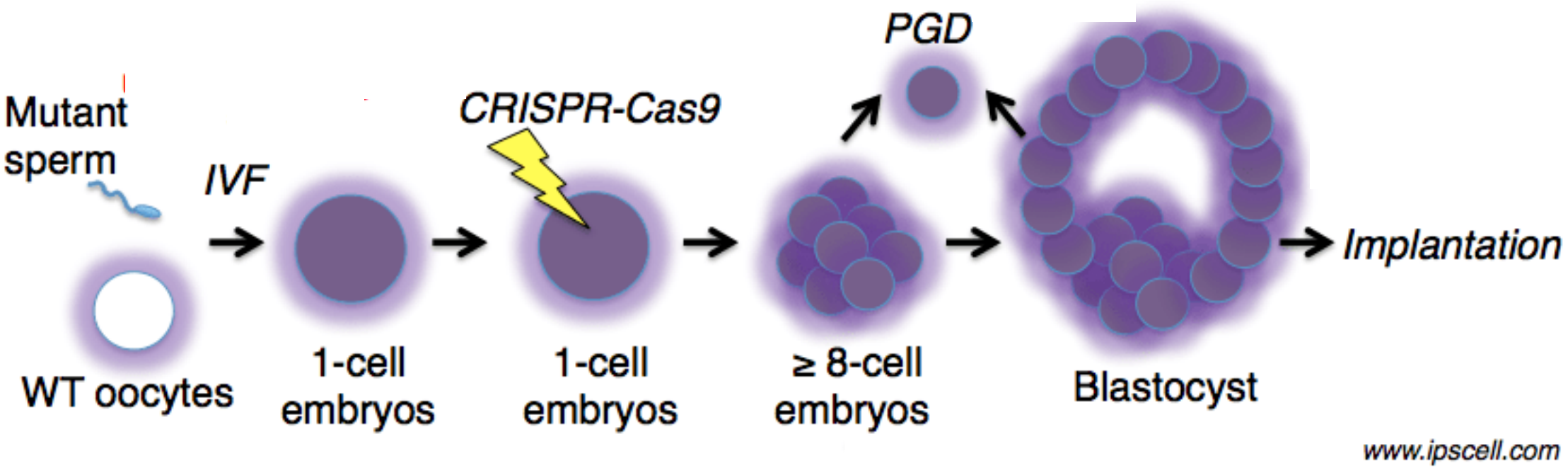
Maeder and Gersbach, *Molecular Therapy* (2016)

Somatic Cell Gene Editing Clinical Trials

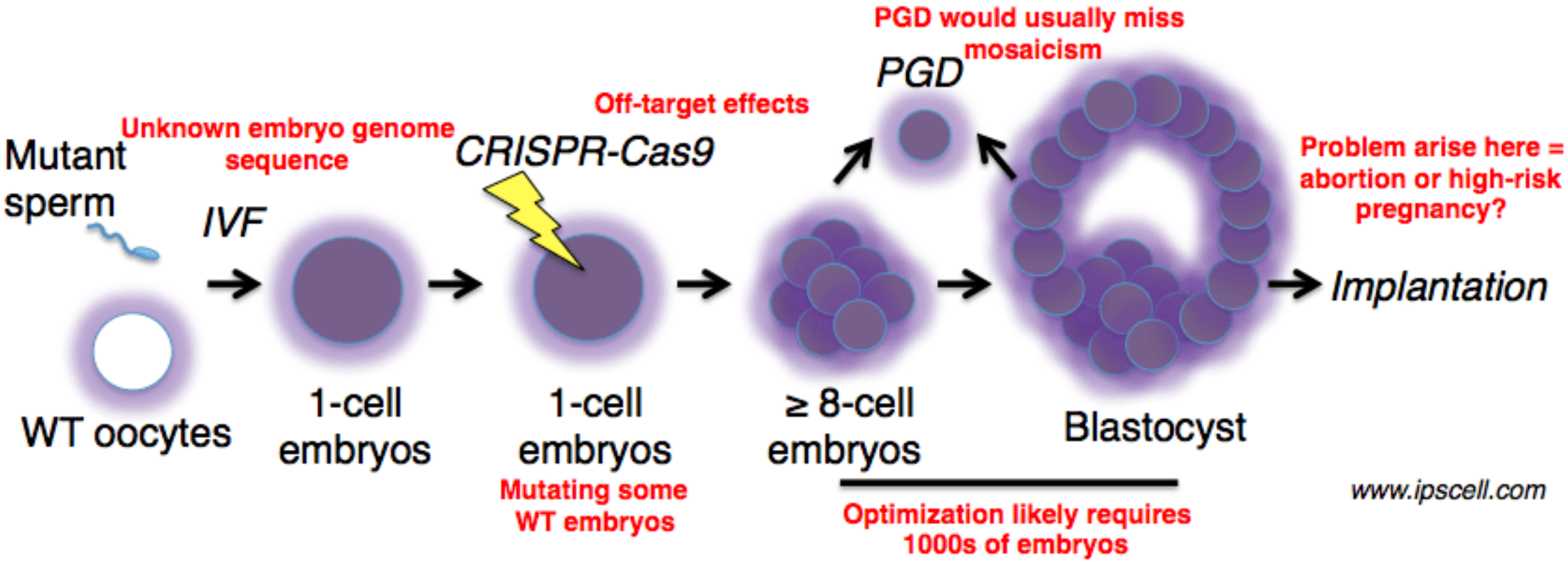
Trial #	Phase	Editor	Target	Gene	Disease	Sponsor	Status
NCT00842634	Phase 1	ZFNs	T cells	CCR5	HIV	Sangamo, Inc.	Completed
NCT01044654	Phase 1	ZFNs	T cells	CCR5	HIV	Sangamo, Inc.	Completed
NCT01082926	Phase 1	ZFNs	T cells	GR	Cancer	Sangamo, Inc.	Completed
NCT01252641	Phase ½	ZFNs	T cells	CCR5	HIV	Sangamo, Inc.	Completed
NCT01543152	Phase ½	ZFNs	T cells	CCR5	HIV	Sangamo, Inc.	Completed
NCT02225665	Phase ½	ZFNs	T cells	CCR5	HIV	Sangamo, Inc.	Active
NCT02500849	Phase 1	ZFNs	HSCs	CCR5	HIV	Sangamo, Inc.	Recruiting
NCT02695160	Phase 1	ZFNs	Liver	Albumin	Hemophilia B	Sangamo, Inc.	Recruiting
NCT02702115	Phase 1	ZFNs	Liver	Albumin	MPS I	Sangamo, Inc.	Recruiting
NCT03041324	Phase 1	ZFNs	Liver	Albumin	MPS II	Sangamo, Inc.	Recruiting
NCT02735083	Phase 1	TALENs	T cells	TCR & CD52	Cancer	Cellectis/Pfizer/Servier, Inc.	Enrolling
NCT03399448	Phase 1	CRISPR	T cells	TCR & PD-1	Cancer	Univ. Pennsylvania	Recruiting
NCT03690011	Phase 1	CRISPR	T cells	CD7	Cancer	Baylor	Not yet recruiting
NCT03655678	Phase ½	CRISPR	HSCs	Globin	Beta Thal	CRISPR Therapeutics	Recruiting
NCT03745287	Phase ½	CRISPR	HSCs	Globin	Sickle cell	CRISPR Therapeutics	Recruiting
	Phase 1	CRISPR	Retina	CEP290	LCA10	Editas Medicine	

Germline Gene Editing

Germline/Embryo Editing



Germline/Embryo Editing



Germline Gene Editing

Rewriting Life

EXCLUSIVE: Chinese scientists are creating CRISPR babies

A daring effort is under way to create the first children whose DNA has been tailored using gene editing.

by Antonio Regalado November 25, 2018

AP

Chinese researcher claims first gene-edited babies

By MARILYNN MARCHIONE November 26, 2018