

Engineering the Genome

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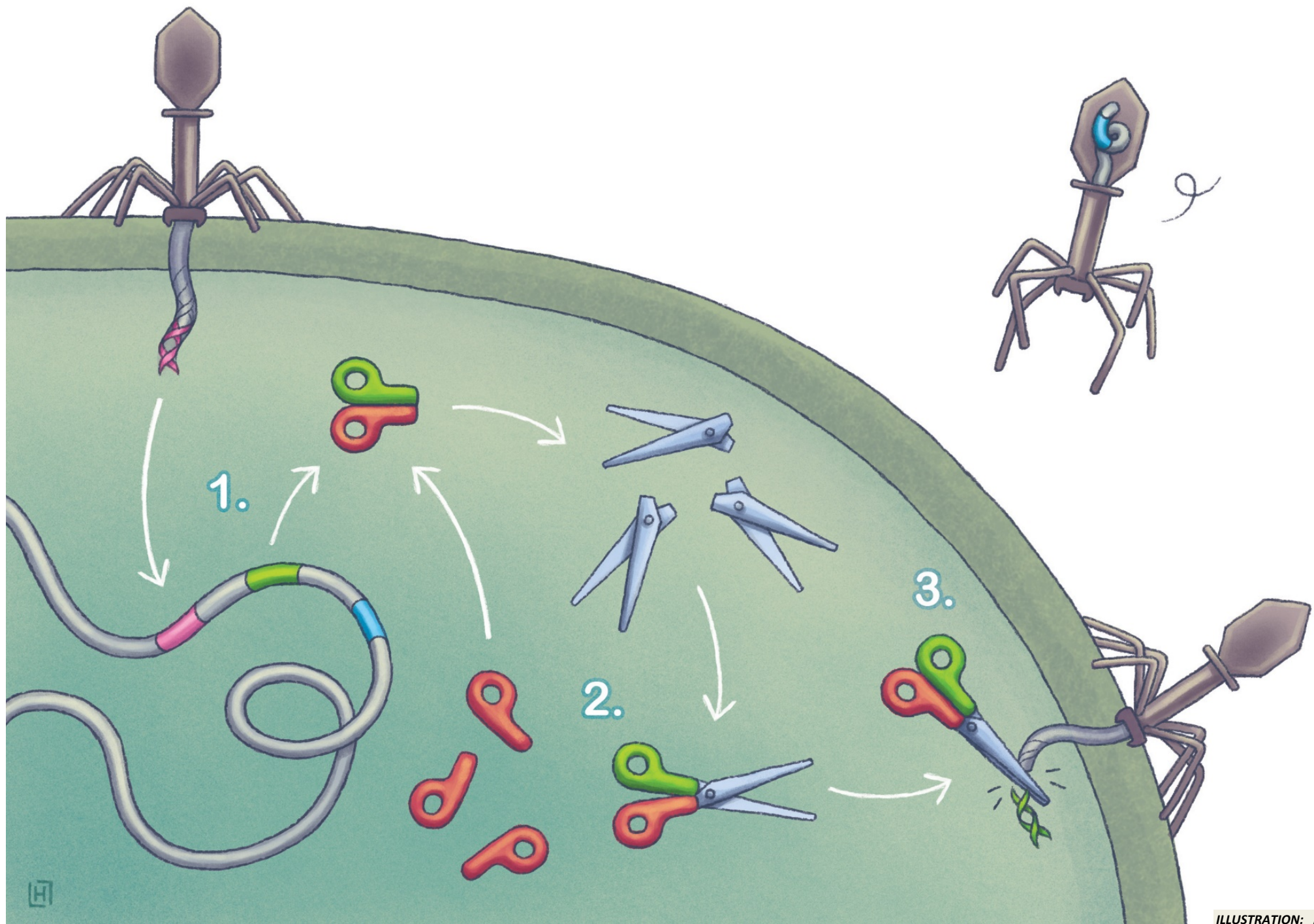




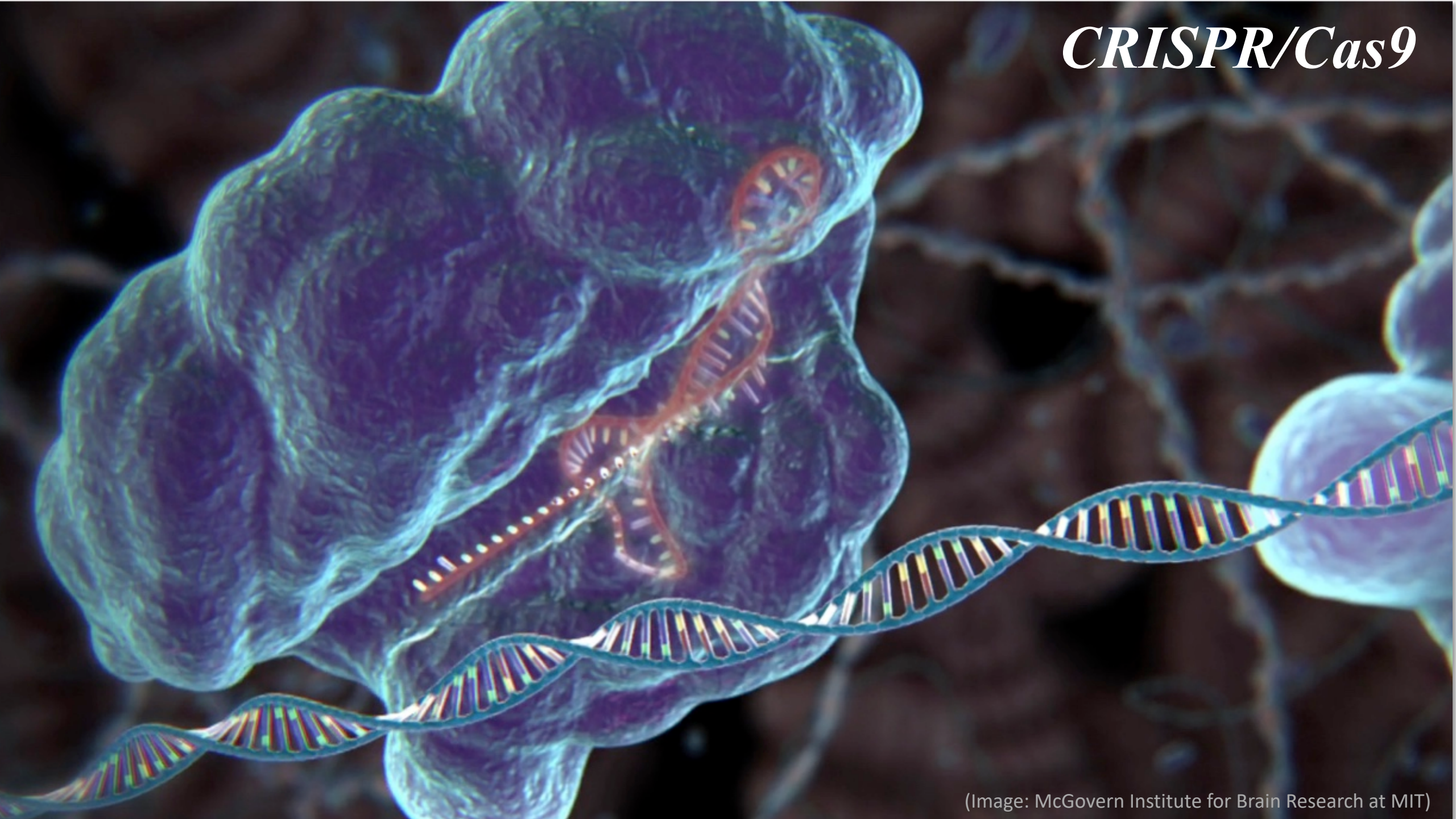


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CRISPR/Cas9



(Image: McGovern Institute for Brain Research at MIT)

SCIENCE

Gene Editing Offers Hope for Treatment of Duchenne Muscular Dystrophy, Studies Find

By NICHOLAS WADE DEC. 31, 2015



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

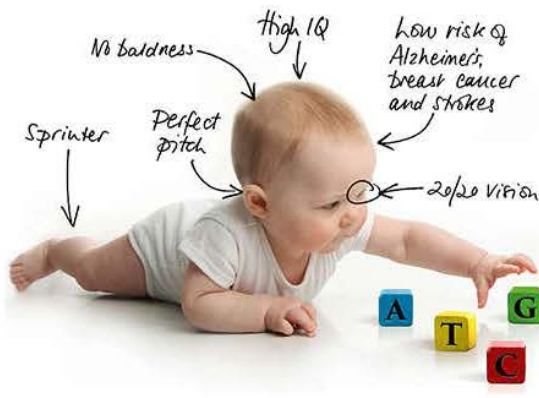


Genetic engineering

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



Shutterstock

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

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



Gene editing

Even CRISPR

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

Oct 3rd 2015 | From the print edition



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



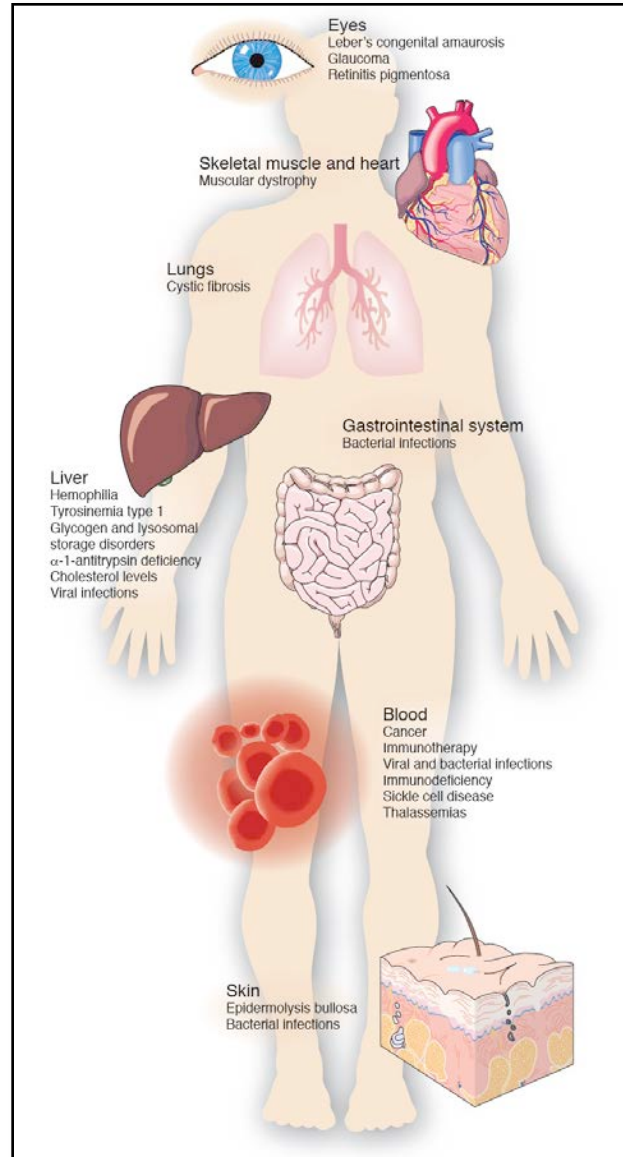
Xenotransplantation



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



Therapeutic Gene Editing



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

Maeder and Gersbach, *Molecular Therapy* (2016)

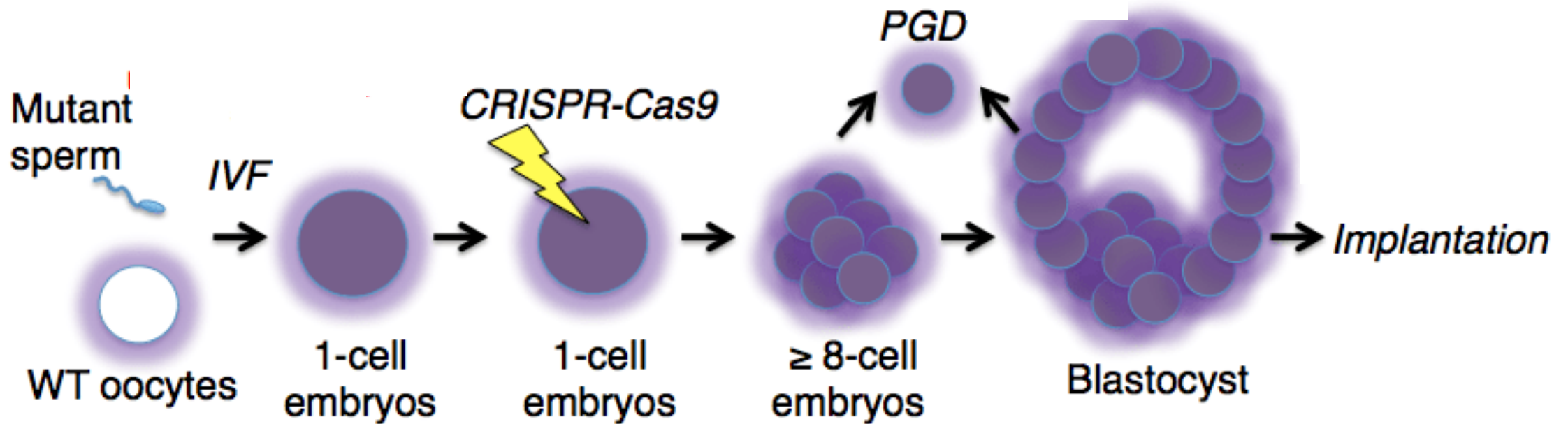
Somatic Cell Gene Editing Clinical Trials

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	Collectis/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	

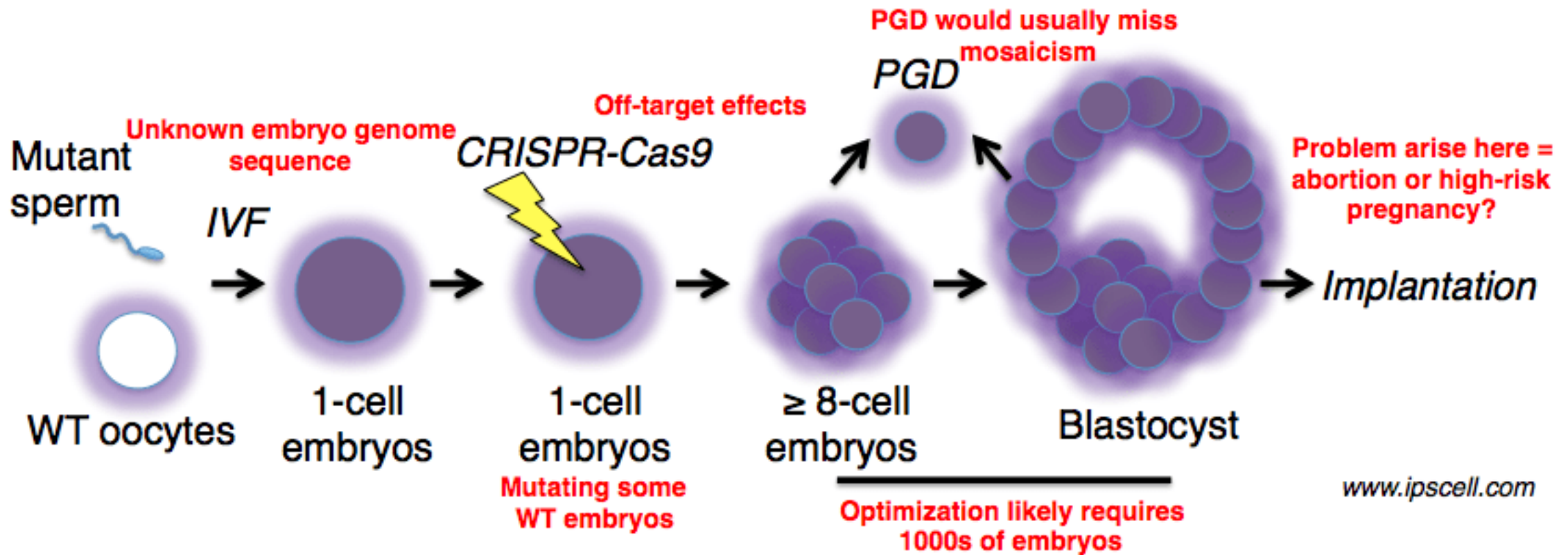
Germline Gene Editing



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