Advancing Gene Editing Medicines from N=1 to N=many: the Unique Urgency and Opportunity of the Present Moment

Fyodor Urnov

Professor of Molecular Therapeutics, Department of Molecular and Cell Biology

Director, Innovative Genomics Institute, UC Berkeley

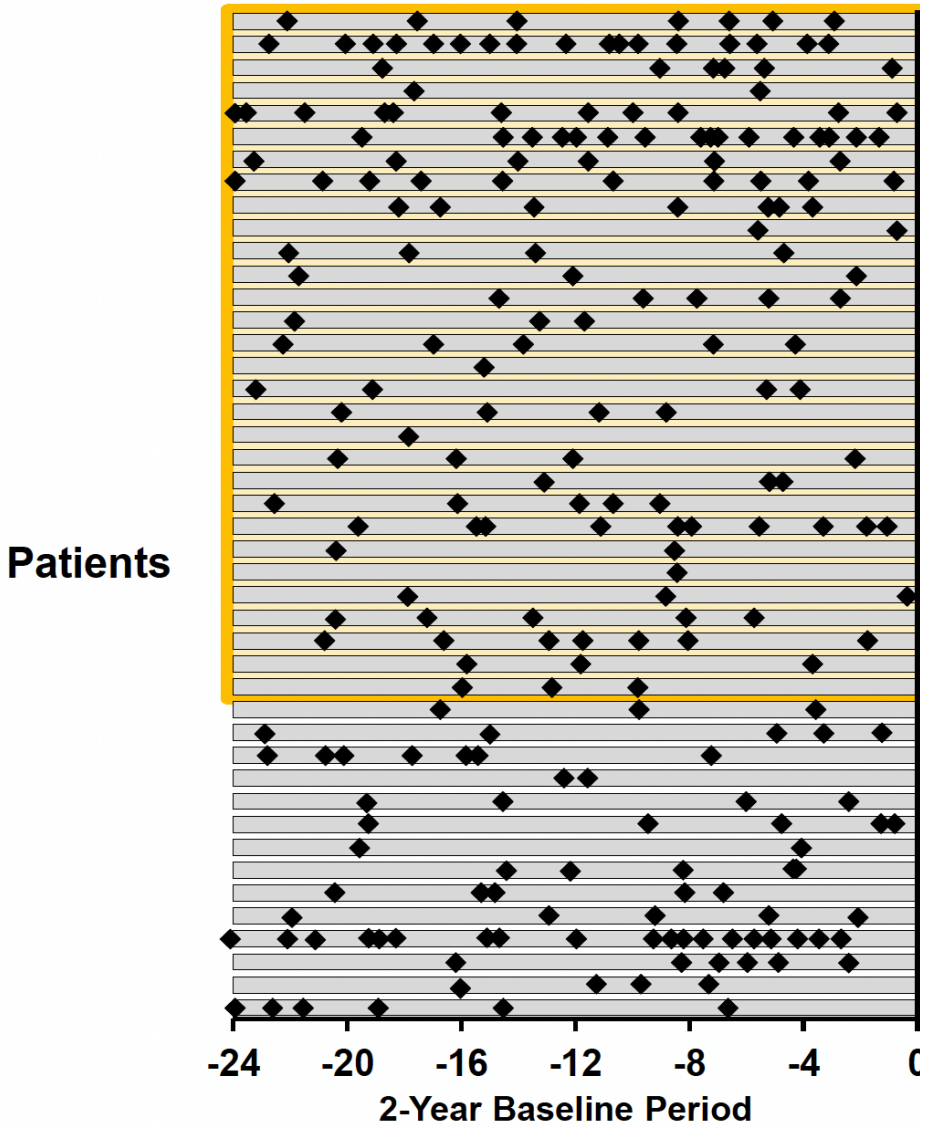
Director, IGI-Danaher Beacon for CRISPR Cures

Director, CZI-IGI Center for Pediatric CRISPR Cures

Fyodor Urnov: disclosures

- Cimeio Therapeutics: SAB chair, paid advisor, hold equity
- Evox Therapeutics: SAB member, paid advisor
- Ionis Pharmaceuticals: paid advisor
- Tune Therapeutics: scientific co-founder, paid advisor, hold equity
- Aurora Therapeutics: scientific co-founder, paid advisor, hold equity
- Vertex Pharmaceuticals: paid consultant on exa-cel program
- Danaher Corporation: salary and research support for work as Director of the IGI-Danaher Beacon for CRISPR Cures

Exa-cel Exhibited Durable Effect in Avoiding Inpatient Hospitalizations Due to VOCs



FAS

Approved Cellular and Gene Therapy Products

Below is a list of licensed products from the Office of Tissues and Advanced Therapies (OTAT).

Approved Products

- ABECMA (idecabtagene vicleucel)
  Celgene Corporation, a Bristol-Myers Squibb Company
- ADSTILADRIN
  Ferring Pharmaceuticals A/S
- ALLOCORD (HPC, Cord Blood)
  SSM Cardinal Glennon Children's Medical Center
- BREYANZI
  Juno Therapeutics, Inc., a Bristol-Myers Squibb Company
- CARVYKTI (ciltacabtagene autoleucel)
  Janssen Biotech, Inc.
- CASGEVY
  Vertex Pharmaceuticals Incorporated
- CLEVECORD (HPC Cord Blood)
  Cleveland Cord Blood Center
- Ducord, HPC Cord Blood
  Duke University School of Medicine
- ELEVIDYS
  delandistrogene moxeparvovec
- GINTUIT (Allogeneic Cultured Keratinocytes and Fibroblasts in Bovine Collagen)
  Organogenesis Incorporated
- HEMACORD (HPC, cord blood)
  New York Blood Center
- HEMGENIX
  CSL Behring LLC
- HPC, Cord Blood
  Clinimmune Labs, University of Colorado Cord Blood Bank
- HPC, Cord Blood - MD Anderson Cord Blood Bank
  MD Anderson Cord Blood Bank
- HPC, Cord Blood - LifeSouth
  LifeSouth Community Blood Centers, Inc.
- HPC, Cord Blood - Bloodworks
  Bloodworks
- IMLYGIC (talimogene laherparepvec)
  BioVex, Inc., a subsidiary of Amgen Inc.
- KYMRIAH (tisagenlecleucel)
  Novartis Pharmaceuticals Corporation
- LANTIDRA (donislecel)
  CellTrans Inc.
- LAVIV (Azficel-T)
  Fibrocell Technologies
- LUXTURNA
  Spark Therapeutics, Inc.
- LYFGENIA
  bluebird bio, Inc.
- MACI (Autologous Cultured Chondrocytes on a Porcine Collagen Membrane)
  Vericel Corp.
- OMISIRGE (omidubicel-onlv)
  Gamida Cell Ltd.
- PROVENGE (sipuleucel-T)
  Dendreon Corp.
- RETHYMIC
  Enzyvant Therapeutics GmbH
- ROCTAVIAN (valoctocogene roxaparvovec-rvox)
  BioMarin Pharmaceutical Inc
- SKYSONA (elivaldogene autotemcel)
  bluebird bio, Inc.
- STRATAGRAFT
  Stratatech Corporation
- TECARTUS (brexucabtagene autoleucel)
  Kite Pharma, Inc.
- VYJUVEK
  Krystal Biotech, Inc.
- YESCARTA (axicabtagene ciloleucel)
  Kite Pharma, Incorporated
- ZYNTEGLO (betibeglogene autotemcel)
  bluebird bio, Inc.
- ZOLGENSMA (onasemnogene abeparvovec-xioi)
  Novartis Gene Therapies, Inc.

CASGEVY

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STN: 125787, 125785
Proper Name: exagamglogene Autotemcel (exa-cel)
Tradename: CASGEVY
Manufacturer: Vertex Pharmaceuticals Incorporated

Indication:

- STN 125787 - Treatment of sickle cell disease (SCD) in patients 12 years and older with recurrent vaso occlusive crises (VOCs).
- STN 125785 - Indicated for the treatment of patients aged 12 years and older with sickle cell disease (SCD) with recurrent vaso occlusive crises (VOCs) and transfusion-dependent ß-thalassemia (TDT).

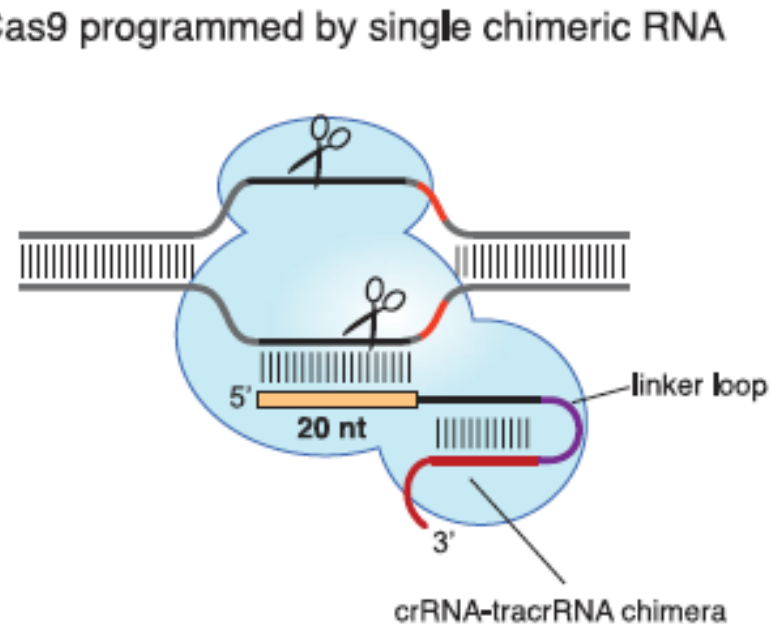
Victoria Gray

https://www.npr.org/sections/health-shots/2023/12/25/1219342935/sickle-cell-patients-journey-leads-to-landmark-approval-of-gene-editing-treatmen

June 28 2012

A Programmable Dual-RNA–Guided DNA Endonuclease in Adaptive Bacterial Immunity

Martin Jinek,[1,2]* Krzysztof Chylinski,[3,4]* Ines Fonfara,[4] Michael Hauer,[2]†
Jennifer A. Doudna,[1,2,5,6]‡ Emmanuelle Charpentier[4]‡



We … show that the Cas9 endonuclease can be programmed … to target and cleave **any dsDNA sequence of interest** … by changing the DNA target-binding sequence in the guide chimeric RNA. … Cas9 … **could offer considerable potential** for gene-targeting and genome-editing applications.

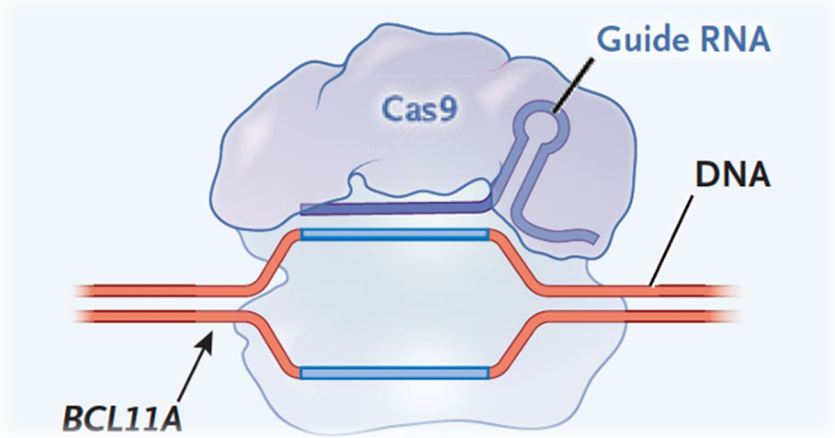
December 5 2020

The NEW ENGLAND JOURNAL of MEDICINE

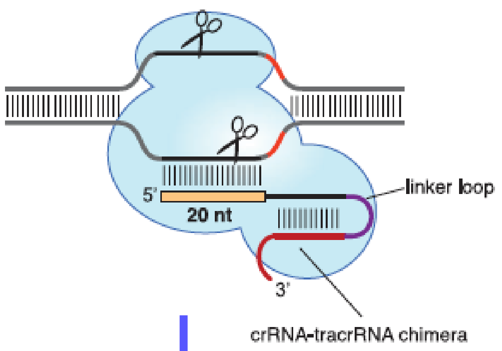
BRIEF REPORT

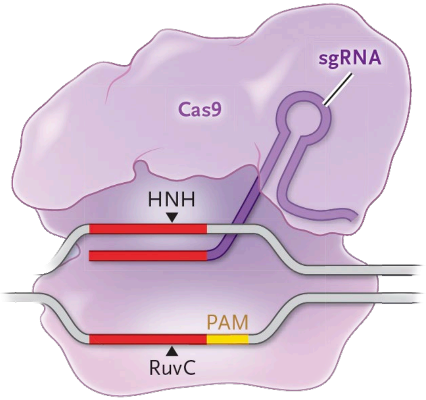
CRISPR-Cas9 Gene Editing for Sickle Cell Disease and β-Thalassemia

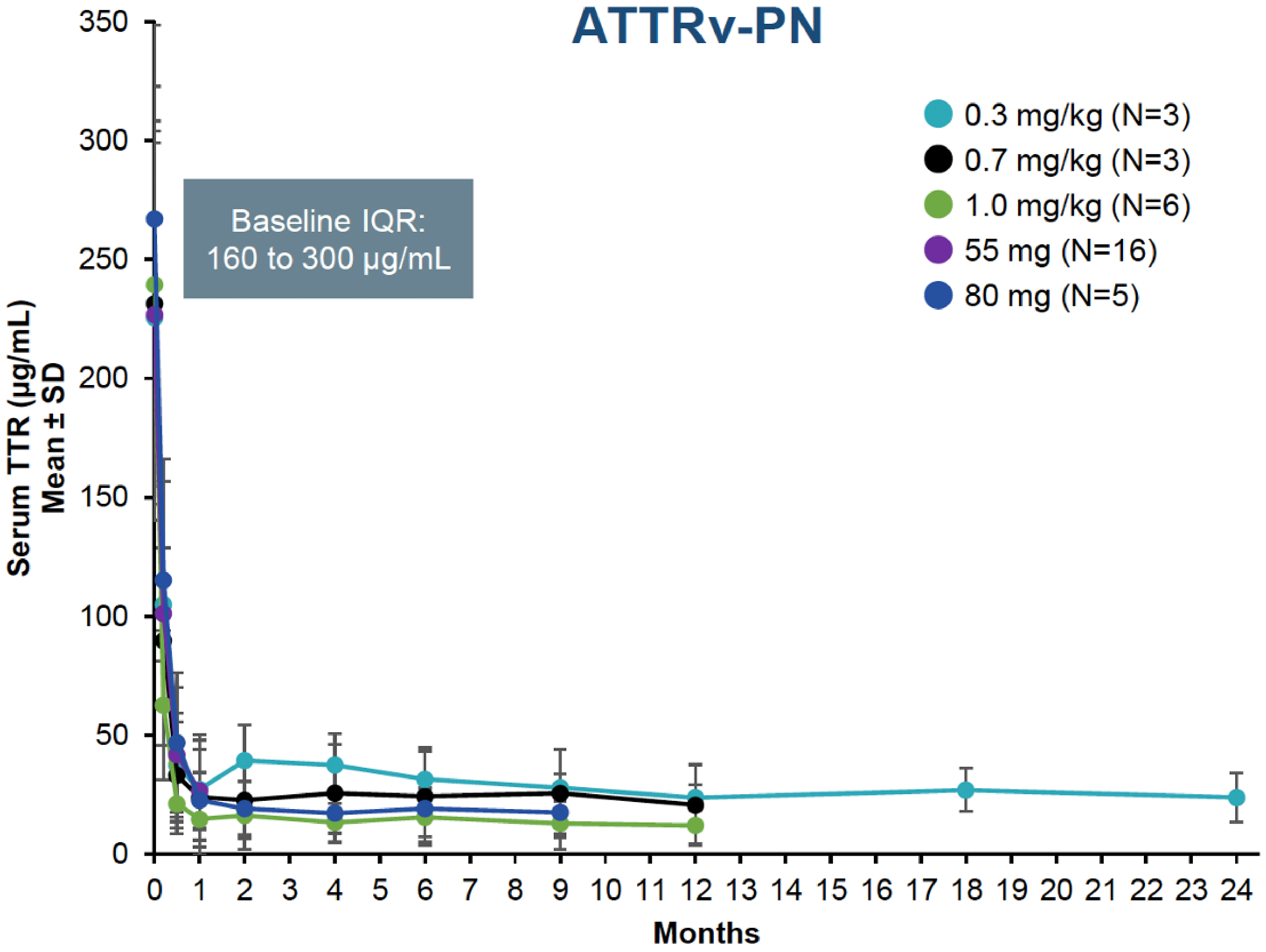
H. Frangoul, D. Altshuler, M.D. Cappellini, Y.-S. Chen, J. Domm, B.K. Eustace, J. Foell, J. de la Fuente, S. Grupp, R. Handgretinger, T.W. Ho, A. Kattamis, A. Kernytsky, J. Lekstrom-Himes, A.M. Li, F. Locatelli, M.Y. Mapara, M. de Montalembert, D. Rondelli, A. Sharma, S. Sheth, S. Soni, M.H. Steinberg, D. Wall, A. Yen, and S. Corbacioglu



Cas9 programmed by single chimeric RNA

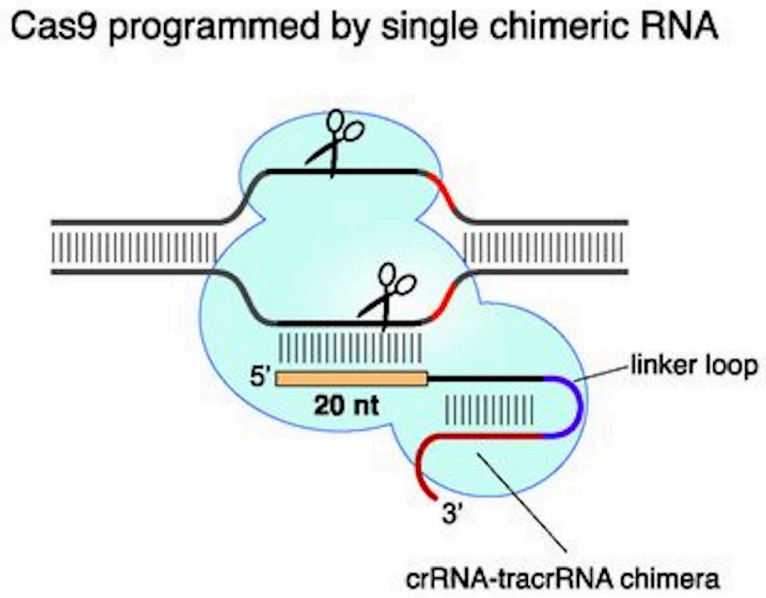


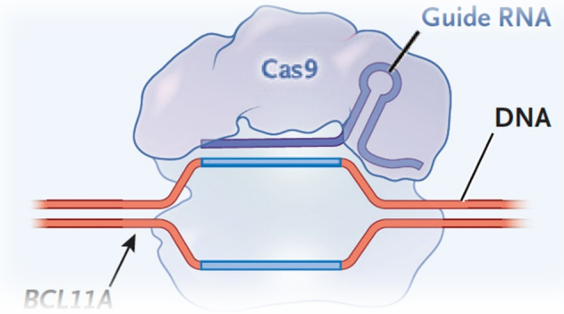


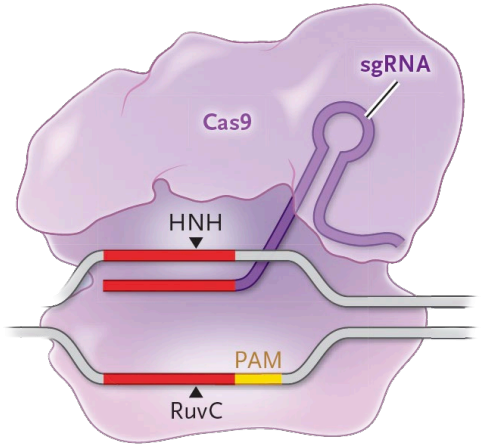


Jinek et al *Science* (2012) 337: 816-821 | Gillmore *NEJM* 2021

CRISPR-Cas Gene Editing Has Exceeded Expectations as a One-Time Curative Treatment for Severe Mendelian Disease in Four Indications







The Genome Editors' Toolbox Is In "Superbloom"

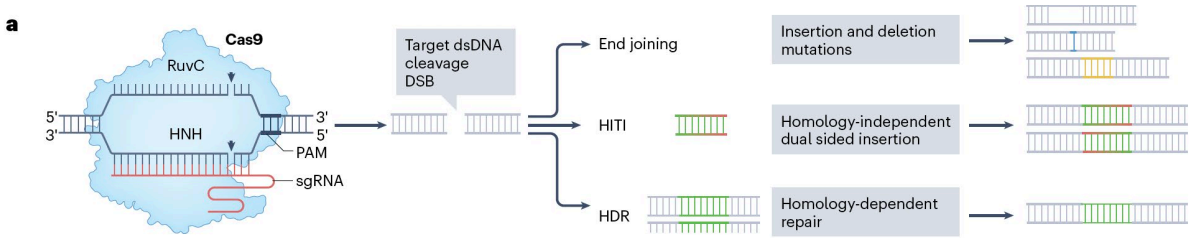


Table 1. Mendelian disease indications currently in the development pipeline of publicly traded gene-editing companies relying on CRISPR-Cas technology

Company	*Mendelian disease indications in the clinic*	*Entering clinic in 2025*
Editas Medicine	Sickle cell disease and transfusion-dependent beta-thalassemia (TDT)	None
CRISPR Therapeutics	Familial hypercholesterolemia (FH) + 3 related	Acute hepatic porphyria
Intellia Therapeutics	TTR amyloidosis (ph 3) Hereditary angioedema (ph 3) Alpha-1 antitrypsin deficiency (targeted integration) Factor IX deficiency (targeted integration)	None
Beam Therapeutics	Sickle cell disease and TDT Alpha-1 antitrypsin deficiency (E342K mutation) Glycogen storage disease 1A (R83C mutation)	None
Verve Therapeutics	Heterozygous familial hypercholesterolemia (FH)	Homozygous FH
Prime Medicine	Chronic granulomatous disease (A314B mutation)	None

All information obtained from company web sites on Oct 7, 2024. Approved (e.g., Casgevy), oncology [i.e., chimeric antigen receptor T cell (CAR-T)], and common (e.g., cardiovascular) disease indications are not included.

Urnov F. (2024) Give Cas a Chance: an Actionable Path to a Platform For CRISPR Cures. *The CRISPR Journal* **7:** 212-219.

Table 1. Mendelian disease indications currently in the development pipeline of publicly traded gene-editing companies relying on CRISPR-Cas technology

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Editas Medicine	HYPERLIPIDEMIA	
CRISPR Therapeutics	Familial hypercholesterolemia (FH) + 3 related	Acute hepatic porphyria
Intellia Therapeutics	TTR amyloidosis (ph 3)	None
	Hereditary angioedema (ph 3)	
	Alpha-1 antitrypsin deficiency (targeted integration)	
	Factor IX deficiency (targeted integration)	
Beam Therapeutics	Sickle cell disease and TDT	None
	Alpha-1 antitrypsin deficiency (E342K mutation)	
	Glycogen storage disease 1A (R83C mutation)	
Verve Therapeutics	Heterozygous familial hypercholesterolemia (FH)	Homozygous FH
Prime Medicine	Alpha-1 antitrypsin deficiency, Wilsons Disease	

Pipeline contraction is *not* driven by CRISPR technology limitations:

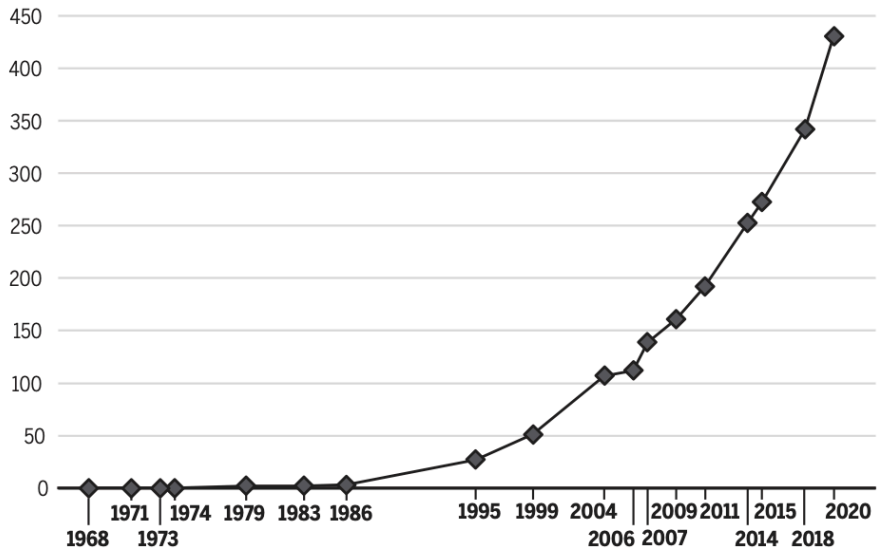
the challenge of clinic-grade on-target potency and genome-wide specificity has been robustly addressed.

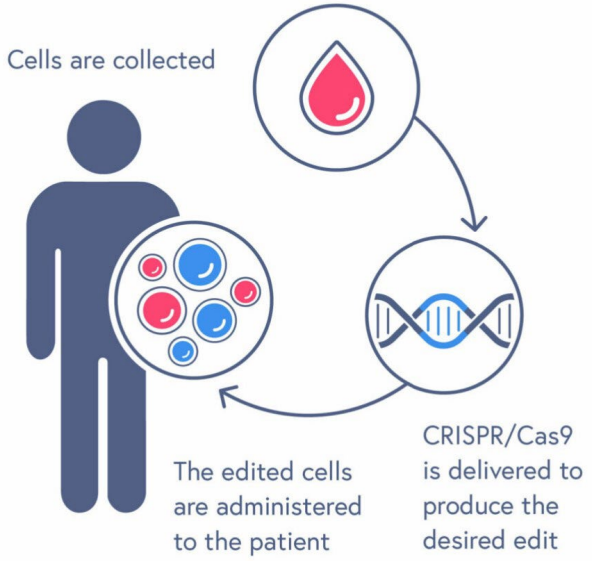
Despite Patient Availability As Well as Technical and Clinical Tractability, There Are ZERO Trials for CRISPR Gene Editing in the IEIs

> 110,000 known persons living with IEIs + > 500 known IEIs and growing! + Casgevy manufacturing process = 0

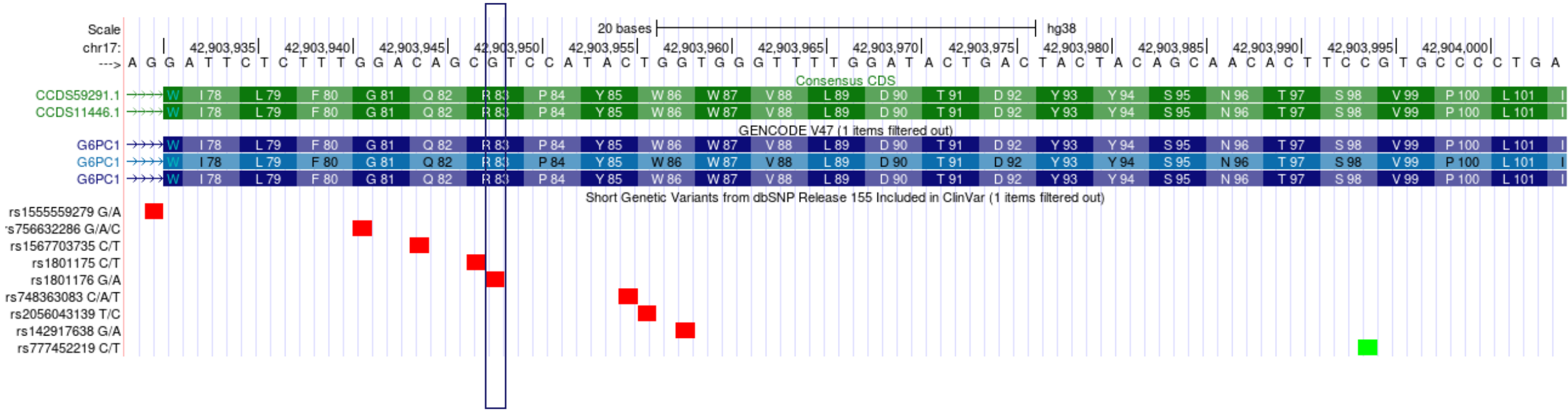
1.	ADA (Adenosine deaminase deficiency), AR	575
2.	AK2 (AK2 Defect), AR	42
3.	B2M (MHC class I deficiency), AR	11
4.	BCL10 (BCL10 deficiency), AR	4
5.	CARD 11 (CARD11 deficiency), AR LOF	31
6.	CD3D (CD3$_d$ deficiency), AR	54
7.	CD3E (CD3$_\varepsilon$ deficiency), AR	26
8.	CD3G (CD3$_\gamma$ deficiency), AR	13
9.	CD3Z (CD3$_\zeta$ deficiency), AR	6
10.	CD40 (CD40 deficiency), AR	104
11.	CD40LG (CD40 ligand (CD154) deficiency), XL	686
12.	CD8A (CD8 deficiency), AR	18
13.	CIITA (MHC class II deficiency group A, B, C, D), AR	97
14.	CORO1A (Coronin-1A deficiency), AR	15
15.	DCLRE1C (Artemis deficiency), AR	268
16.	DOCK2 (DOCK2 deficiency), AR	21
17.	DOCK8 (DOCK8 deficiency), AR	509
18.	FCHO1 (FCHO1 deficiency), AR	10
19.	ICOS (ICOS deficiency), AR	28
20.	ICOSLG (ICOSL deficiency), AR	3
21.	IKBKB (IKBKB deficiency), AR	32
22.	IKZF1 (IKAROS deficiency), AD DN	35
23.	IL21 (IL-21 deficiency), AR	2
24.	IL21R (IL-21R deficiency), AR	21
25.	IL2RG ($_{gc}$ Deficiency, γc SCID, CD132 deficiency), XL	818
26.	IL7R (IL7R$_a$ deficiency), AR	208
27.	ITK (ITK deficiency), AR	34
28.	JAK3 (JAK3 deficiency), AR	250
29.	LAT (LAT deficiency), AR	1
30.	LCK (LCK deficiency), AR	3

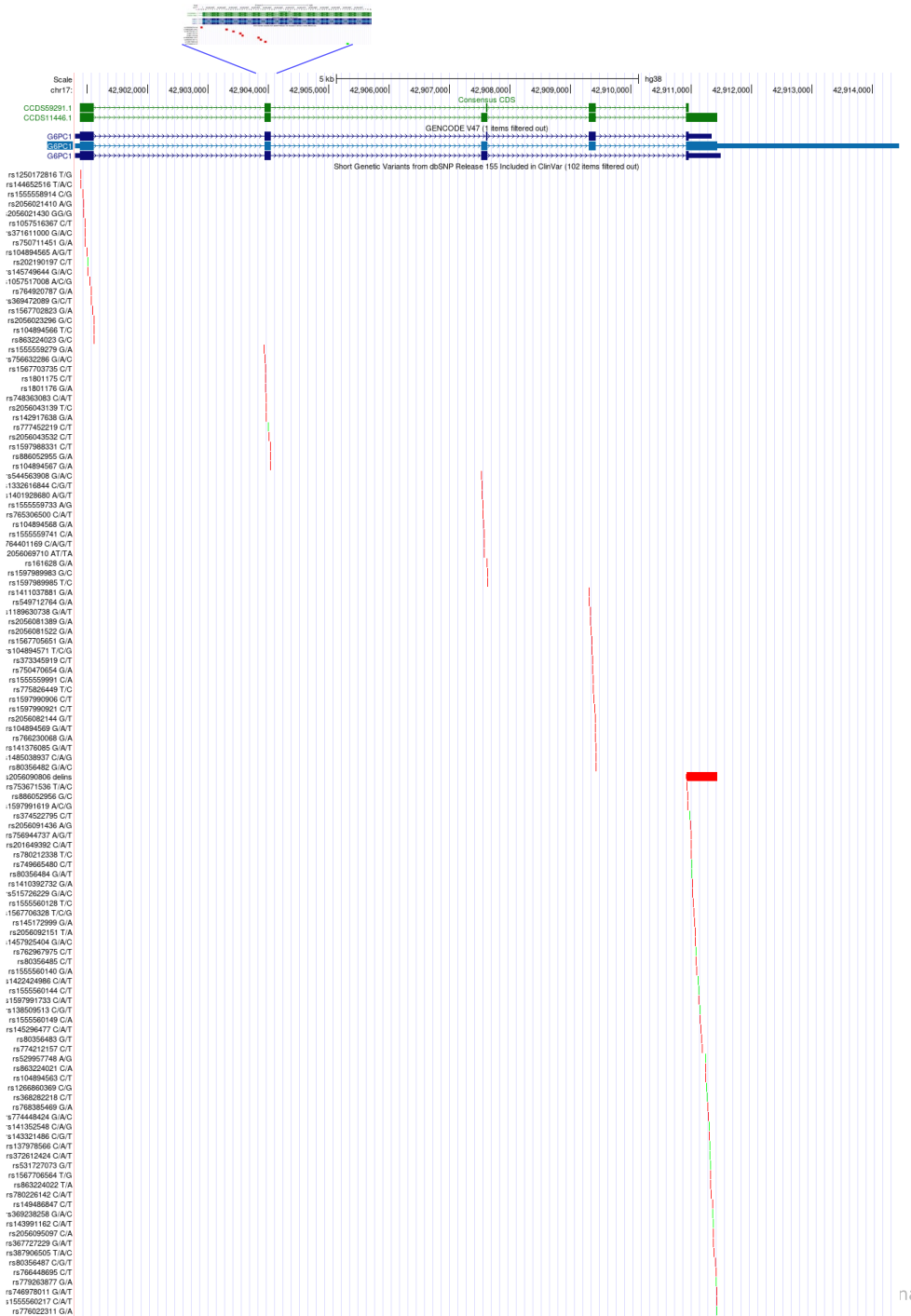
...





Glycogen storage disease 1A (R83C mutation)



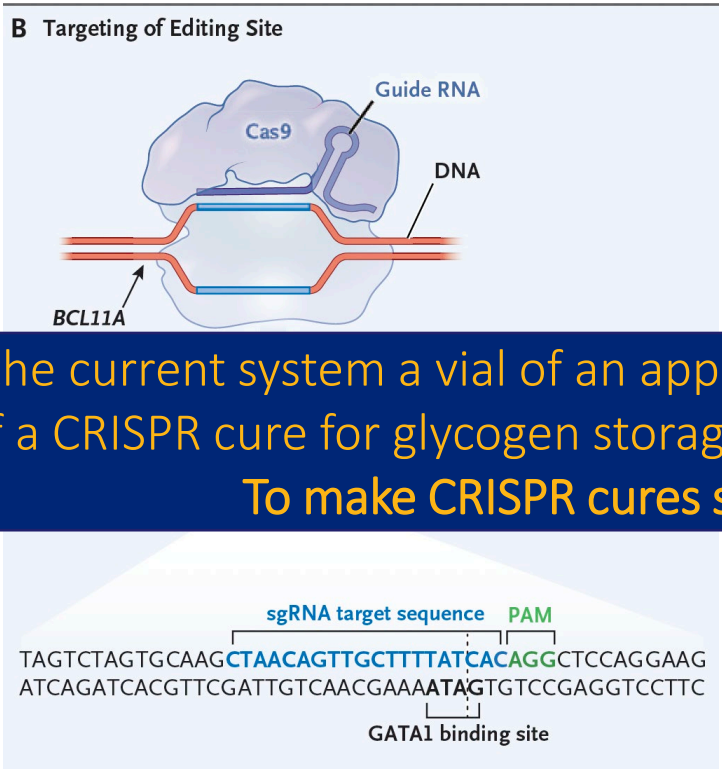


Classification of Glycogen Storage Disorder

GSDs that primarily affect the liver include the following:

- Glycogen synthase-2 deficiency (GSD type 0a)
- Glucose-6-phosphatase deficiency (GSD type Ia)
- Glucose-6-phosphate transporter deficiency (GSD type Ib)
- Glycogen debrancher deficiency (GSD type III)
- Glycogen branching enzyme deficiency (GSD type IV)
- Liver phosphorylase deficiency (GSD type VI)
- Phosphorylase kinase deficiency (GSD type IXa)
- GLUT2 deficiency or Fanconi-Bickel disease

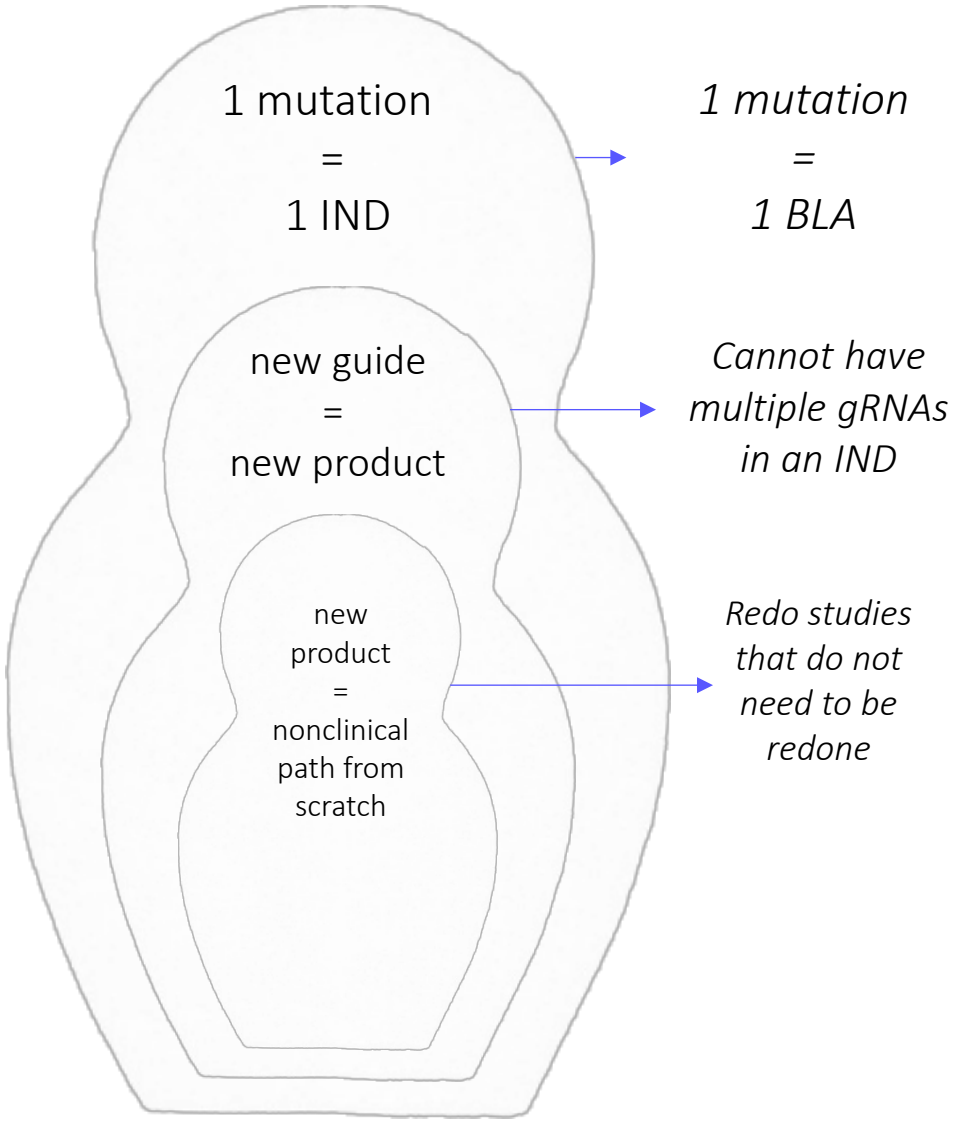
The APIs in Casgevy – Cas9 protein and gRNA – are a specific molecular entity that can be used to near-any person living with SCD or TDT



casgevy®

Under the current system a vial of an approved CRISPR medicine has to contain a single, specific guide RNA. A vial of a CRISPR cure for glycogen storage disease will thus treat a fraction of patients suffering from GSDs.

To make CRISPR cures successful we have to change that and we WILL.



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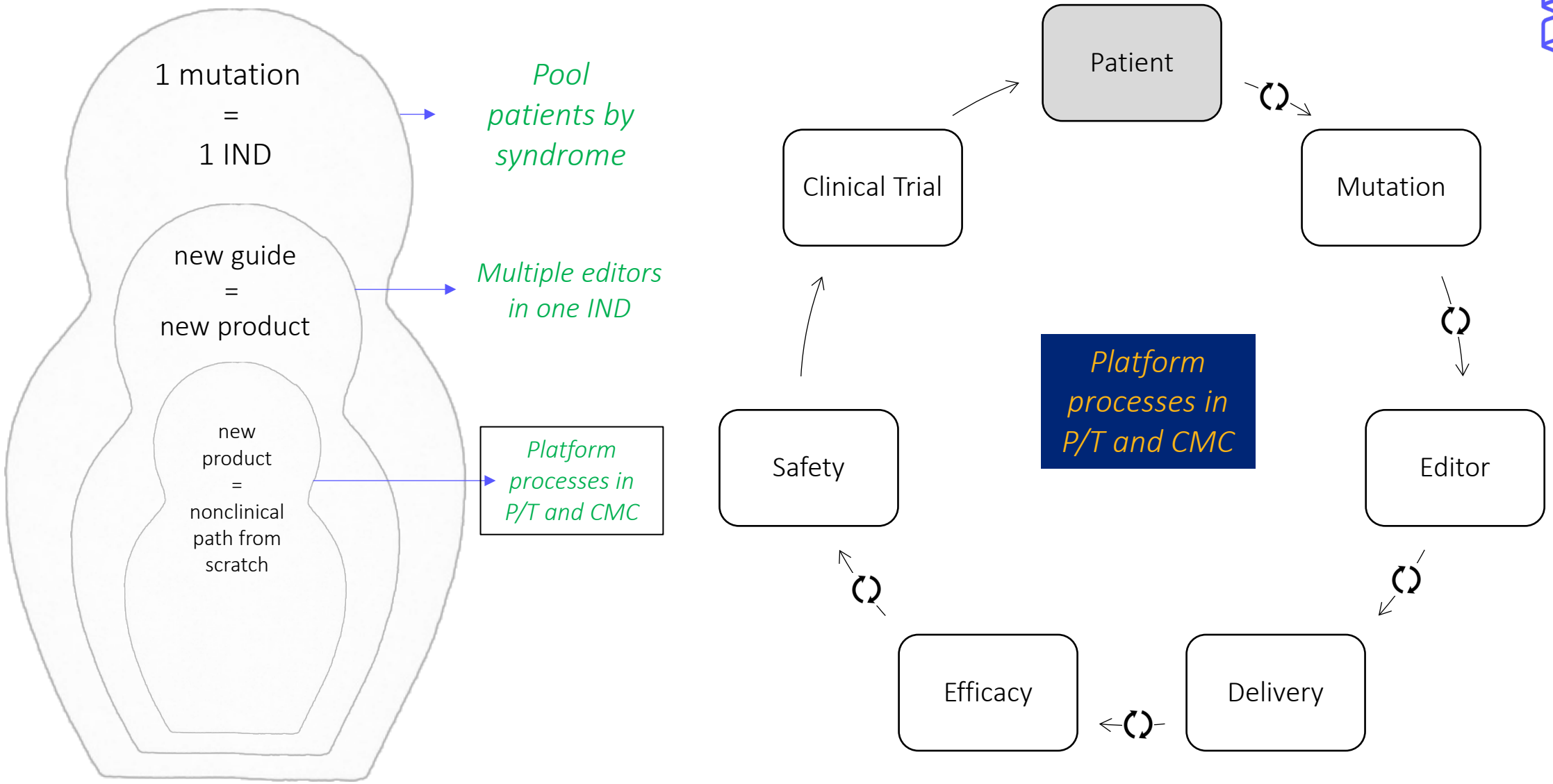


Urnov F. (2024) Give Cas a Chance: an Actionable Path to a Platform For CRISPR Cures. *The CRISPR Journal* **7:** 212-219.

$$1 - 0 = 1$$

$$2 - 1 < 1$$

$$3 - 2 <<< 1$$

With apologies to Brahmagupta and Al-Kwarizmi

2022: Intellia Therapeutics

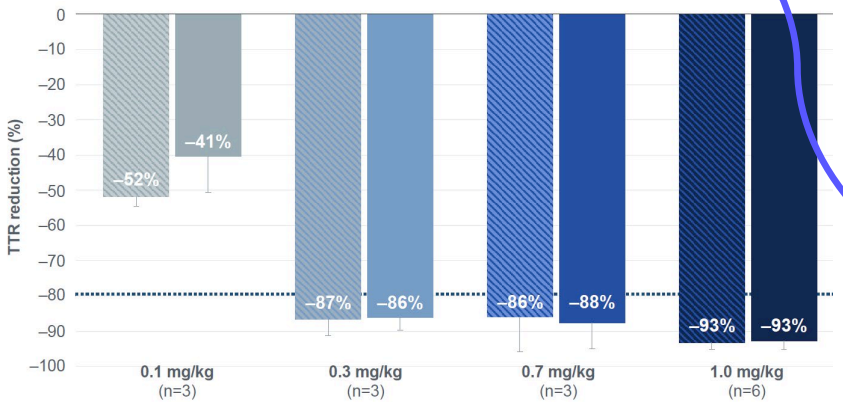
A Programmable Dual-RNA–Guided DNA Endonuclease in Adaptive Bacterial Immunity

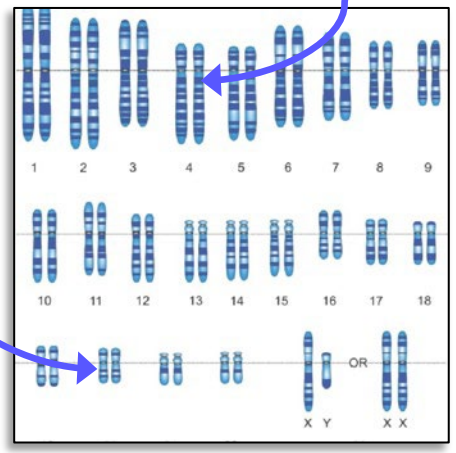
Martin Jinek,[1,2*] Krzysztof Chylinski,[3,4*] Ines Fonfara,[4] Michael Hauer,[2†] Jennifer A. Doudna,[1,2,5,6‡] Emmanuelle Charpentier[4‡]

CRISPR-Cas9 can be programmed to cut a specific sequence of DNA in a patient by simply changing the guide RNA

Disease 1: TTR Amyloidosis

Gene: ATTR on chr 20a

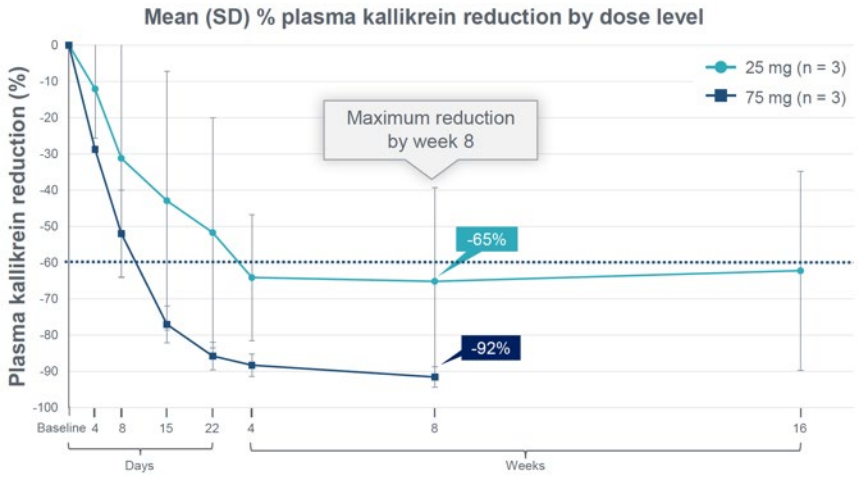




93% target gene knockout in patient liver

Disease 2: Hereditary Angioedema

Gene: KLKB1 on chr 4



92% target gene knockdown in patient liver

Q: your personal dream for CRISPR in next 5-10 years?

A: For CRISPR to become the standard of medical care for certain diseases.

Platform solutions for commercial challenges to expanding patient access and making gene editing sustainable

Sadik H. Kassim, Fyodor Urnov, Kiran Musunuru, Ann Lee, Luis Barrera, Pamela Stetkiewicz, Julianne Bruno, Matthew Hewitt, Troy Lister, Harry Malech, Lindsay Gasch, Matt Diver, Nicholas Gertler, Felix Grignon, Audrey Le, Michael Lehmicke, Edwin M. Horwitz, David R. Liu, Josephine Lembong & Vanessa Almendro-Navarro

Platform-based approaches for gene-editing therapies could markedly improve development efficiency, reduce costs and increase access for patients with rare diseases. Although gene editing has shown remarkable clinical success for a small number of Mendelian disease indications, broader adoption faces substantial hurdles. We propose strategies to overcome these challenges through modular platforms for nonclinical and chemistry, manufacturing and controls (CMC) data reuse, risk-based manufacturing quality, and streamlined umbrella clinical trials for regulatory efficiency and accelerated approval.

Nature Biotechnology 2025

Cytotherapy 2025

Advancing gene-editing platforms to improve the viability of rare-disease therapeutics: key insights from a 2024 Scientific Exchange hosted by ARM, ISCT, and Danaher

Fyodor Urnov[1,$], Sadik Kassim[2,$], Kiran Musunuru[3], David Liu[4,5,6], Ann Lee[7], Luis Barrera[8], Pam Stetkiewicz[9], Julianne Bruno[10], Matthew Hewitt[11], Troy Lister[12], Harry Malech[13], Lindsay Gasch[14], Matt Diver[15], Nicholas Gertler[15], Felix Grignon[16], Audrey Le[16], Michael Lehmicke[17], Vanessa Almendro-Navarro[2,†], Josephine Lembong[17,†,*]

[1] Innovative Genomics Institute, University of California, Berkeley, California, USA
[2] Danaher Corporation, Washington, District of Columbia, USA
[3] Perelman School of Medicine, University of Pennsylvania, Philadelphia, Pennsylvania, USA
[4] Merkin Institute of Transformative Technologies in Healthcare, Broad Institute of Harvard and MIT, Cambridge, Massachusetts, USA
[5] Department of Chemistry and Chemical Biology; Harvard University, Cambridge, Massachusetts, USA
[6] Howard Hughes Medical Institute, Harvard University, Cambridge, Massachusetts, USA
[7] Prime Medicine, Cambridge, Massachusetts, USA
[8] Beam Therapeutics, Cambridge, Massachusetts, USA
[9] Arbor Biotechnologies, Cambridge, Massachusetts, USA
[10] CRISPR Therapeutics, Boston, Massachusetts, USA
[11] Charles River Laboratories, Wilmington, Massachusetts, USA
[12] Verve Therapeutics, Boston, Massachusetts, USA
[13] National Institute of Allergy and Infectious Diseases, National Institutes of Health, Bethesda, Maryland, USA
[14] Capstone Medical Writing & Communications, Lander, Wyoming, USA
[15] Galen/Atlantica, Boston, Massachusetts, USA
[16] International Society for Cell & Gene Therapy, Vancouver, British Columbia, Canada
[17] Alliance for Regenerative Medicine, Washington, District of Columbia, USA
*Correspondence: Josephine Lembong, Alliance for Regenerative Medicine, 1015 18th Street NW, Suite 1102, Washington, DC 20036, USA. *E-mail address:* jlembong@alliancerm.org (J. Lembong).
[$]Co-first authors.
[†]Co-senior authors.

Table 3
Basic details of gene-editing platform case studies.

Presenting academic institution/sponsor/CDMO	Gene-editor/editing method	Delivery vehicle	Target cell type	Disease target
Innovative Genomics Institute/ Danaher Corporation	CRISPR/Cas nuclease[a]	Electroporation (*ex vivo*)[a]	T cell[a]	Inherited hemophagocytic lymphohistiocytosis
University of Pennsylvania	Base editor	LNP (*in vivo*)	Hepatocytes	Urea cycle disorder
Beam Therapeutics	Base editor	LNP (*in vivo*)	Hepatocytes	Glycogen storage disease type 1a
Prime Medicine	Prime editor	LNP (*in vivo*)	Hepatocytes	Wilson's disease[a]
Verve Therapeutics	Not specified	LNP (*in vivo*)	Not specified	Not specified
Arbor Biotechnologies	Small CRISPR/Cas nuclease	AAV (*in vivo*)	Motor neurons	Amyotrophic lateral sclerosis
CRISPR Therapeutics	CRISPR/Cas9[a]	LNP (*in vivo*)	HSCs	Sickle cell disease[a]
Charles River Laboratories[b]	CRISPR/Cas9	Electroporation (*ex vivo*)	T cells	N/A

Table 2. A Stepwise path to expand gene editing in the clinic

Approach	*v1*
Genes edited	One
Editing enzyme	One
Guide RNA	One
Patients eligible	5%

Urnov F. (2024) Give Cas a Chance: an Actionable Path to a Platform For CRISPR Cures. *The CRISPR Journal* **7:** 212-219.
Urnov, Kassim, et al (2025) Advancing gene-editing platforms to improve the viability of rare-disease therapeutics. *Cytotherapy.* 24: S1465

Benefit-risk proportionate development of CRISPR on-demand

“Benefit-risk appropriate path”

Dr Rebecca Ahrens-niklas
CHOP

Drs Cowan & Puck
UCSF

Dr Donald Kohn
UCLA

Dr Michelle Hermiston
UCSF

Dr Claire Booth
GOSH

Open IND for CRISPR in severe pediatric disease

Newborn diagnosed with a new mutation

path

Child dosed before death or severe disability

Case report: HLH due to PRF1 mutation

A 26-month-old previously well boy was brought to the emergency room by his parents with sudden onset of fever, runny nose (rhinorrhea), and decreased activity.
On hospital day 2, the patient developed a decreased level of consciousness and began seizing.
Diagnosed with hemophagocytic lymphohistiocytosis (HLH).
[compound heterozygous point mutations in *PRF1*]
On day 42, … developed acute pulmonary hemorrhage.
Despite extensive resuscitation efforts, the patient developed asystole and died on day 44.

Michelle Hermiston MD PhD
UCSF Health

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[compound heterozygous point mutations in *PRF1*]

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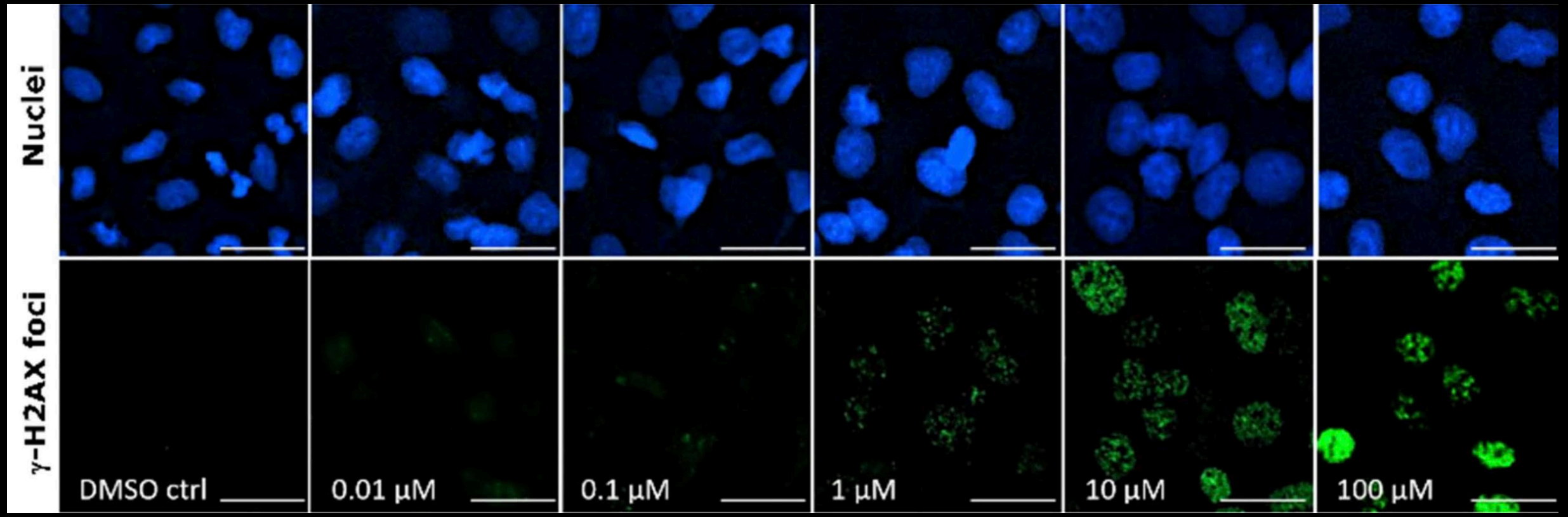
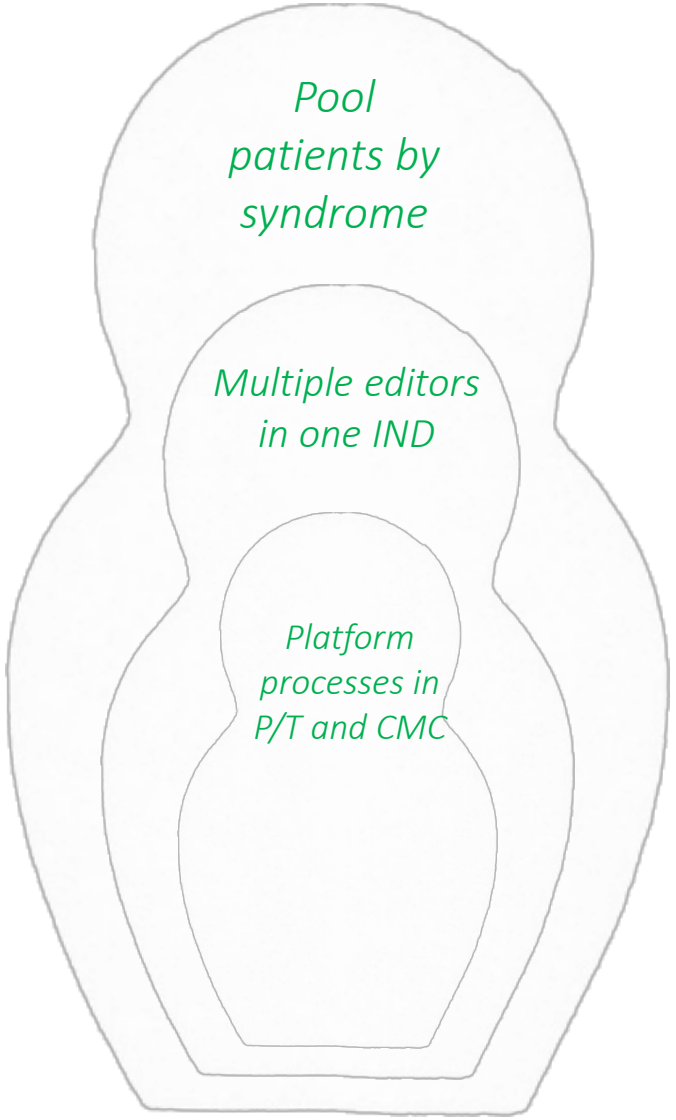
Michelle Hermiston MD PhD

UCSF Health

Infants with fHLH are treated with etoposide as SOC



30% of newborns with fHLH have 2 months to live



Enroll subjects with mutations in multiple genes that cause HLH

Establish a CRISRP-on-demand gene editor design / derisking pathway

Manufacturing scheme for critical reagents and DP identical between subjects

With David Nguyen, Russell Valle, Jerry Lee, Michelle Hermiston, Brian Shy, Petros Giannikopoulos, and Team Danaher (IDT, Aldevron)

Rebecca Ahrens-niklas, CHOP

Kiran Musunuru, Penn Medicine

Sadik Kassim, Danaher

The difference Baby KJ has made

June 5 2025

FORUM I VIRTUAL

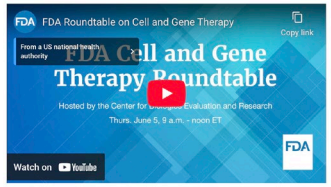
Cell and Gene Therapy Roundtable

JUNE 5, 2025

On This Page

- Meeting Information

Date: June 5, 2025
Time: 9:00 a.m. - 12:00 p.m. ET



Webcase Recording: FDA Roundtable on Cell and Gene Therapy

Media Inquiries: HHS Press Office.

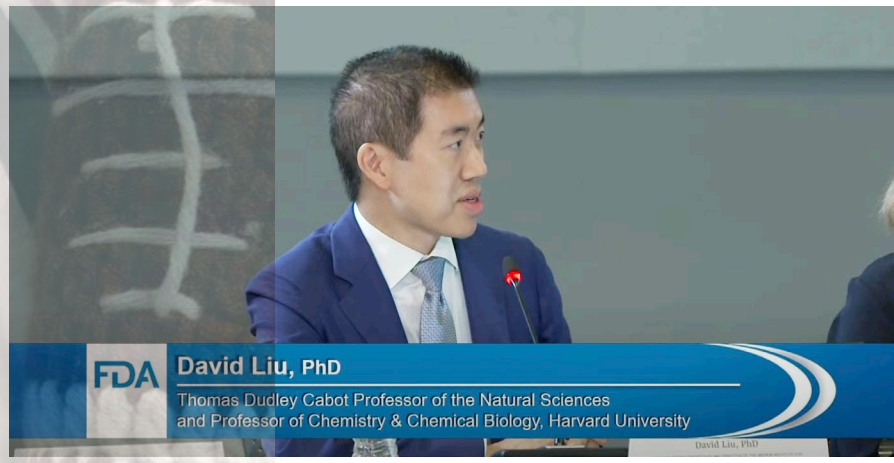
Mehmet Oz
CMS

Robert Kennedy
HHS

Marty Makary
FDA

Jay Bhachattarya
NIH

Vinay Prasad
CBER



Sec Kennedy – “CRISPR gives the gift of life.”

Secretary Kennedy
@SecKennedy

Thursday morning, I had the privilege of speaking with the Muldoon family, whose 10-month-old son, baby KJ, was discharged Wednesday from @ChildrensPhila. Baby KJ was diagnosed with a rare urea cycle disorder and received a first-of-its-kind personalized CRISPR gene-editing therapy—customized specifically for his unique mutation.

Thanks to the extraordinary expertise and compassion of the team at CHOP and the researchers who developed this pioneering treatment, baby KJ now has a second chance at life. The Muldoon family's courage is nothing short of inspiring, and I offer my deepest gratitude to everyone at CHOP and beyond who made this medical milestone possible.

This isn't just a medical success story—it's a powerful glimpse into the future of personalized medicine.

I remain firmly committed to working with @NIHDirector_Jay, @DrMakaryFDA, @DrOzCMS, and others to ensure that:

• HHS continues to invest in cutting-edge research that drives breakthrough therapies
• Our regulatory frameworks keep pace with rapid innovation
• No family is ever denied access to life-saving treatments because of cost

Breakthroughs like this are why we do the work.

HHS.gov
@HHSGov

After this week's cell and gene therapy roundtable at @US_FDA, @SecKennedy joined @DrOzCMS, @DrMakaryFDA, and @NIHDirector_Jay to deliver a message directly to the American people about investing in cutting-edge cures.

Cell and gene therapy holds promise—but it also demands caution, transparency, and rigorous oversight.

Smart innovation will help Make America Healthy Again.

1:35 PM · Jun 6, 2025 · **246.3K** Views

“New FDA approval pathway for n-of-1 therapies coming soon, Prasad says”

Endpoints Sep 8 2025

The success story of baby KJ’s customized CRISPR therapy and the prospects for others like it have the FDA readying a new approval pathway for those with extremely limited treatment options.

The new pathway will be for novel therapies intended for just one or a few patients, the FDA’s Center for Biologics Evaluation and Research Director Vinay Prasad told a Duke-Margolis workshop last week. The agency is planning to outline it in the New England Journal of Medicine, according to Prasad, who described it as “the plausible mechanism pathway.”

The idea was first floated by FDA Commissioner Marty Makary in April, who described it at the time as a “conditional approval” pathway. Prasad said that “it will be a novel pathway for drug developers who are pursuing bespoke therapy. By that, I mean completely individualized therapy that baby A gets one treatment, B gets the next treatment, baby C gets a different treatment.”

Baby KJ, or Kyle Patrick Muldoon Jr., is a pediatric patient born with a rare metabolic disease. His ailment was successfully treated with a custom gene editing therapy by researchers in Philadelphia, accelerating discussions about how to advance similar treatments for tiny populations of patients. The effort has been helped along by the failure in recent years of many such treatments to gain traction with corporate developers, or to gain approval through existing approval pathways.

The upcoming NEJM publication will provide drugmakers with a roadmap for how they can win approval for such therapies, Prasad said, “after treating just several kids or young adults or adults with a certain condition.” Those trials could be as small as five people, he added.

The only way to find out how to safely edit people – is to edit more people.

In the 36 year history of cell and gene therapy practically not a single treatment related SAE was predicted from nonclinical studies

The NEW ENGLAND JOURNAL of MEDICINE

BRIEF REPORT

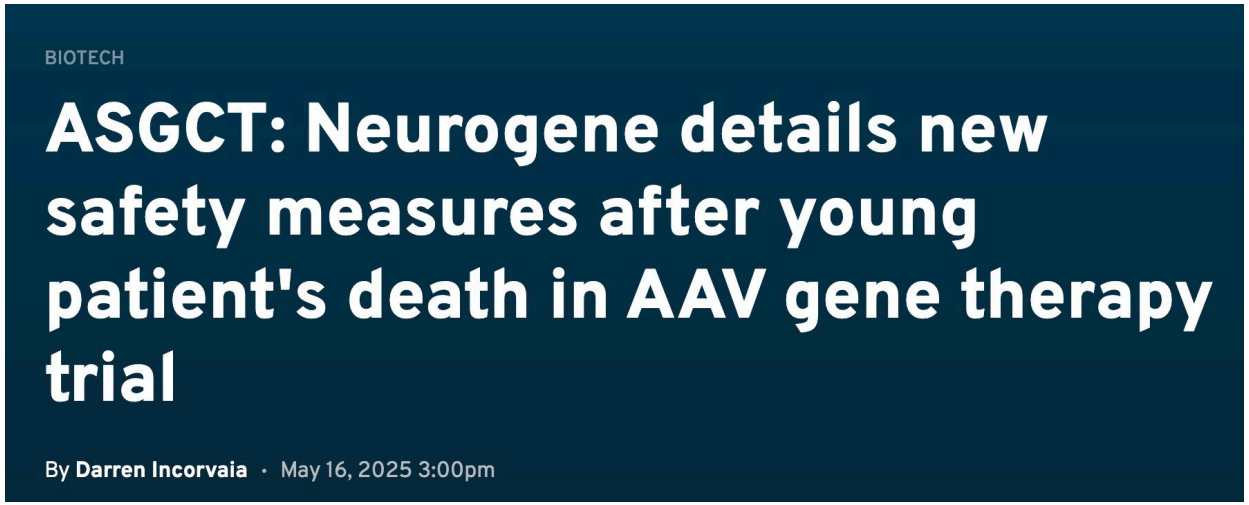
Acute Myeloid Leukemia Case after Gene Therapy for Sickle Cell Disease

Sunita Goyal, M.D., John Tisdale, M.D., Manfred Schmidt, Ph.D., Julie Kanter, M.D., Jennifer Jaroscak, M.D., Dustin Whitney, Ph.D., Hans Bitter, Ph.D., Philip D. Gregory, Ph.D., Geoffrey Parsons, Ph.D., Marianna Foos, M.S., Ashish Yeri, Ph.D., Maple Gioia, A.L.M., Sarah B. Voytek, Ph.D., Alex Miller, B.S., Jessie Lynch, M.S., Richard A. Colvin, M.D., Ph.D., and Melissa Bonner, Ph.D.

The New York Times

Gene Treatment for Rare Epilepsy Causes Brain Side Effect in 2 Children

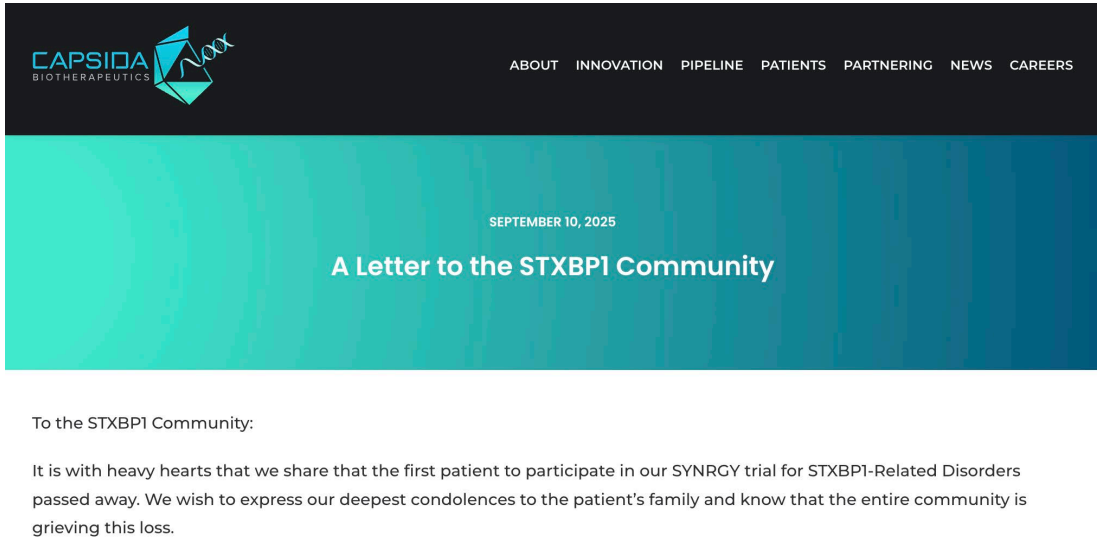
The side effect, a buildup of fluid in the brain, led to the death of one of the children and presents a grave setback for a class of personalized medicine.



BIOTECH

ASGCT: Neurogene details new safety measures after young patient's death in AAV gene therapy trial

By **Darren Incorvaia** · May 16, 2025 3:00pm



CAPSIDA BIOTHERAPEUTICS

ABOUT INNOVATION PIPELINE PATIENTS PARTNERING NEWS CAREERS

SEPTEMBER 10, 2025

A Letter to the STXBP1 Community

To the STXBP1 Community:

It is with heavy hearts that we share that the first patient to participate in our SYNRGY trial for STXBP1-Related Disorders passed away. We wish to express our deepest condolences to the patient's family and know that the entire community is grieving this loss.

May 15 2025

npr

A promising genetic treatment tailor-made for a baby born with a rare disorder

TIME

The Economist

The Washington Post

THE WALL STREET JOURNAL.

ENDPOINTS NEWS

The inside story of the six-month sprint to create the first custom CRISPR therapy for one infant's rare disease

The New York Times

Baby Is Healed With World's First Personalized Gene-Editing Treatment

The technique used on a 9½-month-old boy with a rare condition has the potential to help people with thousands of other uncommon genetic diseases.

May 15 2028?

Baby Rosalind of London

Medicines & Healthcare products Regulatory Agency

Baby Gregor of Brno

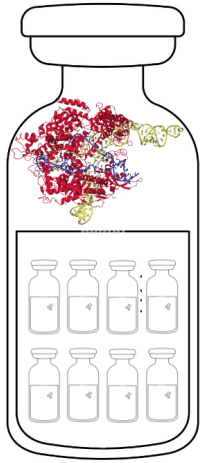
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Baby Barbara of Cold Spring Harbor, NY

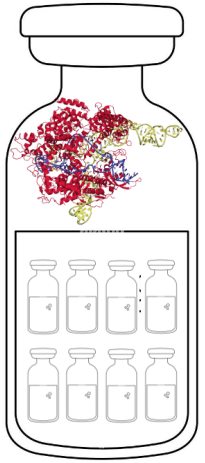
FDA

May 15 2030?

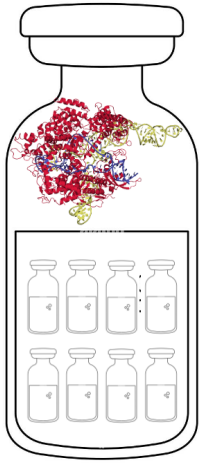
hematologic



metabolic



pulmonary



dermatology

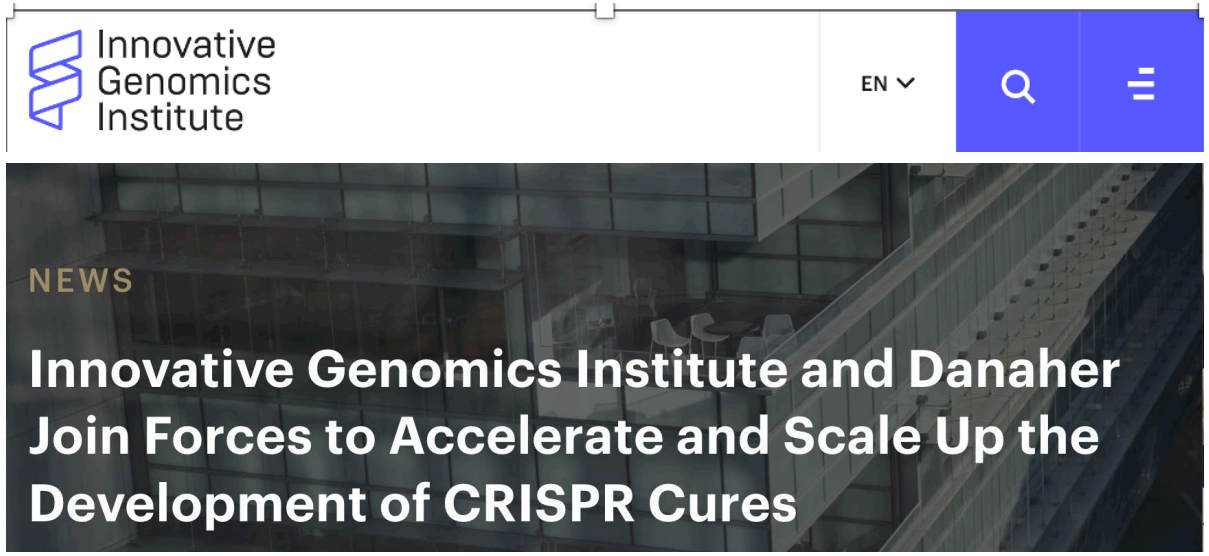
Platform approaches for CRISPR on-demand

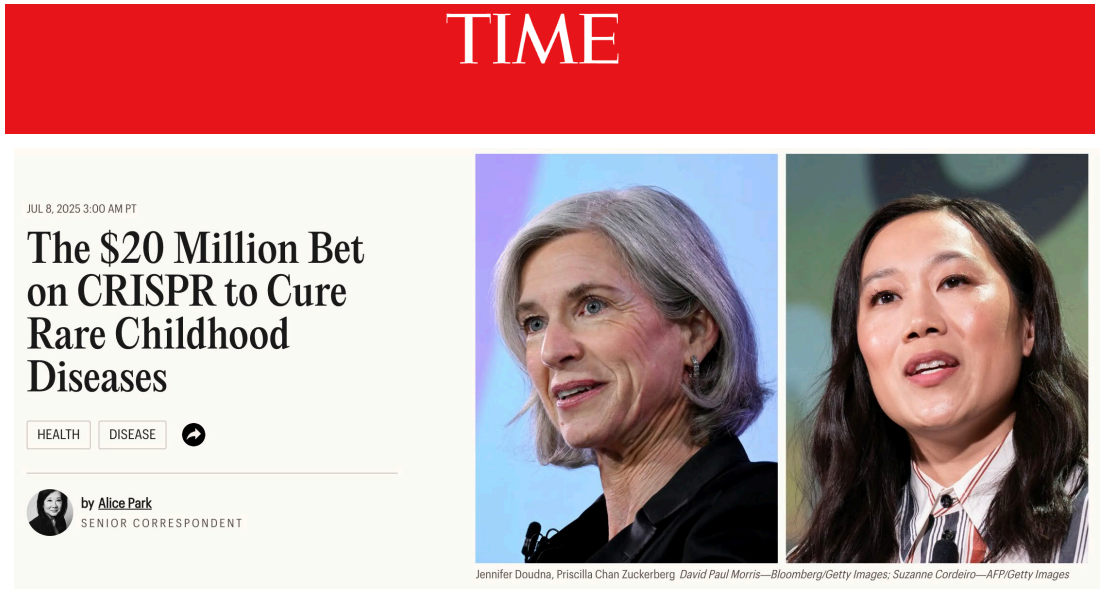
Medicines & Healthcare products Regulatory Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

FDA

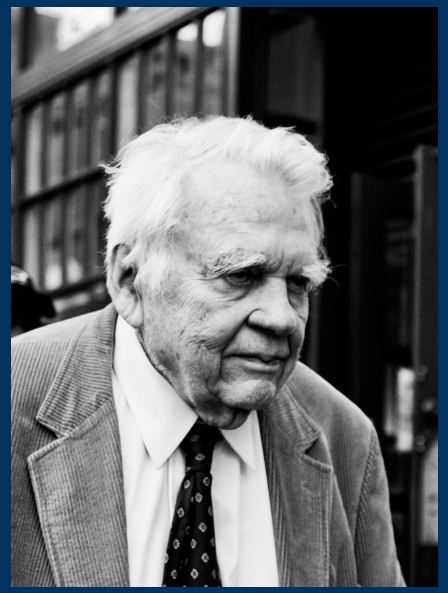
IGI parterships in pediatric CRISPR Cures





Develop platform approaches for rapid design and clinical deployment of CRISPR-on-demand pediatric disease.
Use them to treat children in areas of severe unmet need and no for-profit sector involvement.
Share the nonclinical and clinical data broadly.

"Courage is not being fearless,
but being scared to death
and doing the right thing anyway."



Andy Rooney (1919-2011)