Developing and deploying *N*-of-1 gene-editing therapies

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ADVANCING KNOWLEDGE FOR GOOD

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Relationships with Verve Therapeutics, Sanofi, Tenaya Therapeutics, Lexeo

Typical case of neonatal-onset urea cycle disorder

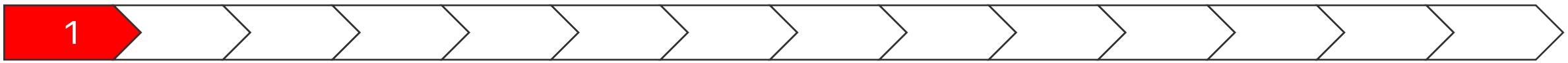
A 1-day-old male infant was noted to have poor feeding and increased sleepiness in the well-baby nursery. He was transferred to the neonatal intensive care unit.

The blood ammonia level was significantly elevated >1000 µmol/L (normal for age is <33 µmol/L).

Over the next 12 hours, he was transferred to a tertiary care pediatric hospital 3 hours away for emergent management, including 48 hours of dialysis.

His newborn screening and initial metabolic labs were consistent with a diagnosis of citrullinemia type 1. He was initiated on ammonia-scavenging medications and medical formula.

He was discharged from the hospital by day of life 20.



months

Typical case of neonatal-onset urea cycle disorder

Unfortunately, he was readmitted at 1 month of life with poor feeding, diarrhea, and an elevated ammonia level of 250 µmol/L.

Over the first 12 months of life, he was readmitted to the hospital 6 more times for recurrent episodes of hyperammonemia.

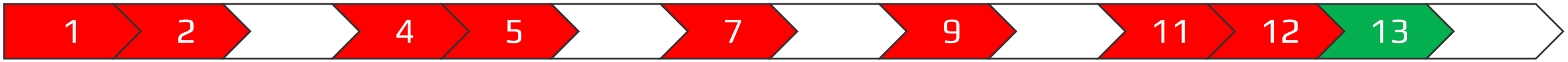
He was also noted to have difficulty feeding, requiring gastrostomy tube placement at 6 months of age.

Typical case of neonatal-onset urea cycle disorder

The family decided to pursue liver transplantation, and he was listed for transplant at 10 months of age after he had grown to an appropriate size. He received a deceased donor transplant when he was 13 months of age.

His post-transplantation period was complicated by a bile leak and episodes of acute rejection requiring increased doses of immunosuppression.

He had no further hyperammonemic crises after transplantation, but he required 4 additional hospitalizations for transplant-related complications.



months

Typical case of neonatal-onset urea cycle disorder

He is currently 6 years old and continues to require post-transplantation immunosuppression.

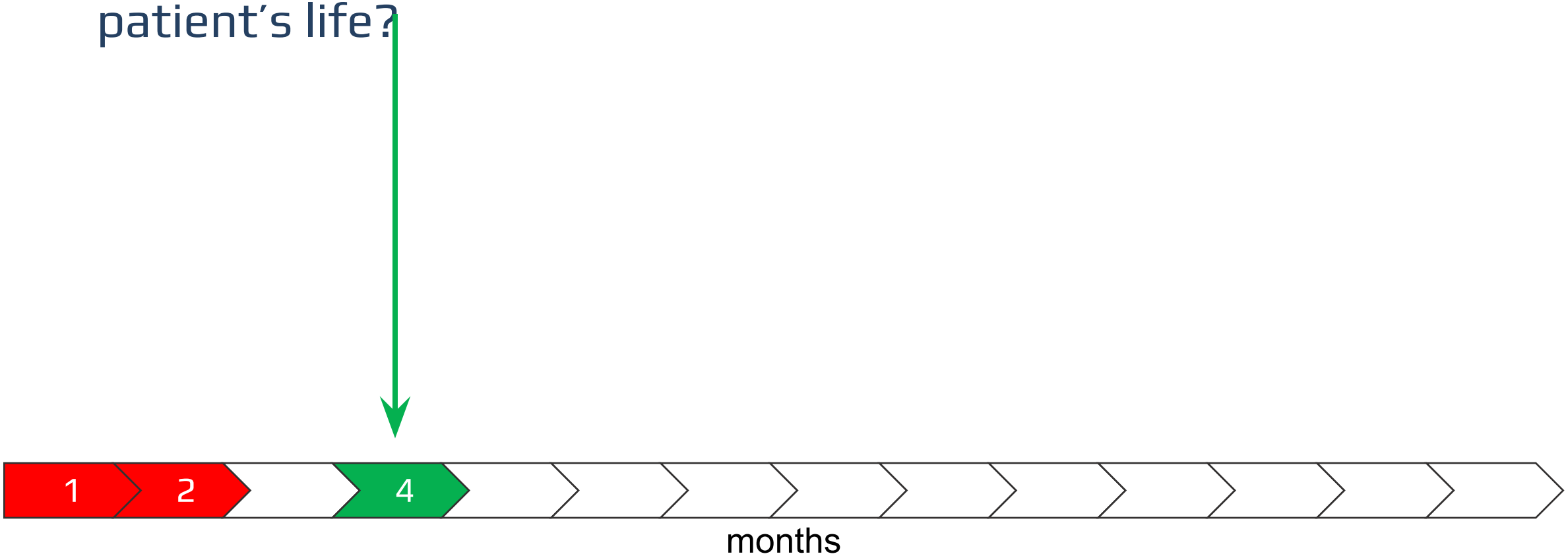
He suffers from global developmental delay.

He requires help with dressing and toileting, but he can speak in 2-word phrases and enjoys attending his special needs kindergarten.

The aggregate health care costs for this individual have totaled > US$ 2 million.

Typical case of neonatal-onset urea cycle disorder

What if we could have intervened with a personalized liver-directed corrective gene-editing therapy early in this patient's life?

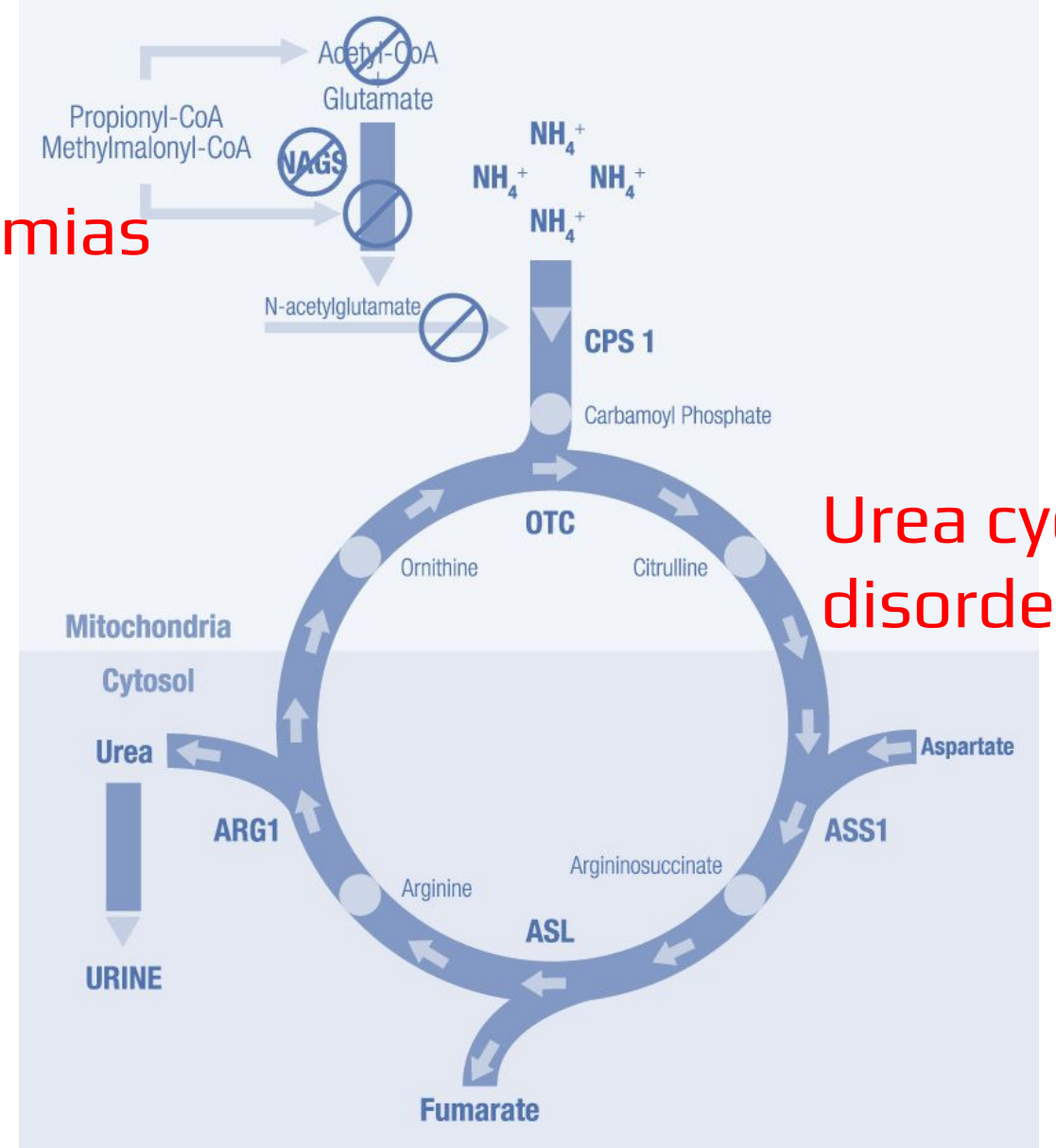


Newborn patients with grievous inborn errors of metabolism

Organic acidemias

Hyperammonemia episodes, starting immediately after birth, cause **progressive brain damage** and **early death**

Liver transplantation—**if available**—has to be deferred until **late infancy** and has substantial morbidity and mortality



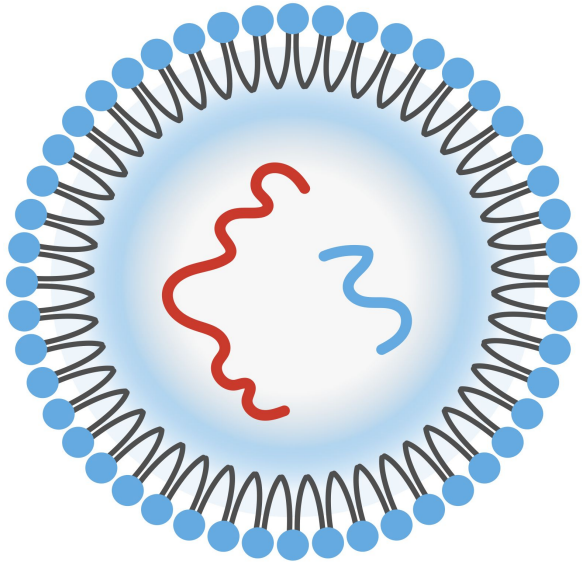
Rebecca Ahrens-Nicklas
CHOP

Fyodor Urnov
IGI/UC Berkeley

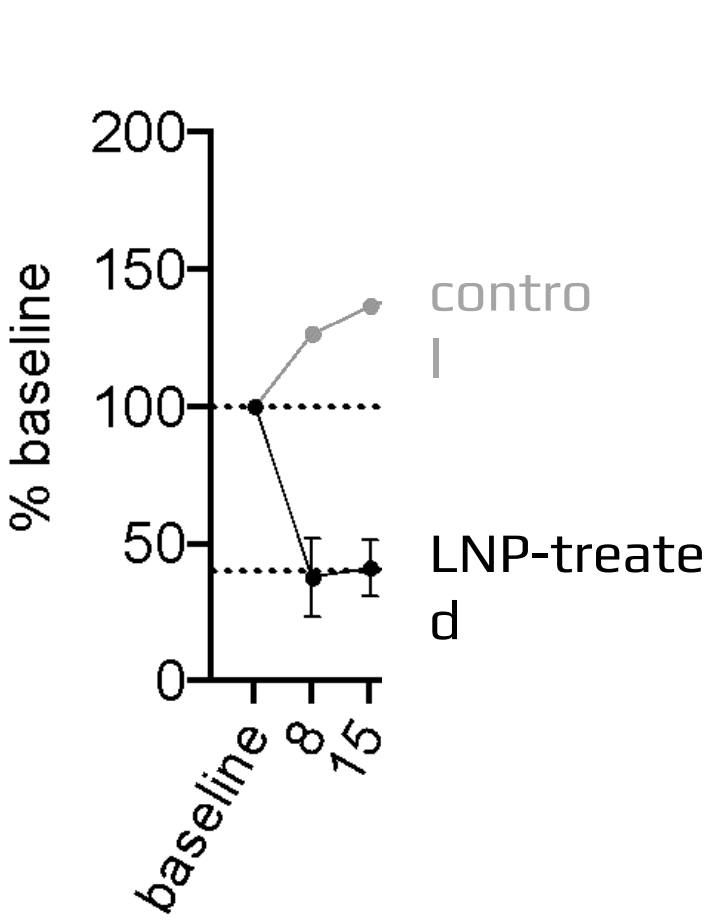
Urea cycle disorders

Protecting against the world’s leading killer

Editing via lipid nanoparticles (LNPs), base editor mRNA, and guide RNA



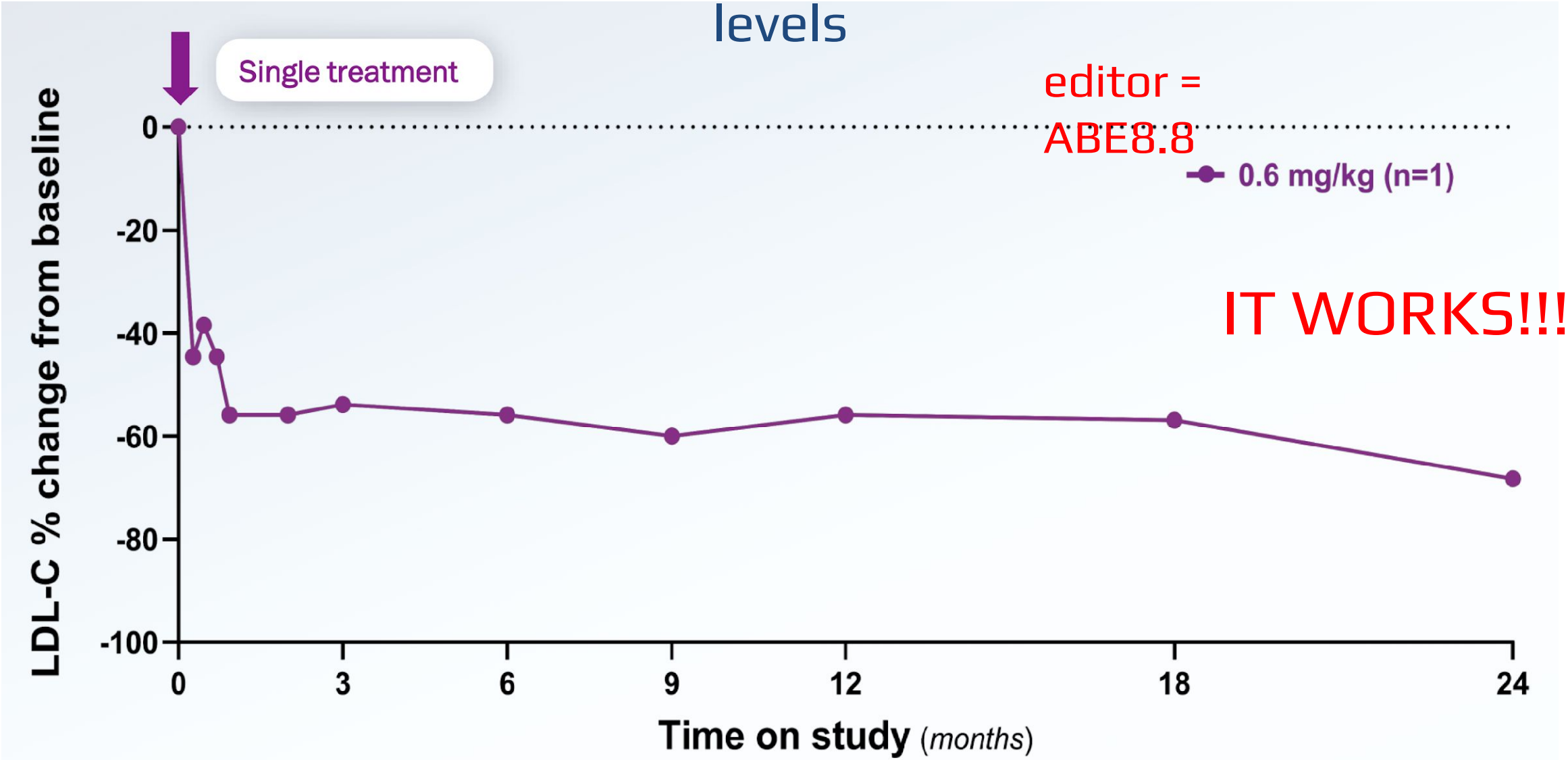
LNP base editing of *PCSK9* in the liver in monkeys – LDL cholesterol levels



editor = ABE8.8

Musunuru et al. *Nature* 2021;

LNP base editing of *PCSK9* in heart disease patients – LDL cholesterol levels



https://www.vervetx.com/sites/default/files/2025-04/VERV%20Heart-2%20Data%20Call%20Deck_041425_PP%20page.pdf

Correcting pathogenic variants to definitively treat rare genetic disorders

Credit: Erik Jacobs

Anthony Parrazzo (center) counts out 11 pills of Kuvan, which he takes every day to help manage his PKU. Lauren Ward (left) and Samantha Parrazzo (right) take a powdered form of the drug.

phenylketonuria (PKU)

=

high phenylalanine levels

=

neurological problems, avoided only with strict diet & daily pills/shots

Base editing for correction of PKU variants in *PAH* gene in the liver

- c.1222C>T (R408W) – 22.4% allele frequency (AF)
- c.1066–11G>A (splice site) – 6.5% AF
- c.782G>A (R261Q) – 5.5% AF
- c.728G>A (R243Q) – 3.7% AF
- c.1315+1G>A (splice site) – 3.6% AF
- c.842C>T (P281L) – 3.2% AF

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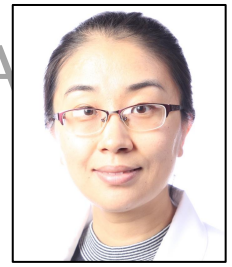

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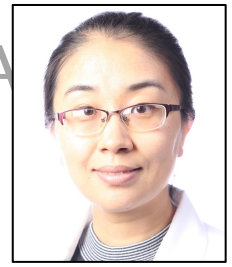

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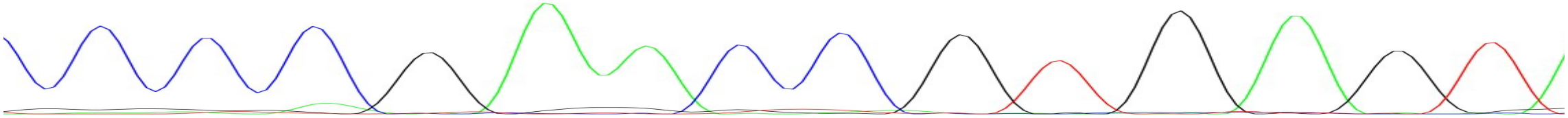
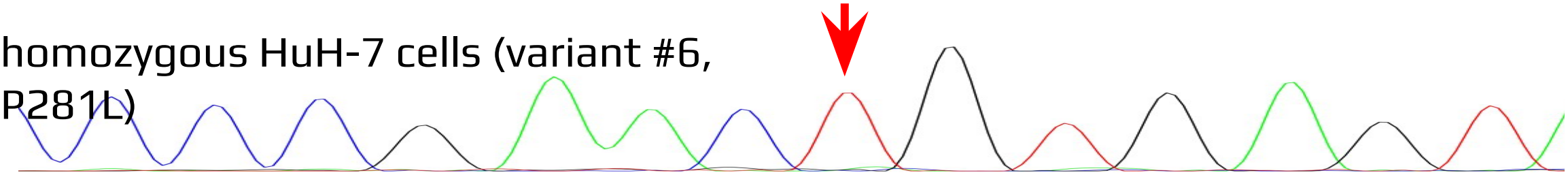
Introducing *PAH* variants into HuH-7 human hepatoma cells via prime editing

wild-type HuH-7 cells



homozygous HuH-7 cells (variant #6, P281L)



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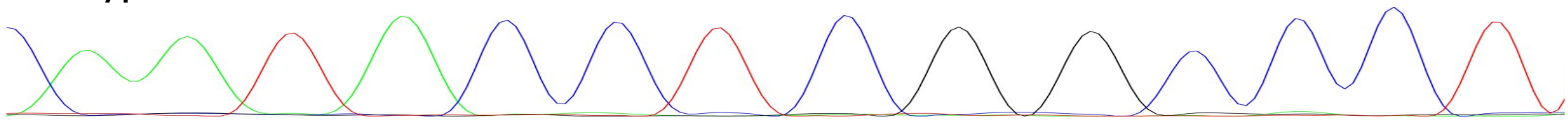
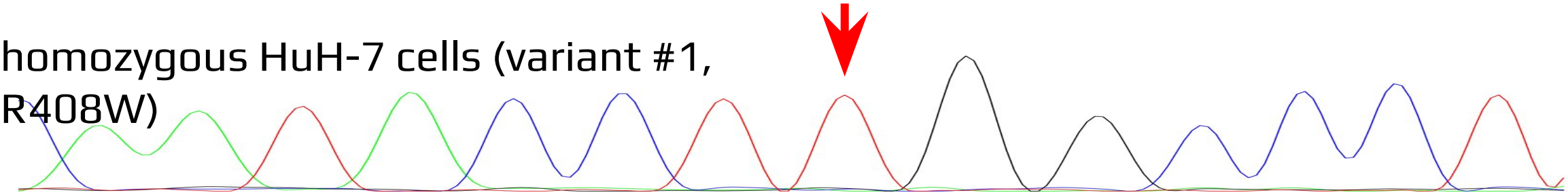
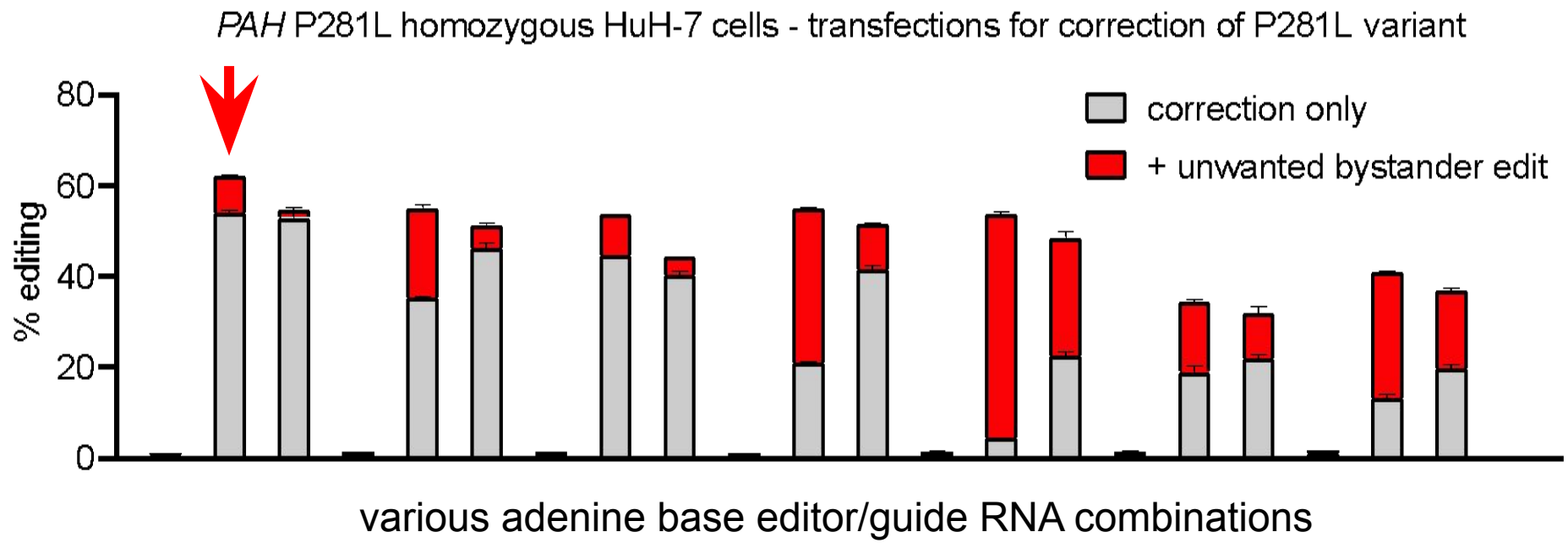


homozygous HuH-7 cells (variant #1, R408W)



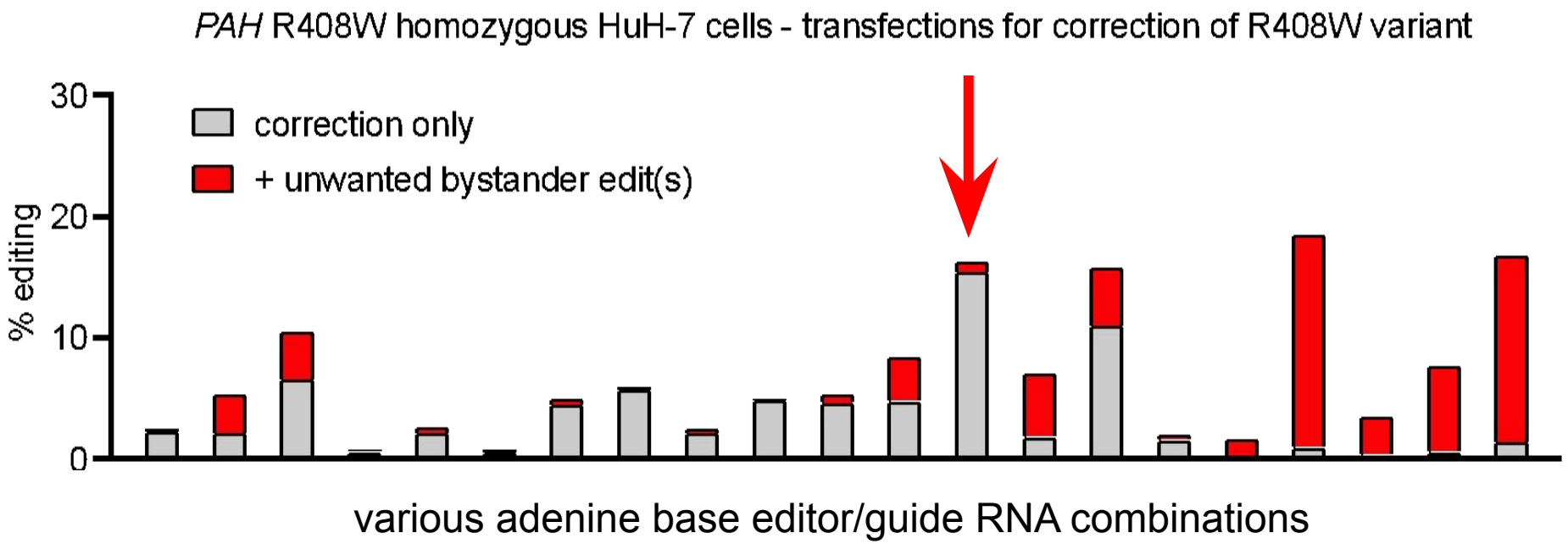
Base editing to correct P281L & R408W variants in HuH-7 cells

variant #6 = P281L editor = ABE8.8

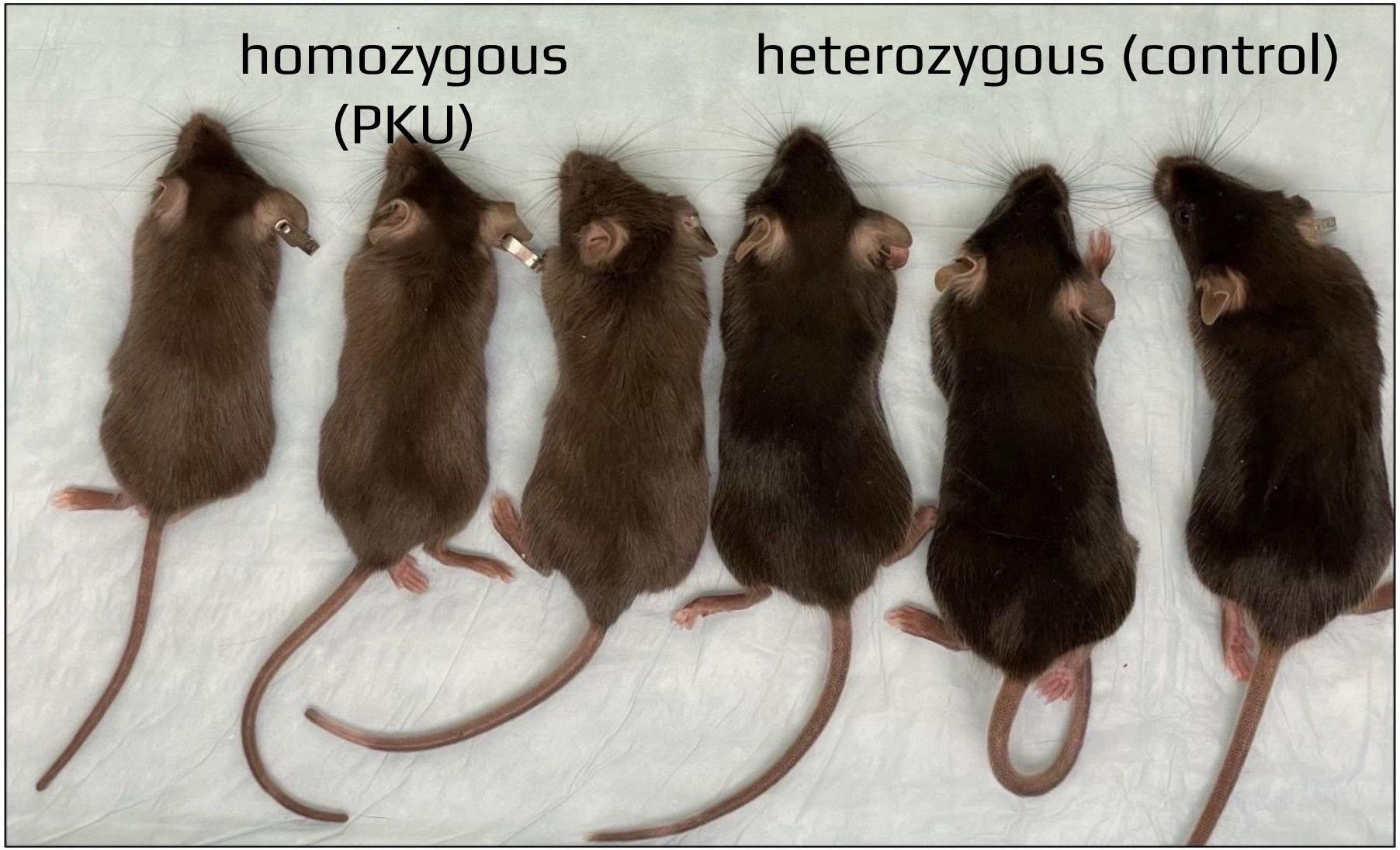


Base editing to correct P281L & R408W variants in HuH-7 cells

variant #1 = R408Weditor = SpRY-ABE8.8

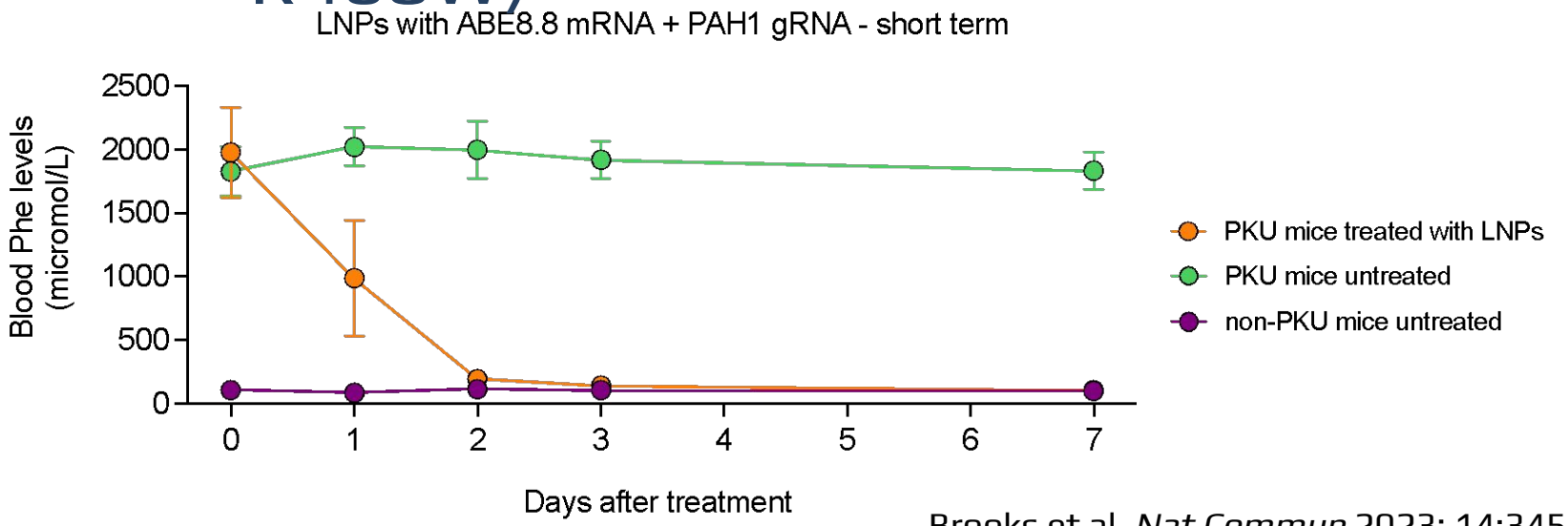


Mouse model with “humanized” *PAH* allele(s)



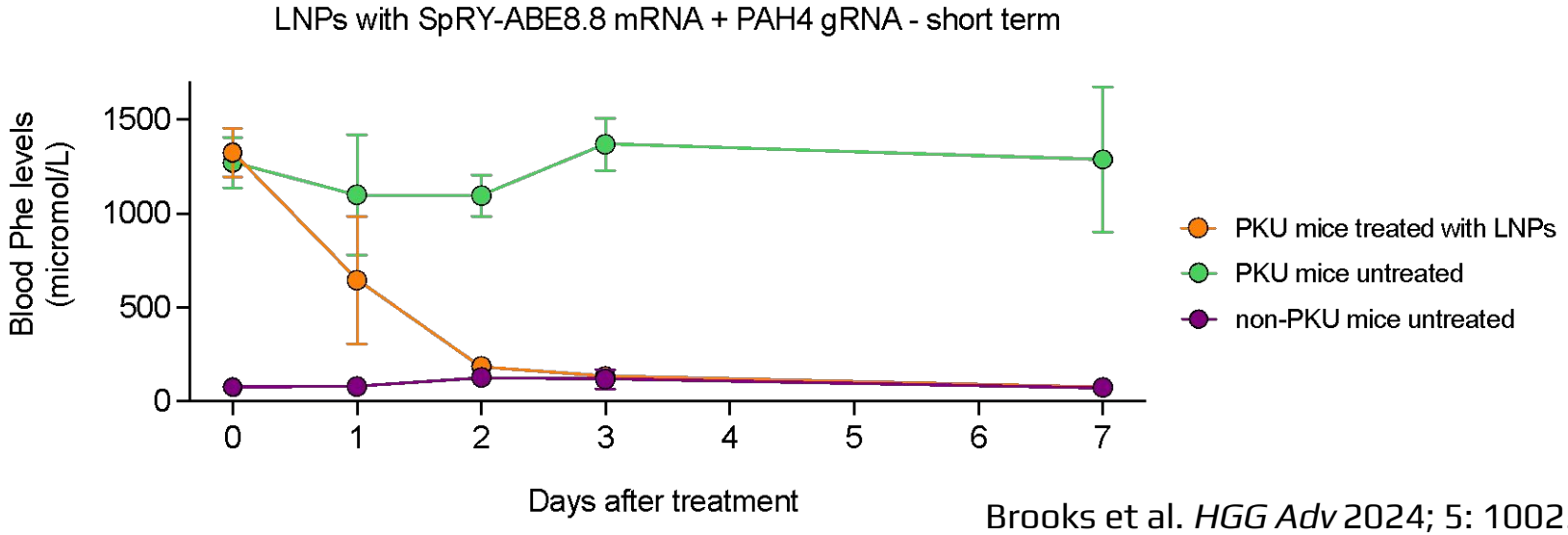
LNP base editing treatment of “humanized” PKU mice (P281L or R408W)

PAH P281L mice (variant #6)



Brooks et al. *Nat Commun* 2023; 14:3451

PAH R408W mice (variant #1)



Brooks et al. *HGG Adv* 2024; 5: 100253

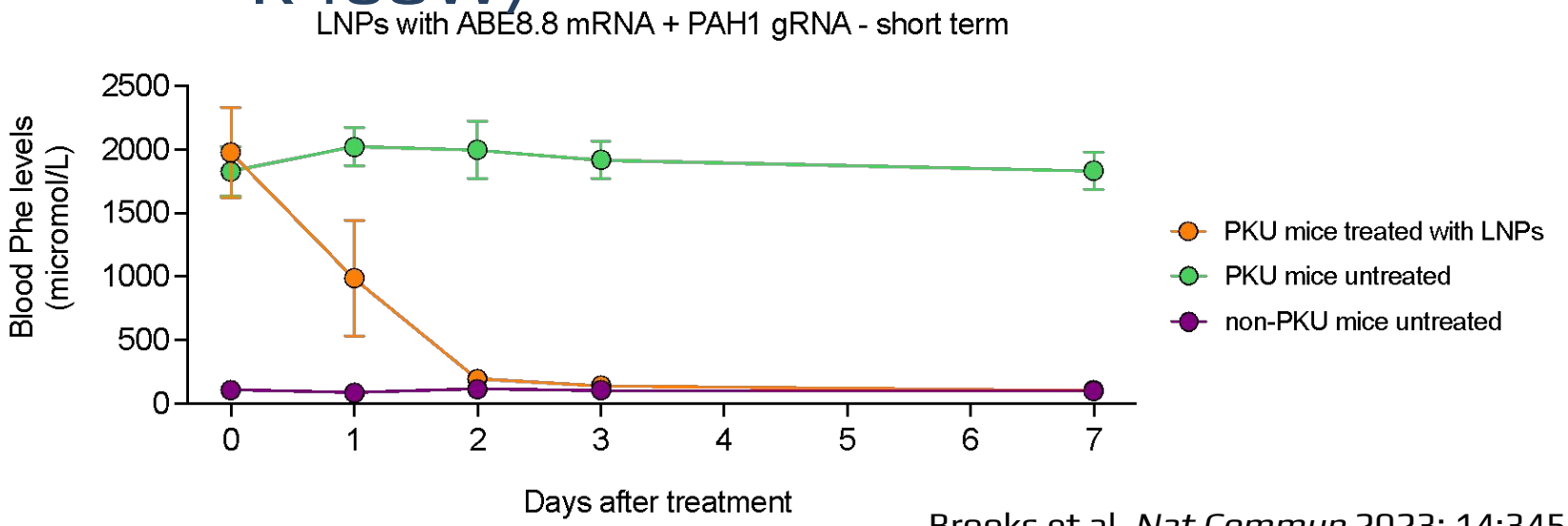
LNP base editing treatment of “humanized” PKU mice (P281L or R408W)

PAH P281L mice (variant #6)

<u>whole-liver editing</u>

on-target correction = 40%

unwanted bystander = 0.8%



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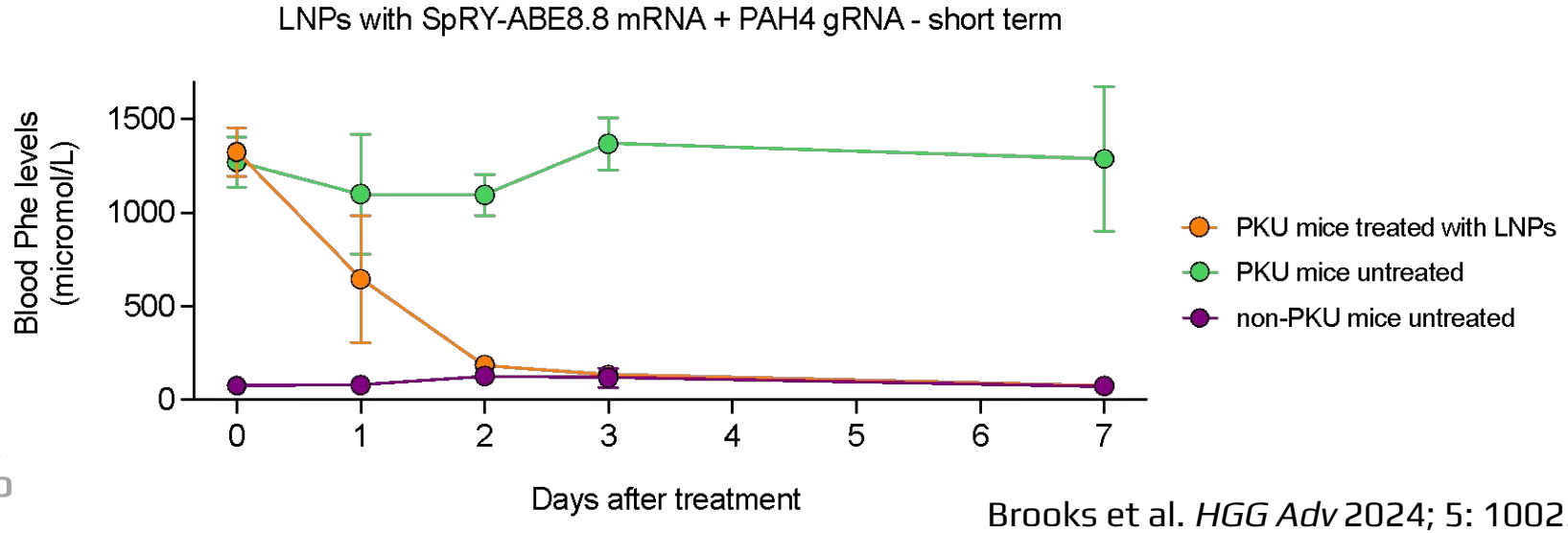
PAH R408W mice (variant #1)

<u>whole-liver editing</u>

on-target correction = 26%

unwanted bystander = 2.8%



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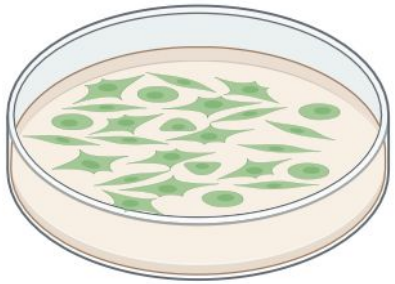
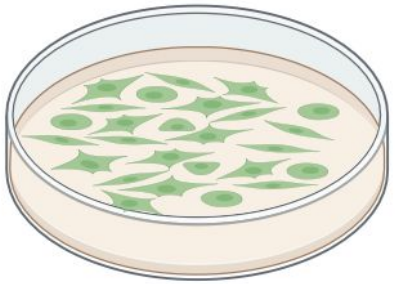
c.611A>G (T204C)	1.4%	c.814G>T (G272X)	0.5%

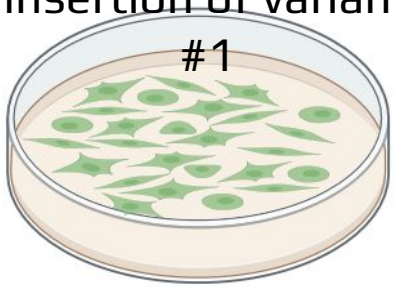
Rapid, standardized screening for corrective editing in cells



HuH-7 cells edited for insertion of variant #1



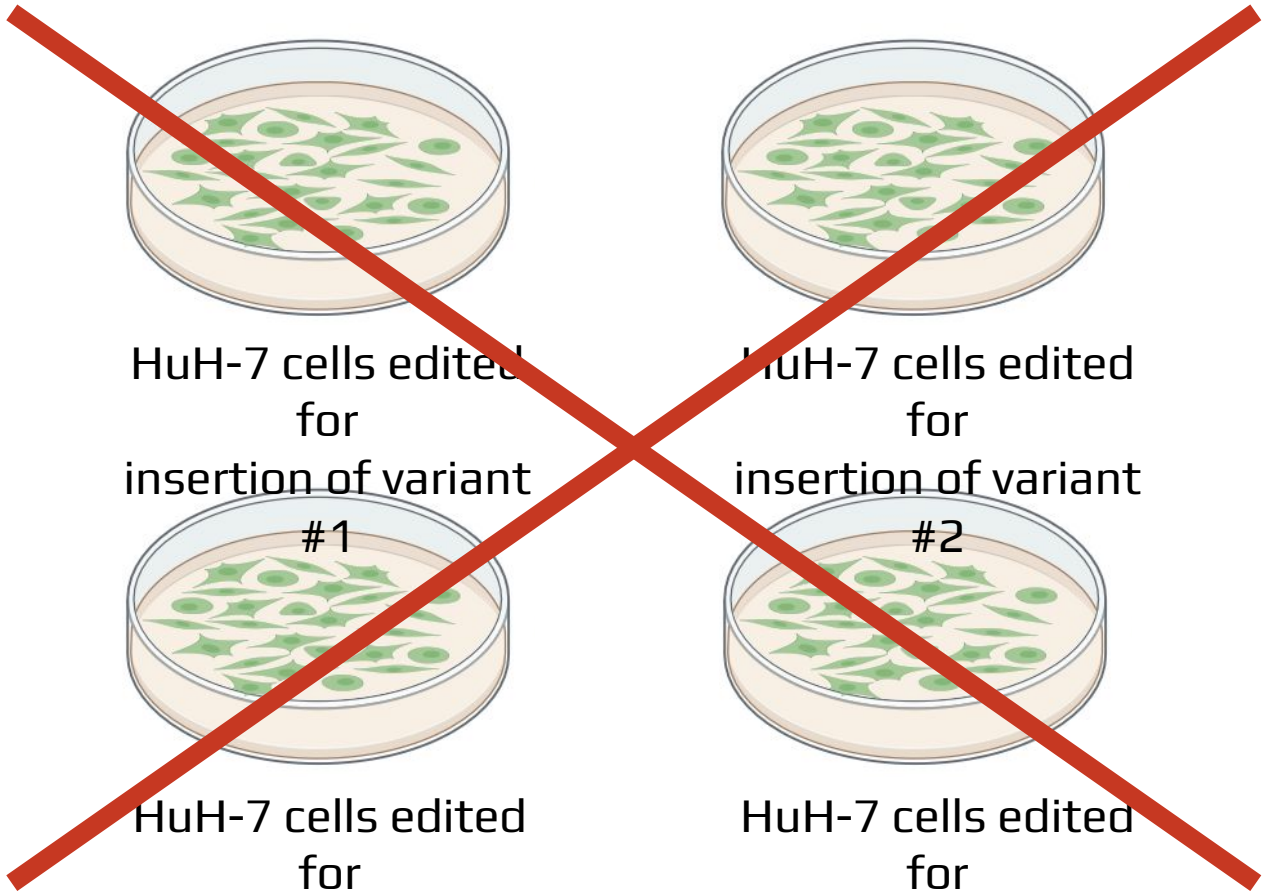
HuH-7 cells edited for insertion of variant #2

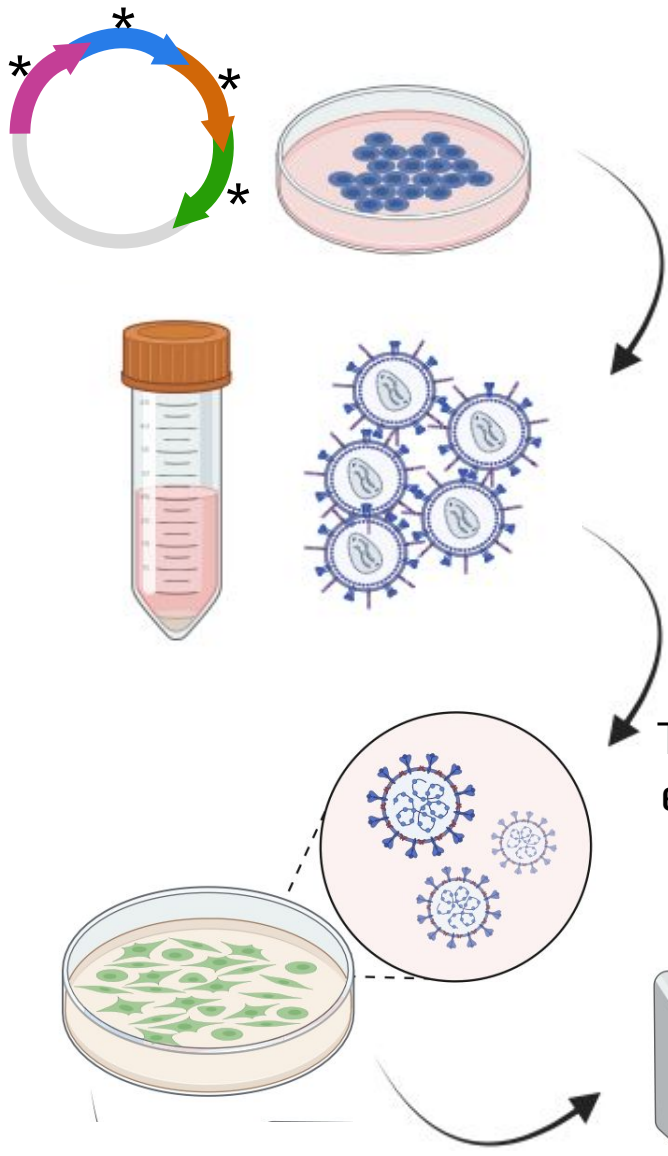


HuH-7 cells edited for insertion of variant #3

HuH-7 cells edited for insertion of variant #4

Rapid, standardized screening for corrective editing in cells



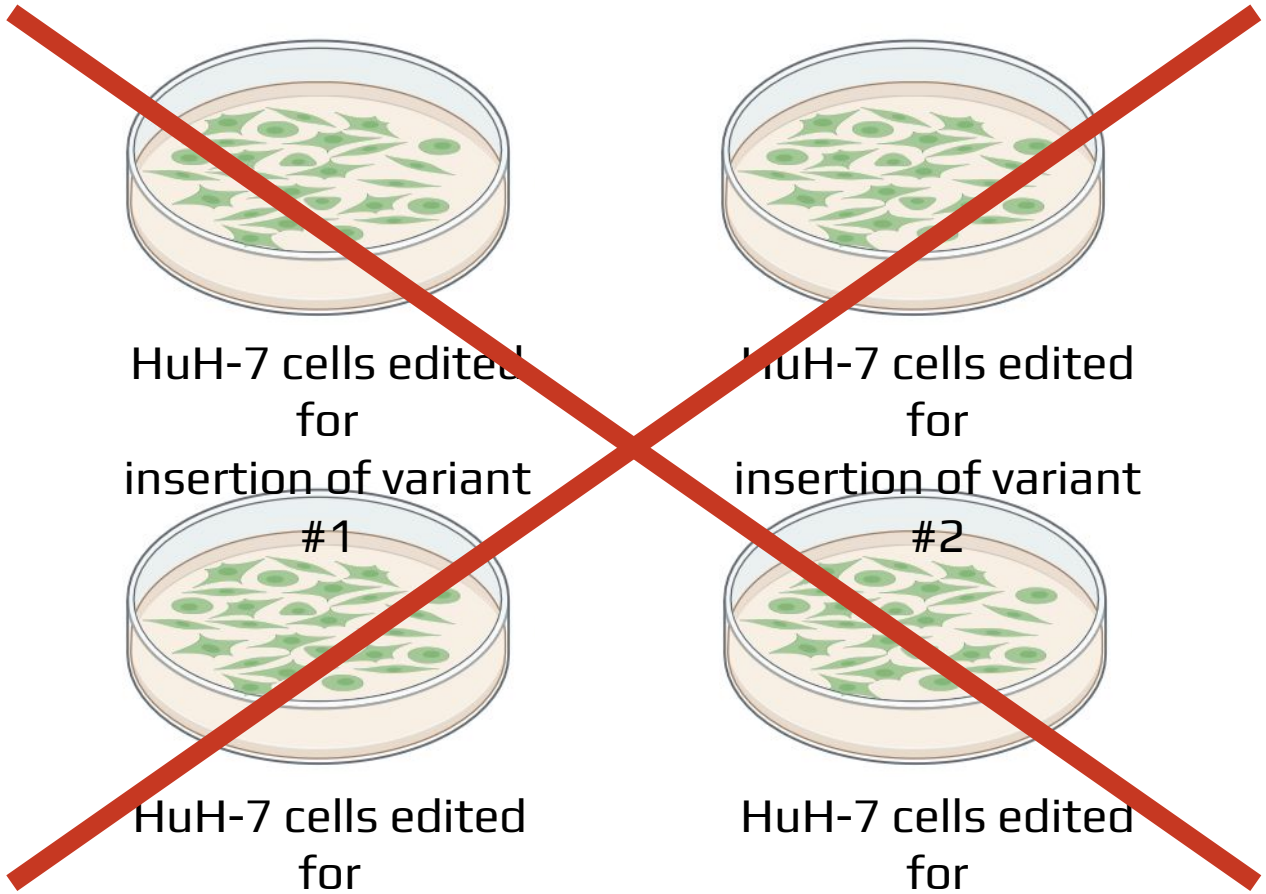


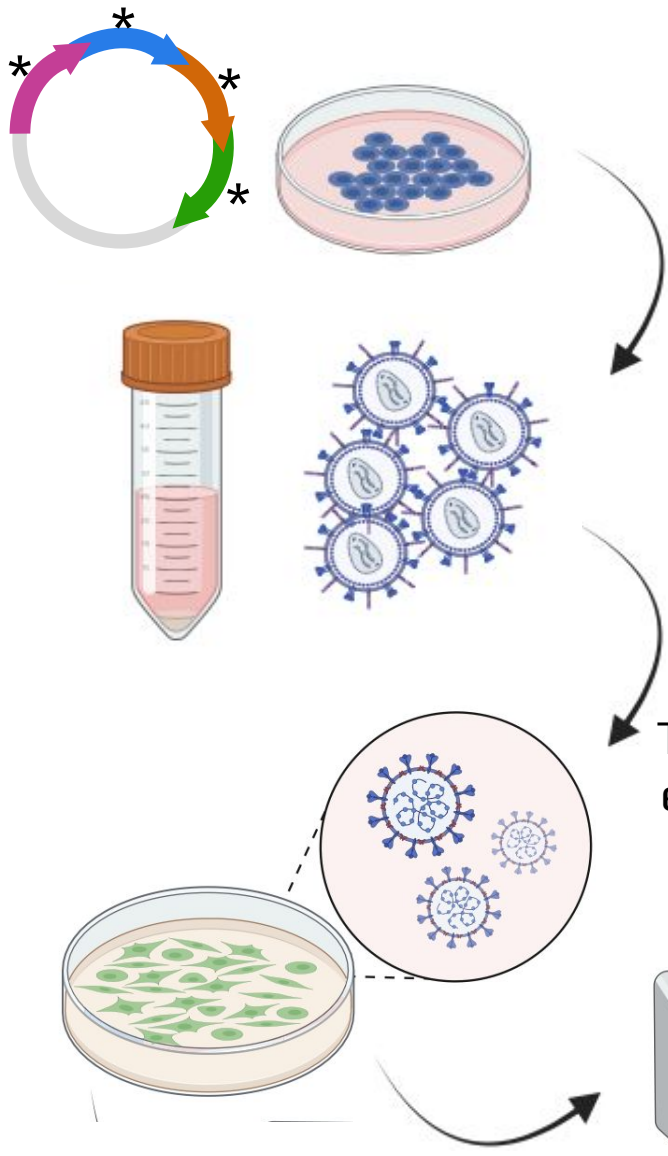
Fabricate a string of multiple variants, insert as a unit into lentiviral vector

Lentiviral infection of HuH-7 cells, placing collinear variants into genomes of cells

Transfections to screen editors/gRNAs—including validated “standards”

Rapid, standardized screening for corrective editing in cells





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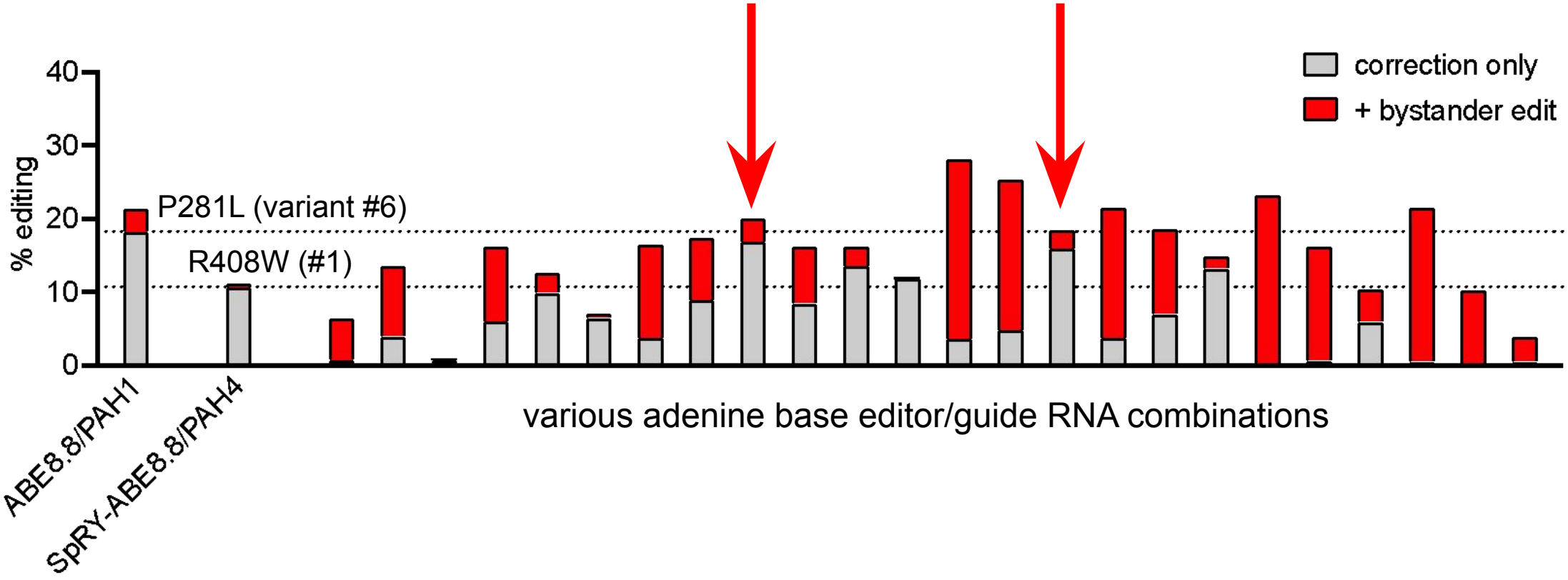
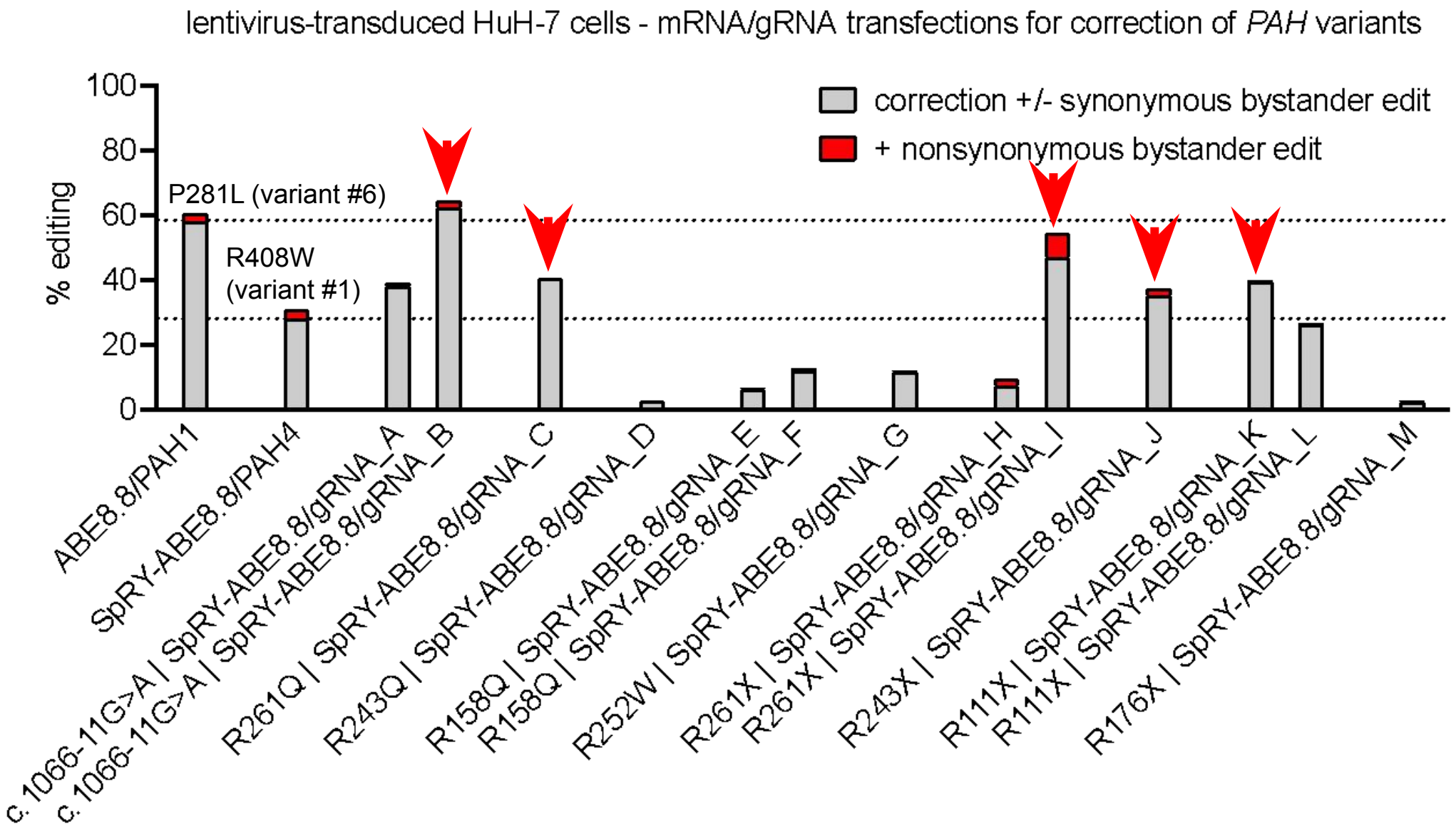


Screening with SpRY-ABE8.8 mRNA + guide RNAs in HuH-7 cells



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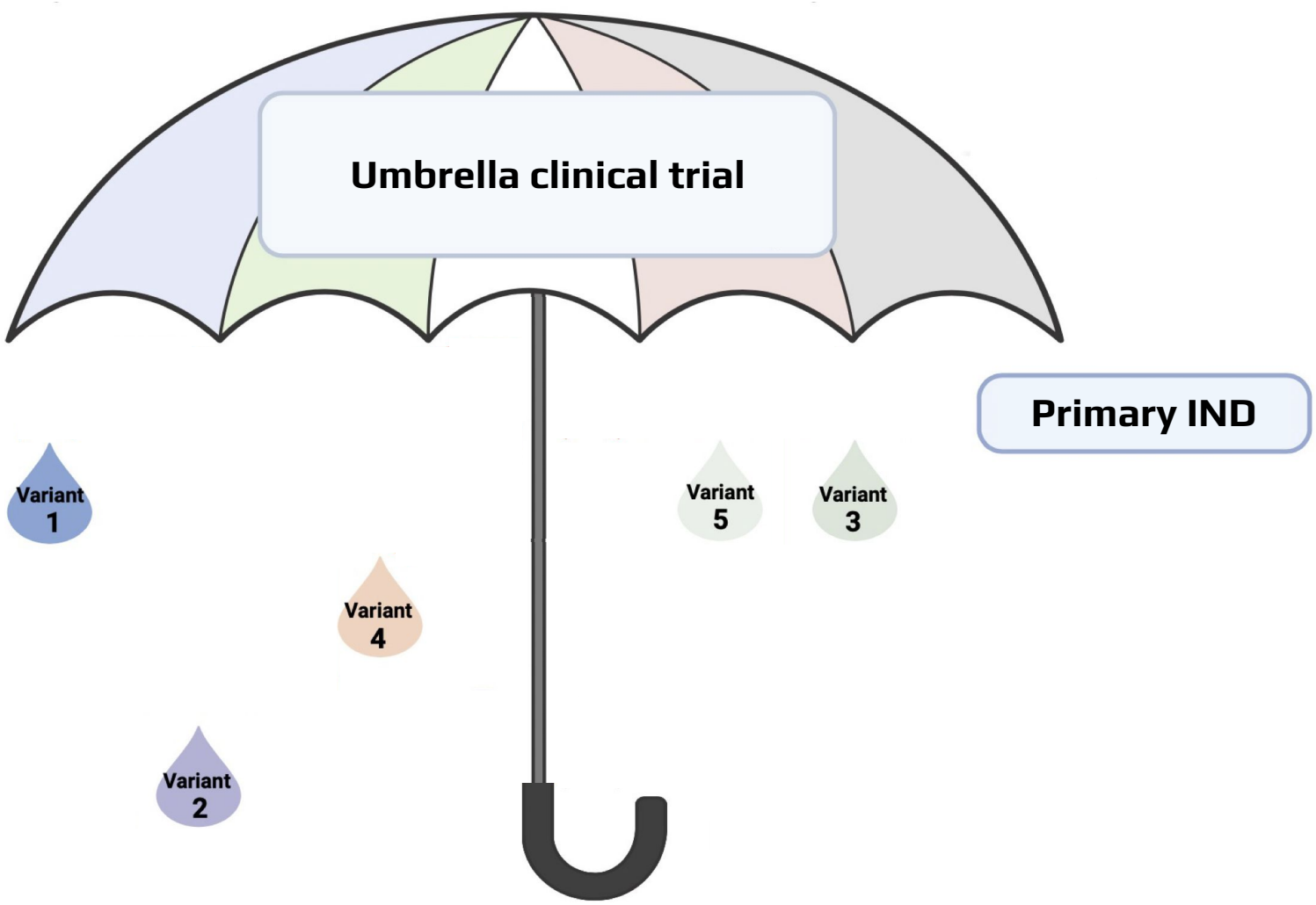
Umbrella clinical trial for PKU



Primary IND

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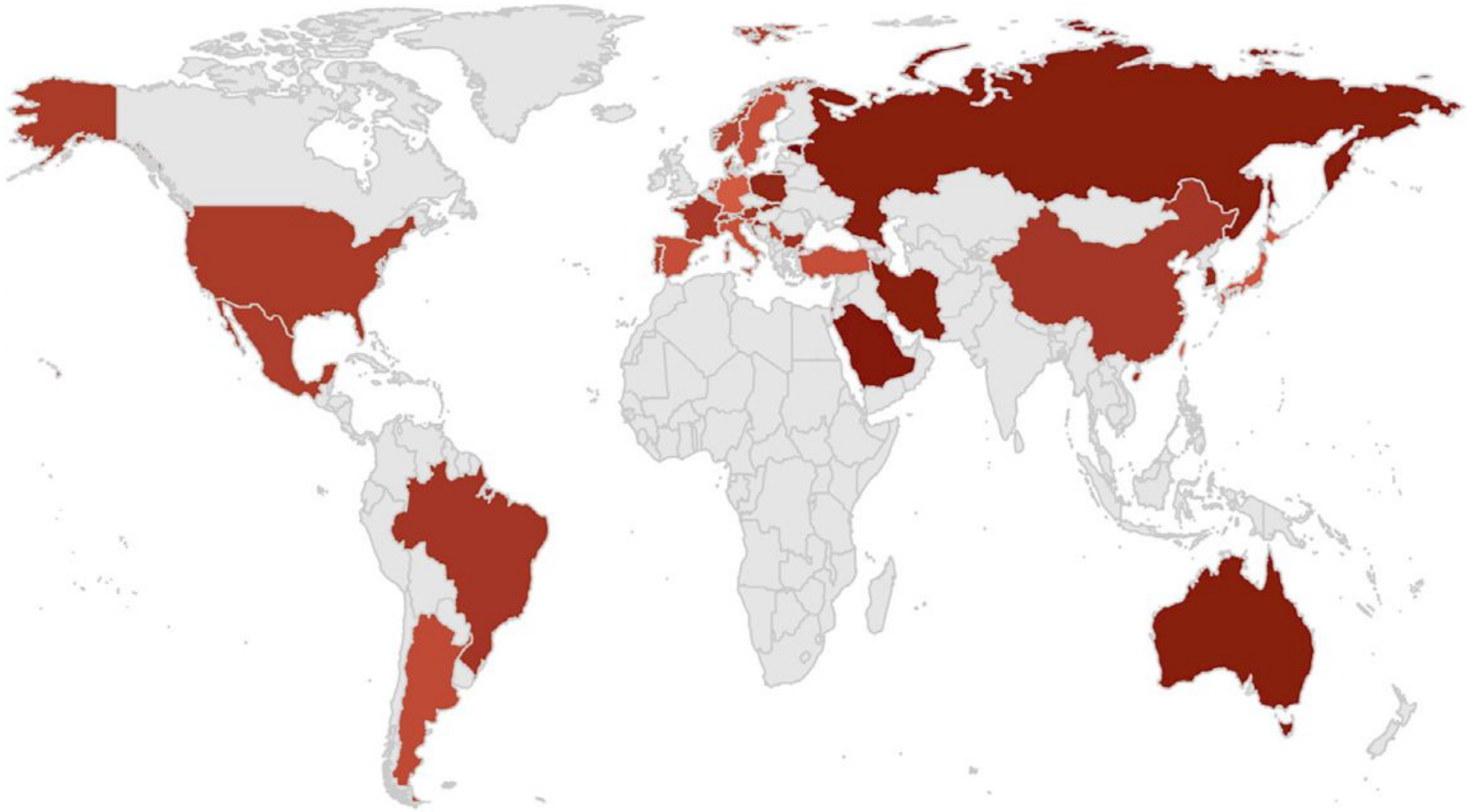
Children's Hospital of Philadelphia (CHOP)

NIH CGE
Somatic Cell Genome Editing

Pathogenic variants in PKU patients

- What about the other 1,000+ cataloged variants?

Pathogenic variants in PKU patients



Hillert et al. *Am J Hum Genet* 2020; 107:234-50

Pathogenic genes and variants

- What about uncataloged variants in low- and middle-income countries without capacity for genetic testing?
- Enormous potential for inequity – drug development biased to certain genes and certain frequent variants in high-income countries?
- Mutational discrimination

How to mitigate mutational discrimination?

~~Focus on most frequent variants in well-studied diseases with larger commercial opportunities~~

Make personalized gene editing therapies for all comers, no matter how rare the disease and how rare the variant (even *N*-of-1)

Newborn patients with grievous inborn errors of metabolism

Organic acidemias

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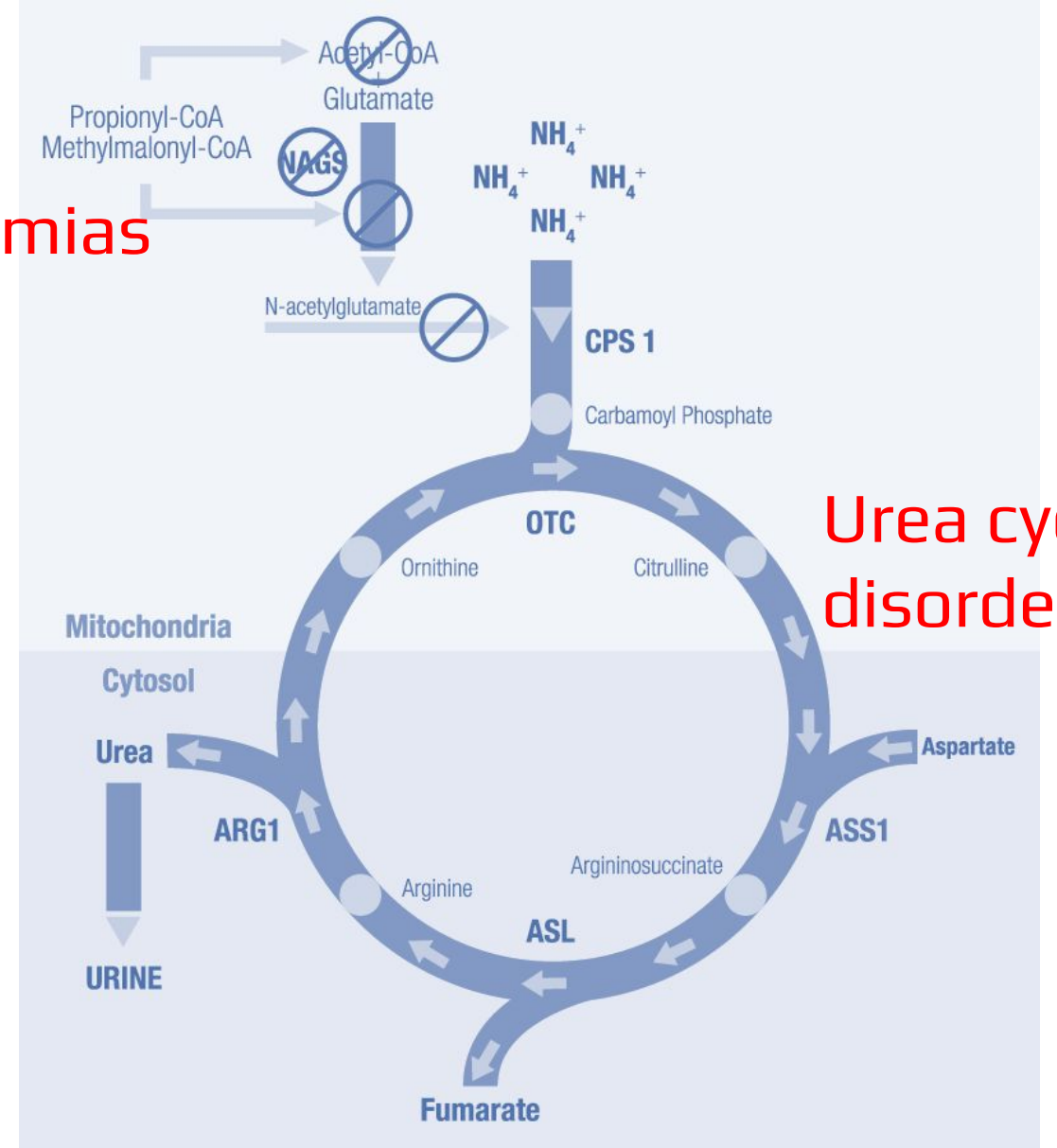


Rebecca Ahrens-Nicklas
CHOP

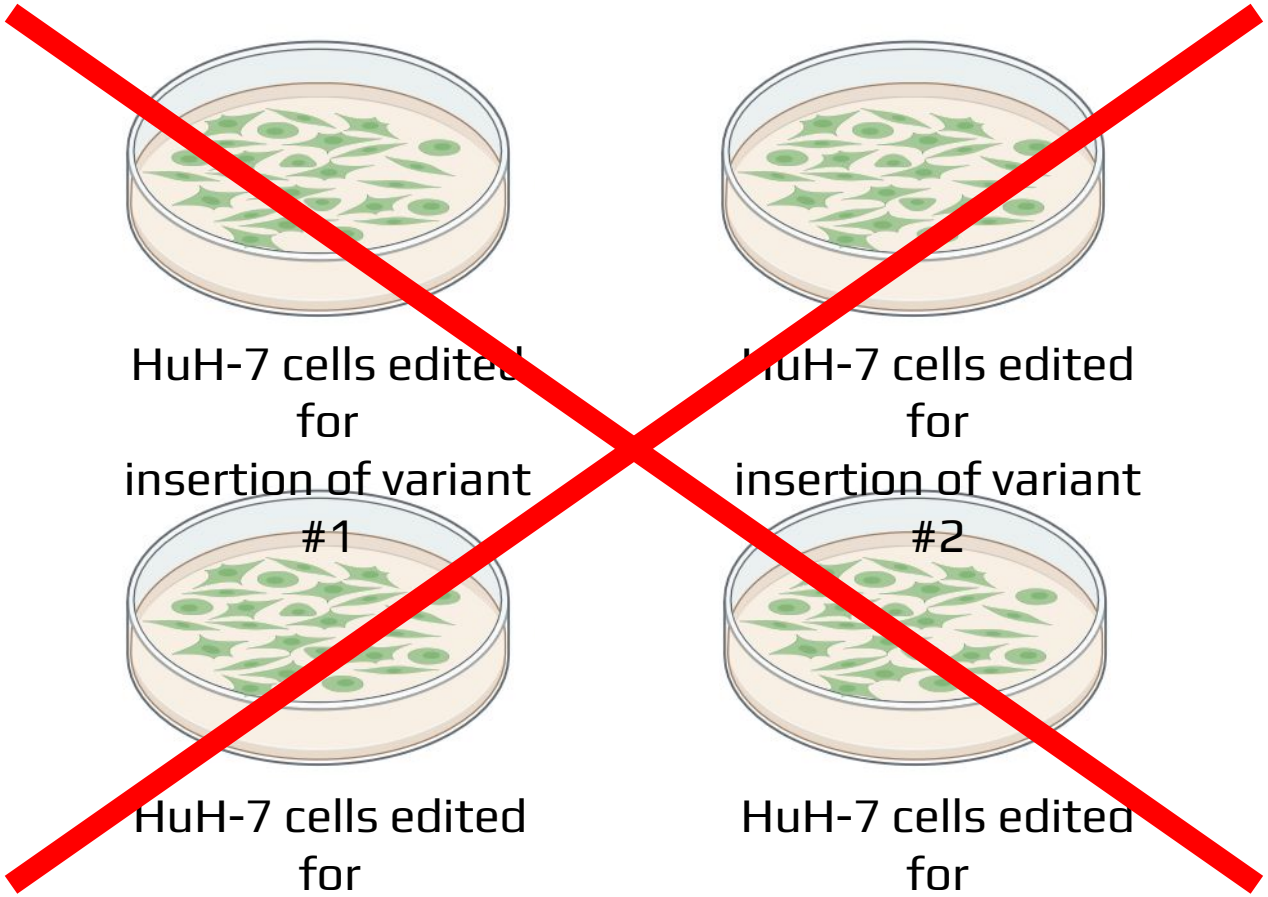
Fyodor Urnov
IGI/UC Berkeley

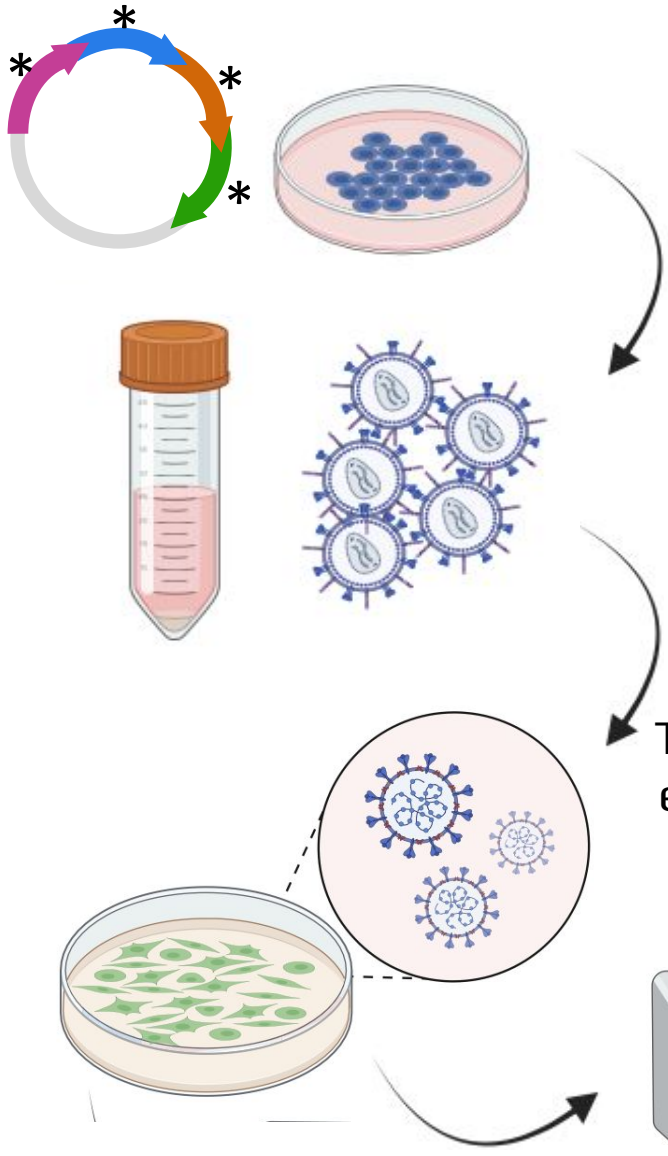
Urea cycle disorders

Variants causing urea cycle disorders and organic acidemias

- citrullinemia type 1 = *ASS1* variants
- argininosuccinic aciduria = *ASL* variants
- CPS1 deficiency = *CPS1* variants
- OTC deficiency = *OTC* variants
- propionic acidemia = *PCCA* or *PCCB* variants
- methylmalonic acidemia = *MMUT* or *MMAB* variants

Rapid, standardized corrective editing in cells





Fabricate a string of multiple variants, insert as a unit into lentiviral vector

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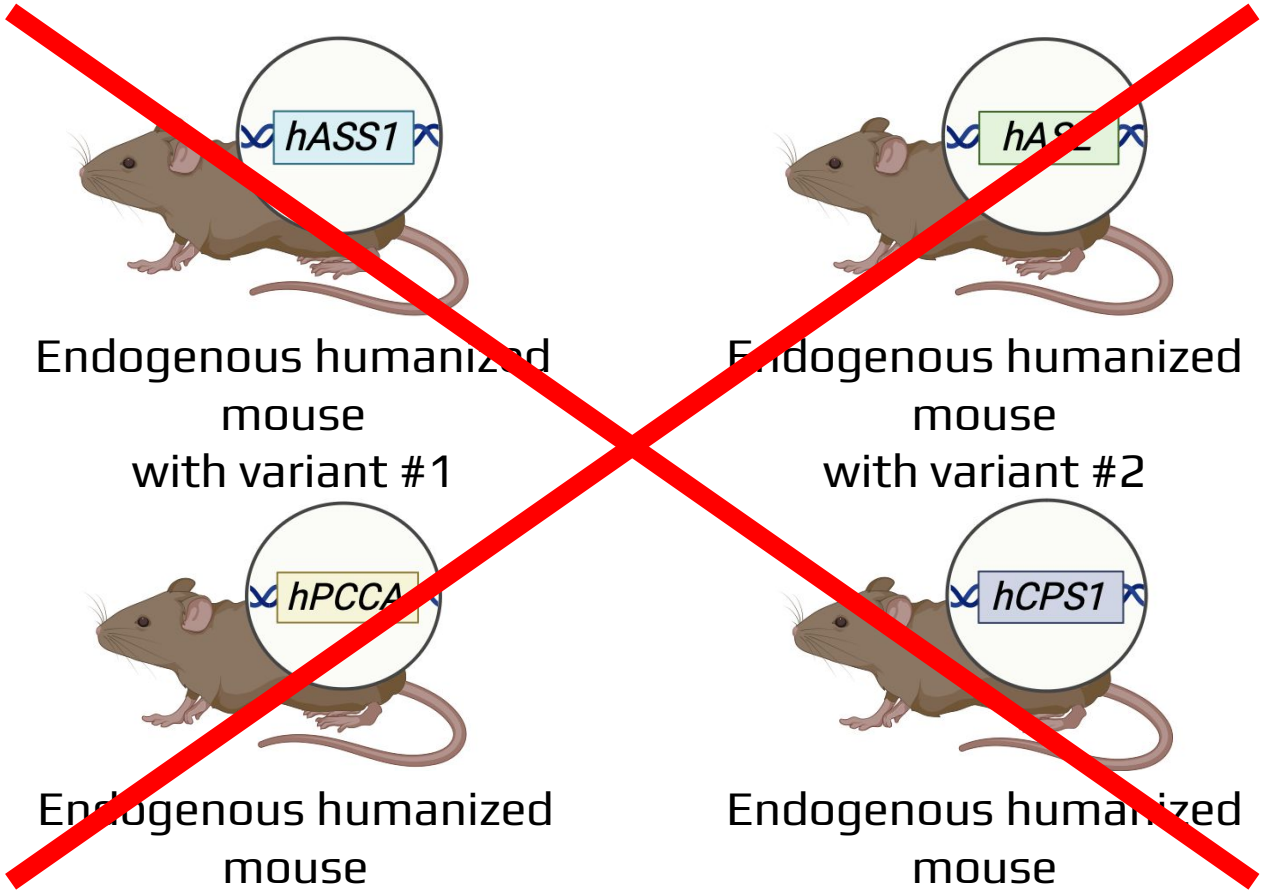
Transfections to screen editors/gRNAs—including validated “standards”

Rapid generation of mice for testing *in vivo* corrective editing

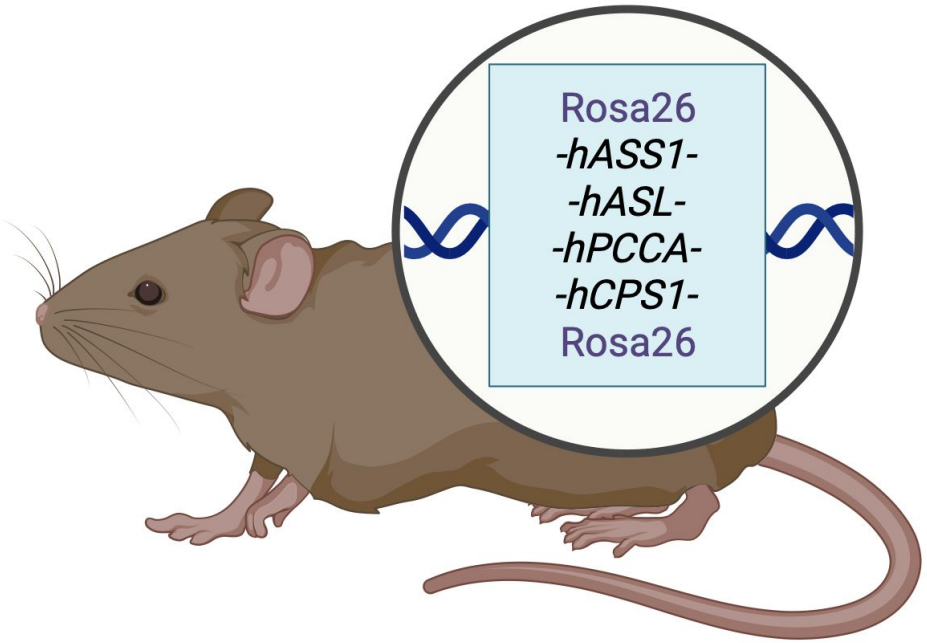


Endogenous humanized mouse with variant #1

Endogenous humanized mouse with variant #2

Endogenous humanized mouse with variant #3

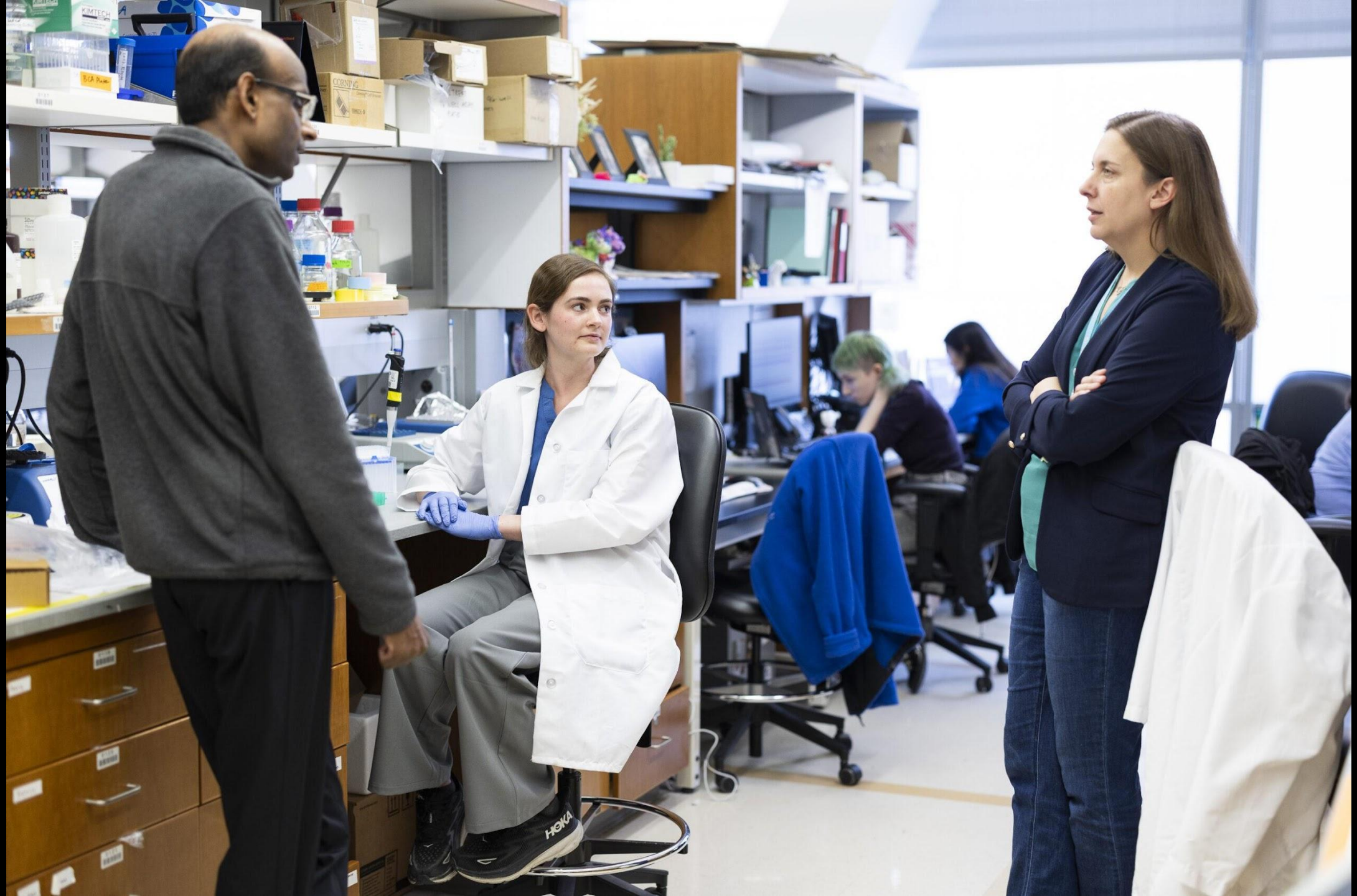
Endogenous humanized mouse with variant #4



Rosa26 safe harbor with all variants
= multi-variant mouse

Variants causing urea cycle disorders and organic acidemias

- citrullinemia type 1 = *ASS1* variants
- argininosuccinic aciduria = *ASL* variants
- CPS1 deficiency = *CPS1* variants
- OTC deficiency = *OTC* variants
- propionic acidemia = *PCCA* or *PCCB* variants
- methylmalonic acidemia = *MMUT* or *MMAB* variants
- newborn case

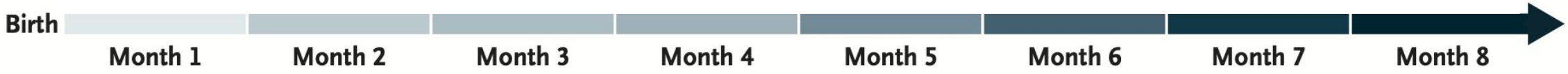

KIMTECH
CORNING
HOKA

New case of neonatal-onset urea cycle disorder

A 2-day-old male infant, named KJ, became lethargic and had respiratory distress.

The blood ammonia level was significantly elevated >1000 µmol/L (normal for age is <33 µmol/L).

He was transferred to the neonatal intensive care unit and rapidly started on life-saving dialysis.



Musunuru, Grandinette ... Ahrens-Nicklas. *N Engl J Med* 2025; online first

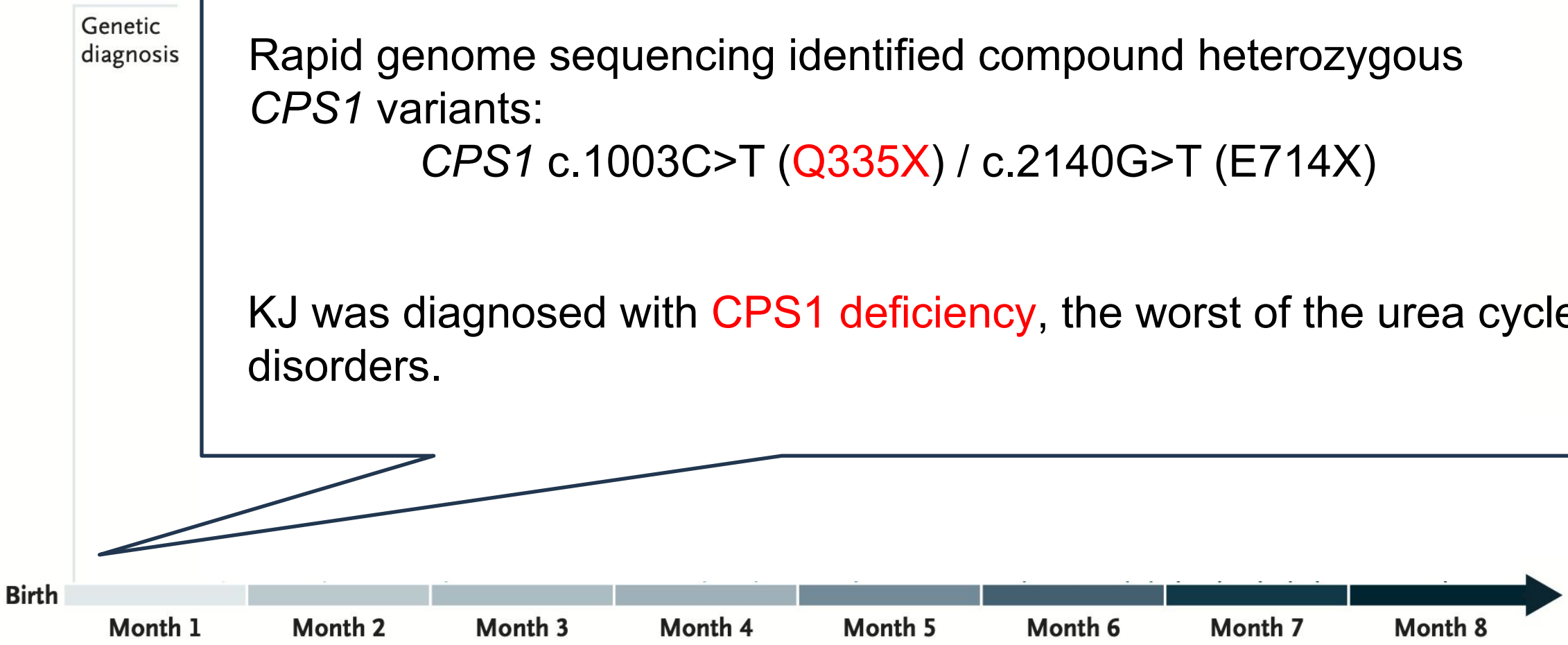
Genetic diagnosis

Metabolic testing confirmed a proximal urea cycle defect.

Rapid genome sequencing identified compound heterozygous *CPS1* variants:

CPS1 c.1003C>T (Q335X) / c.2140G>T (E714X)

KJ was diagnosed with CPS1 deficiency, the worst of the urea cycle disorders.

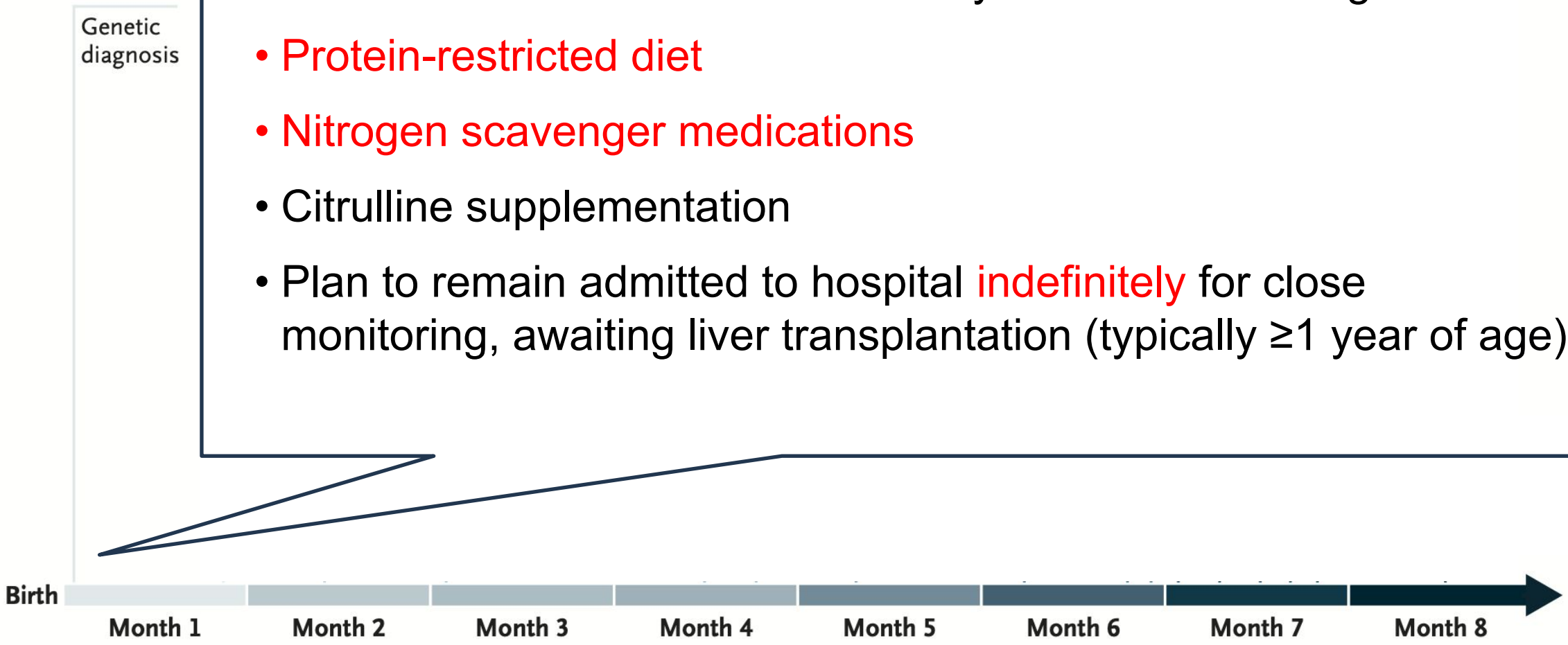


Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Genetic diagnosis

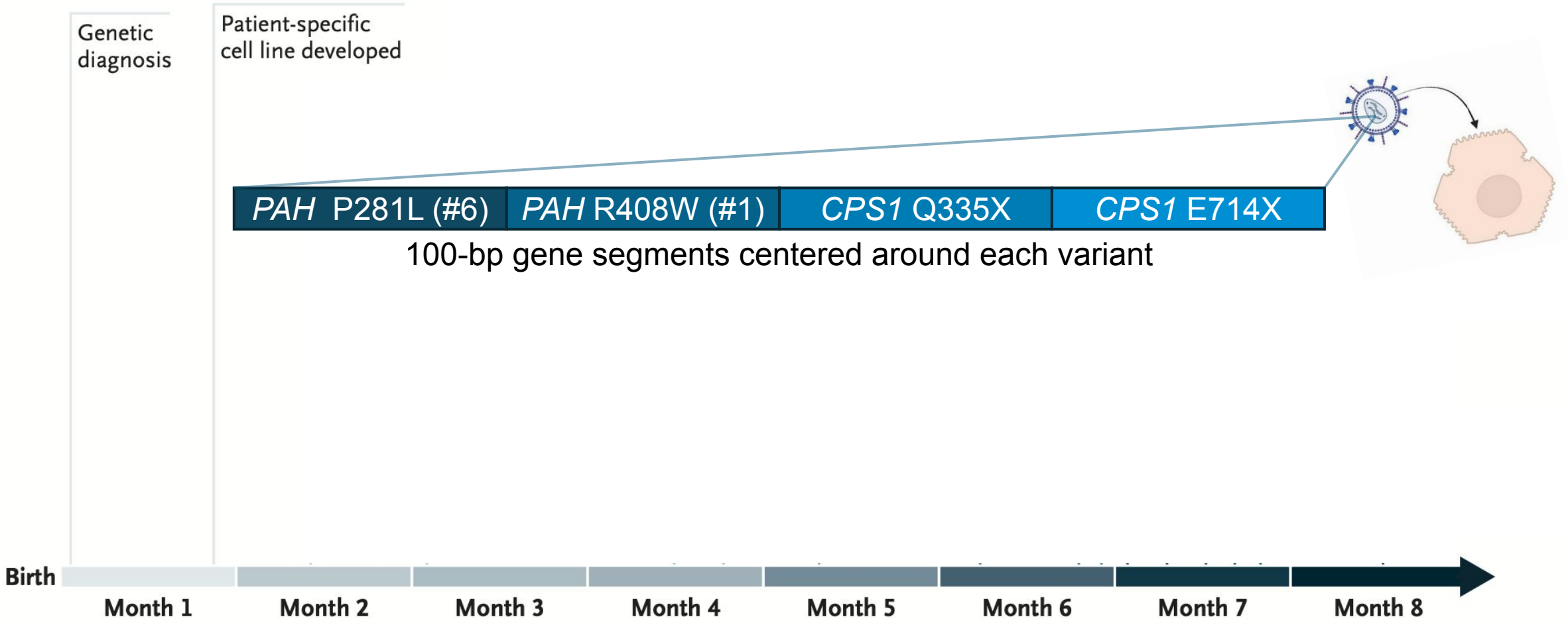
KJ was transitioned to chronic urea cycle disorder management:

- Protein-restricted diet
- Nitrogen scavenger medications
- Citrulline supplementation
- Plan to remain admitted to hospital indefinitely for close monitoring, awaiting liver transplantation (typically ≥1 year of age)



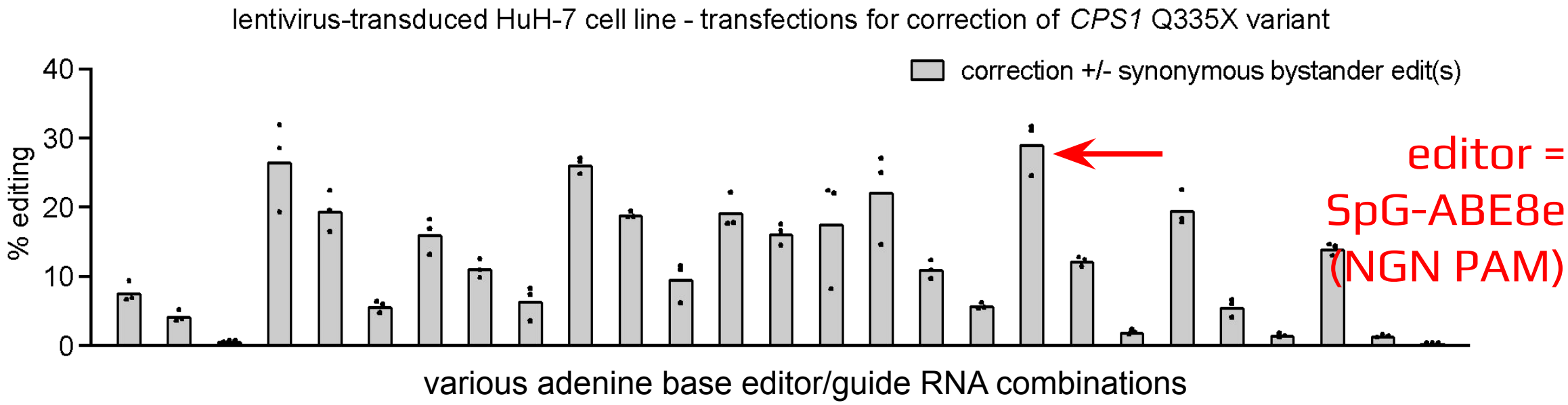
Musunuru, Grandinette ... Ahrens-Nicklas. *N Engl J Med* 2025; online first

Screening of base editors for correction of *CPS1* Q335X variant



Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

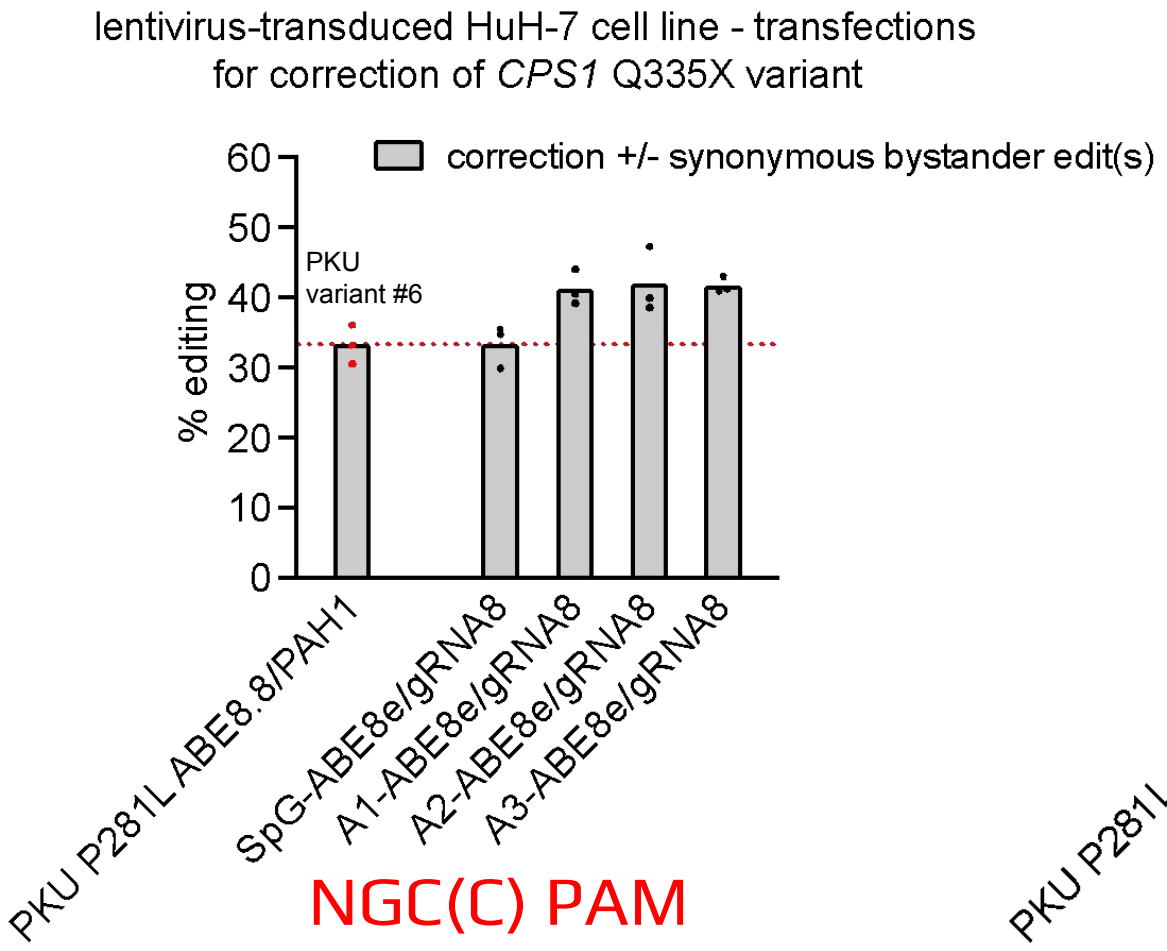
Screening of base editors for correction of *CPS1* Q335X variant



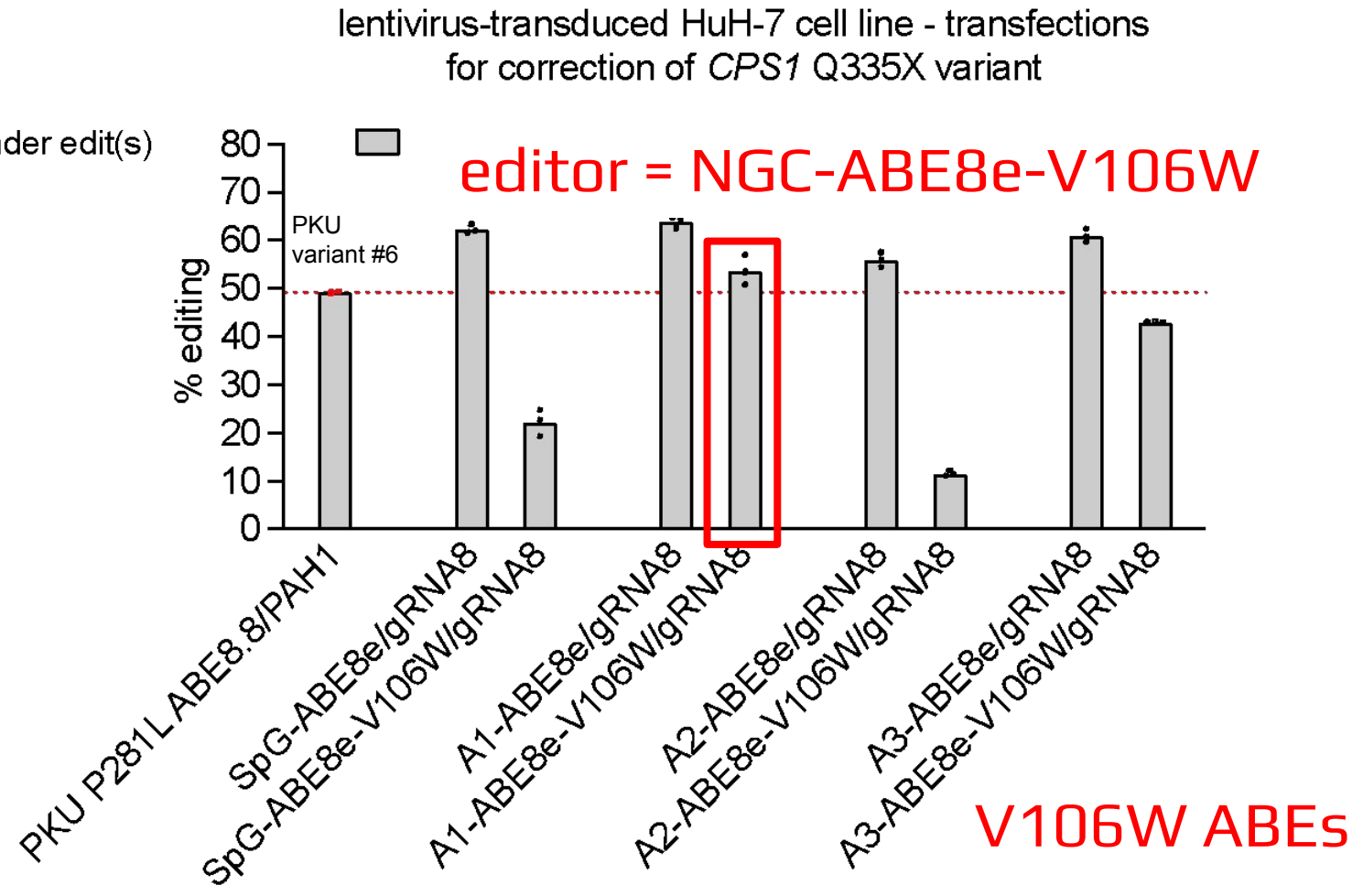
Results in less than 4 weeks

Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Screening of base editors for correction of *CPS1* Q335X variant



NGC(C) PAM editors

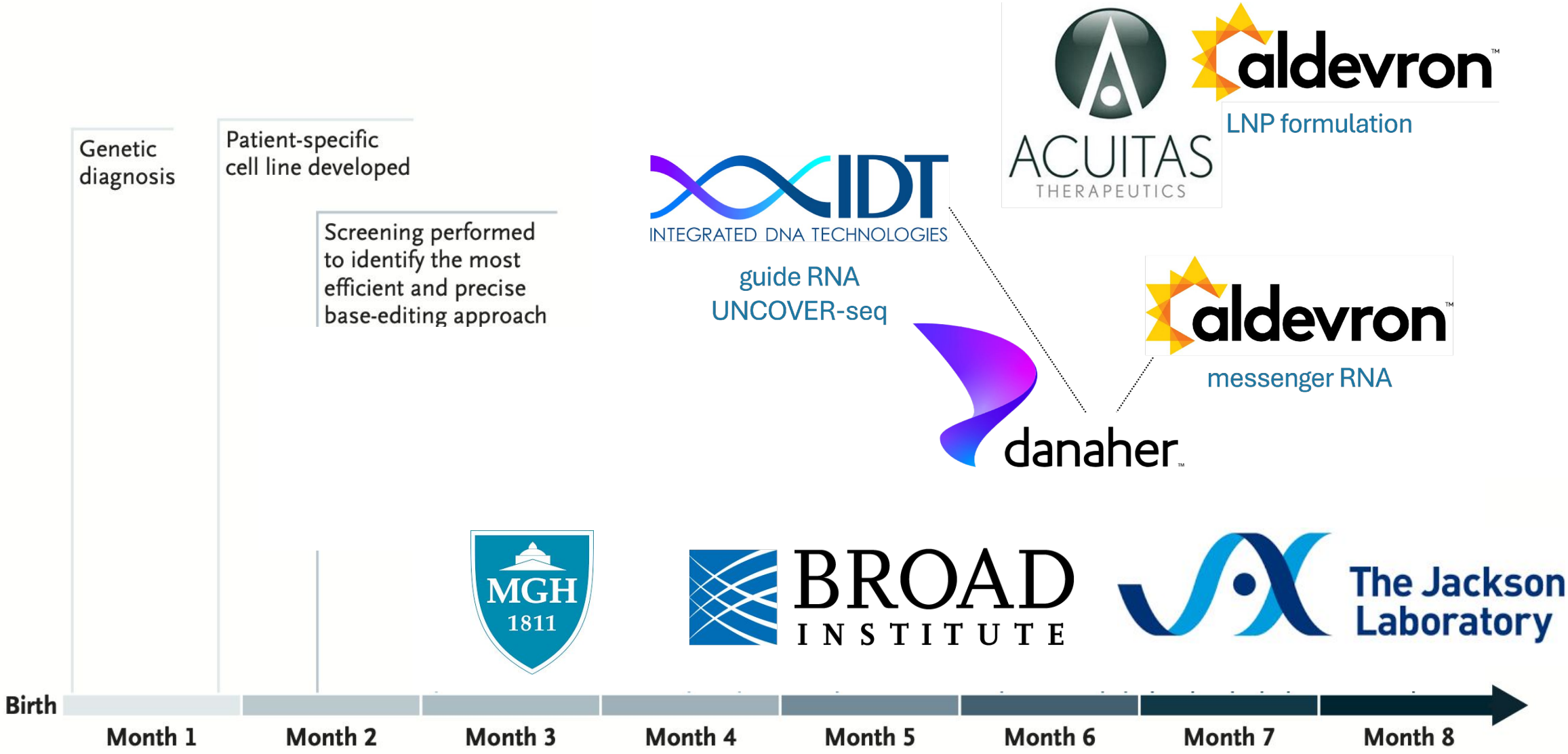


V106W ABEs

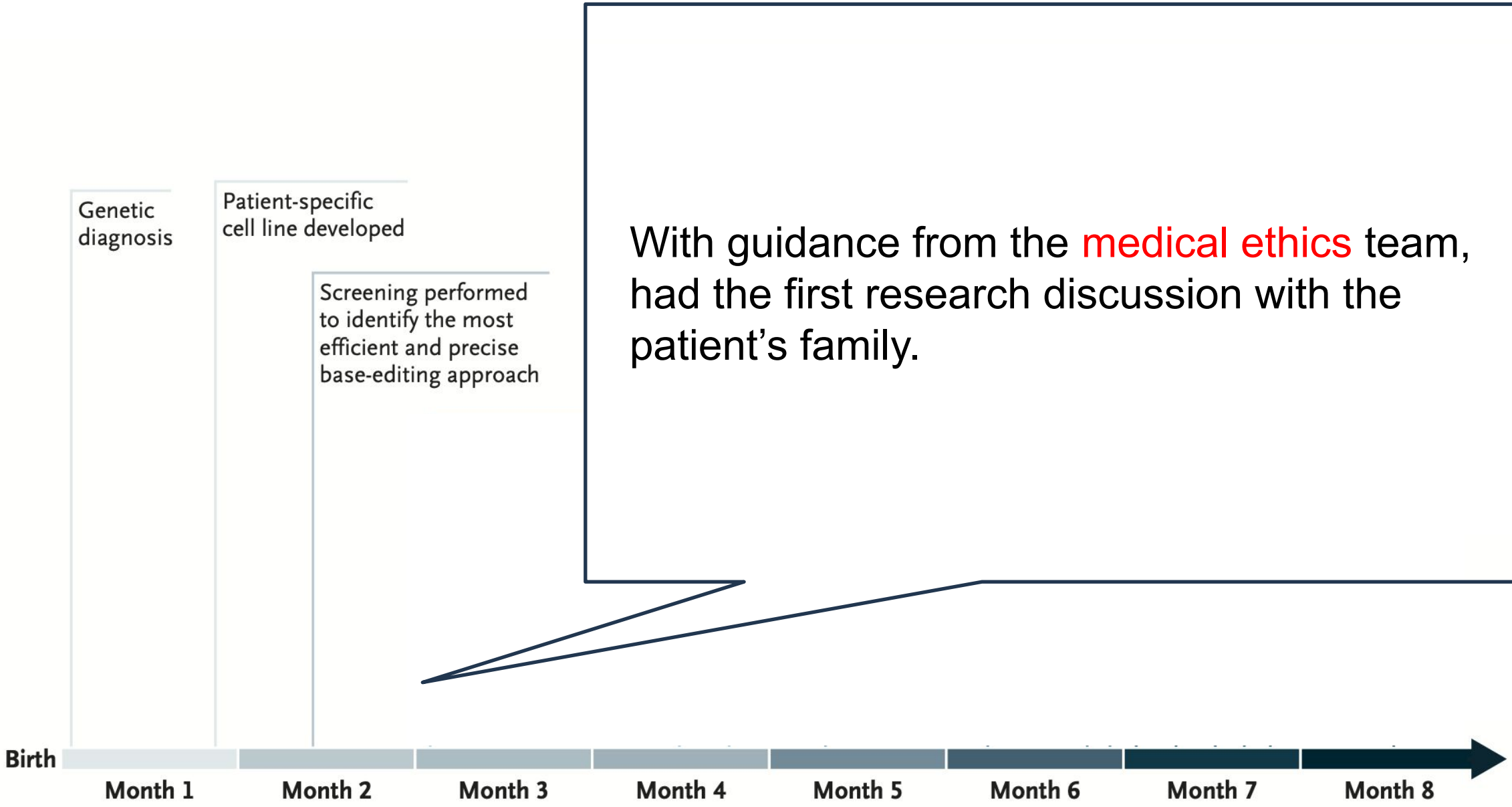
Completed in 6 weeks

Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

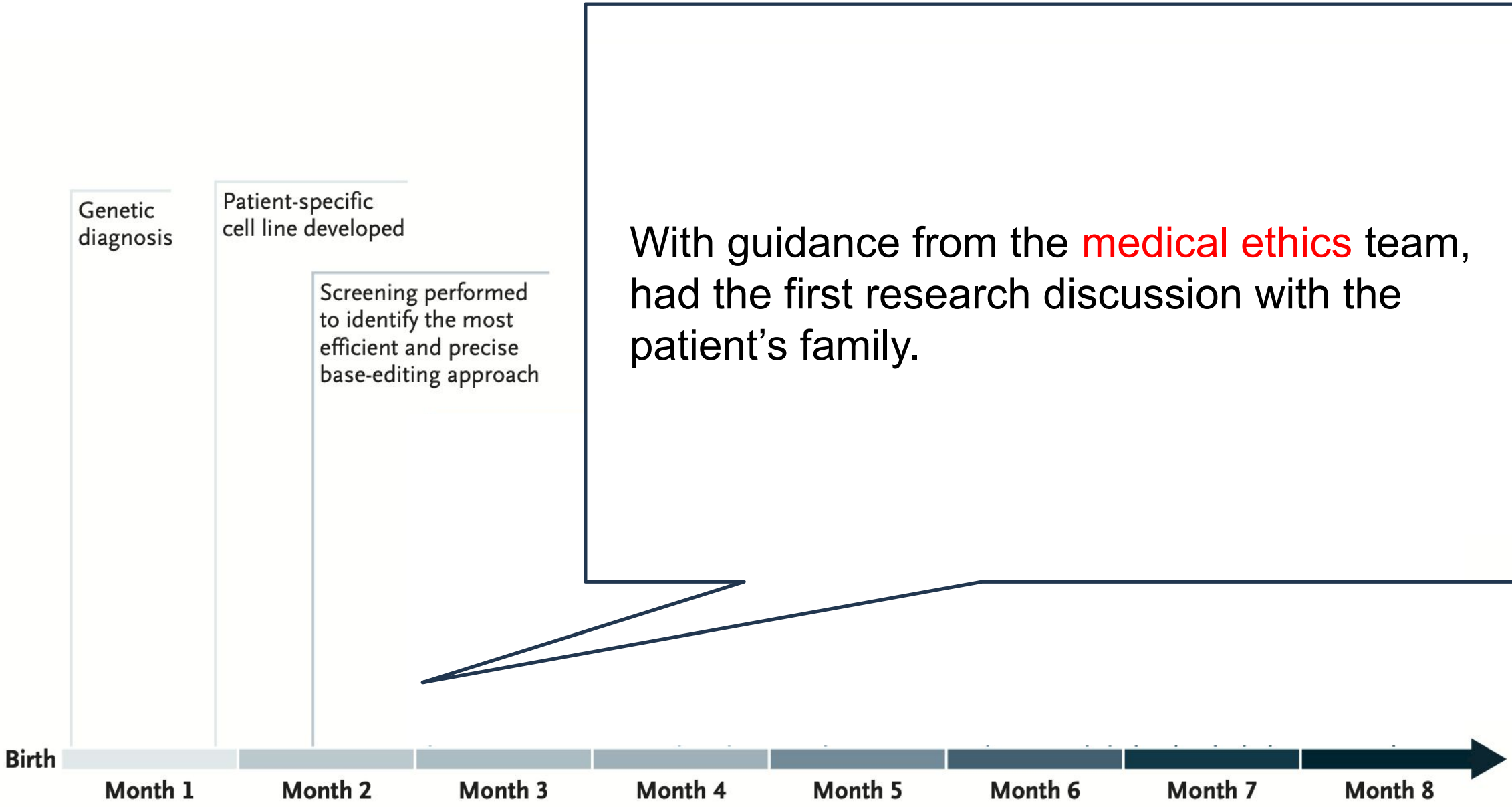
Assembly of a team of academic and industry partners



Musunuru, Grandinette ... Ahrens-Nicklas. *N Engl J Med* 2025; online first

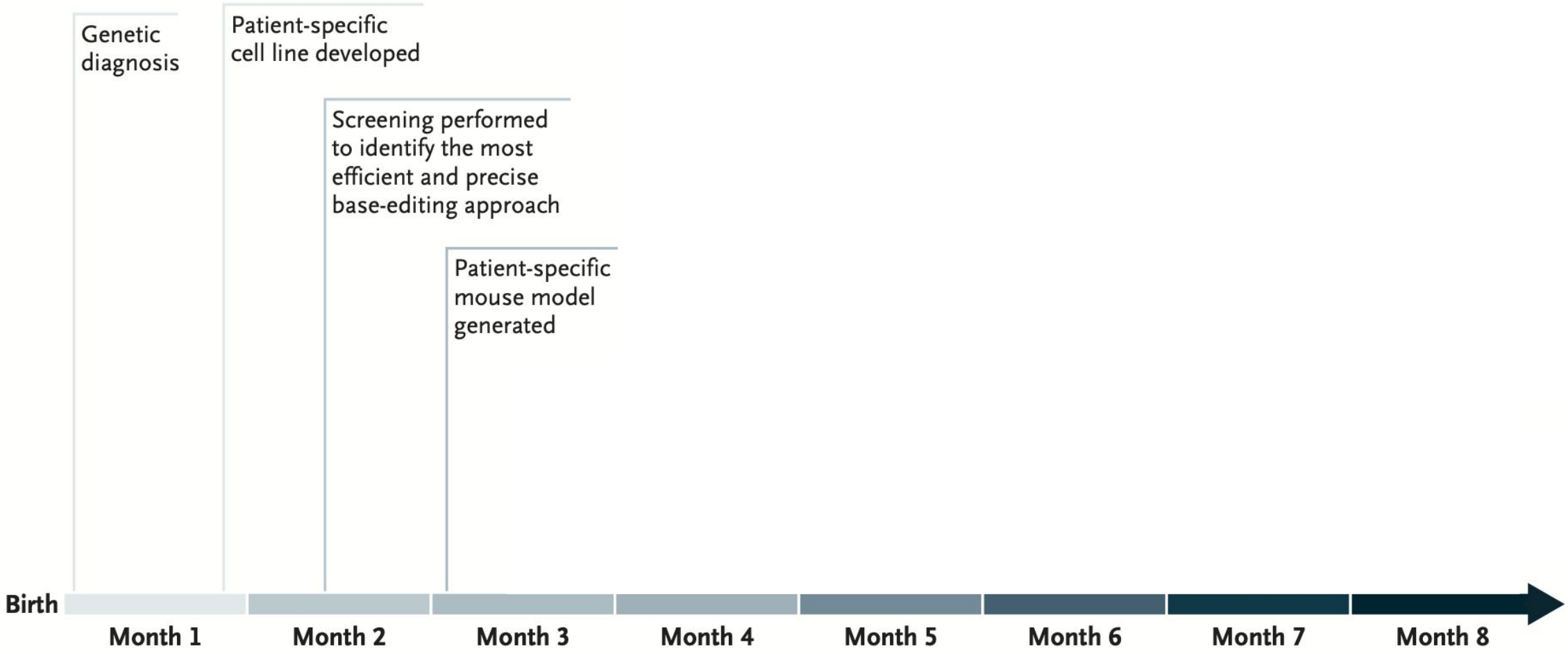


Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first



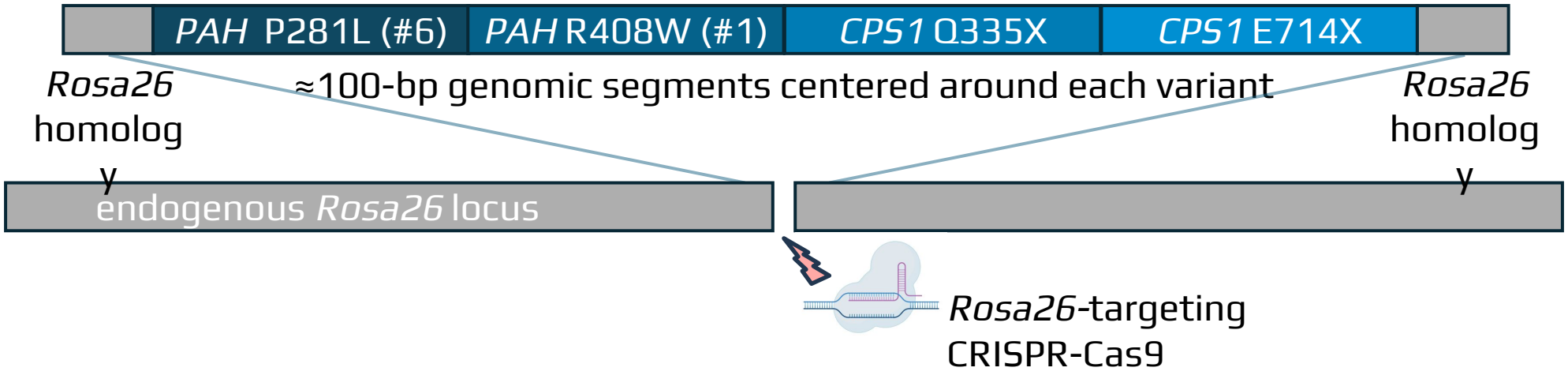
Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Generation of patient-specific Q335X mice for *in vivo* testing



Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Rosa26 multi-variant mice (and endogenous *Cps1*-Q335X mice)



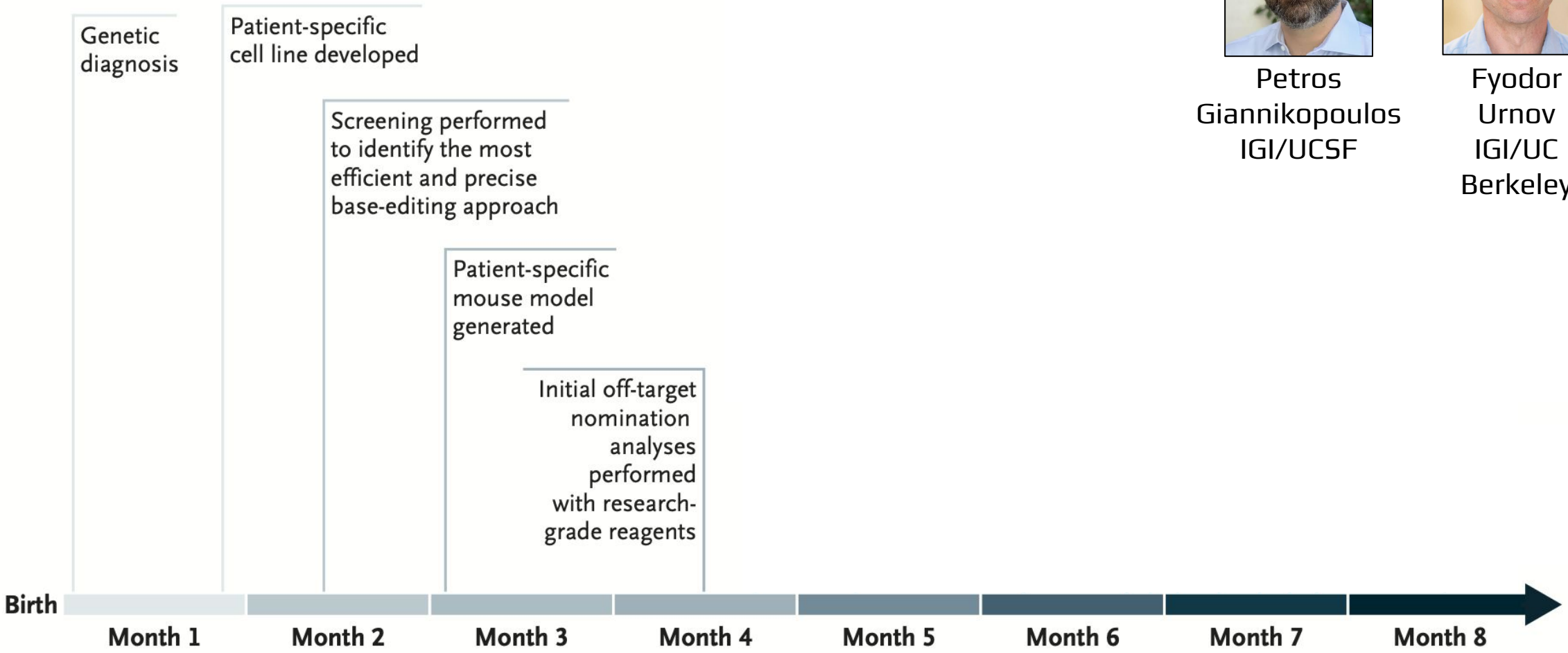
Obtained two founder Rosa26 *multi-variant mice (and one founder* Cps1*-Q335X mouse) in 2 months*

Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Initial off-target assessment

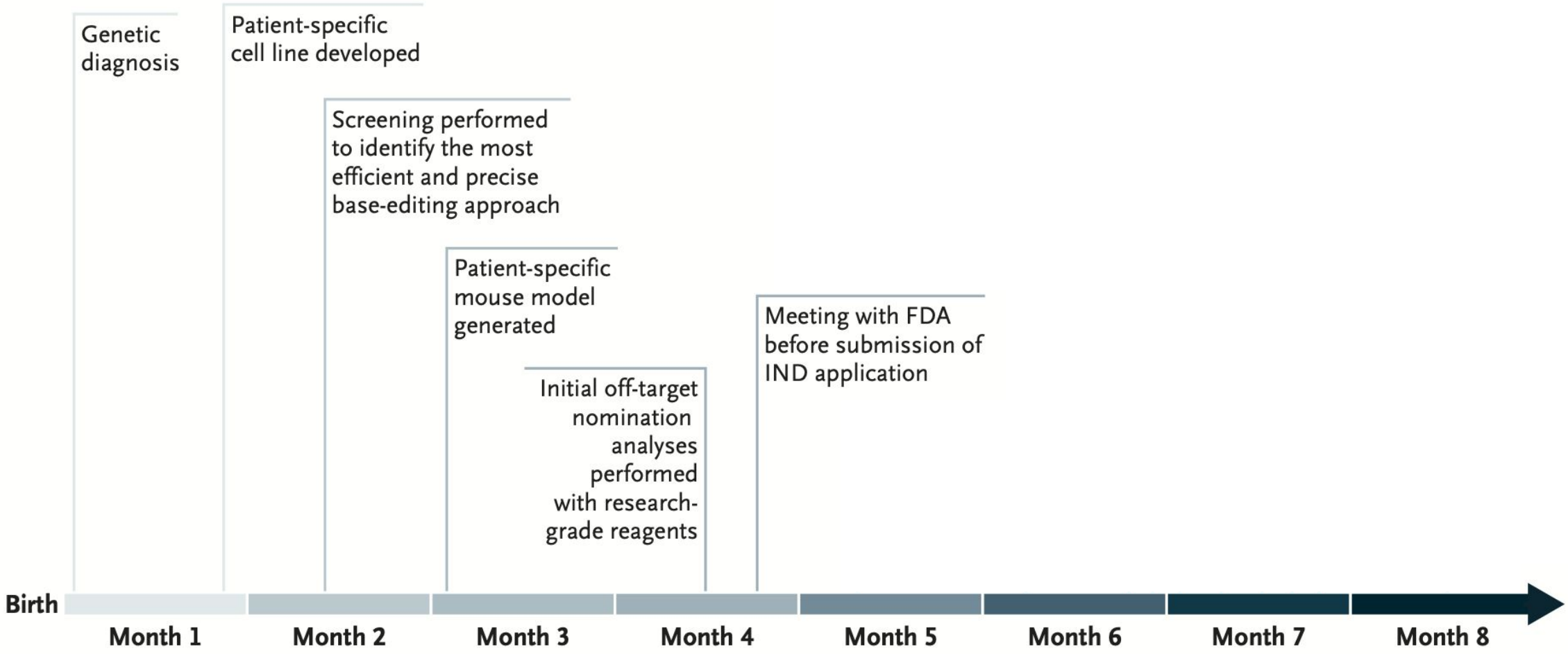
Petros Giannikopoulos
IGI/UCSF

Fyodor Urnov
IGI/UC Berkeley



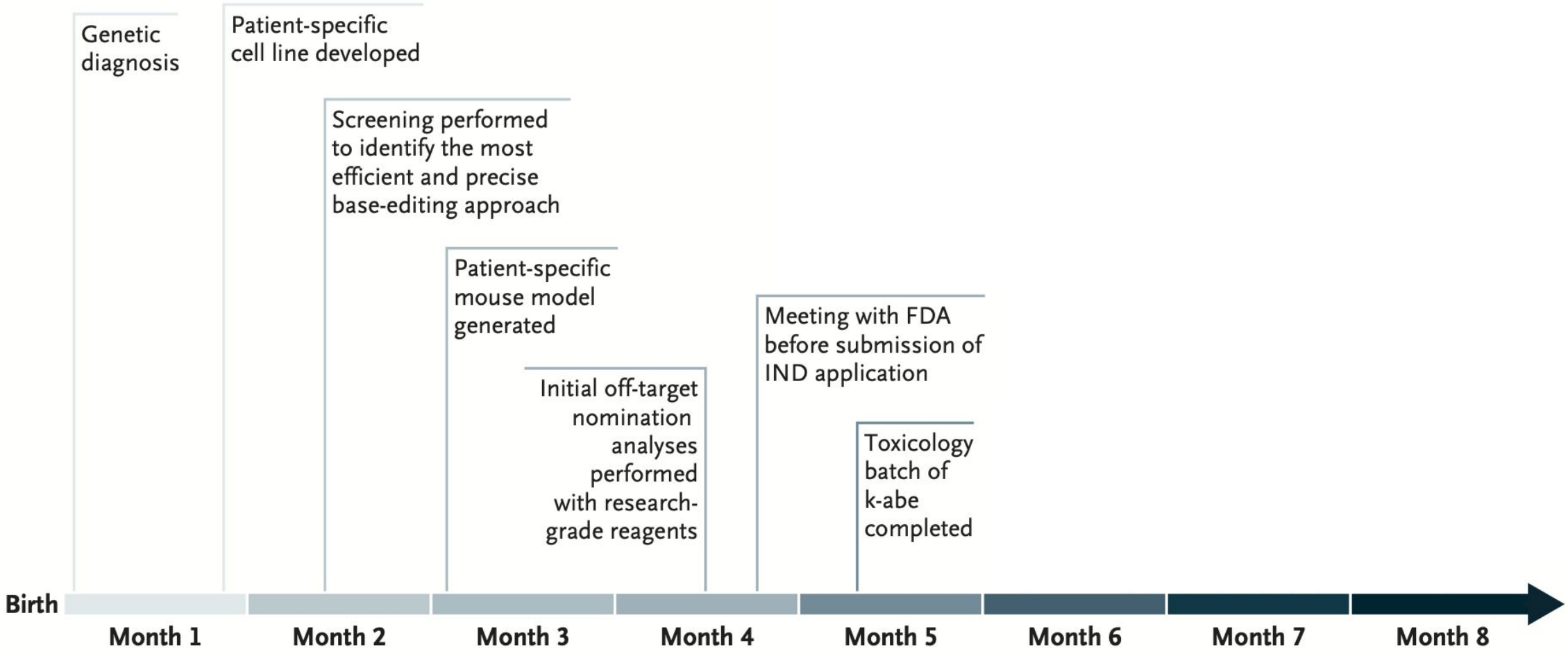
Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Pre-IND meeting with U.S. Food and Drug Administration (FDA)



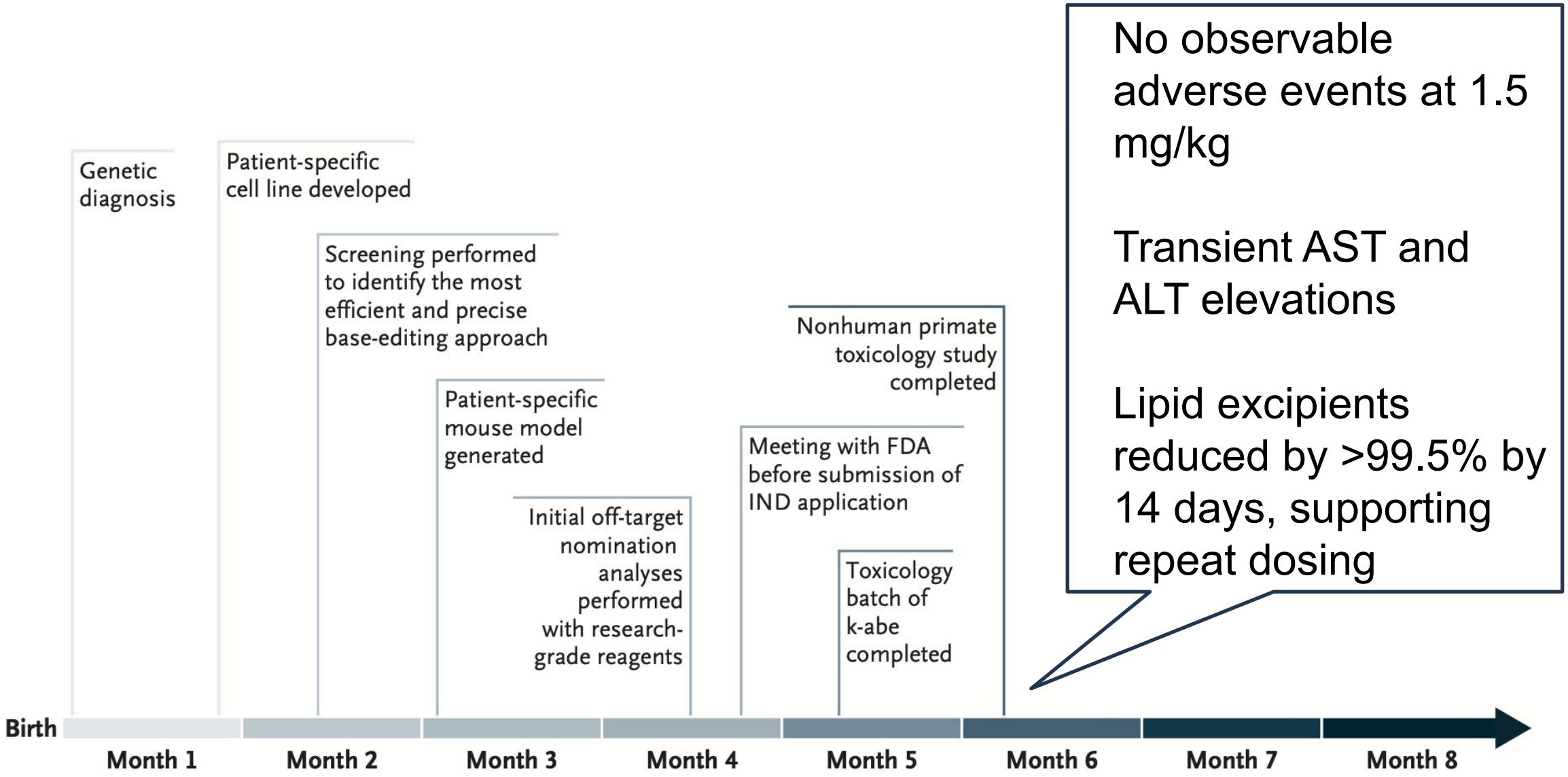
Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Toxicology batch of kayjayguran abengcemeran (k-abe)



Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Nonhuman primate toxicology study



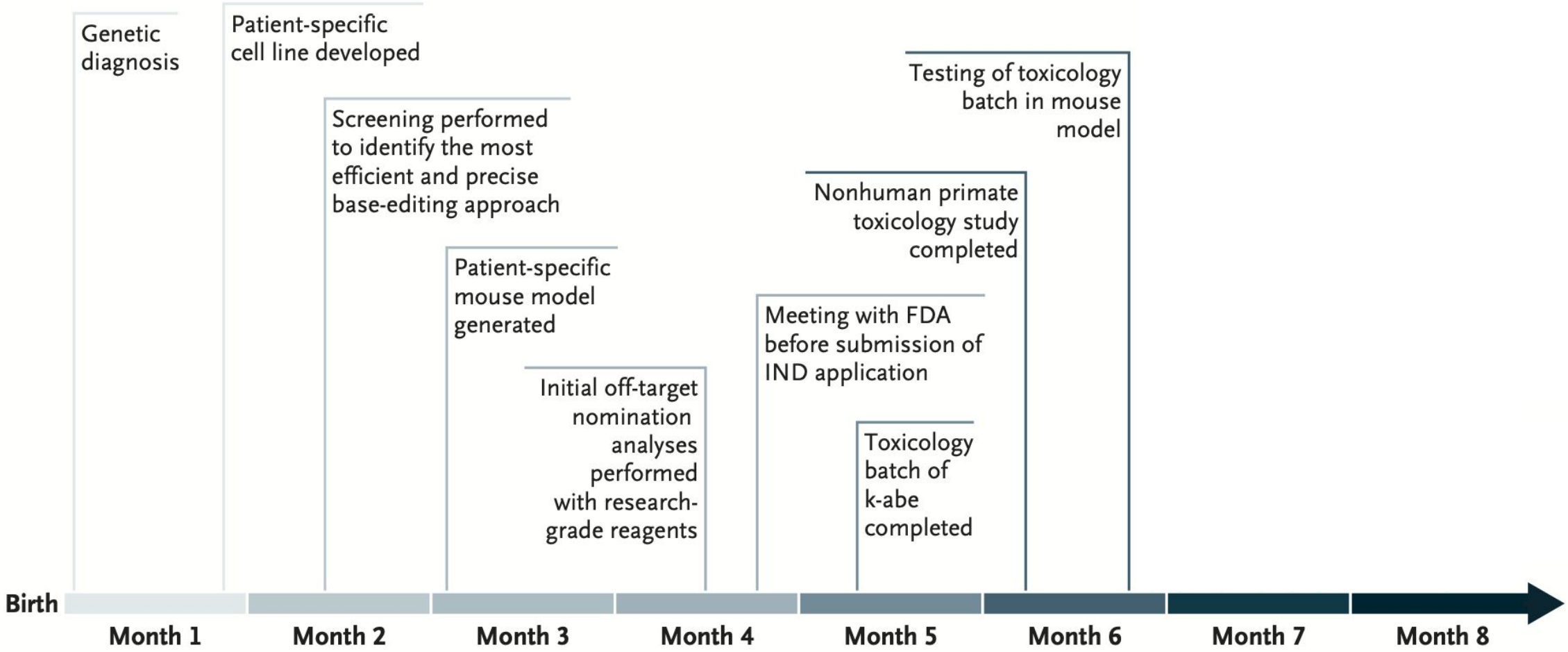
No observable adverse events at 1.5 mg/kg

Transient AST and ALT elevations

Lipid excipients reduced by >99.5% by 14 days, supporting repeat dosing

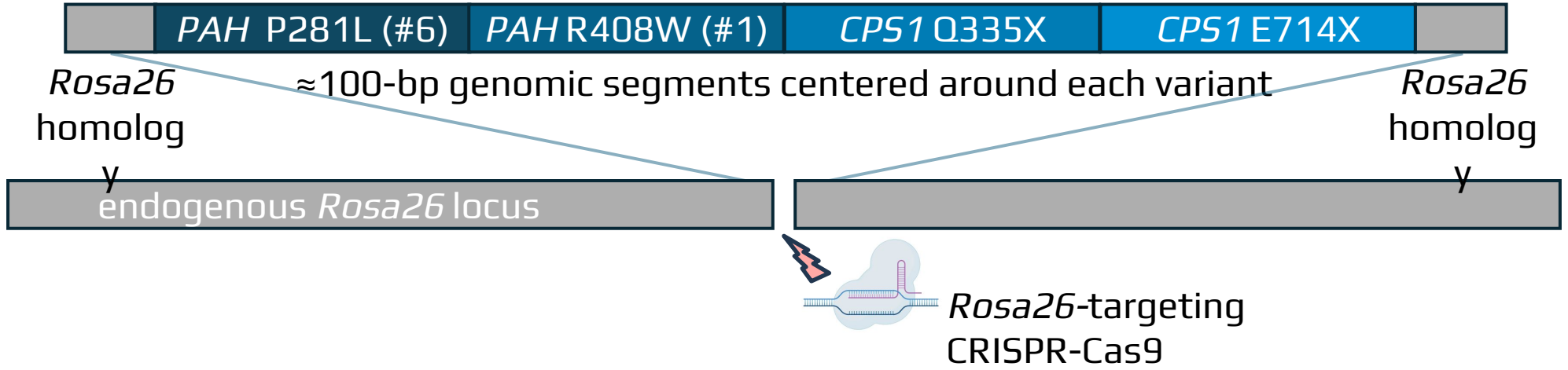
Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

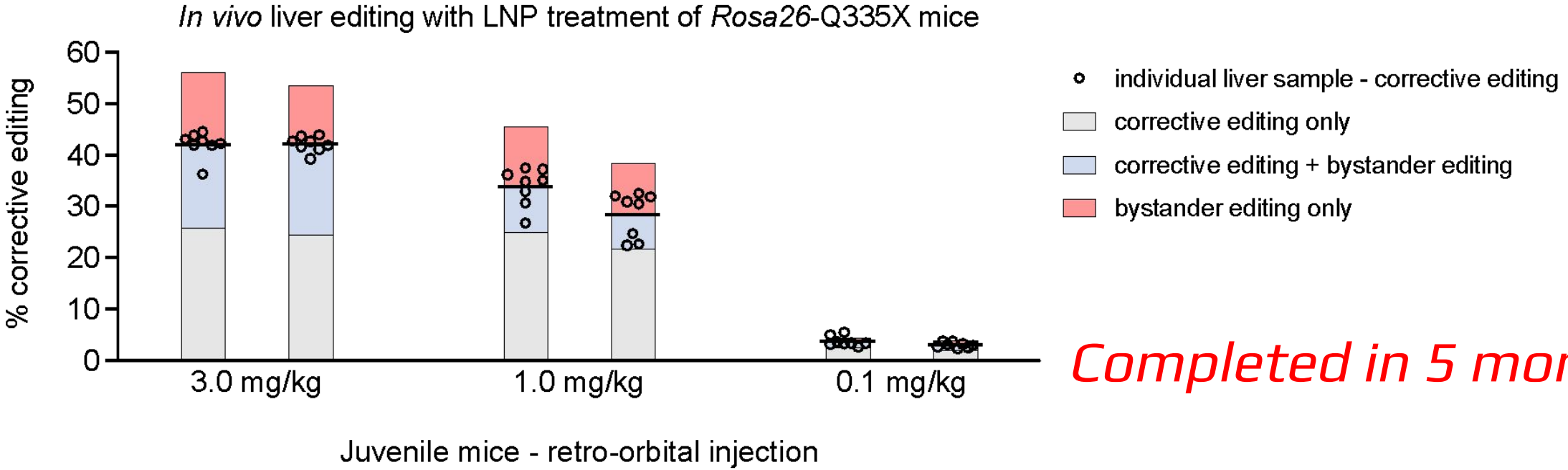
Testing of toxicology batch of k-abe in *Rosa26* multi-variant mice



Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Correction of *CPS1* Q335X variant in *Rosa26* multi-variant mice

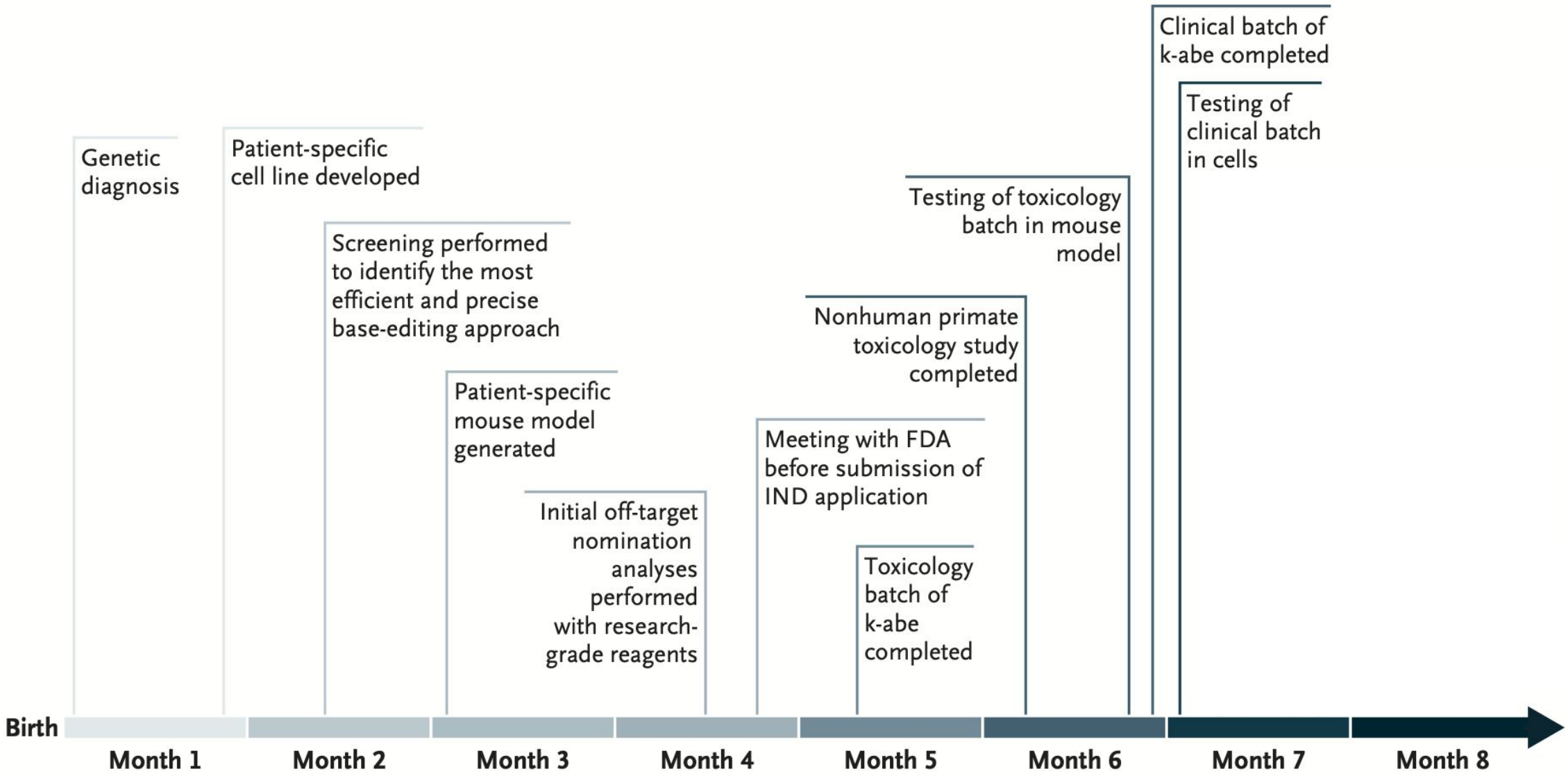




Completed in 5 months

Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

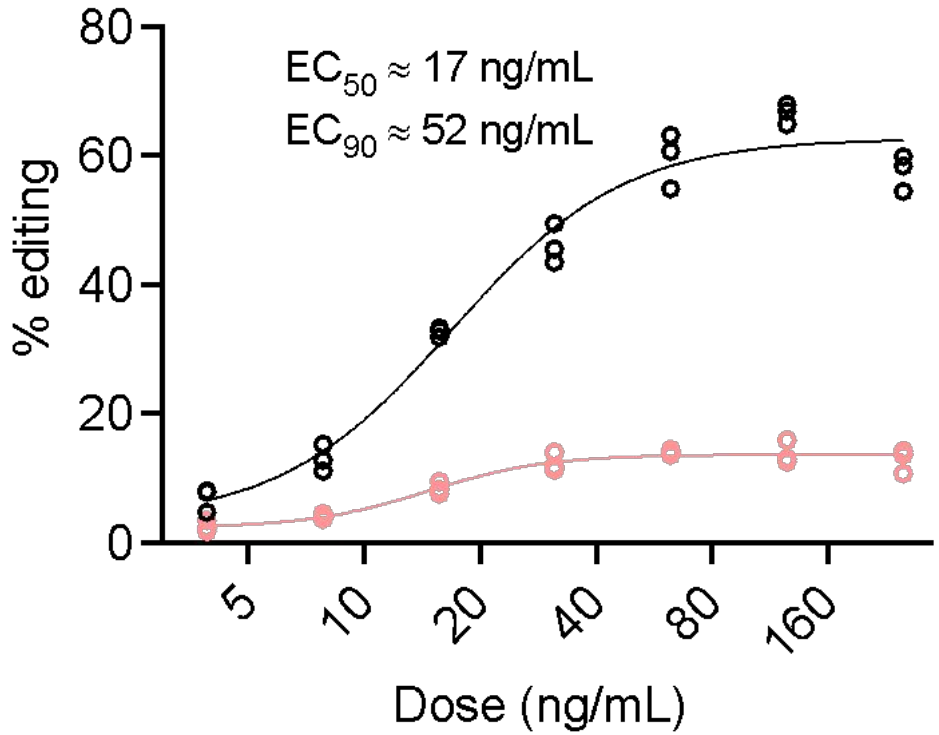
Manufacturing of clinical batch of k-abe and testing in cells



Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Manufacturing of clinical batch of k-abe and testing in cells

Q335X lentivirus-transduced HuH-7 cells treated with k-abe

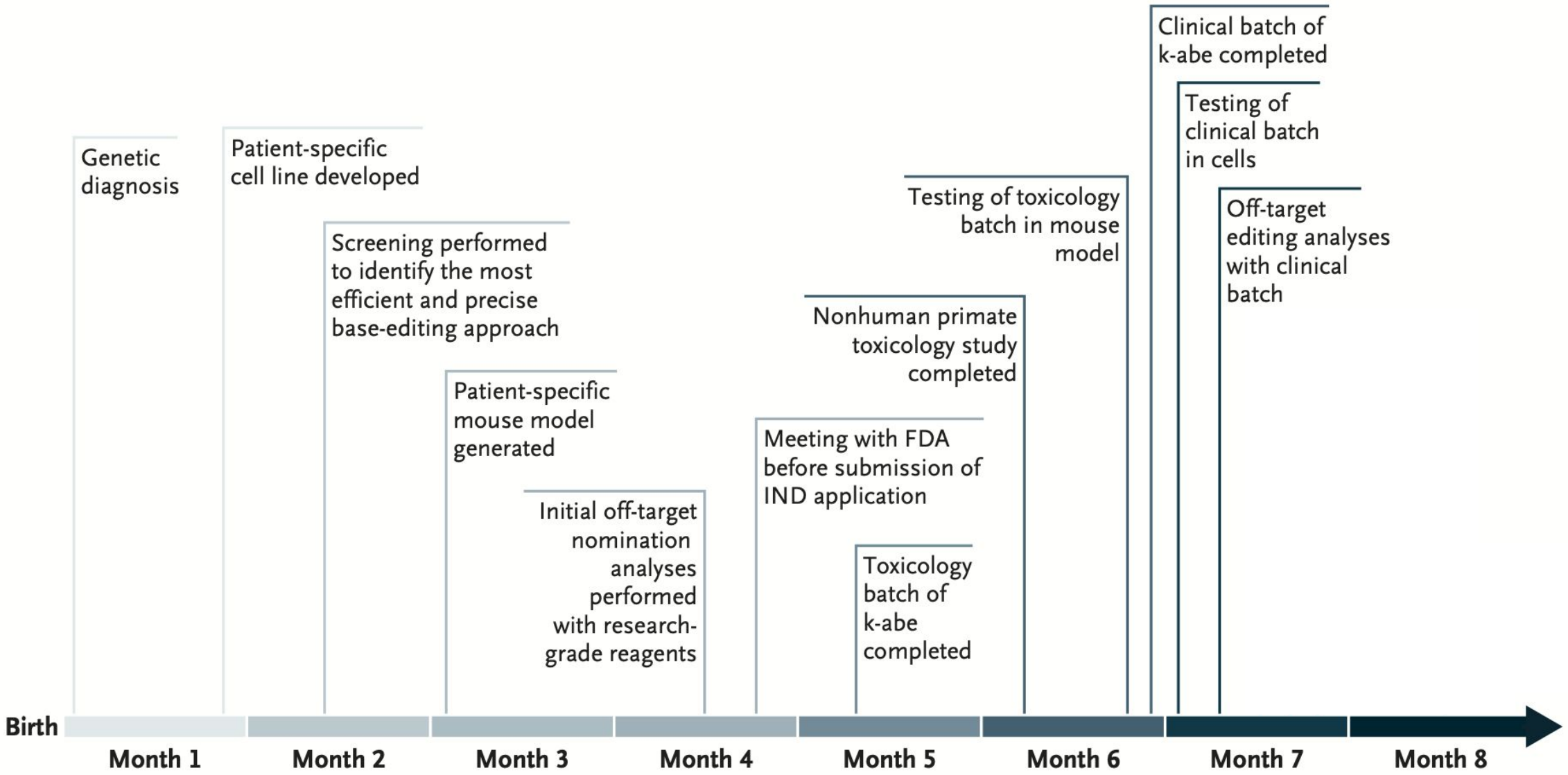


corrective editing +/- bystander editing

bystander editing alone

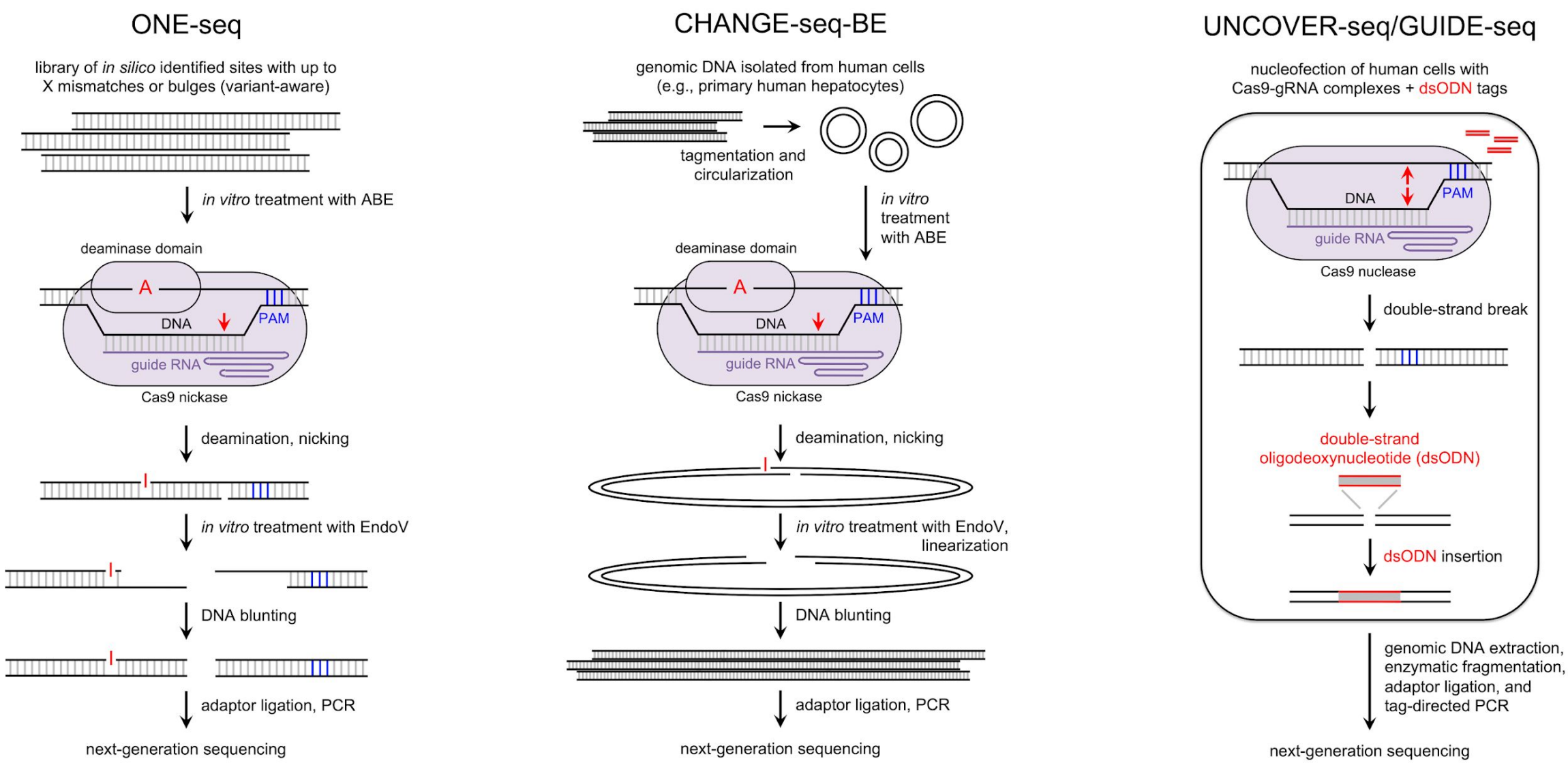
Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Off-target analyses with clinical batch of k-abe



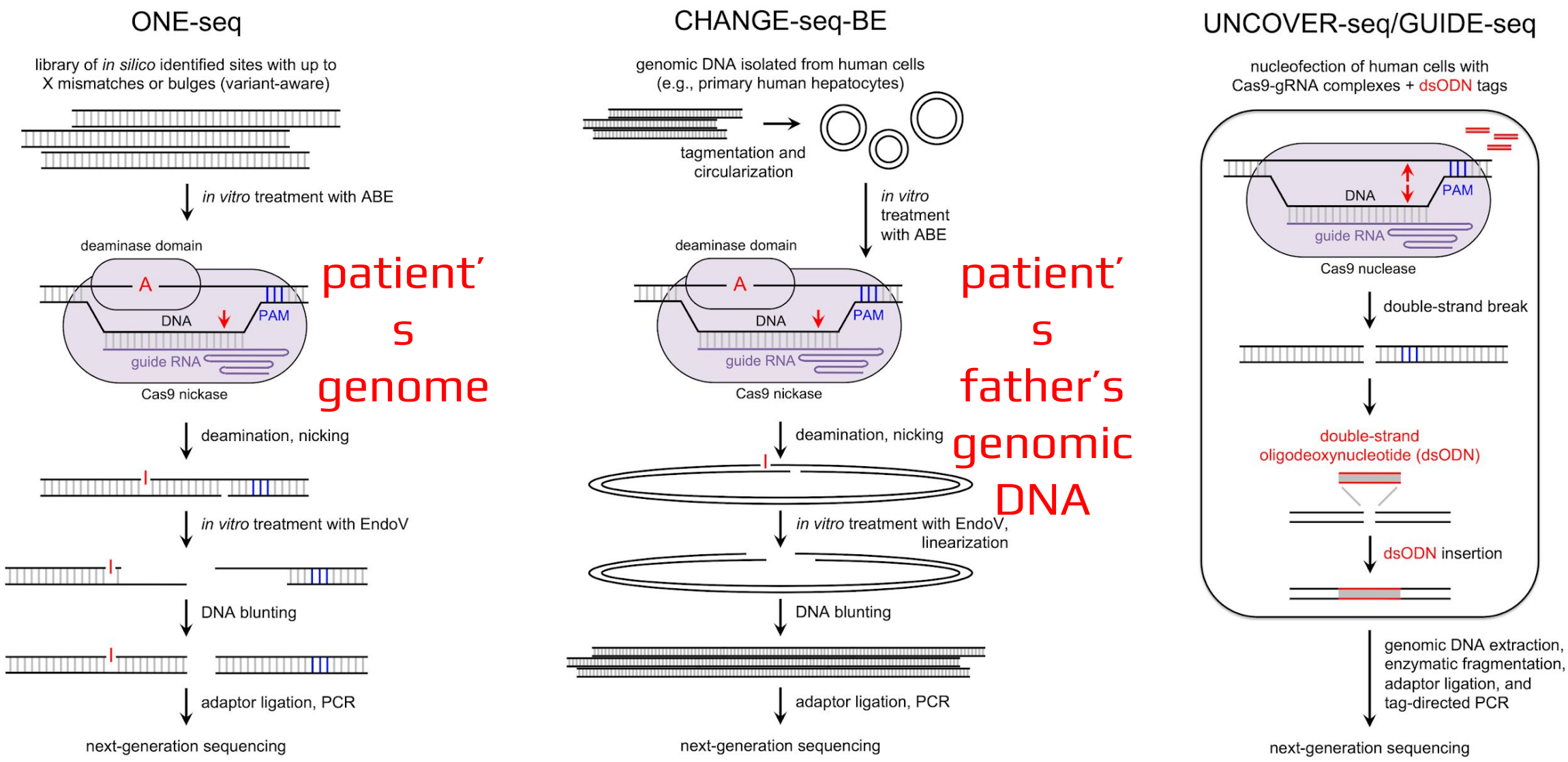
Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Patient-specific off-target analyses



Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Patient-specific off-target analyses



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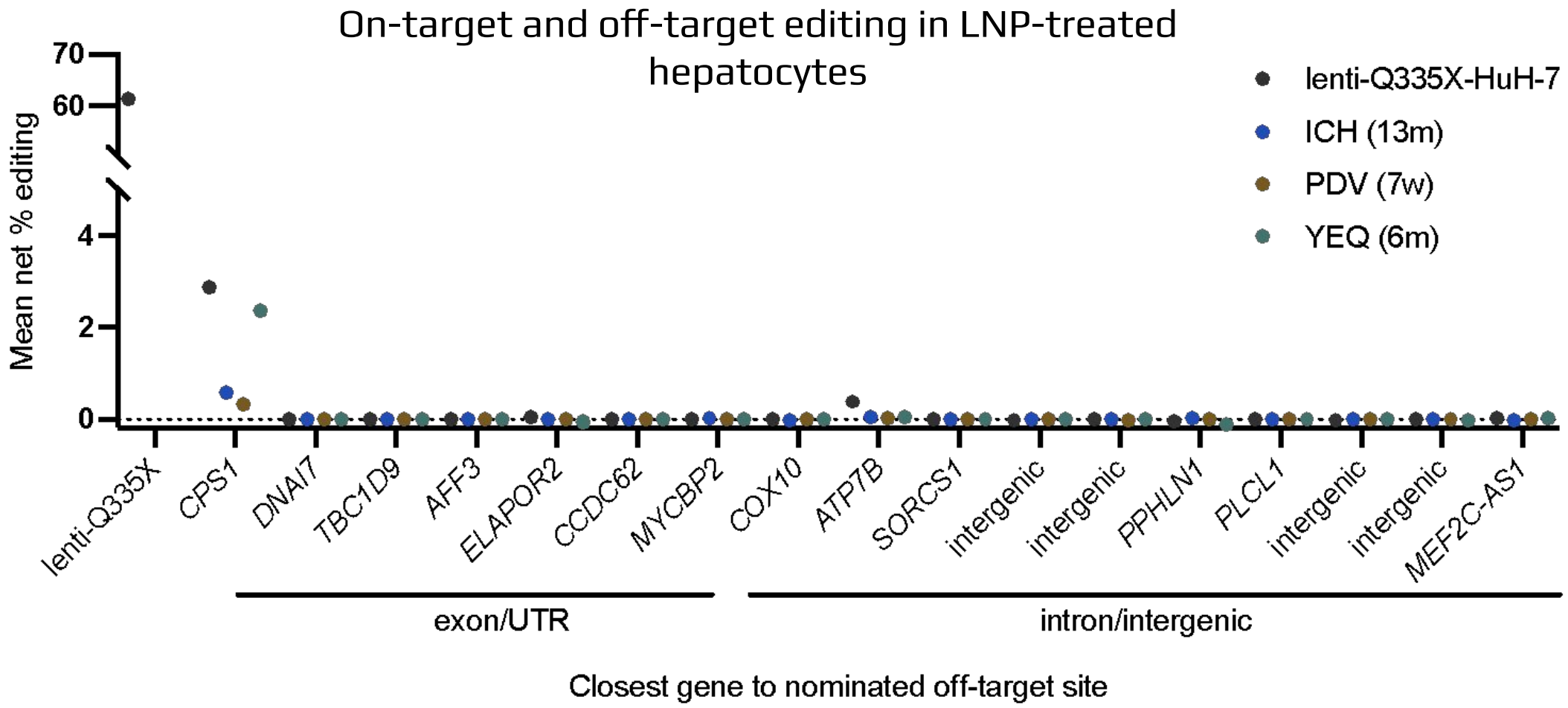
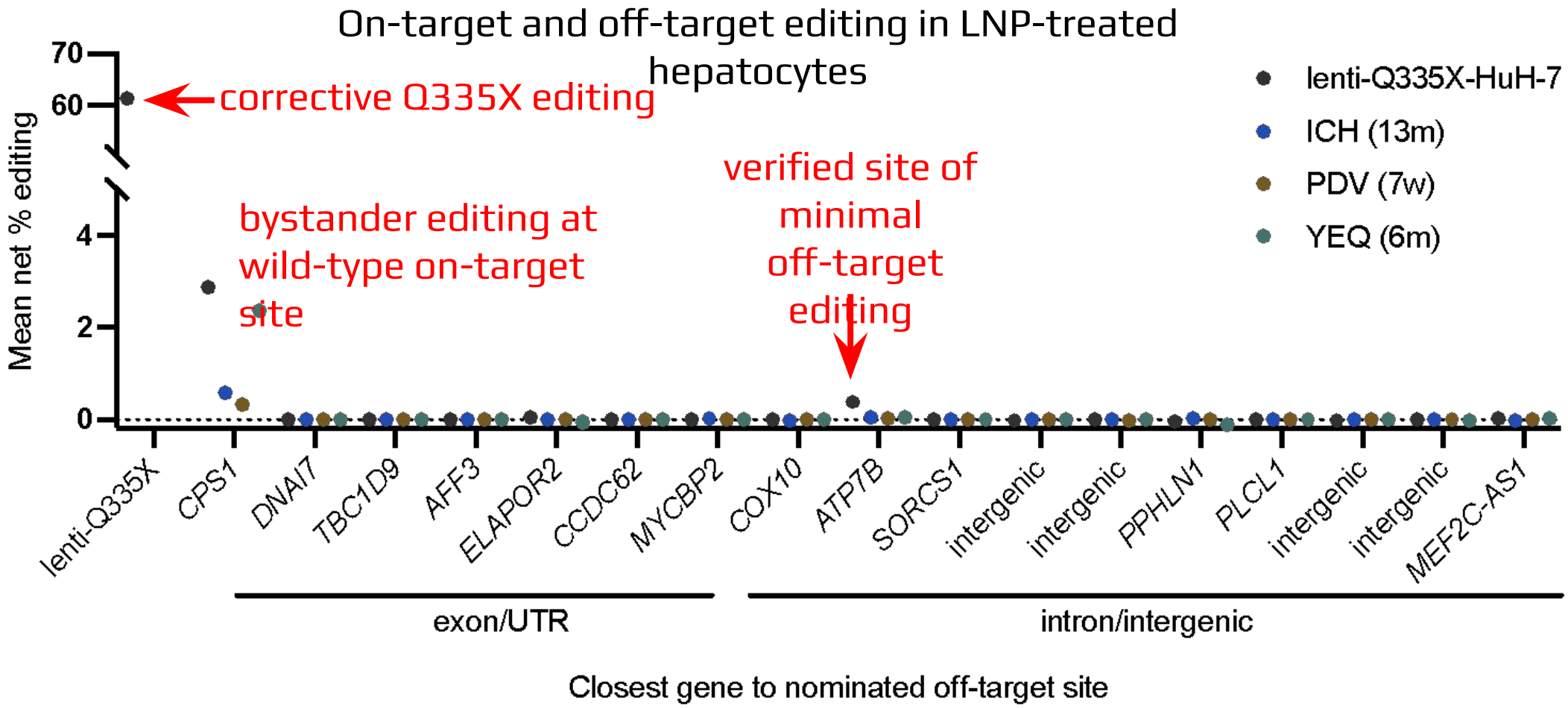
Patient-specific off-target analyses



Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

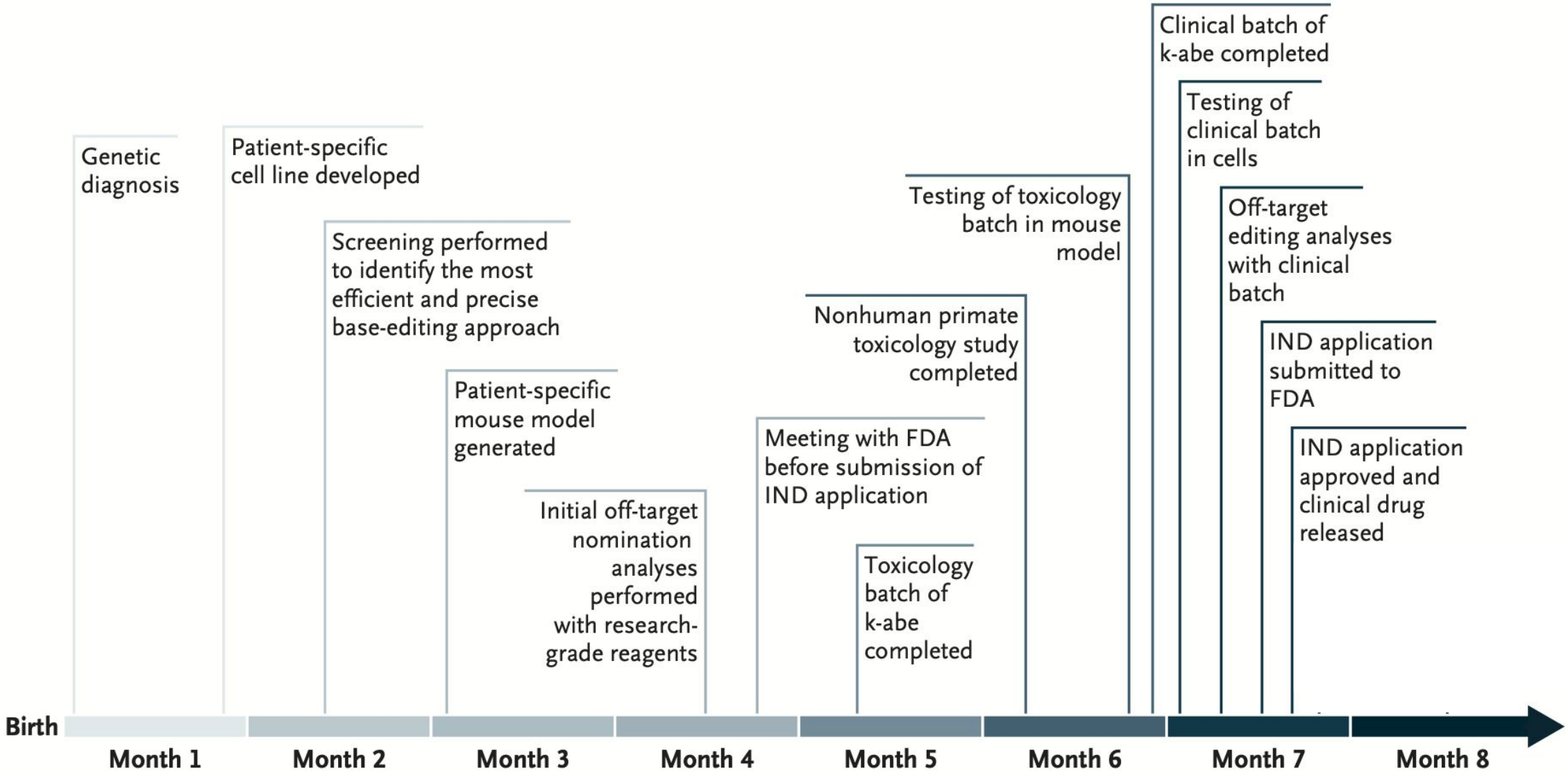
Patient-specific off-target analyses



Completed in 6 months

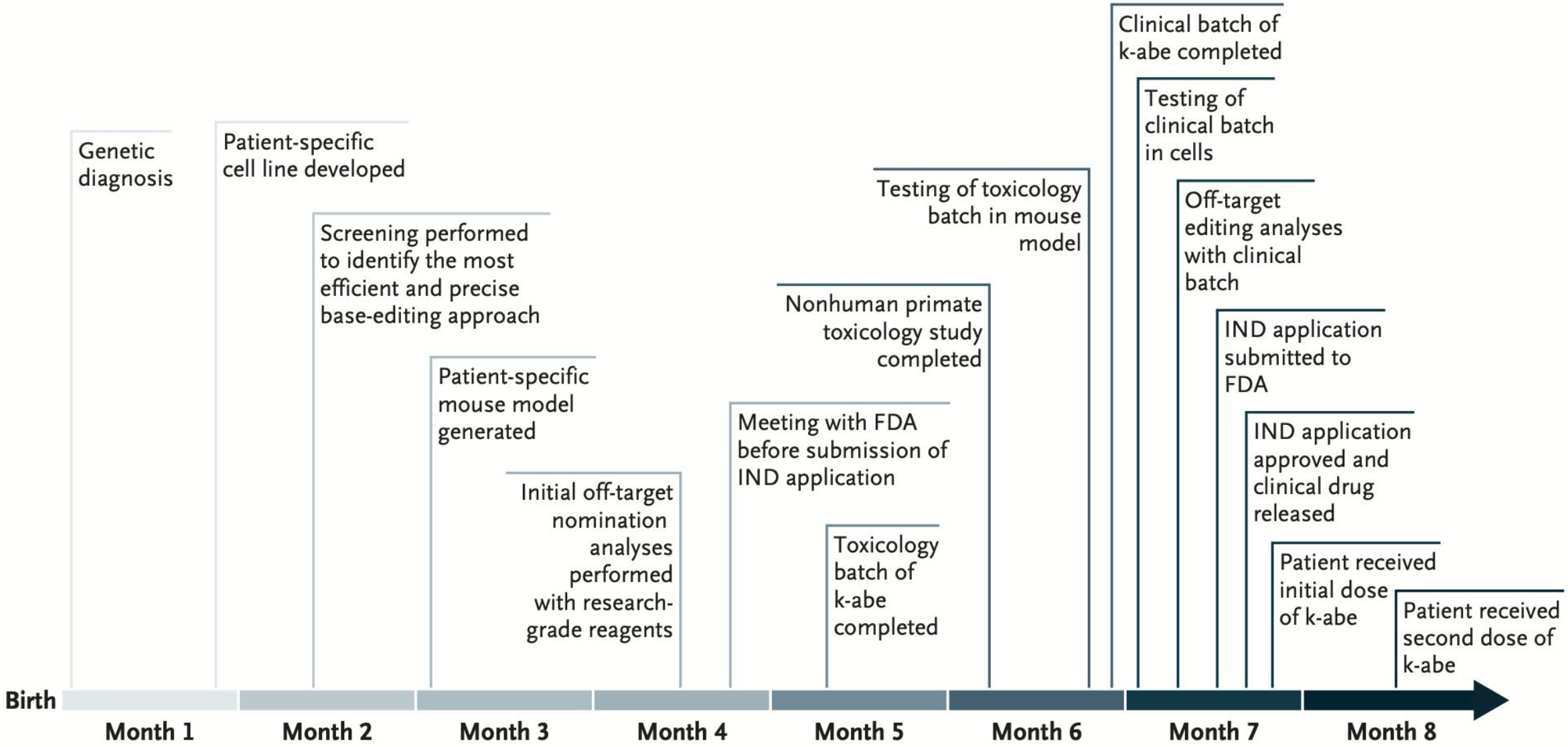
Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Single patient expanded access IND application to FDA



Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Initial treatment with k-abe (day 208 after birth)



Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Single-patient dose escalation plan as part of clinical care

- Initial low dose (0.1 mg/kg) to ensure safety
- Maximum of 3 doses
- At least 21 days between doses
- All doses must be given by 120 days
- As he is presumed CRIM-negative (no full-length protein), given steroid-sparing immunosuppression regimen with sirolimus and tacrolimus
- Decisions to re-dose made by clinical oversight committee with members from:
  - Metabolism
  - Liver transplant
  - Immunology
  - Hematology
  - Gene therapy team
  - Medical ethics

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  - Hematology
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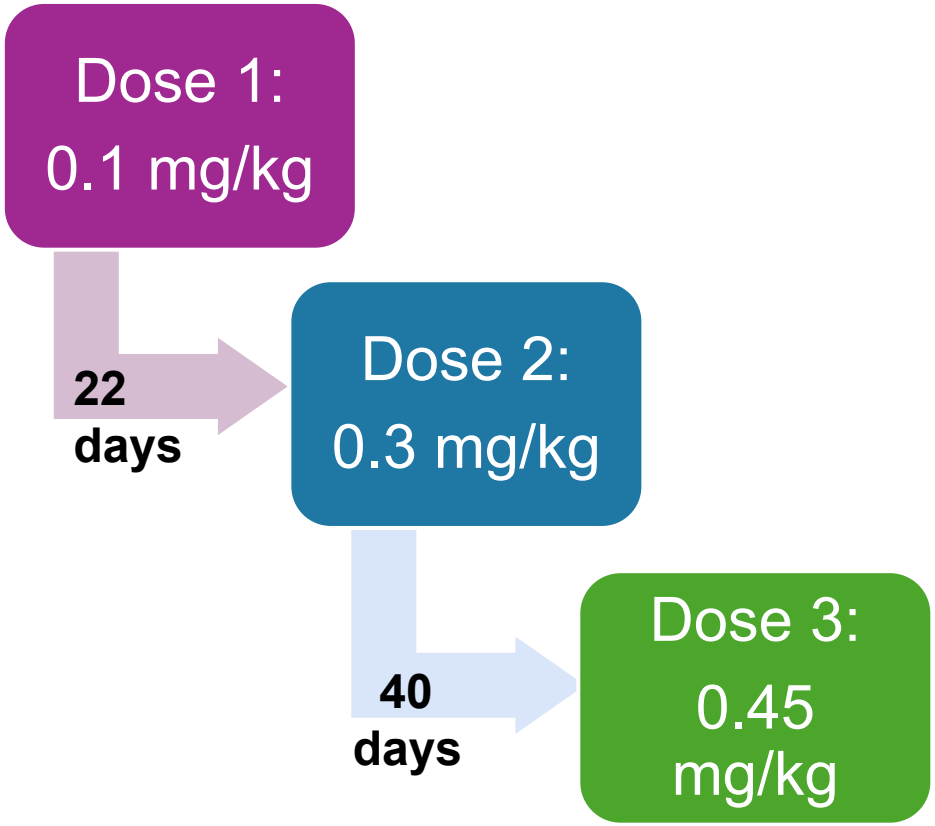
  - Medical ethics

Actual dosing schedule



Treatment with k-abe resulted in no serious adverse events

- Brief coughing episode at beginning of dose 2 and of dose 3; after initial episode, no further cough and able to tolerate full rate of infusion
- Low-grade fever and transient rash after dose 3
- Mild, transient, asymptomatic, dose-dependent increases in ALT with no other liver function abnormalities

= viral illnesses

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Higher protein tolerance, weaning of nitrogen scavenger medication

= viral illnesses

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Higher protein tolerance, weaning of nitrogen scavenger medication

= viral illnesses

Musunuru, Grandinette … Ahrens-Nicklas. *N Engl J Med* 2025; online first

Longer follow-up is need to understand efficacy and safety

Dose 1: 0.1 mg/kg

- No serious adverse events
- Able to increase dietary protein intake to 100% recommended dietary allowance
- Unable to wean nitrogen scavenger medications

22 days

Dose 2: 0.3 mg/kg

- No serious adverse events
- Able to wean nitrogen scavengers by half
- Recovered from viral illnesses without decompensation

40 days

Dose 3: 0.45 mg/kg

- No serious adverse events
- More time needed to evaluate response

Liver biopsy to assess *CPS1* Q335X editing was not completed due to the risk of the procedure

Conclusions, challenges, and opportunities

- KJ will likely continue to need some urea cycle management, but early signs suggest that his disease may be less severe
- It is possible to develop a personalized gene-editing therapy in 6 months
- Repeated doses of an LNP base-editing therapy can be safely given to an infant
- Longer follow-up and studies of additional non-invasive markers are needed to quantify potential benefit and durability
- Ultimately, we need to move from *N*-of-1 studies to platform trials

Conclusions, challenges, and opportunities

- KJ will likely continue to need some urea cycle management, but early signs suggest that his disease may be less severe
- It is possible to develop a personalized gene-editing therapy in 6 months
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- Longer follow-up and studies of additional non-invasive markers are needed to quantify potential benefit and durability
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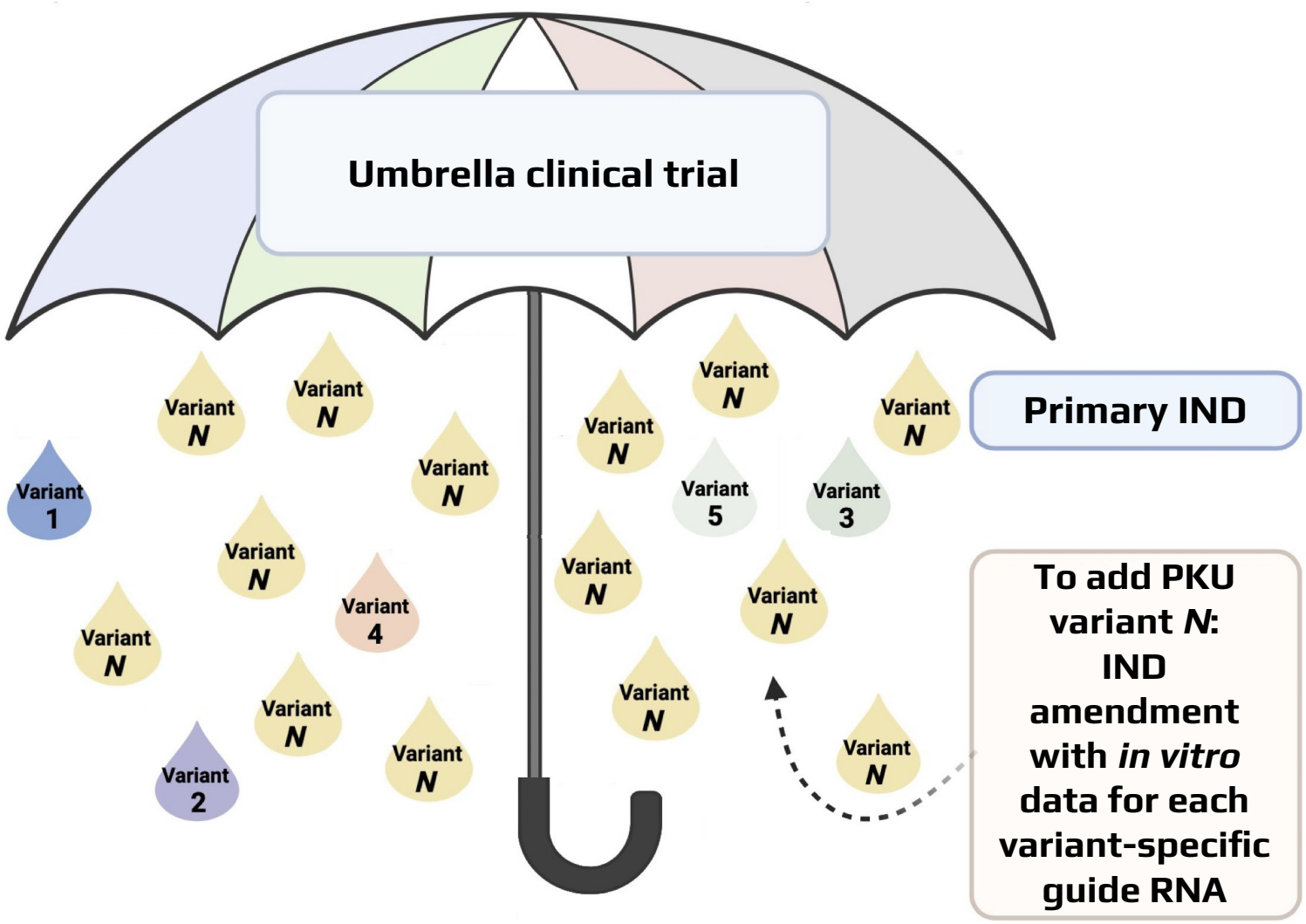
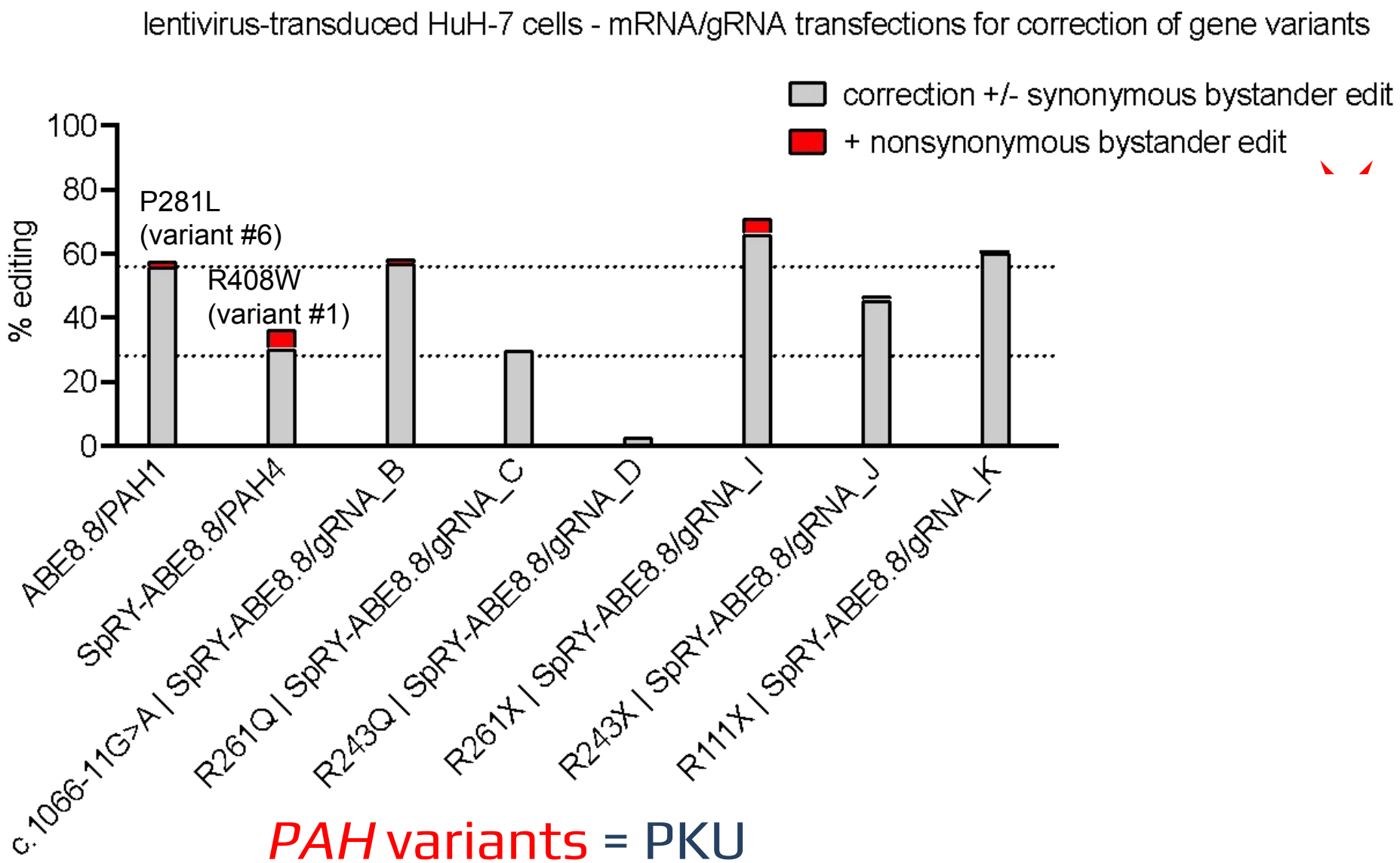
Umbrella clinical trial for PKU



Rebecca Ahrens-Nicklas
Children's Hospital of Philadelphia (CHOP)

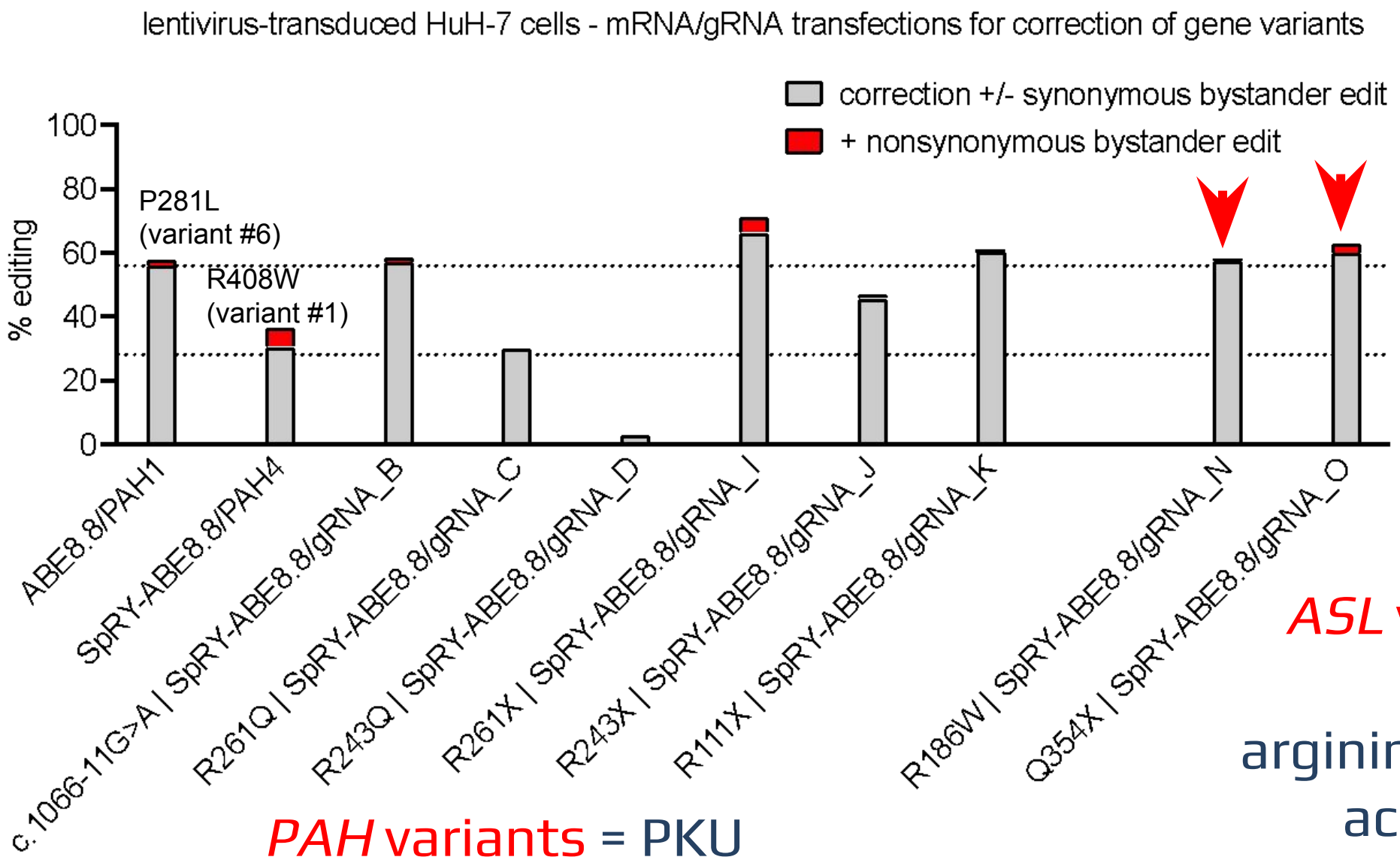
NIH CGE
Somatic Cell Genome Editing

Screening with SpRY-ABE8.8 mRNA + guide RNAs in HuH-7 cells



PAH variants = PKU

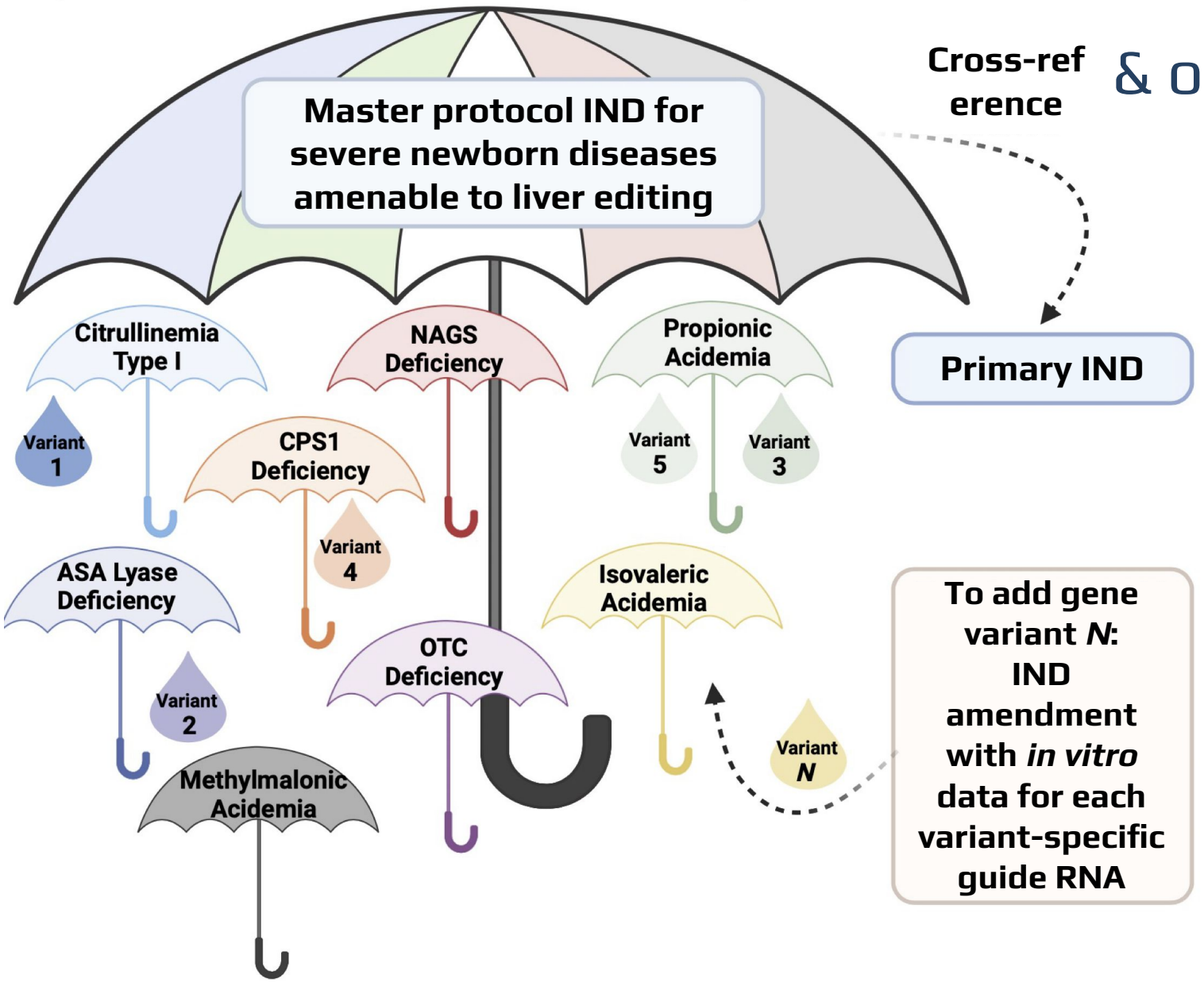
Screening with SpRY-ABE8.8 mRNA + guide RNAs in HuH-7 cells



PAH variants = PKU

ASL variants
=
argininosuccinic aciduria

Master protocol for urea cycle disorders & organic acidemias



Rebecca Ahrens-Nicklas
Children's Hospital of Philadelphia (CHOP)

NIH CGE Somatic Cell Genome Editing

KJ and his parents

University of Pennsylvania
Xiao Wang
Sarah Grandinette
Dominique Brooks
Madelynn Whittaker
Aidan Quigley
Anne Marie Berry
Julia Hacker
Lauren Testa
Elena Kahn
Ananya Talikoti
Emily Feierman
Delaney Rutherford
Ping Qu
Nataliya Petrenko

Innovative Genomics Institute
Fyodor Urnov
Petros Giannikopoulos
Taylor Hudson
Kevin Briseno
Aysel Ogul

Children's Hospital of Philadelphia
Rebecca Ahrens-Nicklas
Nancy Robinson-Garvin
Juliana Small
Sarah McCague
Samantha Burke
Christina Wright
Sarah Bick
Lindsey George
Kim Ng
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Alvin Hsu

Mass General Brigham
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Acuitas Therapeutics
Ying Tam
Christopher Barbosa
Sean Semple
Kamila Wlodarczyk
Nicholas Tougas
Robert Leone
Jon Le Huray

Aldevron
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Michael Jepperson
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A.J. Muehlberg
Megan Wohl
Jenna Sjoerdsma
Bezhin Mesho
Jacob Scherb
Helen Velishek
Jon Cooper

Danaher Corporation
Sadik Kassim

Integrated DNA Technologies
Christopher Vakulskas
Michael Collingwood
Katie Keogh
Ashley Jacobi
Morgan Sturgeon
Christian Brommel
Ellen Schmaljohn
Gavin Kurgan
Thomas Osborne
He Zhang
Kyle Kinney
Garrett Rettig

The Jackson Laboratory
Cathleen Lutz

NIH Somatic Cell Genome Editing program
P.J. Brooks
Tim LaVaute
Chris Boshoff