Liver-targeted Genetic Disease Franchise Update and FY 2025 Earnings

FEBRUARY 24, 2026

NASDAQ: BEAM

Beam THERAPEUTICS

Beam event participants

TOPIC	PARTICIPANT
Introduction	**Holly Manning** Vice President, Investor Relations & External Communications
Beam Overview & Introduction to New Liver-targeted Genetic Disease Program	**John Evans** Chief Executive Officer
PKU Disease Overview and Standard of Care	**Amy Simon, M.D.** Chief Medical Officer
Beam PKU Program Review	**Gopi Shanker, Ph.D.** Chief Scientific Officer
Emerging Approaches to Developing Customized Genetic Medicines	**Kiran Musunuru, M.D., Ph.D.** University of Pennsylvania
Financial Updates	**Sravan Emany** Chief Financial Officer
Closing Remarks and Q&A	**Mr. Evans**

Cautionary note regarding forward-looking statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements include statements regarding: the therapeutic applications and potential of our technology, including with respect to SCD, AATD, GSDIa, PKU and beta-thalassemia; our plans, and anticipated timing, to advance our programs, including the clinical trial designs and expectations for risto-cel, BEAM-103, BEAM-301, BEAM-304 and BEAM-302; our plans and anticipated timing to present data from ongoing clinical trials; our anticipated regulatory interactions and filings; our current expectations and anticipated results of operations, including our expected use of capital; the sufficiency of our capital resources to fund operating expenses and capital expenditure requirements and the period in which such resources are expected to be available; and the therapeutic applications and potential of our technology, including our potential to develop lifelong, curative, precision genetic medicines for patients through base editing, including potential safety advantages, all of which are subject to known and unknown important risks, uncertainties and other factors that may cause our actual results, performance or achievements, market trends, or industry results to differ materially from those expressed or implied by such forward-looking statements. Therefore, any statements contained herein that are not statements of historical fact may be forward-looking statements and should be evaluated as such. Without limiting the foregoing, the words "anticipate," "expect," "suggest," "plan," "vision," “strategy,” “possibility,” “promise,” "believe," "intend," "project," "forecast," "estimates," "targets," "projections," "potential," "should," "could," "would," "may," "might," "will," and the negative thereof and similar words and expressions are intended to identify forward-looking statements.

Each forward-looking statement is subject to important risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement, including, without limitation, risks and uncertainties related to: our ability to develop, obtain regulatory approval for, and commercialize our product candidates, which may take longer or cost more than planned; our ability to raise additional funding, which may not be available; our ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; that preclinical testing of our product candidates and preliminary or interim data from preclinical studies and clinical trials may not be predictive of the results or success of ongoing or later clinical trials; that initiation and enrollment of our clinical trials may take longer than expected; that our product candidates or the delivery modalities we rely on to administer them may cause serious adverse events; the uncertainty that our product candidates will receive regulatory approval necessary to initiate or continue human clinical trials, that our product candidates may experience manufacturing or supply interruptions or failures; risks related to competitive products; and the other risks and uncertainties identified under the headings "Risk Factors Summary" and "Risk Factors" and elsewhere in our annual report on Form 10-K for the year ended December 31, 2025, our quarterly reports on Form 10-Q, and in any subsequent filings with the Securities and Exchange Commission (the "SEC") which are available on the SEC's website at www.sec.gov. Additional information will be made available by our annual and quarterly reports and other filings that we make from time to time with the SEC. These forward-looking statements speak only as of the date of this presentation. Factors or events that could cause our actual results to differ may emerge from time to time, and it is not possible for us to predict all of them. We undertake no obligation to update any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by applicable law.

Our vision is to provide lifelong cures
for patients suffering from serious diseases

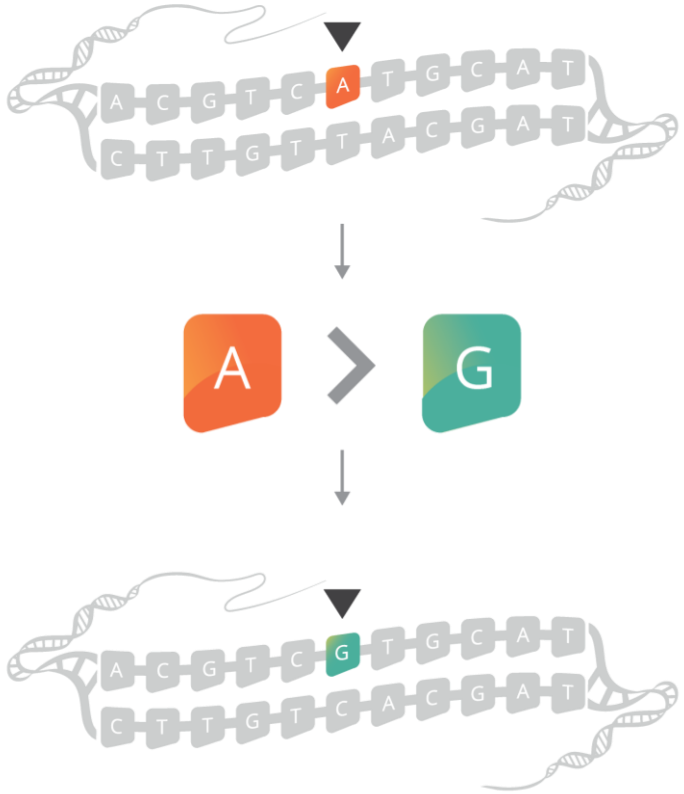
GENE EDITING FOR
rare and common diseases

POTENTIAL FOR
one-time, curative therapies

PLATFORM FOR
rapidly programmable precision medicines

Beam was founded on a simple concept with profound implications

BASE EDITING
TECHNOLOGY



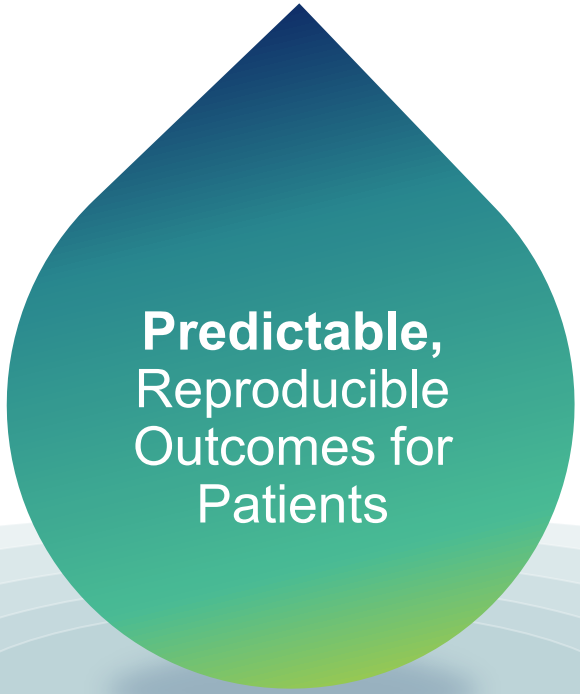
CONSISTENT gene sequence outcomes

DURABLE correction for one-time cures

LESS GENOTOXICITY than traditional gene editing

Predictable, Reproducible Outcomes for Patients

Predictability as a driver of progress:
The potential to ripple through the broader healthcare ecosystem



Streamlined R&D Cycles
- Reduced development risk

Regulatory Acceleration
- Efficient regulatory pathways

Physician Confidence
- Predictable safety and durability
- Clarity in treatment decisions

Patient Experience Improvement
- Reliable therapeutic outcomes
- Enhanced quality of daily living

Payer & System Impact
- Sustainable, outcomes-aligned payer models
- Reduced lifetime healthcare utilization

The power of predictability:
Beam is building a reliable model for advancing genetic medicine

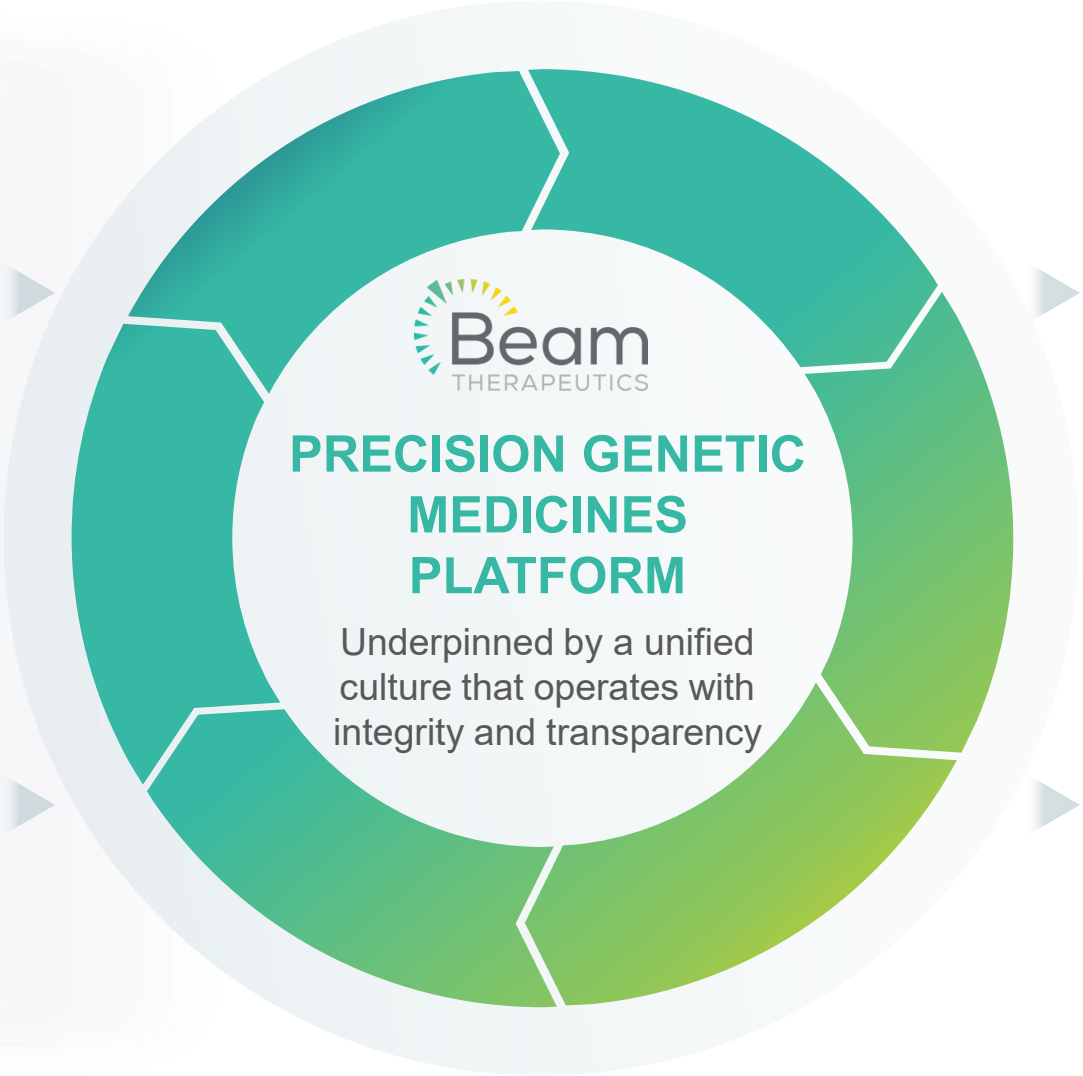
Easily **ADAPTABLE** to new targets

REUSABLE delivery technology

CONSISTENT preclinical and clinical outcomes

HIGHLY SCALABLE internal manufacturing

FLEXIBLE emerging regulatory framework



Multiple growing, high-value franchises

Active pipeline **expansion**

Creative platform **partnerships**

Rapidly advancing and growing a portfolio of liver-targeted *in vivo* programs for genetic diseases

Potential **best-in-class** and **first-in-class** AATD program

Strategic **pipeline expansion into PKU**

Industry-leading LNP capabilities

Platform synergies well positioned for **novel regulatory pathways**

PROGRAM	DISEASE	DELIVERY	EDITING APPROACH	RESEARCH	LEAD OPTIMIZATION	IND ENABLING	PHASE I/II	PIVOTAL
BEAM-302	Alpha-1 antitrypsin deficiency (AATD)	*In vivo* LNP	Correction of E342K mutation					
BEAM-301	Glycogen storage disease type Ia (GSDIa)	*In vivo* LNP	Correction of R83C mutation					
BEAM-304	Phenylketonuria (PKU)	*In vivo* LNP	Correction of multiple mutations					

LNP = lipid nanoparticle

PKU represents strategic expansion of the portfolio and ideal application of Beam's genetic medicines platform

Relevant Technology and Expertise

- PKU often caused by point mutations in phenylalanine hydroxylase (PAH)
- Beam has potential to address mutations found in majority of PKU patients
- PAH primarily expressed in hepatocytes, targetable via LNP delivery
- Utilizes Beam's emerging expertise in metabolic disease

Large Addressable Market

- Approximately 20,000 people living with PKU in the U.S.
- Potential to address nearly half of PKU patients by targeting two most common mutations initially
- PKU unmet need exists due to severe diet limitations, burden of available treatments and uncontrolled disease

Existing and Novel Regulatory Pathways

- Bar for full phenylalanine (Phe) normalization expected to be low (~10-20% correction of one allele)
- Blood Phe reduction established as previously approvable study endpoint
- Potential to utilize novel regulatory pathways to address multiple mutations

Source: Stone WL, Los E. Phenylketonuria (PKU). StatPearls Publishing; 2025 Jan

PKU: Disease Overview & Current Standard of Care

Amy Simon, M.D., Chief Medical Officer

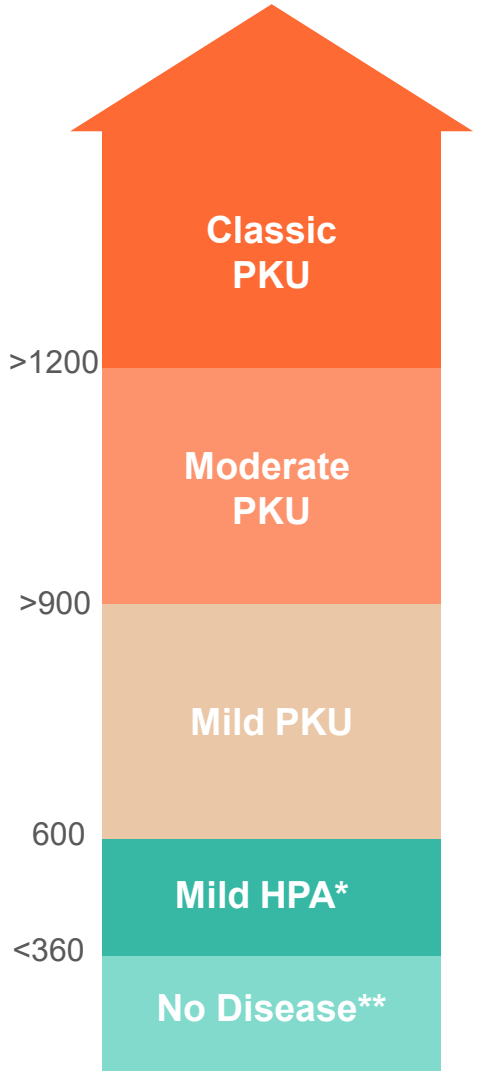
PKU is a genetic metabolic disease in which failure to metabolize phenylalanine leads to neurotoxicity

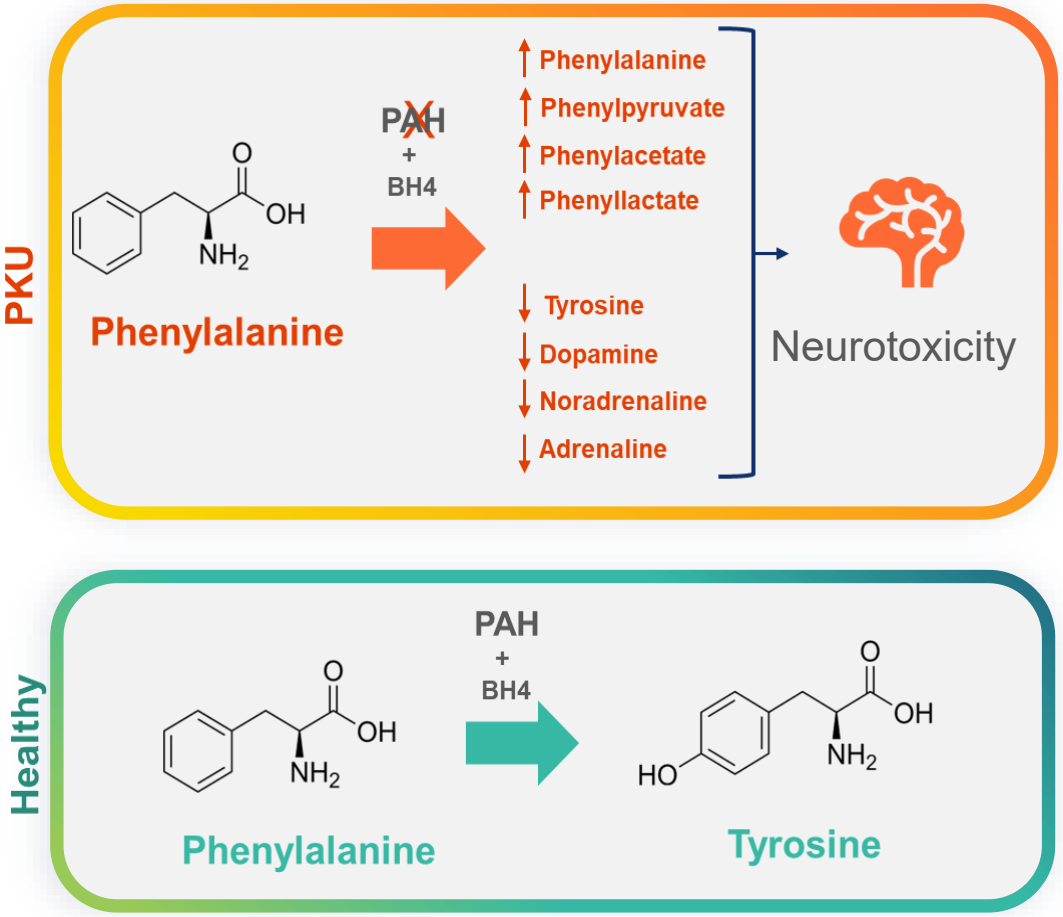
PKU is caused by mutations in *PAH* gene, resulting in loss of PAH activity, failure to metabolize Phe and elevated Phe levels

U.S. PKU patients identified with widespread newborn screening

- Majority, but not all, patients are genotyped

Majority of patients have uncontrolled disease, above the recommended guidelines of blood Phe <360μM



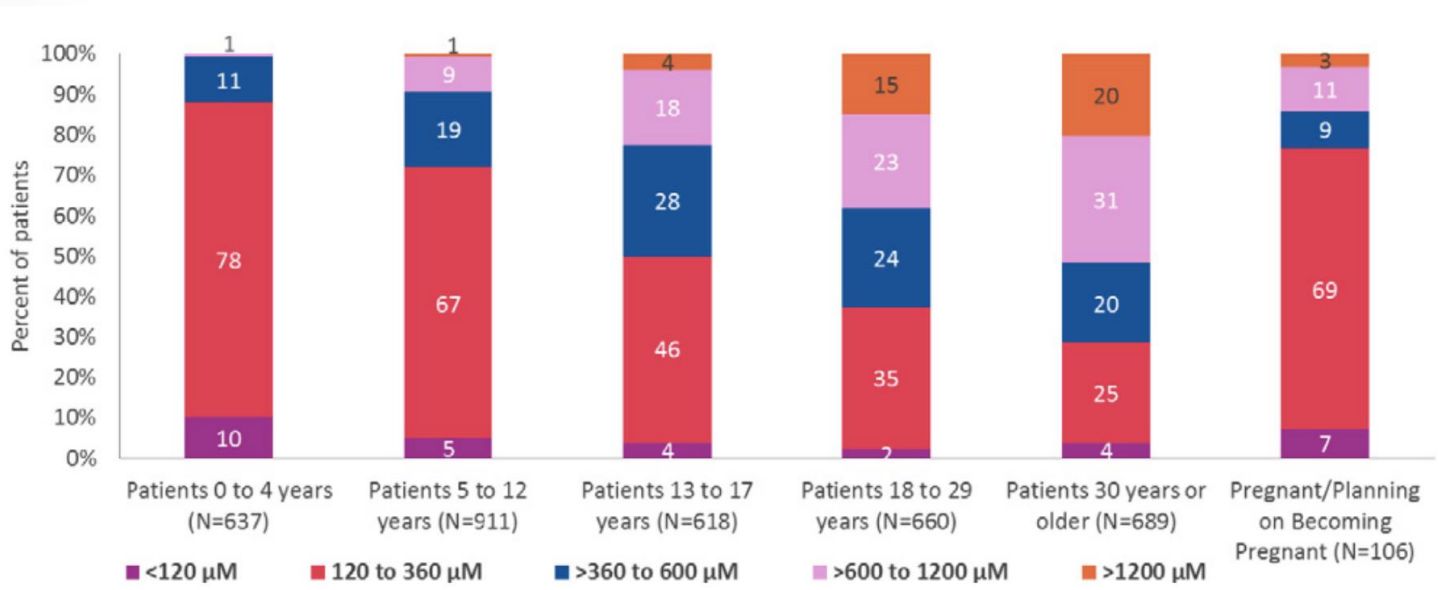


*HPA: hyperphenylalaninemia
**Normal Phe <120; treatment target <360

Significant impact on health and quality of life for people with PKU, and most patients are not within recommended Phe levels

- In children, very elevated Phe levels can lead to impaired brain development, intellectual disability and seizures
- In adults, with decreased adherence, increased Phe results in cognitive impairment (e.g., confusion, headaches, impaired decision making), anxiety, depression and ADHD
- Pregnant women with PKU need strict Phe control (≤ 240µM) to prevent microcephaly in their newborns (even if newborn does not have PKU mutation)

Blood Phe Concentrations Increase with Age



Source: E.R. Jurecki et al. / Molecular Genetics and Metabolism 120 (2017) 190–197

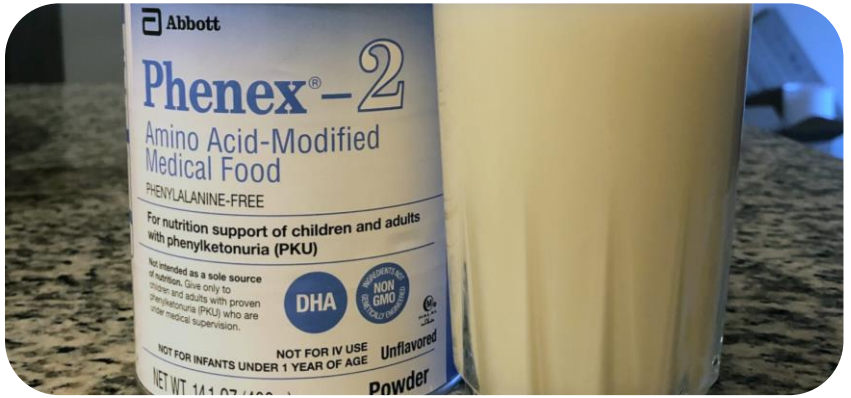
PKU unmet need exists due to severe diet limitations and burden of available treatments

- **Available therapies impose significant burden on patients** given the lifetime need for diet restrictions, poor taste of medical foods and cost—all leading to diminished quality of life and compliance over time
- **Lifelong, Phe-restricted diet:** Phe exists in most foods (meat, dairy, grains, vegetables and fruits), severely restricting diet and requiring medical food
- **BH4 for mild patients:** a cofactor used to stimulate PAH enzyme in those with existing enzyme (generally milder disease)
- **Enzyme replacement therapy for moderate-to-severe patients:** requires daily SC injections; Phe <360µM in 60% patients after 2 years of treatment; requires labs to adjust diet; significant discontinuations due to immune reactions and hypersensitivity

Most classical PKU patients can handle only 5-10 g of protein/day from diet

1 slice cheese	1 drumstick	1 slice bread	1 egg
6.5 g	18 g	3 - 4 g	6.5 g

Most protein intake for PKU patients comes from medical formula



Sources: U.S. Department of Agriculture. Agricultural Research Service/Food Data Central/Food Search; PBS Wisconsin; Thomas J., et al, Mol Genet Metab. 2018;124(1):27. Epub 2018 Mar 31.

Target product profile for PKU gene therapy driven by established regulatory precedents, the literature and clinician feedback

- Blood Phe has been an accepted surrogate endpoint for patients with PKU for full approval in U.S. and EU
- Diet normalization can be demonstrated in clinical setting
- Phe reduction associated with positive trends in patient outcomes, including improvements in inattention and confusion, but has not been required for approval

Target profile for successful clinical uptake of PKU gene therapy:

- **Significant and sustained reduction in Phe levels** (<360µM)
- **Well tolerated**
- **Diet normalization** (no medical food or Phe restrictions)
- **One-time therapy for lifelong Phe control**

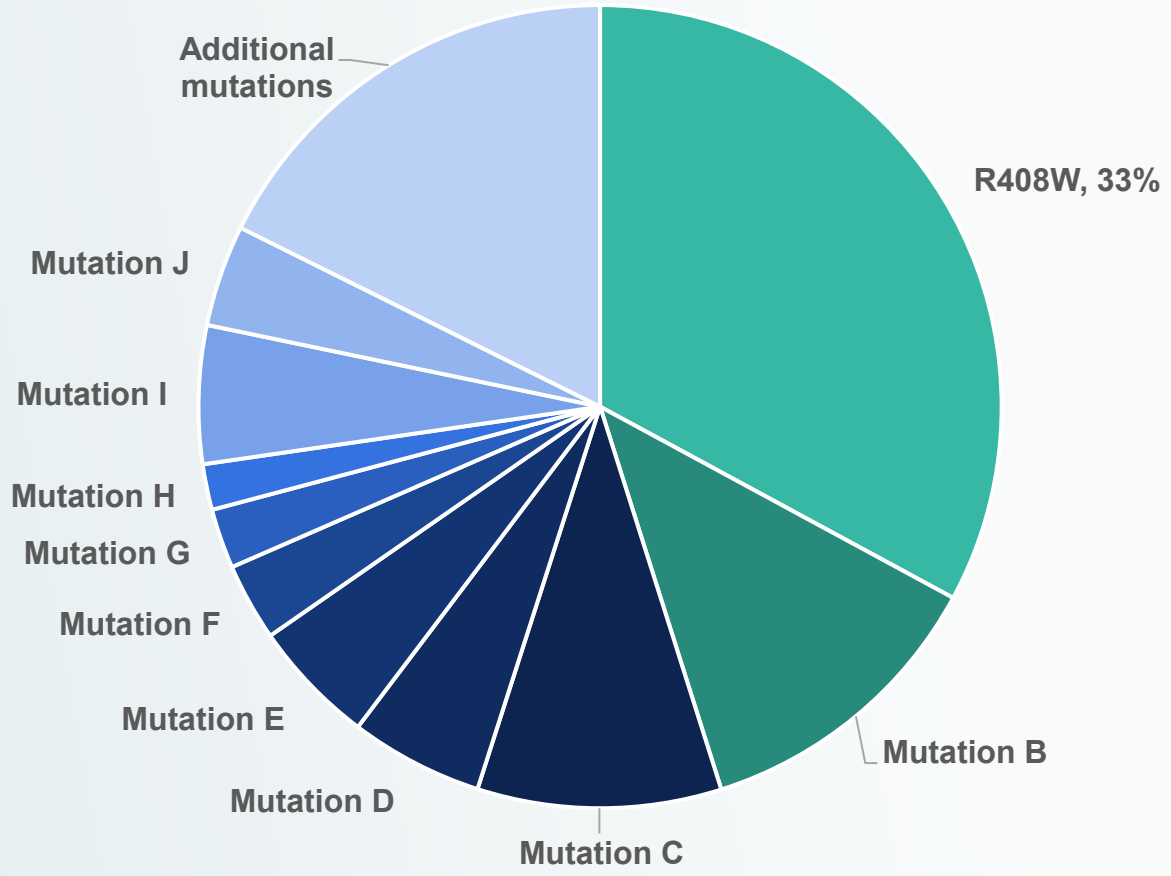
Sources: Kuvan prescribing information; Palynziq prescribing information; EMA CHMP assessment report for Palynziq 2019; ACMG Board of Directors et al. Genetics in Medicine, Volume 27, Issue 1, 101289

Base Editing as a Potential One-time Treatment Option for PKU

Gopi Shanker, Ph.D., Chief Scientific Officer

Beam has potential to address vast majority of patients with PKU, leveraging an efficient development approach

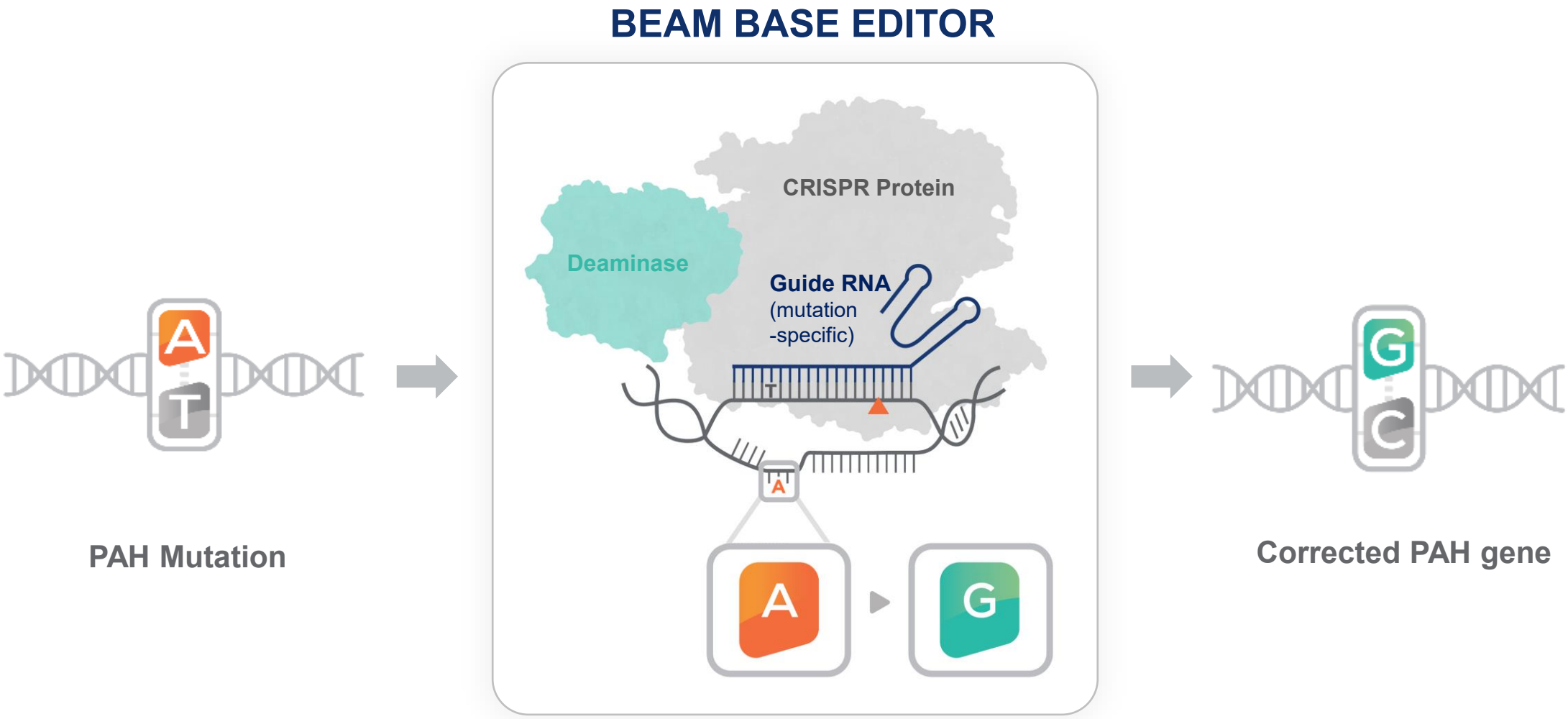
~20,000 PKU Individuals in U.S.



- Two identified development candidates have potential to address nearly half of PKU patients
- Ongoing research efforts focused on correcting additional mutations
- Opportunity to leverage novel regulatory and clinical approach to efficiently address vast majority of PKU

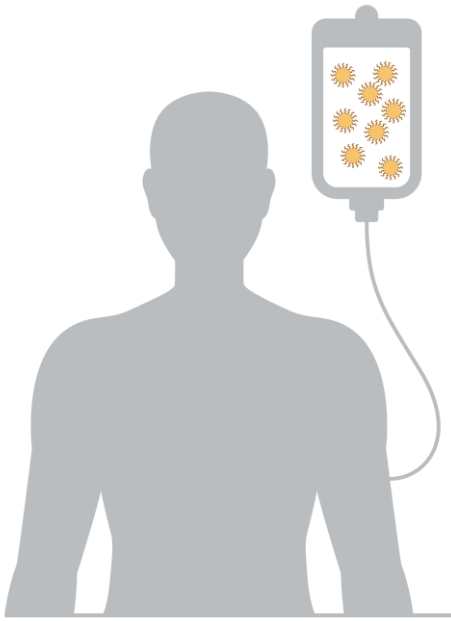
Source: Beam internal data; Hillert, Alicia et al. The American Journal of Human Genetics, Volume 107, Issue 2, 234 – 250; Stone WL, Los E. Phenylketonuria (PKU). StatPearls Publishing; 2025 Jan

BEAM-304 program uses base editing to directly correct multiple mutations causing PKU

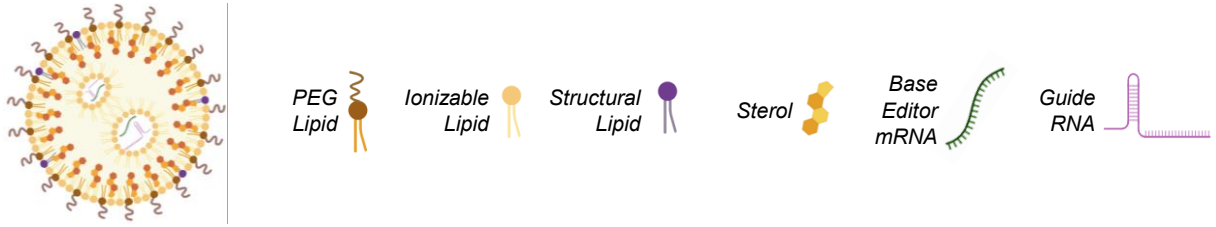


Beam has established leading LNP capabilities for *in vivo* treatment of liver-targeted genetic diseases

Advantages of LNP delivery



- ✓ Outpatient dosing via simple IV infusion
- ✓ Titratable and redosable
- ✓ Synthetic and highly scalable manufacturing process
- ✓ Once optimized, potential for predictable and reproducible platform
- ✓ Low cost of goods

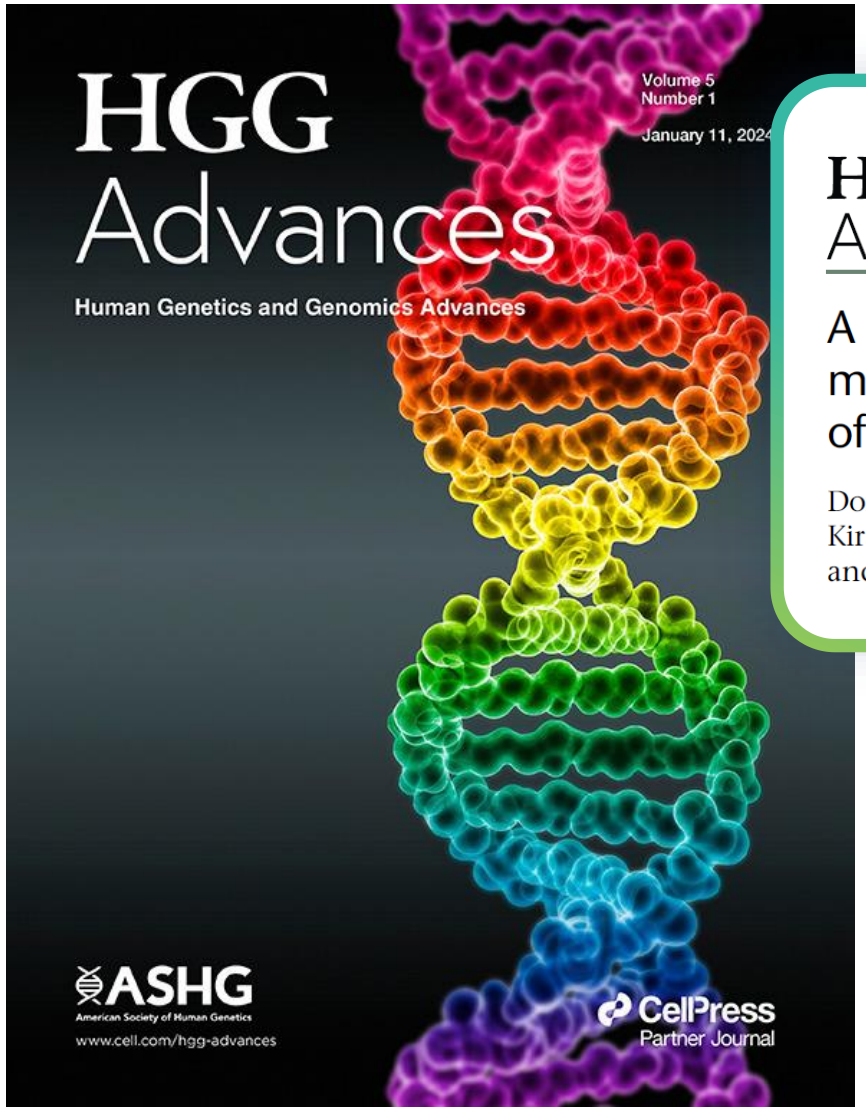


Beam's LNP Leadership Position

- Significant investment and expertise in LNP optimization and process development
- Internal GMP capabilities in NC facility to manufacture at scale
- Re-usable platform for future liver programs – changing cargo not expected to change LNP tolerability or efficacy
- Actively exploring potential to retarget LNPs to other tissues (e.g., HSCs)

GMP = good manufacturing practice; HSCs = hemopoietic stem cells

Collaboration with the Musunuru lab at UPenn to bring base editing to patients with PKU



HGG Advances **REPORT**

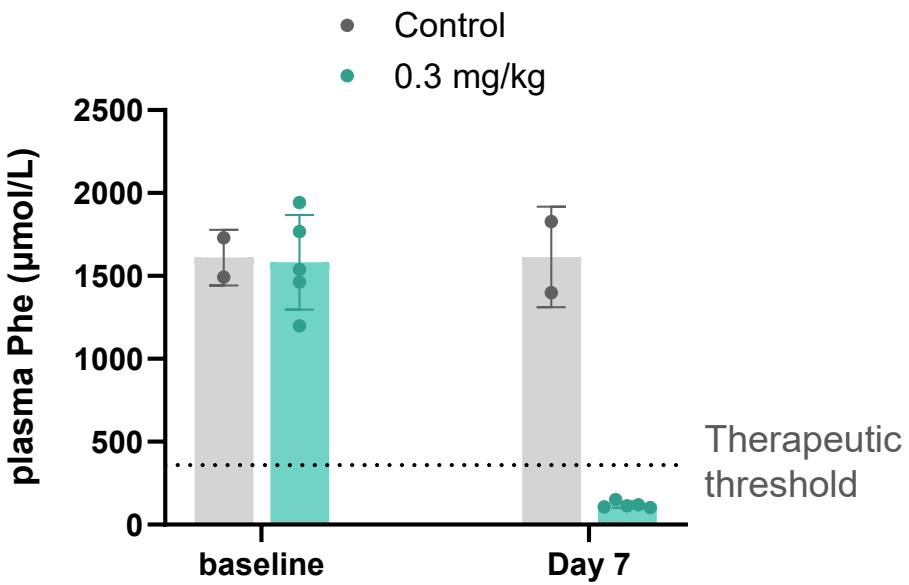
A base editing strategy using mRNA-LNPs for *in vivo* correction of the most frequent phenylketonuria variant

Dominique L. Brooks,[1,2,3] Madelynn N. Whittaker,[1,2,3,4] Hooda Said,[5] Garima Dwivedi,[6] Ping Qu,[1,2,3] Kiran Musunuru,[1,2,3,11,*] Rebecca C. Ahrens-Nicklas,[3,7,8,11] Mohamad-Gabriel Alameh,[5,9,10,11] and Xiao Wang[1,2,3,11]

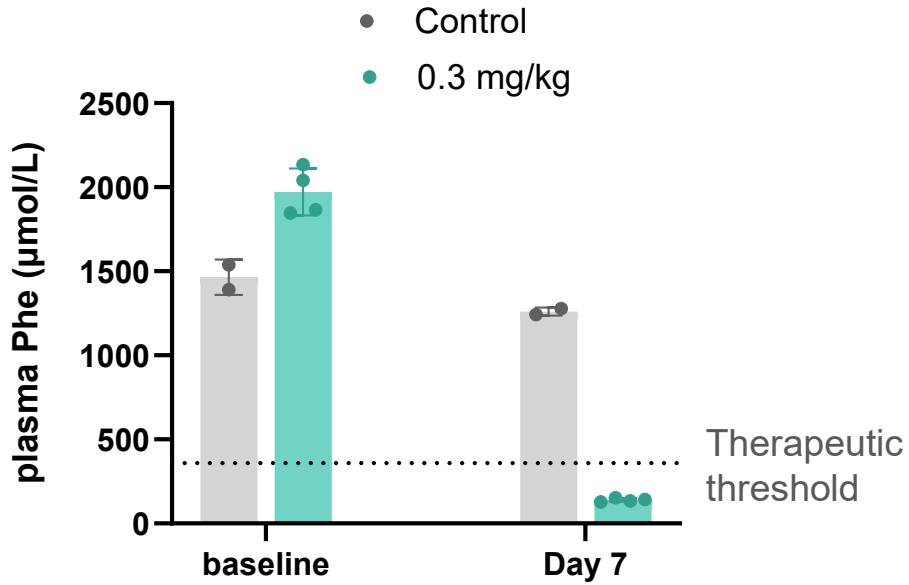
- Musunuru lab established preclinical proof-of-concept for base editing in PKU
- Beam leveraged expertise and platform capability to rapidly advance program to IND in <2 years

Preclinical data demonstrate the potential of BEAM-304 to correct underlying PKU mutations and rapidly normalize plasma Phe

R408W MUTATION



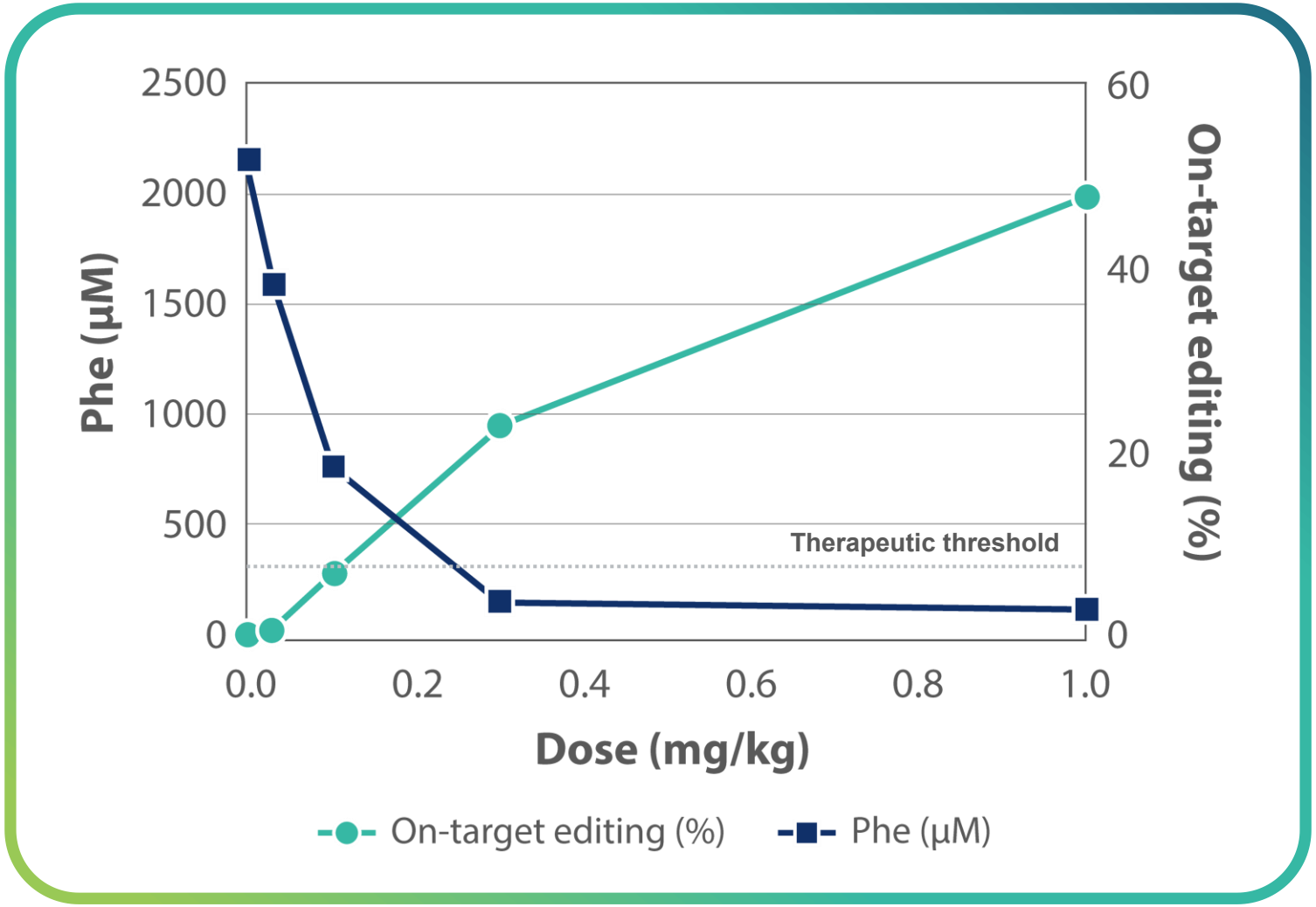
MUTATION B



For both target mutations, BEAM-304 normalized plasma Phe in mice consuming standard protein-containing feed

Source: Beam internal data

Low-level editing sufficient to normalize plasma Phe in mouse models at clinically relevant doses



Source: Beam internal data

BEAM-304 Phase 1/2 trial in PKU patients designed to achieve early clinical proof of concept

- Beam will initially focus development of BEAM-304 in U.S.
- Potential to rapidly expand to other mutations
- Productive pre-IND interaction with FDA

PHASE 1/2

Open-label, single-ascending dose trial

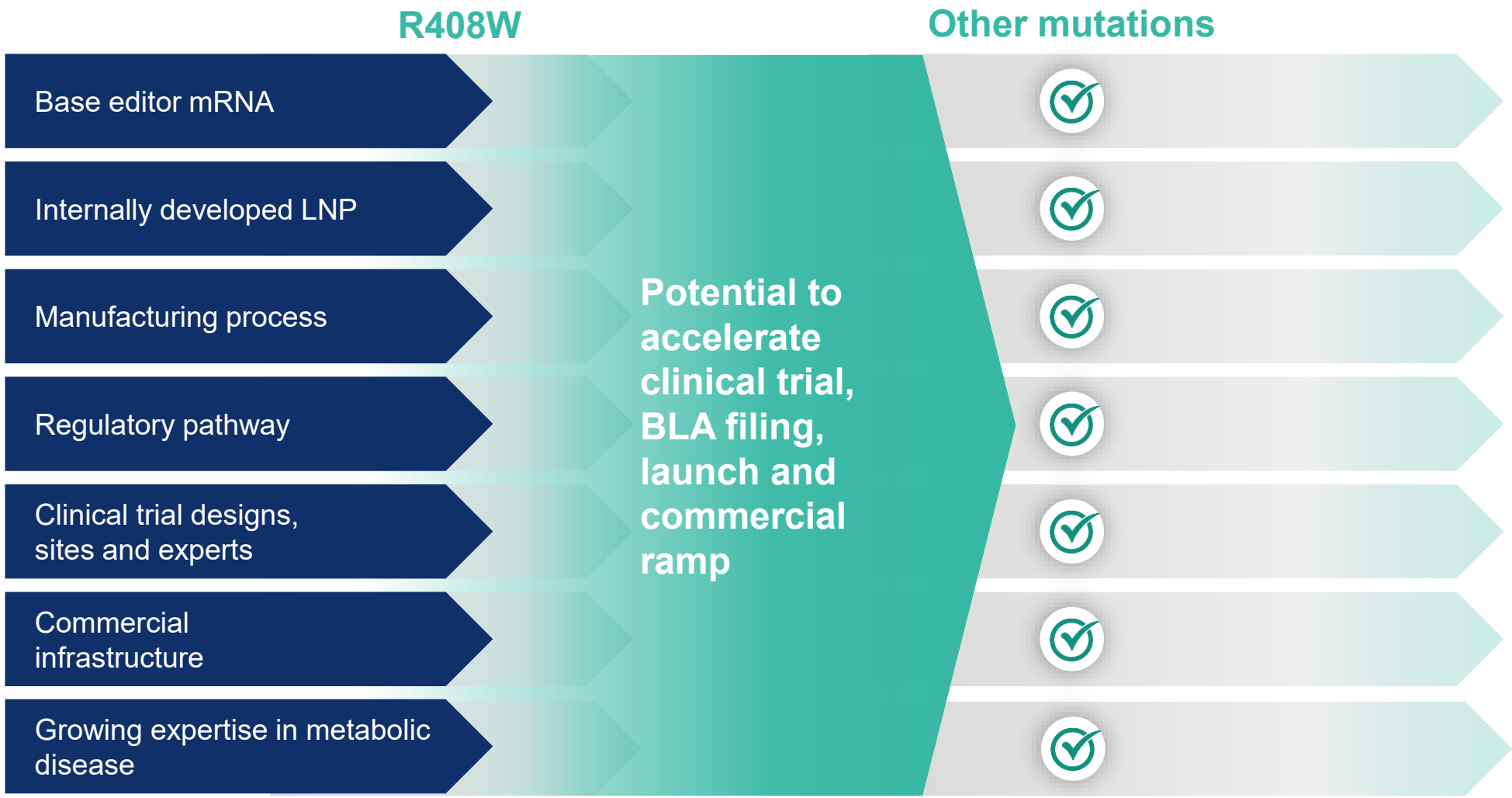
DOSE EXPLORATION ▶ **DOSE EXPANSION**

KEY ENDPOINTS

- Safety and tolerability
- Reduction of blood Phe concentration

Expect to file IND in 2026

Initial learnings from BEAM-304 in R408W expected to provide predictable path for accelerated development for future mutations

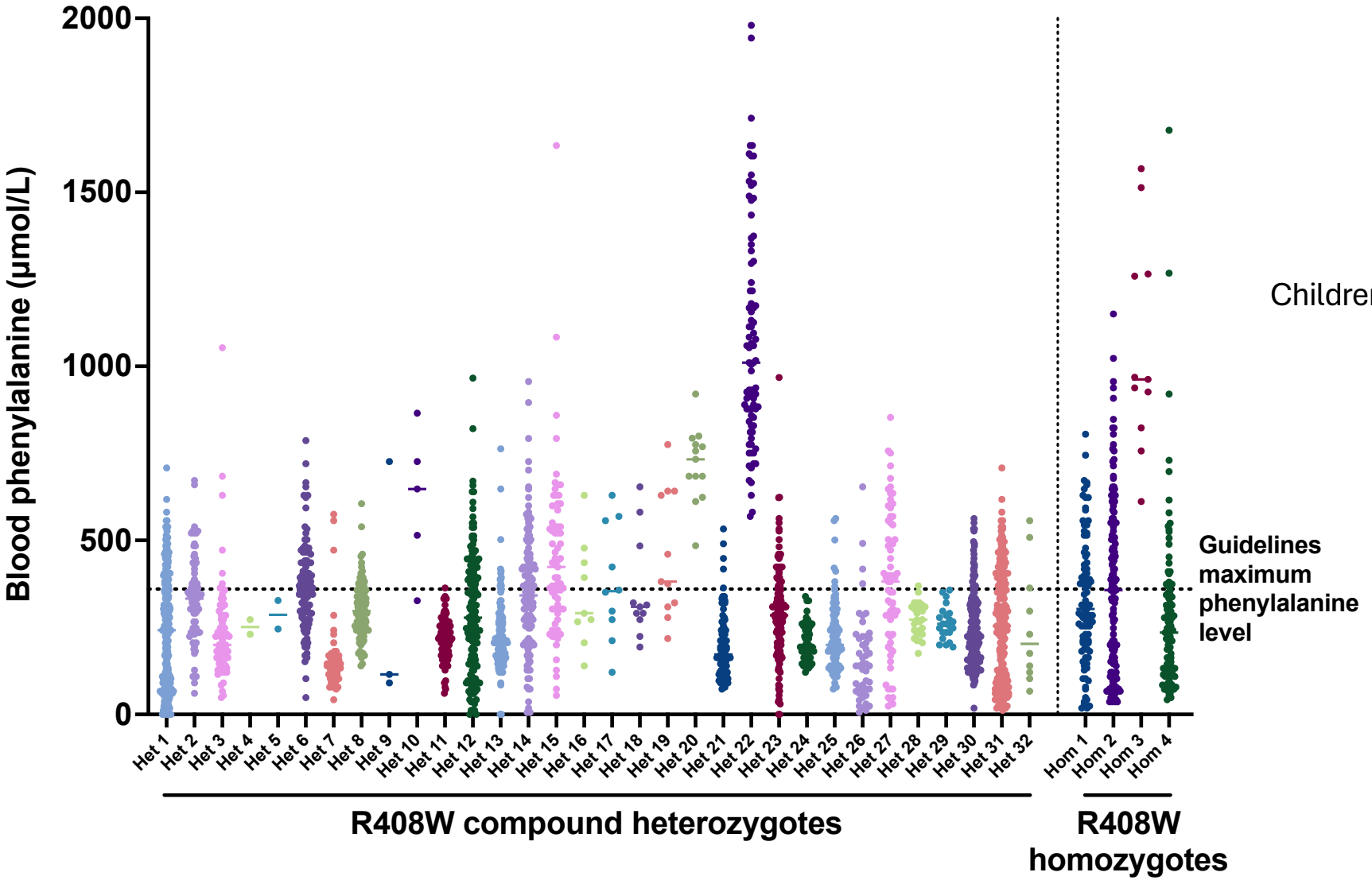


Emerging Approaches to Developing Customized Genetic Medicines

Kiran Musunuru, M.D., Ph.D., University of Pennsylvania

Beam THERAPEUTICS

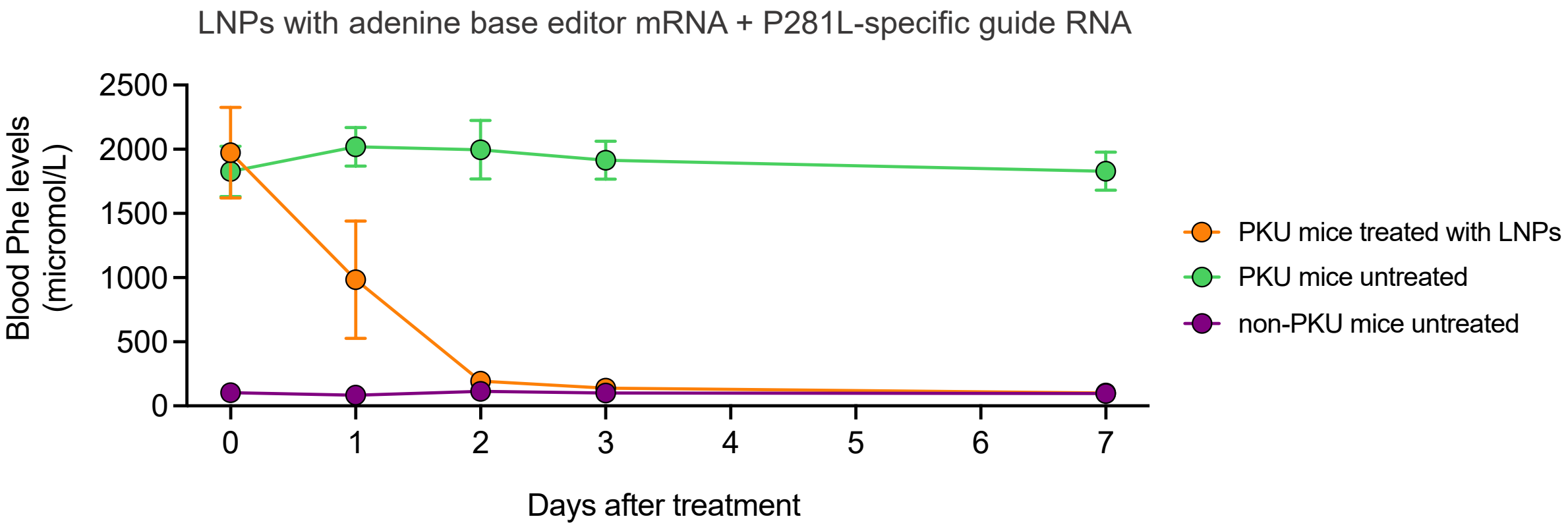
Poor metabolic control in PKU patients



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

Brooks et al. *Am J Hum Genet* 2023;110:2003-14

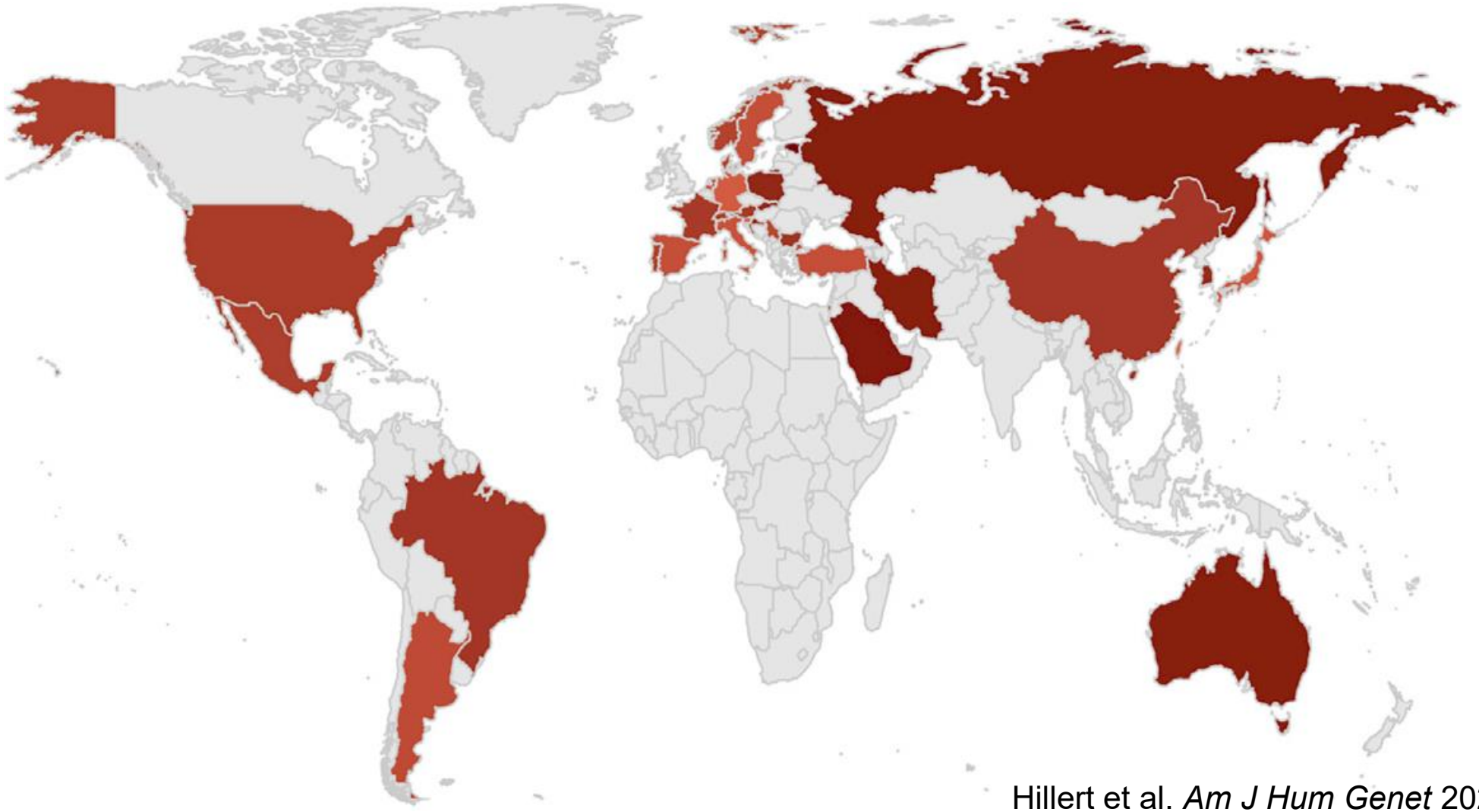
LNP base editing treatment of “humanized” PKU mice (P281L)



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

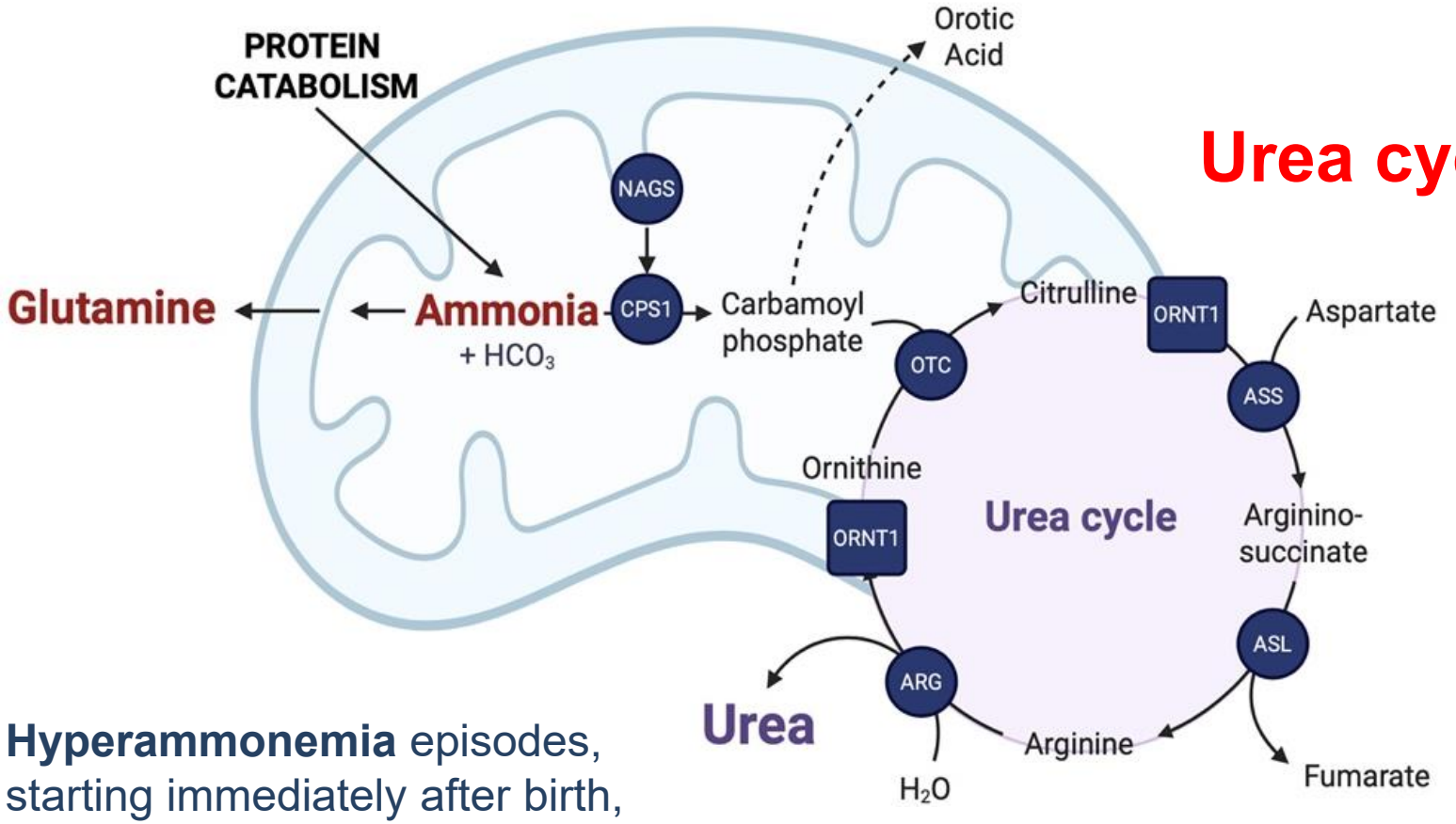
Pathogenic *PAH* variants in PKU patients

What about the 1,000+ other cataloged variants? And all the variants not yet cataloged …



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

Newborn patients with grievous inborn errors of metabolism



Urea cycle disorders

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

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

Interactions with the FDA

FORUM

How to create personalized gene editing platforms: Next steps toward interventional genetics

Rebecca C. Ahrens-Nicklas[1,2,3,*] and Kiran Musunuru[1,2,3,4,5,*]

How do we go from a single individual receiving a personalized gene-editing therapy to a future of "interventional genetics" in which such therapies are the standard of care? First and foremost: regulatory innovation.

Phenylketonuria (PKU) INTERACT:

Major question: Could a single biodistribution/toxicology study be used to support multiple variant-specific LNP-editor drug products?

Answer: Maybe, we will discuss further at the Pre-IND meeting.

PKU Pre-IND:

Major question: Could many different variant-specific versions of an LNP-editor drug product targeting a single gene be included in a single IND?

Answers:

- A single IND application is appropriate.
- A single biodistribution/toxicology study of a single LNP formulation tailored to one variant is sufficient to cover all editable variants.
- A single biodistribution/toxicology study in a single species is sufficient.

2024 → 2025

CPS1 deficiency single-patient Pre-IND and IND:

Major question: Do our proof-of-concept studies, toxicology data, off-target editing analysis, and CMC plan support a repeat dosing protocol in a single infant with CPS1 deficiency? (Musunuru … Ahrens-Nicklas, *N. Engl. J. Med.* 2025;392:2235-43.)

Answer: Yes, and comments were provided on how to adapt specific aspects of the nonclinical, pharmacology/toxicology, off-target editing, CMC, and clinical plans for a future Phase I/II trial.

Urea cycle disorder (UCD) platform Pre-IND:

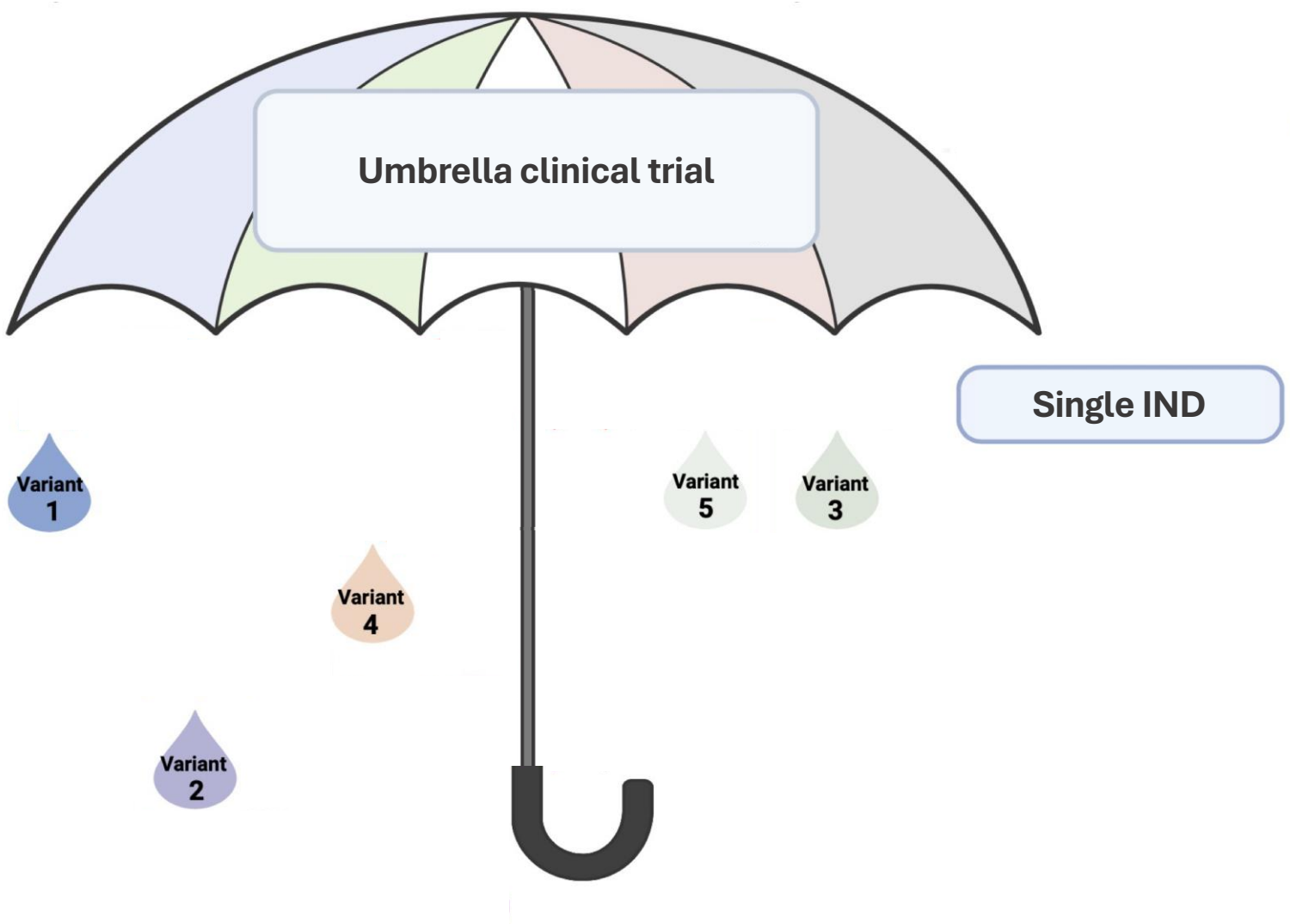
Major question: Can multiple variant-specific LNP-editor drug products targeting different variants in any of **seven UCD genes** be included in a single IND? Can they be evaluated in a single master protocol clinical trial?

Answers:

- All versions can be evaluated in a single Phase I/II master protocol trial.
- A separate IND should be filed for each gene, but all IND components should heavily cross-reference the first IND.
- The Agency strongly prefers that all future cases be treated in a formal clinical trial, **NOT** under compassionate-use, single-patient INDs.

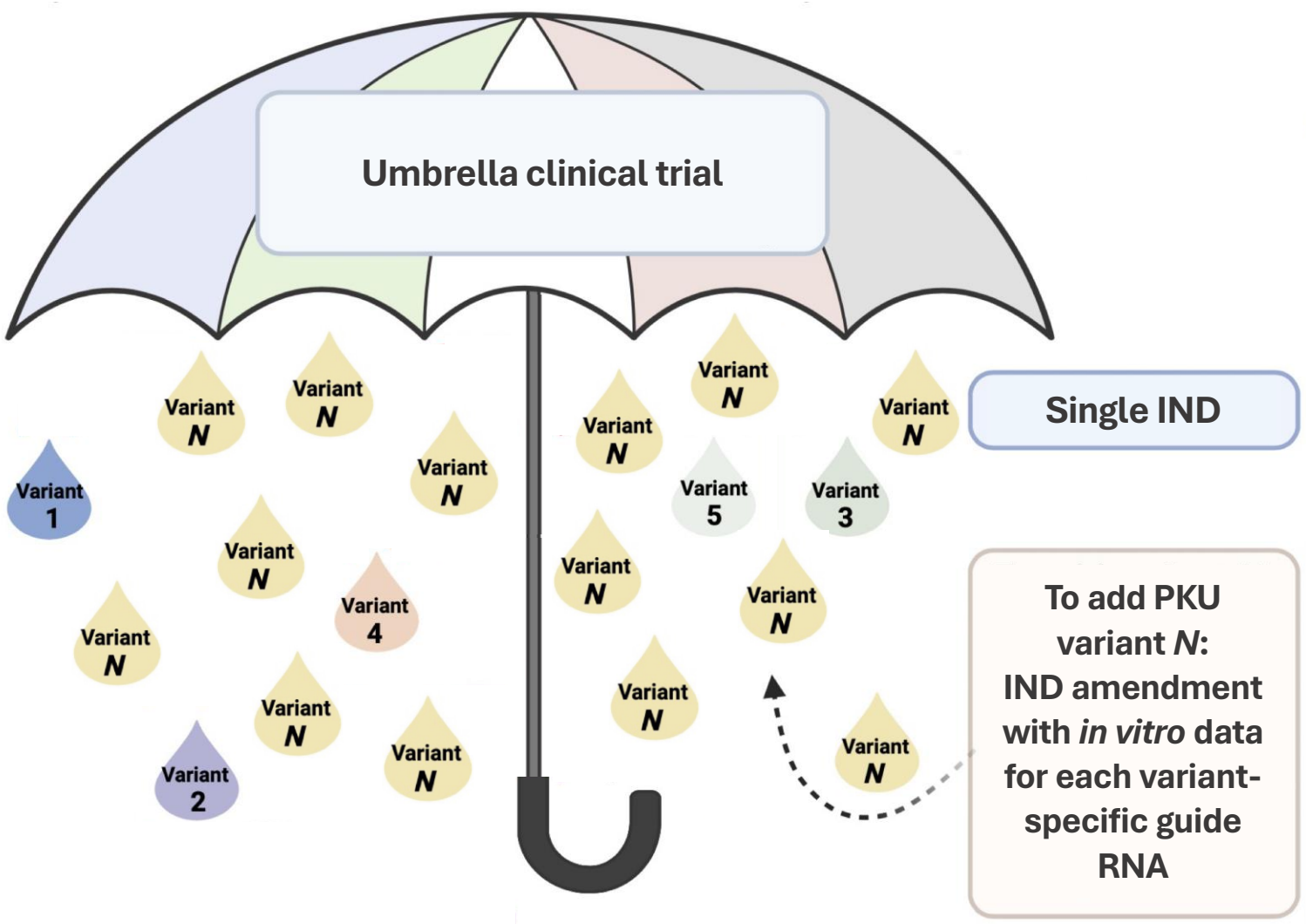
Ahrens-Nicklas & Musunuru. *Am J Hum Genet* 2025;112:2826-9

Umbrella clinical trial for PKU – pre-specified variants



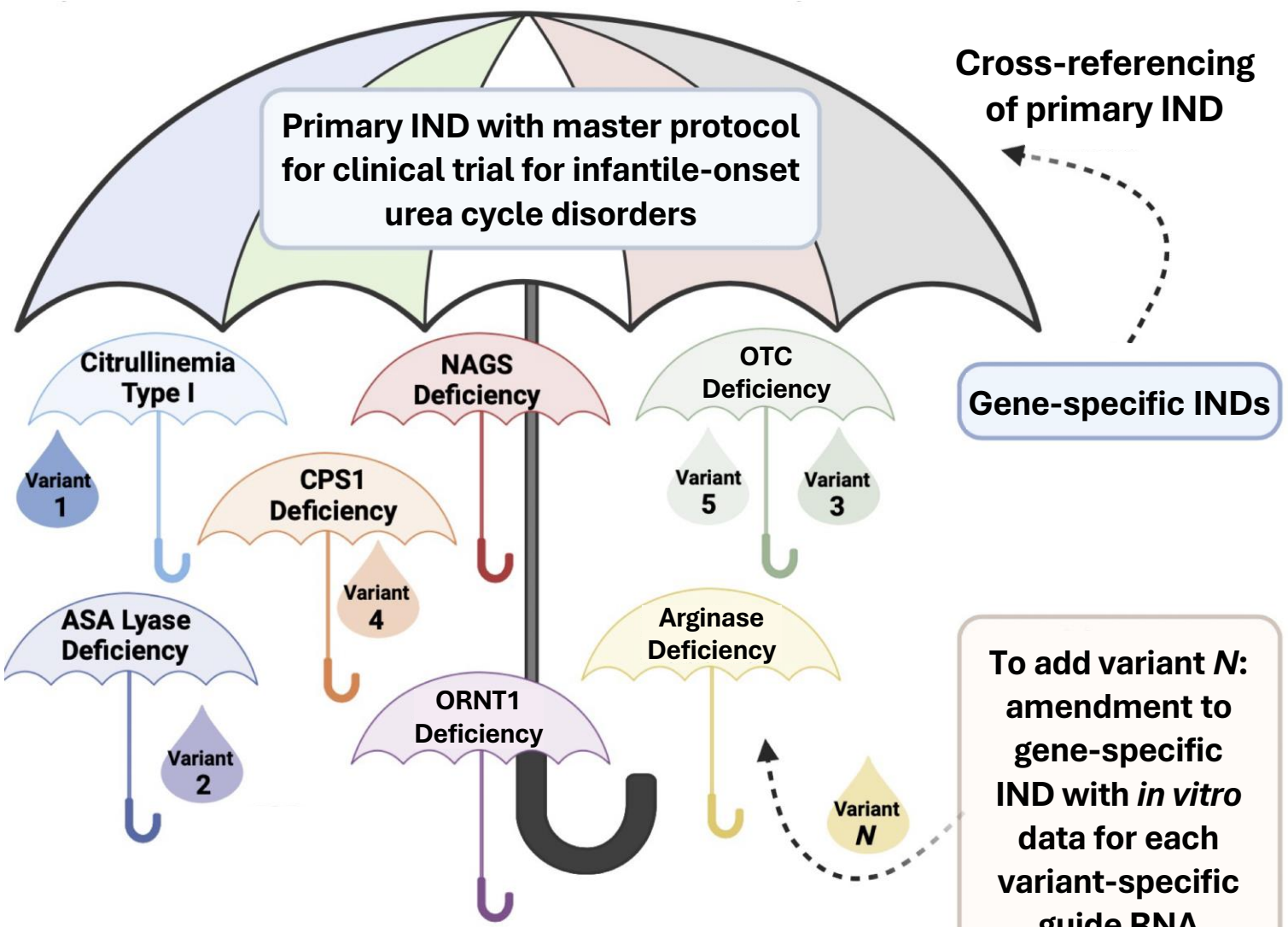
Ahrens-Nicklas & Musunuru. *Am J Hum Genet* 2025;112:2826-9

Umbrella clinical trial for PKU – rapid addition of new variants



Ahrens-Nicklas & Musunuru. *Am J Hum Genet* 2025;112:2826-9

Master protocol clinical trial for 7 urea cycle disorders



Ahrens-Nicklas & Musunuru. *Am J Hum Genet* 2025;112:2826-9

Financial Update

Sravan Emany, Chief Financial Officer

Beam THERAPEUTICS

Beam announces $500 million strategic financing facility with Sixth Street to support risto-cel launch

Balance sheet strengthened with long-term, non-dilutive capital

- Flexible repayment mechanics during the seven-year term

$100 million funded at close with the potential to draw up to an additional $400 million

Enhances Beam's ability to advance risto-cel towards a potential 2027 launch

Enables continued investment in future growth and innovation across our pipeline

Establishing a foundation of financial strength for sustainable growth

Financial Strength

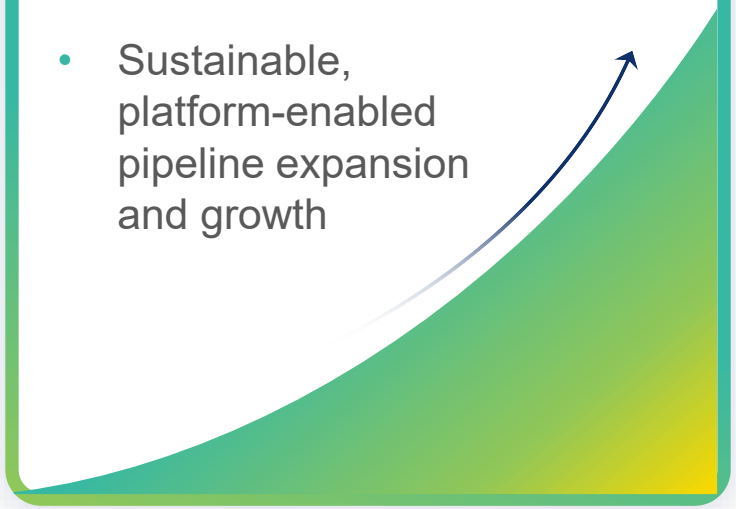
- With $1.25 billion in cash as of Dec. 31, 2025* and the anticipated $200 million minimum drawdown from Sixth Street facility, expected operating runway now into mid-2029
- Anticipate funding through risto-cel launch, execution of BEAM-302 pivotal development plan, and clinical POC for BEAM-304 in PKU

Focused Investment

- Optimized spend and expense management strategy in place
- Efficient commercial build for potential risto-cel launch
- Potential accelerated pathway for BEAM-302 development
- Efficient, novel development path for BEAM-304 in PKU

Clear Path to Value and Sustainable Growth

- Wholly owned pipeline
- Significant addressable markets
- Sustainable, platform-enabled pipeline expansion and growth



*Inclusive of cash, cash equivalents, and marketable securities.

Wrap up

Beam THERAPEUTICS

PKU illustrates the power and potential of Beam's genetic medicines platform

Base editing addresses the underlying genetic cause of disease

Rapid adaptation for additional mutations, enabled by novel regulatory pathways

Precision medicine with potential for early POC in clinic and established regulatory pathway

Large initial addressable market with significant growth opportunity

Beam is well positioned to realize the power of predictability in 2026 through key anticipated milestones

Pursue Path to Approval for Lead Programs

- Report updated Phase 1/2 data for BEAM-302 and provide next steps for pivotal development by the end of Q1 2026
- Plan to submit risto-cel BLA as early as YE 2026

Advance and Expand Pipeline

- File IND for BEAM-304 in 2026
- Report initial BEAM-301 data by YE 2026
- Complete BEAM-103 healthy volunteer study in 1H 2026
- Advance *in vivo* HSC editing program

Maintain Financial Strength

- $1.25 billion in cash as of Dec. 31, 2025*
- Expected runway into mid-2029 through anticipated risto-cel launch, execution of BEAM-302 pivotal development plan and clinical proof of concept for BEAM-304

*Inclusive of cash, cash equivalents, and marketable securities.

Q&A