



## **Beam Therapeutics Announces Clearance of Investigational New Drug Application for BEAM-304 for the Treatment of Phenylketonuria (PKU) by the United States (U.S.) Food and Drug Administration**

June 18, 2026

*BEAM-304 Development Approach Has Potential to Create Transformative, One-time Therapies for the Majority of Patients with PKU*

*BEAM-304 Program Designed as Platform-based Approach Reflecting Emerging FDA Guidance for Accelerated Development of Genome Editing Therapies*

*New Preclinical Data for BEAM-304 to be Presented at FASEB Genome Engineering: Research and Applications Conference*

CAMBRIDGE, Mass., June 18, 2026 (GLOBE NEWSWIRE) -- [Beam Therapeutics Inc.](#) (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced that the United States (U.S.) Food and Drug Administration (FDA) has cleared the investigational new drug (IND) application for BEAM-304 for the treatment of phenylketonuria (PKU). PKU is a rare, inherited metabolic disorder that results in toxic accumulation of phenylalanine (Phe), leading to serious neurologic and neurocognitive impairments and requires strict, lifelong dietary management. Beam is advancing BEAM-304 using an innovative development approach in which multiple mutation-specific base editors are developed efficiently within a single clinical program using *in vivo* delivery, in accordance with the FDA's recent efforts to accelerate development of genome editing therapies.<sup>1</sup>

"PKU affects approximately 20,000 people in the U.S., with significant unmet need for therapies that address the underlying cause of disease," said Giuseppe Ciaramella, Ph.D., president of Beam. "FDA clearance of our IND for BEAM-304 supports our novel approach of developing multiple mutation-specific base editors through a single clinical platform program, leveraging emerging FDA guidance intended to accelerate development of base editing therapeutics. We look forward to initiating our Phase 1/2 trial, intending to establish clinical proof of concept for base editing in PKU."

"Many PKU-causing mutations are single-base changes, making the disease particularly well suited for correction through base editing," said Gopi Shanker, Ph.D., chief scientific officer of Beam. "By leveraging the same underlying base editing technology, LNP delivery system, and manufacturing approach across multiple mutation-specific editors, we believe we can establish a scalable development pathway that expands access to potentially transformative therapies for people living with PKU and may serve as a model for addressing other genetically diverse liver diseases in the future."

BEAM-304 leverages Beam's proprietary and clinically validated base editing technology and lipid nanoparticle (LNP) delivery capabilities to directly and durably correct mutations in the phenylalanine hydroxylase (*PAH*) gene that cause PKU. By correcting mutations in the *PAH* gene, BEAM-304 aims to restore *PAH* enzyme activity in order to reduce toxic Phe to the recommended guideline levels ( $\leq 360 \mu\text{mol/L}$ ) while enabling diet normalization and freedom from medical food.

Preclinical data demonstrate that BEAM-304 normalized plasma Phe levels in PKU mouse models at clinically relevant doses with robust on-target editing in the liver. Updated preclinical data for BEAM-304 will be presented at the Federation of American Societies for Experimental Biology (FASEB) Genome Engineering: Research and Applications Conference, taking place July 6-9, 2026, in Porto, Portugal.

The planned Phase 1/2 trial will initially evaluate safety, tolerability, reduction of blood Phe levels and diet liberalization in PKU patients with the R408W mutation, followed by a base editor designed to address a second mutation, with the goal of establishing clinical proof of concept for base editing in PKU.

### **About BEAM-304**

BEAM-304 is a liver-targeting lipid-nanoparticle (LNP) formulation of base editing reagents designed to correct mutations in the phenylalanine hydroxylase (*PAH*) gene that cause phenylketonuria (PKU). By correcting mutations in the *PAH* gene, BEAM-304 aims to reduce toxic Phe to within recommended guidelines while enabling normalization of diet and freedom from medical food. BEAM-304 is delivered via an intravenous infusion. BEAM-304 will be evaluated in a Phase 1/2, open-label, dose exploration and dose expansion clinical trial to investigate its safety, tolerability, pharmacodynamics, pharmacokinetics and efficacy in PKU patients. Beam is advancing BEAM-304 using an innovative development approach in which multiple mutation-specific base editors are developed efficiently within a single clinical program. Initial clinical development will focus on base editors addressing the two most prevalent variants found in nearly half of patients with PKU in the U.S., with ongoing research efforts to address additional pathogenic mutations.

### **About Phenylketonuria (PKU)**

Phenylketonuria (PKU) is a rare, inherited metabolic disorder caused by pathogenic variants in the phenylalanine hydroxylase (*PAH*) gene, resulting in the inability to properly metabolize phenylalanine (Phe), an essential amino acid. Elevated blood Phe levels can lead to serious and irreversible brain damage and neurological complications, including cognitive impairment, developmental delays, and psychiatric symptoms, if not adequately controlled. PKU affects approximately 20,000 individuals in the United States and is typically diagnosed in infancy through newborn screening. There are no currently approved curative treatments for PKU. Current treatment options often require lifelong dietary restriction and chronic disease management, and many patients continue to experience significant unmet medical need.

### **About Beam Therapeutics**

Beam Therapeutics (Nasdaq: BEAM) is a biotechnology company committed to establishing the leading, fully integrated platform for precision genetic medicines. To achieve this vision, Beam has assembled a platform with integrated gene editing, delivery and internal manufacturing capabilities. Beam's suite of gene editing technologies is anchored by base editing, a proprietary technology that is designed to enable precise, predictable and efficient single base changes, at targeted genomic sequences, without making double-stranded breaks in the DNA. This has the potential to enable a

wide range of potential therapeutic editing strategies that Beam is using to advance a diversified portfolio of base editing programs. Beam is a values-driven organization committed to its people, cutting-edge science, and a vision of providing lifelong cures to patients suffering from serious diseases.

#### **Cautionary Note Regarding Forward-Looking Statements**

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Investors are cautioned not to place undue reliance on these forward-looking statements, including, but not limited to, statements related to: the therapeutic applications and potential of our technology, including with respect to PKU; our plans, and anticipated timing, to advance our PKU program; the clinical trial designs and expectations for BEAM-304; our expected presentation at the FASEB conference; our anticipated regulatory interactions and filings; and our ability to develop lifelong, curative, precision genetic medicines for patients through base editing. 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; the uncertainty that our product candidates will receive regulatory approval necessary to initiate or continue human clinical trials; 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, and anticipated timing to advance, our clinical trials may take longer than expected; that our product candidates, including the delivery modalities we rely on to administer them, may cause serious adverse events; 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" in our Annual Report on Form 10-K for the year ended December 31, 2025, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, and in any subsequent filings with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this press release. 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.

#### **Contacts:**

Investors:

Holly Manning

Beam Therapeutics

[hmanning@beamtx.com](mailto:hmanning@beamtx.com)

Media:

Josie Butler

1AB

[josie@1abmedia.com](mailto:josie@1abmedia.com)

---

<sup>1</sup> U.S. Food and Drug Administration. FDA Issues Draft Guidance to Help Accelerate Cell and Gene Therapies for Patients. Press release. June 2, 2026. Available at: <https://www.fda.gov/news-events/press-announcements/fda-issues-draft-guidance-help-accelerate-cell-and-gene-therapies-patients>. Accessed June 11, 2026.