UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): November 22, 2021

bluebird bio, Inc.

(Exact name of Registrant as Specified in Its Charter)

Delaware	**001-35966**	**13-3680878**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**
60 Binney Street, Cambridge, MA		**02142**
(Address of Principal Executive Offices)		**(Zip Code)**

Registrant's Telephone Number, Including Area Code: (339) 499-9300

Not Applicable
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instructions A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.01 par value per share	BLUE	The NASDAQ Stock Market LLC

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 8.01 Other Events.

On November 22, 2021, bluebird bio, Inc. ("bluebird") issued a press release announcing that the U.S. Food and Drug Administration (FDA) has accepted the Biologics License Application (BLA) for betibeglogene autotemcel (beti-cel) for priority review. Beti-cel is a potentially transformative gene therapy for adult, adolescent and pediatric patients with β-thalassemia across all genotypes who require regular red blood cell (RBC) transfusions. If approved, beti-cel will be the first one-time treatment that addresses the underlying genetic cause of disease for patients living with β-thalassemia in the United States, and an alternative to regular RBC transfusions and iron chelation therapy. The agency has set a Prescription Drug User Fee Act goal date of May 20, 2022.

The full text of bluebird's press release regarding the announcement is filed as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press release issued by bluebird bio, Inc. on November 22, 2021.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: November 22, 2021

bluebird bio, Inc.

By: /s/ Helen C. Fu

Helen C. Fu

Senior Vice President, General Counsel and Secretary

Exhibit 99.1

bluebird bio Announces FDA Priority Review of Biologics License Application for beti-cel Gene Therapy for Patients with β-thalassemia Who Require Regular Red Blood Cell Transfusions

If approved, beti-cel will be the first one-time treatment option to address the underlying genetic cause of disease

Current standard of care relies on regular red blood cell transfusions and iron management that carry the risk of progressive multi-organ damage and increased risk of morbidity and mortality

FDA set PDUFA date of May 20, 2022

CAMBRIDGE, Mass. — (BUSINESS WIRE) — November 22, 2021— bluebird bio, Inc. (Nasdaq: BLUE) today announced that the U.S. Food and Drug Administration (FDA) has accepted the Biologics License Application (BLA) for betibeglogene autotemcel (beti-cel) for priority review. Beti-cel is a potentially transformative gene therapy for adult, adolescent and pediatric patients with β-thalassemia across all genotypes who require regular red blood cell (RBC) transfusions. If approved, beti-cel will be the first one-time treatment that addresses the underlying genetic cause of disease for patients living with β-thalassemia in the U.S.—offering an alternative to regular RBC transfusions and iron chelation therapy. The agency has set a Prescription Drug User Fee Act (PDUFA) goal date of May 20, 2022.

“The FDA’s acceptance of our BLA for beti-cel brings us one step closer to potentially providing a one-time treatment that can address the underlying cause of β-thalassemia and offer patients freedom from regular transfusions,” said Andrew Obenshain, chief executive officer, bluebird bio. “It’s also a critical milestone for bluebird bio as an independent severe genetic disease company. We are moving forward with great discipline and exceptional care to deliver on our commitments to patients and achieve our near-term goal of launching three first-in-class gene therapies in the U.S.”

The BLA for beti-cel is based on data from bluebird bio’s Phase 3 studies HGB-207 (Northstar-2) and HGB-212 (Northstar-3), the Phase 1/2 HGB-204 (Northstar) and HGB-205 studies, and the long-term follow-up study LTF-303. Together, these studies represent more than 220 patient-years of experience with beti-cel. As of March 9, 2021, the results include a total of 63 pediatric, adolescent and adult patients, including long-term efficacy and safety results in two patients with more than seven years follow-up. Additional data through August 2021 will be presented at the 63rd American Society of Hematology (ASH) Annual Meeting and Exposition, taking place December 11-14, 2021.

“For too long, people with β-thalassemia who rely on regular transfusions have had to live with extraordinary burdens associated with their disease. beti-cel works uniquely to help patients produce adult hemoglobin at normal or near-normal levels, which can eliminate their need for chronic transfusions and chelation that only temporarily relieve the symptoms of anemia and are associated with serious health risks and reduced quality of life,” said Anne-Virginie Eggimann, chief regulatory officer, bluebird bio. “This BLA acceptance represents the culmination of contributions from many, including the patients involved in the clinical program, their caregivers, and the study investigators. We look forward to working closely with the FDA to bring this treatment to patients in need.”

The FDA previously granted beti-cel Orphan Drug status and Breakthrough Therapy designation.

About β-thalassemia
β-thalassemia is a severe genetic disease for those requiring regular red blood cell (RBC) transfusions, caused by mutations in the β-globin gene, which may cause significantly reduced adult hemoglobin (Hb).

This can result in severe anemia and lifelong dependence on RBC transfusions. Patients who require regular RBC transfusions to maintain adequate Hb levels typically undergo the 4-7-hour process every 3-4 weeks. While transfusions temporarily relieve symptoms associated with severe anemia, including fatigue, weakness, and shortness of breath, they do not address the underlying genetic cause of β-thalassemia and can lead to unavoidable iron overload and serious complications, including progressive multi-organ damage and organ failure. Iron overload resulting from β-thalassemia or ongoing RBC transfusions requires chronic treatment with chelation therapy; even with chelation therapy, some patients remain significantly iron overloaded, and only 63% of patients are adherent, due in part to tolerability issues. Despite advances in treatment and improved transfusion techniques, people with β-thalassemia who require regular transfusions have an increased risk for morbidity and mortality.

About betibeglogene autotemcel (beti-cel)
betibeglogene autotemcel (beti-cel) (pronounced BEH tee cell) is a one-time gene therapy custom-designed to treat the underlying cause of β-thalassemia in patients who require regular red blood cell (RBC) transfusions. Beti-cel adds functional copies of a modified form of the β-globin gene ($\beta^{A\text{-}T87Q}$-globin gene) into a patient's own hematopoietic (blood) stem cells (HSCs) in order to correct the deficiency of adult hemoglobin that is the hallmark of β-thalassemia. Once a patient has the $\beta^{A\text{-}T87Q}$-globin gene, they have the potential to produce beti-cel-derived adult hemoglobin (HbA^{T87Q}) at levels that may eliminate the need for transfusions. In Phase 3 beti-cel studies, 89% (32/36) of evaluable patients across all ages and genotypes, including pediatric patients as young as four years of age and those with the most severe (β^0/β^0) genotypes, achieved transfusion independence, which is defined as no longer needing RBC transfusions for at least 12 months while maintaining a weighted average Hb of at least 9 g/dL.

beti-cel is manufactured using the BB305 lentiviral vector (LVV), a third-generation, self-inactivating LVV that has been studied for more than a decade across multiple therapeutic areas.

Adverse reactions considered related to beti-cel were uncommon and consisted primarily of non-serious infusion-related reactions that occurred on the day of infusion (e.g. abdominal pain, hot flush, dyspnea, tachycardia and non-cardiac chest pain) and cytopenias (e.g. thrombocytopenia, leukopenia and neutropenia). Pain in extremity shortly after treatment was also documented. One of these adverse events (AE) was a serious adverse event (SAE) of thrombocytopenia considered possibly related to beti-cel and has resolved.

The majority of AEs and SAEs in the beti-cel clinical development program were unrelated to beti-cel and consistent with the known side effects of HSC collection and busulfan conditioning regimen (including several SAEs of veno-occlusive disease that resolved with treatment).

The Phase 3 Northstar-2 (HGB-207) and Northstar-3 (HGB-212) studies evaluating beti-cel are ongoing; enrollment is complete, and all patients have been treated. bluebird bio is also conducting a long-term follow-up study, LTF-303, to monitor safety and efficacy for people who have participated in bluebird bio-sponsored beti-cel clinical studies through 15 years post-treatment.

About bluebird bio, Inc.
bluebird bio is pursuing curative gene therapies to give patients and their families more bluebird days.

With a dedicated focus on severe genetic diseases, bluebird has industry-leading clinical and research programs for sickle cell disease, β-thalassemia and cerebral adrenoleukodystrophy and is advancing research to apply new technologies to these and other diseases. We custom design each of our therapies to address the underlying cause of disease and have developed in-depth and effective analytical methods to understand the safety of our lentiviral vector technologies and drive the field of gene therapy forward.

Founded in 2010, bluebird has the largest and deepest ex-vivo gene therapy data set in the world—setting the standard for industry. Today, bluebird continues to forge new paths, combining our real-world experience with a deep commitment to patient communities and a people-centric culture that attracts and grows a diverse flock of dedicated birds.

For more information, visit **bluebirdbio.com** or follow us on social media at **@bluebirdbio**, **LinkedIn**, **Instagram** and **YouTube**.

bluebird bio Cautionary Statement Regarding Forward-Looking Statements
This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. All statements that are not statements of historical facts are, or may be deemed to be, forward-looking statements. Such forward-looking statements are based on historical performance and current expectations and projections about our future goals, plans and objectives and involve inherent risks, assumptions and uncertainties, including internal or external factors that could delay, divert or change any of them in the next several years, that are difficult to predict, may be beyond our control and could cause our future goals, plans and objectives to differ materially from those expressed in, or implied by, the statements. No forward-looking statement can be guaranteed. Forward-looking statements in this press release should be evaluated together with the many risks and uncertainties that affect bluebird bio's business, particularly those identified in the risk factors discussion in bluebird bio's Annual Report on Form 10-K, as updated by our subsequent Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and other filings with the Securities and Exchange Commission. These risks and uncertainties include, but are not limited to: the risk that the efficacy and safety results from our prior and ongoing clinical trials will not continue or be repeated in our ongoing or planned clinical trials; the risk that additional insertional oncogenic or other safety events associated with lentiviral vector, drug product, or myeloablation will be discovered or reported over time; and the risk that any one or more of our product candidates, will not be successfully developed, approved or commercialized. The forward-looking statements included in this document are made only as of the date of this document and except as otherwise required by applicable law, bluebird bio undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events, changed circumstances or otherwise.

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