# 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): May 23, 2018**

# 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 (Address of Principal Executive Offices) | | 02142 (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))

Indicate by check mark whether the registrant is an emerging growth company as defined in 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 May 23, 2018, bluebird bio, Inc. ("bluebird") issued a press release to announce that the U.S. Food and Drug Administration has granted Breakthrough Therapy designation to bluebird's Lenti-D product candidate for the treatment of patients with cerebral adrenoleukodystrophy. A copy of the press release 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

| **xhibit No.** | **Description** |
|---|---|
| 9.1 | Press release issued by bluebird bio, Inc. on May 23, 2018. |

**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: May 23, 2018

**bluebird bio, Inc.**

By:/s/ Jason F. Cole
Jason F. Cole
*Chief Legal Officer*

**Exhibit 99.1**

**FDA Grants Breakthrough Therapy Designation to**
**Lenti-D™ for the Treatment of Cerebral Adrenoleukodystrophy**

**CAMBRIDGE, Mass., May 23, 2018** – bluebird bio, Inc. (Nasdaq: BLUE) today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to Lenti-D™ for the treatment of patients with cerebral adrenoleukodystrophy (CALD), a rare, serious and life-threatening hereditary neurological disorder.

Breakthrough Therapy designation is designed to expedite the development and review of a drug intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints.

"The founding of bluebird was inspired by the potential to develop a one-time gene therapy for boys suffering from this potentially fatal form of adrenoleukodystrophy," said David Davidson, M.D., chief medical officer, bluebird bio. "With Lenti-D, we hope the modified, autologous hematopoietic stem cells will keep these boys alive and free from major functional disabilities while avoiding many of the safety risks of the current standard of care, allogeneic hematopoietic stem cell transplant. The FDA's Breakthrough Therapy designation for Lenti-D brings us one step closer to realizing this mission to bring new hope to the patients and families affected by this devastating disease. We look forward to continuing to work closely with the FDA and EMA to expedite development of Lenti-D as a treatment for CALD."

Breakthrough Therapy designation is supported by preliminary data from the ongoing Phase 2/3 Starbeam Study (ALD-102) evaluating Lenti-D investigational gene therapy in boys with CALD, 17 years of age or less who do not have a matched sibling donor. Findings from 17 patients were published in the *New England Journal of Medicine* in October 2017 and showed that 15 of the 17 patients (88 percent) infused with Lenti-D remained alive and free of major functional disabilities at 2 years post-treatment, the primary efficacy endpoint of the trial. Results also showed that the safety profile of Lenti-D remains consistent with myeloablative chemotherapy. Additionally, no engraftment failure, graft versus host disease or treatment-related mortality occurred, nor was there any evidence of insertional oncogenesis.

bluebird's Lenti-D investigational gene therapy previously was granted Orphan Drug designation by the FDA and European Medicines Agency (EMA), as well as Rare Pediatric Disease designation by the FDA for the treatment of adrenoleukodystrophy (ALD). Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives a marketing application approval for a rare pediatric indication may be eligible for a Priority Review Voucher, which can be redeemed to obtain Priority Review for a subsequent marketing application for a different product.

**About the Starbeam (ALD-102) Study**
Starbeam is a two-year study assessing the safety and efficacy of an investigational gene therapy in boys 17 years of age and under with CALD. This therapy is comprised of a patient's own immature bone marrow cells that are modified to include a functional copy of the ABCD1 gene to express functional ALD protein (ALDP) which is lacking in patients with CALD.

When the modified cells are provided back to the patient, they develop into different cell types, including brain cells. Presumably, expression of ALDP in the brain can lead to a reduction in very long chain fatty acids (VLCFA). The accumulation of VLCFA in the brain is what is thought to contribute to potentially fatal neurodegeneration in CALD. Patients who complete the Starbeam study subsequently enroll in LTF-304, a long-term follow-up study of patients with CALD who were treated with Lenti-D.

**About CALD**
Also known as Lorenzo's Oil disease, adrenoleukodystrophy (ALD) is estimated to affect one in every 21,000 male births worldwide. Forty percent of boys born with ALD will progress to the cerebral form of the disease, cerebral adrenoleukodystrophy (CALD), a potentially fatal form of ALD. CALD involves a breakdown of the protective sheath of the nerve cells in the brain that are responsible for thinking and muscle control.

Currently, the only therapeutic option for patients with CALD is allogeneic hematopoietic stem cell transplant (HSCT); beneficial effect has been reported if performed early in the course of CALD progression. Potential complications of allogeneic HSCT, which can be fatal, include graft failure, graft versus host disease (GVHD) and opportunistic infections, particularly in patients who undergo allogeneic HSCT using cells from a donor who is not a matched, unaffected sibling.

Early diagnosis of CALD is important, as the outcome of HSCT varies with clinical stage of the disease at the time of transplant. Favorable outcomes have been observed in patients who undergo transplant in the early stages of cerebral disease. Newborn screening for ALD is a critical enabler of early diagnosis and successful treatment of ALD. In the United States, newborn screening for ALD was added to the Recommended Universal Screening Panel (RUSP) in February 2016. Newborn screening for ALD is active in a limited number of states in the U.S.

**About bluebird bio, Inc.**
With its lentiviral-based gene therapies, T cell immunotherapy expertise and gene editing capabilities, bluebird bio has built an integrated product platform with broad potential application to severe genetic diseases and cancer. bluebird bio's gene therapy clinical programs include Lenti-D™ for the treatment of cerebral adrenoleukodystrophy, and LentiGlobin™ for the treatment of transfusion-dependent β-thalassemia, also known as β-thalassemia major, and severe sickle cell disease. bluebird bio's oncology pipeline is built upon the company's leadership in lentiviral gene delivery and T cell engineering, with a focus on developing novel T cell-based immunotherapies, including chimeric antigen receptor (CAR T) and T cell receptor (TCR) therapies. bluebird bio's lead oncology programs, bb2121 and bb21217, are anti-BCMA CAR T programs partnered with Celgene. bluebird bio also has discovery research programs utilizing

megaTAL/homing endonuclease gene editing technologies with the potential for use across the company's pipeline.

bluebird bio has operations in Cambridge, Massachusetts, Seattle, Washington, Durham, North Carolina and Zug, Switzerland.

LentiGlobin and Lenti-D are trademarks of bluebird bio, Inc.

**Forward-Looking Statements**
*This release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the clinical and market potential of the Company's Lenti-D product candidate. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to, risks that the preliminary efficacy and safety data for our Lenti-D product candidate from the Starbeam Study will not continue or persist, the risk of cessation or delay of any of the ongoing clinical studies and/or our development of Lenti-D, the risks regarding future potential regulatory approvals of Lenti-D, and the risk that any one or more of our product candidates will not be successfully developed, approved or commercialized. For a discussion of other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in our annual report on Form 10-K, as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and bluebird bio undertakes no duty to update this information unless required by law.*

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