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): September 5, 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 September 5, 2018, bluebird bio, Inc. ("bluebird") issued a press release announcing updated data results from the Phase 2/3 Starbeam study (ALD-102) of its investigational Lenti-D gene therapy product candidate in boys 17 years of age and under with cerebral adrenoleukodystrophy (CALD), and initial data from ALD-103, the ongoing observational study of outcomes from allogeneic hematopoietic stem cell transplant (allo-HSCT) in boys 17 years of age and under with CALD. The data results were presented at the Society for the Study of Inborn Errors of Metabolism (SSIEM) 2018 Symposium in Athens, Greece.

bluebird also announced that it has reached general agreement with the U.S. Food and Drug Administration and the European Medicines Agency on the clinical development program to support future marketing applications for the Lenti-D product candidate in the treatment of CALD.

The full text of the 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 September 5, 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: September 5, 2018

bluebird bio, Inc.

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

Exhibit 99.1

bluebird bio Presents Updated Data from Phase 2/3 Starbeam Study of Investigational Lenti-D™ Gene Therapy for CALD and Initial Data from Observational Study ALD-103 of Allogeneic Hematopoietic Stem Cell Transplant in CALD at 2018 SSIEM

– Updated Efficacy and Safety Data Consistent with Previously Reported Phase 2/3 Starbeam Results –

– General Agreement Reached on Regulatory Pathway for Lenti-D in the U.S. and EU –

CAMBRIDGE, Mass. – September 5, 2018 – bluebird bio, Inc. (Nasdaq: BLUE) announced updated results from the Phase 2/3 Starbeam study (ALD-102) of its investigational Lenti-D™ gene therapy in boys 17 years of age and under with cerebral adrenoleukodystrophy (CALD), and initial data from ALD-103, the ongoing observational study of outcomes from allogeneic hematopoietic stem cell transplant (allo-HSCT) in boys 17 years of age and under with CALD. The data were presented today at the Society for the Study of Inborn Errors of Metabolism (SSIEM) 2018 Symposium in Athens, Greece.

bluebird bio has also reached general agreement with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) on the clinical development program to support future marketing applications for Lenti-D in CALD.

“Cerebral adrenoleukodystrophy is a genetic disease primarily affecting young boys that causes progressive damage to the brain, leading to the permanent loss of physical and cognitive function, and it is often fatal,” said David Davidson, M.D., chief medical officer, bluebird bio. “The data we presented from our Phase 2/3 Starbeam study are consistent with the results we have observed with Lenti-D to date, with the additional follow-up showing durable lack of progression to major functional disabilities (MFD) in boys who were MFD-free at 24 months post-treatment. The data from the ALD-103 study highlight the increased risks of mortality and morbidity in boys without a matched sibling donor who undergo allogeneic HSCT. We look forward to working with the FDA and EMA to advance our goal of delivering a gene therapy for patients with this terrible disease.”

Updated Results: Phase 2/3 Starbeam Study

The Phase 2/3 Starbeam study has met its enrollment goal. All reported data are as of April 25, 2018 and reflect a total study population of 31 patients. Of these 31 patients, 29 have received Lenti-D and the median follow-up for all treated patients was 34 months (0.4 – 54 months).

The primary efficacy endpoint of the Phase 2/3 Starbeam study is the proportion of patients who are alive and free of major functional disabilities (MFD) at month 24. MFDs are six severe disabilities commonly attributed to CALD and thought to have the most profound impact on a

patient's ability to function independently: loss of ability to communicate, cortical blindness, need for tube feeding, total incontinence, wheelchair dependence, and complete loss of voluntary movement.

As previously reported in the *New England Journal of Medicine* (October 2017), of the 17 patients treated with Lenti-D who completed 24 months of follow-up, 15 patients (88 percent) were alive and MFD-free. One patient withdrew from the study, and one died following rapid development of MFDs post-treatment. All patients who were MFD-free at 24 months (n=15) continued to be MFD-free. The median follow-up for the initial group (n=17) was 41.4 months (13.4 - 54.0 months).

An additional 12 patients have received Lenti-D in the Phase 2/3 Starbeam study. While these patients have not reached the primary endpoint of 24-month follow-up, there have been no MFDs reported as of April 25, 2018. The median follow-up for this additional cohort of patients is 4.2 months (0.4 – 11.7 months).

Secondary efficacy endpoint data for gadolinium-enhancement (GdE) and Neurologic Function Scores (NFS) were also reported. GdE was assessed by magnetic resonance imaging (MRI) every six months following transplant up to 24-months, and then every 12 months. In the 15 patients who completed 24 months of follow-up, 14 were negative for GdE as of their last MRI. Eleven patients in the study had intermittent re-emergence of GdE-positivity at various follow-up assessments, however, post-treatment GdE positivity was markedly reduced in intensity and does not appear to correlate with clinical outcome.

NFS was used to quantify the severity of neurologic dysfunction based on 15 symptoms. All patients had an NFS of ≤1 at time of Lenti-D treatment. In 15 patients who met 24 months of follow up by April 25, 2018; 14 had an NFS score ≤1 at their last follow-up visit, and one patient had an increase in NFS from 1 to 2, due to vision impairment and non-febrile seizures. One patient had rapid disease progression beginning early in their participation in the study, resulting in multiple MFDs and an NFS of 17 at last follow-up.

The primary safety endpoint of the Phase 2/3 Starbeam study is the proportion of patients who experienced grade ≥2 acute graft-versus-host disease (GvHD) or chronic GvHD by 24 months post-treatment. No acute or chronic GvHD has been reported post-Lenti-D treatment as of April 25, 2018. There has been no graft failure, insertional oncogenesis or replication competent lentivirus detected. The safety profile of Lenti-D is generally consistent with myeloablative conditioning with busulfan and cyclophosphamide. Three adverse events (AE) have been deemed potentially related to treatment with Lenti-D and include BK-mediated viral cystitis (grade 3), tachycardia (grade 1), and vomiting (grade 1).

"As a physician who treats boys with CALD, I see its devastating effects on young children and families," said Professor Paul Gissen, Consultant in Pediatric Metabolic Diseases at Great Ormond Street Hospital, London, United Kingdom, and an investigator in the Phase 2/3

Starbeam study. “Data from the Phase 2/3 Starbeam study suggest that Lenti-D, which utilizes a child’s own cells and does not require a matched donor, may be a potential treatment for CALD.”

Initial Results from ALD-103 Study

Allo-HSCT has been successfully used to treat CALD but may be associated with adverse immunologic complications. The ongoing observational study ALD-103 is designed to assess safety and efficacy outcomes of this treatment option in boys 17 years of age or younger with CALD.

As of April 25, 2018, 41 pediatric patients were enrolled in the ALD-103 study and had undergone allo-HSCT. Thirty-one patients received cells from unrelated donors and 10 received cells from a related donor. The median baseline NFS for the group was 0.0 (min – max, 0.0 - 4.0). The median Loes score, a 34-point scale that measures the location and extent of demyelination through MRI, was 3.0 (min – max, 0.0 - 16.0). Twenty-five patients had early cerebral disease, defined as having evidence of cerebral disease established by GdE positivity or Loes score ≥0.5, Loes score ≤9.0, and NFS ≤1.

Initial results were in line with safety and efficacy outcomes reported in the literature for allo-HSCT in patients with CALD. Two-year Kaplan-Meier estimates of MFD-free survival post-allo-HSCT, were 78 percent for patients with early disease (n=25) and 71 percent for all patients (n=41).

Transplant-related mortality was defined as death due to any transplantation-related cause other than disease progression. There were six transplant-related deaths at one year (14.6 percent); none of these patients had a matched sibling donor. Engraftment failure was reported in five patients (12 percent); none of these patients had a matched sibling donor and all received a second transplant.

Of the 41 patients who received allo-HSCT, 34 percent (n=14) experienced either grade ≥2 acute GvHD or chronic GvHD.

Lenti-D Data at SSIEM

Oral Presentation: Lenti-D hematopoietic stem cell gene therapy for cerebral adrenoleukodystrophy: safety and efficacy outcomes from an ongoing Ph 2/3 trial

Presenter: Paul Gissen, MBChB, Ph.D., FRCPCH, Pediatric Metabolic Diseases at Great Ormond Street Hospital, London, United Kingdom
Date & Time: Wednesday, September 5, 2018, 9:00 – 10:30 a.m. EEST (2:00 – 3:30 a.m. EDT)

Oral Presentation: An observational study of patients with cerebral adrenoleukodystrophy (CALD) treated with allogeneic hematopoietic stem cell transplant

Presenter: Robert Chiesa, M.D., Pediatric Bone Marrow Transplantation at Great Ormond Street Hospital, London, United Kingdom
Date & Time: Wednesday, September 5, 2018, 9:00 – 10:30 a.m. EEST (2:00 – 3:30 a.m. EDT)

Regulatory Progress for Lenti-D in Cerebral Adrenoleukodystrophy
bluebird bio reached general agreement with the FDA and EMA on the clinical development program to support future marketing applications for Lenti-D in the treatment of CALD.

The primary efficacy endpoint in the Phase 2/3 Starbeam study is the proportion of patients at month 24 who achieve MFD-free survival. This endpoint will be compared to a clinically meaningful benchmark that is based on literature and data collected in ALD-101, a retrospective analysis that assessed the natural history of CALD as well as outcomes from allo-HSCT treatment in patients with CALD.

The primary safety endpoint in the Phase 2/3 Starbeam study is the proportion of patients who experience grade ≥2 acute GvHD or chronic GvHD at 24 months post treatment. Due to the potential increased risk of immunologic complications when randomizing subjects with a less than ideal HLA-matched donor to an allo-HSCT trial arm, an external control approach has been adopted to establish the benefit-risk profile of Lenti-D.

The safety results from the Phase 2/3 Starbeam study will be compared with data collected from study ALD-103, a multinational, multi-site, prospective and retrospective observational study designed to evaluate outcomes of allo-HSCT in subjects with CALD aged 17 years or younger.

ALD-103 is running concurrently with ALD-102 and its study design is consistent with ALD-102.
bluebird bio is conducting a long-term safety and efficacy follow-up study (LTF-304) for patients who have participated in ALD-102 and were treated with Lenti-D.

About Cerebral Adrenoleukodystrophy
Adrenoleukodystrophy (ALD) is a rare, X-linked metabolic disorder that is estimated to affect one in 21,000 male newborns worldwide. ALD is caused by mutations in the *ABCD1* gene that affect the production of adrenoleukodystrophy protein (ALDP) and subsequently cause toxic accumulation of very long chain fatty acids (VLCFAs) in the adrenal cortex and white matter of the brain and spinal cord.

Approximately 35-40 percent of boys with ALD will develop cerebral ALD (CALD), the most severe form of ALD. CALD is a progressive neurogenerative disease that involves breakdown of myelin, the protective sheath of the nerve cells in the brain that are responsible for thinking and muscle control. Symptoms of CALD usually occur in early childhood and progress rapidly, if untreated, leading to severe loss of neurologic function, and eventual death, in most patients.

Currently, the only therapeutic option for patients with CALD is allo-HSCT. Beneficial effects have been reported if allo-HSCT is performed early in the course of CALD progression. Potential complications of allo-HSCT, which can be fatal, include graft failure, graft-versus-host disease, and opportunistic infections, particularly in patients who undergo transplant with non-sibling matched donor cells.

Early diagnosis of CALD is important, as the outcome of treatment varies with the clinical stage of the disease at the time of transplant. 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 in February 2016 but is currently active in only a limited number of states.

About bluebird bio, Inc.
With its lentiviral-based gene therapies, T cell immunotherapy expertise and gene editing capabilities, bluebird bio has built a pipeline with broad potential application in severe genetic diseases and cancer.

bluebird bio's gene therapy clinical programs include investigational treatments for cerebral adrenoleukodystrophy, 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 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. The company's lead oncology programs are anti-BCMA CAR T programs partnered with Celgene.

bluebird bio's discovery research programs include utilizing meatal/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.

Lenti-D is a trademark 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, clinical development and anticipated regulatory approval pathways, and market potential of the Company's Lenti-D product candidate to treat cerebral adrenoleukodystrophy. 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, including the risk that the Starbeam Study will be insufficient to support regulatory submissions or marketing approval in the US and EU, 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 most recent Form 10-Q, 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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