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): October 2, 2020

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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 October 2, 2020, bluebird bio, Inc. ("bluebird"or the “Company”) issued a press release announcing that the European Medicines Agency (EMA) accepted the Company’s marketing authorization application (MAA) for its investigational elivaldogene autotemcel (eli-cel, Lenti-D™) gene therapy for the treatment of patients with cerebral adrenoleukodystrophy (CALD).

The full text of bluebird’s press release is being furnished 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 October 2, 2020.
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: October 2, 2020

bluebird bio, Inc.

By: /s/ Jason F. Cole

Jason F. Cole

Chief Operating and Legal Officer

Exhibit 99.1

bluebird bio Announces European Medicines Agency's Acceptance of Marketing Authorization Application (MAA) for elivaldogene autotemcel (eli-cel, Lenti-D™) Gene Therapy for Cerebral Adrenoleukodystrophy (CALD)

European Medicines Agency will evaluate eli-cel MAA under accelerated assessment

CAMBRIDGE, Mass. – (BUSINESS WIRE) October 2, 2020 – bluebird bio, Inc. (Nasdaq: BLUE) today announced that the European Medicines Agency (EMA) accepted the company's marketing authorization application (MAA) for its investigational elivaldogene autotemcel (eli-cel, Lenti-D™) gene therapy for the treatment of patients with cerebral adrenoleukodystrophy (CALD). CALD is a fatal neurodegenerative disease primarily affecting young boys.

"CALD is a devastating disease, often marked by rapid neurodegeneration, the development of major functional disabilities, and eventual death. The acceptance of the MAA for eli-cel is a critical milestone in our continued collaboration with the EMA to potentially deliver an autologous gene therapy for boys with CALD," said Gary Fortin, Ph.D., SVP, severe genetic diseases, bluebird bio. "Data from clinical studies conducted in patients with early CALD suggest eli-cel stabilizes the progression of the disease. If approved, eli-cel would represent the first therapy for CALD that uses a patient's own hematopoietic stem cells, potentially mitigating the risk of life-threatening immune complications associated with transplant using cells from a donor."

Data from the Phase 2/3 Starbeam study (ALD-102) formed the basis of the MAA, which is also supported with data from the ongoing Phase 3 ALD-104 study and the long-term follow-up study (LTF-304). The most recent results from these studies were presented at the 46th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT 2020) in August 2020.

Eli-cel is a one-time investigational gene therapy designed to add functional copies of the *ABCD1* gene into a patient's own hematopoietic (blood) stem cells (HSCs) that have been transduced *ex vivo* with the Lenti-D lentiviral vector (LVV). The addition of a functional gene allows patients to produce the adrenoleukodystrophy protein (ALDP), which is thought to allow for the breakdown of very-long-chain fatty acids (VLCFAs) that accumulate to toxic levels in the brain. There is no need for donor HSCs from another person.

The EMA accepted eli-cel gene therapy for the treatment of CALD into its Priorities Medicines scheme (PRIME) in July 2018, and previously granted Orphan Medicinal Product designation to eli-cel. In July 2020, the Committee for Medicinal Products for Human Use (CHMP) of the EMA granted an accelerated assessment to eli-cel, potentially reducing the EMA's active review time of the MAA from 210 days to 150 days.

The U.S. Food and Drug Administration (FDA) granted eli-cel Orphan Drug status, Rare Pediatric Disease designation, and Breakthrough Therapy designation for the treatment of CALD. bluebird bio is currently on track to submit the Biologics License Application (BLA) in the U.S. in mid-2021.

Eli-cel is not approved for any indication in any geography.

bluebird bio is currently enrolling patients for a Phase 3 study (ALD-104) designed to assess the efficacy and safety of eli-cel after myeloablative conditioning using busulfan and fludarabine in patients with CALD. Contact clinicaltrials@bluebirdbio.com for more information and a list of study sites.

Additionally, bluebird bio is conducting a long-term safety and efficacy follow-up study (LTF-304) for patients who have been treated with eli-cel for CALD and completed two years of follow-up in bluebird bio-sponsored studies.

The Phase 2/3 Starbeam study (ALD-102) has completed enrollment.

For more information about bluebird bio-sponsored studies visit: www.bluebirdbio.com/our-science/clinical-trials or clinicaltrials.gov.

About Cerebral Adrenoleukodystrophy (CALD)
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) primarily in the adrenal cortex and white matter of the brain and spinal cord.

Approximately 40% of boys with adrenoleukodystrophy will develop CALD, the most severe form of ALD. CALD is a progressive neurodegenerative 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. CALD is associated with six major functional disabilities (MFDs), which severely compromise a patient's ability to function independently: loss of communication, cortical blindness, need for tube feeding, total incontinence, wheelchair dependence, and complete loss of voluntary movement.

Although allogeneic hematopoietic stem cell transplantation (allo-HSCT) has been shown to have a beneficial effect on clinical indices of disease and long-term survival and can arrest disease progression if performed at the early stage of cerebral involvement, it has significant associated risks, such as transplant-related mortality (TRM), graft failure or rejection, graft-versus-host disease, and potential for opportunistic infections. Safety outcomes are typically more favorable if allo-HSCT is performed using cells from a human leukocyte antigen (HLA)-matched sibling donor.

Early diagnosis of CALD is important, as the outcome of available treatment varies with the clinical stage of the disease. Newborn screening for ALD is a critical enabler of early diagnosis and thus of successful treatment of ALD. Once a patient has been diagnosed with ALD, regular MRI scans are critical to detect white matter changes indicative of progression to CALD.

In the U.S., newborn screening for ALD was added to the Recommended Universal Screening Panel in February 2016 and is currently active in 17 states and Washington, D.C., accounting for at least 58% of U.S. newborns. Outside the U.S., the Minister of Health in the Netherlands has approved the addition of ALD to their newborn screening program. Even though ALD newborn screening has not been implemented in most EU countries, efforts to begin pilot programs are slowly progressing.

About bluebird bio, Inc.
bluebird bio is pioneering gene therapy with purpose. From our Cambridge, Mass., headquarters, we're developing gene and cell therapies for severe genetic diseases and cancer, with the goal that people facing potentially fatal conditions with limited treatment options can live their lives fully. Beyond our labs, we're working to positively disrupt the healthcare system to create access, transparency and education so that gene therapy can become available to all those who can benefit.

bluebird bio is a human company powered by human stories. We're putting our care and expertise to work across a spectrum of disorders, including cerebral adrenoleukodystrophy, sickle cell disease, β-thalassemia and multiple myeloma, using gene and cell therapy technologies including gene addition, and (megaTAL-enabled) gene editing.

bluebird bio has additional nests in Seattle, Wash.; Durham, N.C.; and Zug, Switzerland. For more information, visit **bluebirdbio.com**.

Follow bluebird bio on social media: @bluebirdbio, LinkedIn, Instagram and YouTube.

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 company's expectations and plans for regulatory approval for and commercialization of eli-cel in the U.S. and E.U. 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 efficacy and safety results for eli-cel from the Starbeam Study seen to date will not continue or persist, the risk of cessation or delay of any of the ongoing clinical studies and/or our development of eli-cel, the risks regarding future potential regulatory approvals of eli-cel, including the risk that the Starbeam Study will be insufficient to support regulatory submissions or marketing approval in the U.S., the risk that our submissions for regulatory approvals will not be submitted or accepted for filing by the regulatory authorities on the timeframe we expect or at all, 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-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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