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): February 23, 2021

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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 2.02 Results of Operations and Financial Condition.

On February 23, 2021, bluebird bio, Inc. ("bluebird" or the "Company") announced its financial results for the year and three months ended December 31, 2020. A copy of the press release is being furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in this Report on Form 8-K and Exhibit 99.1 attached hereto is intended to be furnished and shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934 (the “Exchange Act”) or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933 or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press release issued by bluebird bio, Inc. on February 23, 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: February 23, 2021 bluebird bio, Inc.

By: /s/ Chip Baird
Chip Baird
Chief Financial Officer, Principal Financial Officer and Principal Accounting Officer

Exhibit 99.1

bluebird bio Reports Fourth Quarter and Full Year 2020 Financial Results and Highlights Operational Progress

CAMBRIDGE, Mass. – **Feb. 23, 2021** – bluebird bio, Inc. (NASDAQ: BLUE) today reported financial results and business highlights for the fourth quarter and full year ended December 31, 2020 and shared recent operational progress.

“For over ten years, bluebird bio has been pioneering gene therapies for patients with rare diseases and cancer, and our work continues with the analysis of the recent safety events identified in our HGB-206 study in patients with sickle cell disease. We have seen the transformative benefit of these therapies and are conducting a scientific and medical investigation to best inform our path forward on behalf of the patients we hope to serve with LentiGlobin,” said Nick Leschly, chief bluebird. “While this work is ongoing, we remain focused on the upcoming PDUFA action due date for ide-cel for multiple myeloma next month, the potential EU approval of eli-cel for CALD mid-year and the planned US filings for β-thalassemia and CALD later in the year. Through these challenges, we are keeping patients at the center and were very excited to reach a major milestone of the first commercial infusion of ZYNTEGLO in Germany earlier this month.”

RECENT HIGHLIGHTS

COMPANY

- **CLINICAL HOLD OF PHASE 1/2 AND PHASE 3 STUDIES OF LENTIGLOBIN GENE THERAPY FOR SICKLE CELL DISEASE AND TEMPORARY SUSPENSION OF MARKETING OF ZYNTEGLO IN EUROPE** – On February 16, 2021, bluebird bio announced that due to a Suspected Unexpected Serious Adverse Reaction (SUSAR) of acute myeloid leukemia and a SUSAR of myelodysplastic syndrome in our HGB-206 clinical study, the FDA has placed our clinical studies of LentiGlobin for SCD on clinical hold. We are investigating these events and plan to continue to work closely with the FDA in their review of these events. In addition, we are also engaged with the EMA in discussions regarding our proposed development plans for LentiGlobin for SCD in Europe. In light of these SUSARs, we have temporarily suspended marketing of ZYNTEGLO in Europe. Additionally, the EMA has paused the renewal procedure for ZYNTEGLO's conditional marketing authorization while the EMA's pharmacovigilance risk assessment committee reviews the risk-benefit assessment for ZYNTEGLO and determines whether any additional pharmacovigilance measures are necessary.

- **INTENT TO SEPARATE** – On January 11, 2021, bluebird bio announced its intent to separate its severe genetic disease and oncology businesses into two independent, publicly traded companies (bluebird bio and “Oncology Newco”). The separation is expected to be completed by year-end 2021 and it is anticipated that the spin out of Oncology Newco is to be tax-free to shareholders, subject to receipt of a favorable Internal Revenue Service (IRS) ruling.
    - Following the separation, bluebird bio intends to focus on delivery of its Core 3 therapies in β-thalassemia, cerebral adrenoleukodystrophy and sickle cell disease, expand access and reimbursement for our commercial product, ZYNTEGLO, in Europe and continue to explore innovative tools and technologies to ultimately bring these transformative medicines to more patients.

- Following the separation, Oncology Newco plans to support commercial success of ide-cel and continued development of bb21217, deliver on the oncology pipeline of cellular therapies with a focus on non-Hodgkin's lymphoma, acute myeloid leukemia, next-generation multiple myeloma and solid tumors and advance next generation product cycling engine with an overarching goal of 1-2 investigational new drugs (INDs) in each of the years 2021 and 2022.
- At the time of separation, bluebird bio plans to capitalize each business with sufficient capital to achieve value creating milestones and intends to provide additional financial details closer to the date of separation.

TRANSFUSION DEPENDENT β-THALASSEMIA

- **FIRST PATIENT TREATED WITH ZYNTEGLO GENE THERAPY** - bluebird bio is announcing today that a patient was treated earlier this month with the first commercial infusion of ZYNTEGLO [betibeglogene autotemcel (beti-cel)]. The patient was treated at a Qualified Treatment Center (QTC) in Germany.

- **TDT DATA AT ASH –** On December 5, 2020, bluebird bio presented new data showing that all patients that achieved transfusion independence continue to remain free from transfusions up to six years in the ongoing long-term follow-up study (LTF-303) of beti-cel gene therapy in patients with TDT at the 62nd American Society of Hematology (ASH) Annual Meeting. The company also presented updated efficacy and safety results for pediatric patients in the Phase 3 HGB-207 (Northstar-2) and HGB-212 (Northstar-3) studies.

SICKLE CELL DISEASE

- **FIRST PATIENT TREATED IN CONFIRMATORY HGB-210 STUDY –** bluebird bio is announcing today that within the first quarter, the first patient was infused in HGB-210, a Phase 3 confirmatory study of LentiGlobin™ gene therapy (bb1111) for adults and pediatric subjects ≥2 and ≤50 years of age with sickle cell disease (SCD).

- **SCD DATA AT ASH -** On December 7, 2020, bluebird bio presented new data showing a complete elimination of severe vaso-occlusive events (VOEs) through 24 months of follow-up in patients who had a history of at least four severe VOEs and at least six months follow-up in Group C of its ongoing Phase 1/2 HGB-206 study of bb1111 for patients with SCD at the 62nd ASH Annual Meeting.

- **MAGENTA COLLABORATION** – On December 4, 2020, bluebird bio and Magenta Therapeutics announced an exclusive clinical trial collaboration to evaluate the utility of MGTA-145, in combination with plerixafor, for mobilization and collection of stem cells in patients with SCD. The combination approach has the potential to achieve safe, rapid and reliable mobilization of sufficient quantities of high-quality stem cells to ultimately improve outcomes associated with stem cell transplantation. The companies will co-fund the clinical trial and Magenta will retain all rights to its product candidate.

MULTIPLE MYELOMA

- **CRB-401 DATA AT ASH –** On December 5, 2020, bluebird bio and Bristol-Myers Squibb presented longer-term results showing ongoing deep and durable responses in the original

Phase 1 study (CRB-401) of the companies' investigational B-cell maturation antigen (BCMA) directed chimeric antigen receptor (CAR) T cell therapy, idecabtagene vicleucel (ide-cel), in patients with relapsed and refractory multiple myeloma at the 62nd ASH Annual Meeting.

- **BB21217 DATA AT ASH -** On December 5, 2020, bluebird bio and Bristol-Myers Squibb presented updated safety and efficacy results showing promising response rates and durability from the ongoing Phase 1 study (CRB-402) of bb21217, an investigational BCMA-directed CAR T cell therapy in patients with relapsed and refractory multiple myeloma at the 62nd ASH Annual Meeting. The company announced the study has completed enrollment and follow-up is ongoing as data continue to mature and the durability of response at the recommended phase 2 dose is assessed.

UPCOMING ANTICIPATED MILESTONES

Regulatory Outlook

- Multiple Myeloma: The FDA has set a Prescription Drug User Fee Act (PDUFA) goal date of March 27, 2021 for the approval of ide-cel (bb2121) in patients with relapsed and refractory multiple myeloma.
- SCD: The company is investigating the recently-reported safety events and plans to continue to work closely with the FDA in their review of these events to provide an update on the Company's development plan and timeline for submission for regulatory approval.
- TDT: The company is on track to complete its rolling BLA submission to the U.S. FDA for beti-cel in mid-2021, contingent upon successful resolution of any U.S. FDA concerns applicable to the program arising out of the recently-reported safety events in the SCD program. This submission is anticipated to include patients with transfusion dependent β-thalassemia across all genotypes (including non-β0/β0 genotypes and β0/β0 genotypes).
- CALD: The company is on track to complete its BLA submission to the U.S. FDA for elivaldogene autotemcel (eli-cel) in mid-2021. The company plans to receive European approval for eli-cel in patients with cerebral adrenoleukodystrophy (CALD) in mid-2021.

Clinical Updates and Milestones

- Updated data from ongoing clinical study in patients with SCD by the end of 2021.
- Updated data from ongoing clinical studies in patients with TDT in mid-2021.
- Updated data from ongoing clinical studies in patients with CALD in mid-2021.
- bb21217 clinical data from the ongoing CRB-402 study in patients with multiple myeloma by the end of 2021.
- Submission of 1 - 2 investigational new drug (IND) applications by the end of 2021.

FOURTH QUARTER AND FULL YEAR 2020 FINANCIAL RESULTS

- **Cash Position:** Cash, cash equivalents and marketable securities as of December 31, 2020 and December 31, 2019 were $1.27 billion and $1.24 billion, respectively. The increase in cash, cash equivalents and marketable securities is primarily a result of proceeds received from the May 2020 public offering of the Company's common stock and a one-time upfront payment received in connection with the Company's amended collaboration with BMS, partially offset by cash used in support of ordinary course operating and commercial-readiness activities.
- **Revenues:** Total revenues were $10.7 million for the three months ended December 31, 2020 compared to $10.0 million for the three months ended December 31, 2019. Total revenues were $250.7 million for the twelve months ended December 31, 2020 compared to $44.7 million

for the twelve months ended December 31, 2019. The increase for the three-month period was primarily driven by an increase in royalty and other revenue. The increase for the twelve-month period was primarily driven by the amended BMS collaboration and monetization for ex-U.S. milestones and royalties from ide-cel and bb21217, with the majority of the revenue recognized relating to ide-cel license and manufacturing services.

- **R&D Expenses:** Research and development expenses were $137.1 million for the three months ended December 31, 2020 compared to $161.8 million for the three months ended December 31, 2019. Research and development expenses were $588.0 million for the twelve months ended December 31, 2020 compared to $582.4 million for the twelve months ended December 31, 2019. The decrease for the three-month period was primarily driven by a decrease in manufacturing costs, partially offset by increased costs incurred through the amended BMS collaboration. The increase for the twelve-month period was primarily driven by an overall increase in costs incurred to advance and expand the company's pipeline, partially offset by a decrease in manufacturing costs.
- **SG&A Expenses:** Selling, general and administrative expenses were $77.0 million for the three months ended December 31, 2020 compared to $76.2 million for the three months ended December 31, 2019. Selling, general and administrative expenses were $286.9 million for the twelve months ended December 31, 2020 compared to $271.4 million for the twelve months ended December 31, 2019. The increase for both periods was largely attributable to increased headcount and costs incurred to support the Company's ongoing operations and growth of its pipeline, partially offset by a decrease in costs related to commercial readiness activities due to delays during 2020 as a result of the COVID-19 pandemic.
- **Net Loss:** Net loss was $199.9 million for the three months ended December 31, 2020 compared to $223.3 million for the three months ended December 31, 2019. Net loss was $618.7 million for the twelve months ended December 31, 2020 compared to $789.6 million for the twelve months ended December 31, 2019.

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: 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 financial condition, results

of operations, as well as statements regarding the Company's timing and expectations regarding its investigation of the relationship of the safety events in HGB-206 to the use of lentiviral vector BB305 in LentiGlobin gene therapy for SCD, and any myeloablation regimen used in connection with treatment; the plans for regulatory submissions for beti-cel (marketed as ZYTENGLO in the European Union), eli-cel, ide-cel, and LentiGlobin for SCD, including anticipated endpoints to support regulatory submissions and timing expectations; the company's expectations regarding the potential for the suspension-based manufacturing process for lentiviral vector; the company's expectations and execution under its revised operating plan, including its cash runway; its expectations for commercialization efforts for ZYNTEGLO in Europe; and the company's expectations for the amended collaboration agreement with BMS; as well as the company's intentions regarding the timing for providing further updates on the development and commercialization of ZYNTEGLO and the company's product candidates; the timing, leadership, structure, including the division of assets among bluebird bio and Oncology Newco, and the impact of a separation; as well as the company's intention to provide further updates on the separation and the related financing strategies for bluebird bio and Oncology Newco; and the tax free nature of the separation. 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, the risk that the Company may not be able to definitively determine whether the lentiviral vector BB305 used in LentiGlobin gene therapy for SCD and in beti-cel is related to the safety events in a timely manner, or at all; the risk that the lentiviral vector BB305 has caused insertional oncogenic events, including acute myeloid leukemia; the risk that insertional oncogenic events associated with lentiviral vector or additional safety events associated with myeloablation will be discovered or reported over time; the risk that we may not be able to address regulatory authorities' concerns quickly or at all and may not be able to resume our HGB-206 or HGB-210 studies in a timely manner, or at all; the risk that we may not resume patient treatment with ZYNTEGLO in the commercial context in a timely manner or at all; the risk that our lentiviral vector platform across our severe genetic disease programs may be implicated, affecting the development and potential approval elivaldogene autotemcel; the risks that we may not complete the separation on the terms or timeline currently contemplated if at all, achieve the expected benefits of a separation, and a separation could harm our business, results of operations and financial condition; the risk that the transaction might not be tax-free; we may be unable to make, on a timely or cost-effective basis, the changes necessary to operate as independent companies; Oncology Newco's lack of independent operating history and the risk that its accounting and other management systems may not be prepared to meet the financial reporting and other requirements of operating as an independent public company; dedicated financial and/or strategic funding sources may not be available on favorable terms; a separation or announcement thereof may adversely impact our ability to attract or retain key personnel; a separation may adversely impact the effectiveness of development and commercialization efforts by us and our partners; our businesses may be disrupted as a result of the announcement or pendency of the separation; the COVID-19 pandemic and resulting economic conditions will have a greater impact on the company's operations and plans than anticipated; that our amended collaboration with BMS will not continue or be successful; that preliminary positive efficacy and safety results from our prior and ongoing clinical trials will not continue or be repeated in our ongoing or future clinical trials; the risk that our plans for submitting a BLA for LentiGlobin for SCD may be delayed if the FDA does not accept our comparability plans for the use of the suspension-based manufacturing process for LVV; the risk that the BLA for ide-cel is approved by the FDA in the timeline we expect, or at all; the risk of cessation or delay of any of the ongoing development activities, including clinical studies, and commercialization efforts due to delays from the COVID-19 pandemic's impact on healthcare systems; the risk that the current or planned clinical trials of our product candidates will be insufficient to support regulatory submissions or marketing approval in the United States and European Union; the risk that regulatory authorities will require

additional information regarding our product candidates, resulting in delay to our anticipated timelines for regulatory submissions, including our applications for marketing approval; the risk that we will encounter challenges in the commercial launch of ZYNTEGLO in the European Union, including in managing our complex supply chain for the delivery of drug product, or in obtaining sufficient coverage or reimbursement for our products; 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.

Investors & Media

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bluebird bio, Inc.
Condensed Consolidated Statements of Operations
(in thousands, except per share data)
(unaudited)

	For the three months ended December 31,		For the twelve months ended December 31,	
	2020	**2019**	**2020**	**2019**
Revenue:				
Service revenue	$ 5,522	$ 5,827	$ 114,064	$ 30,729
Collaborative arrangement revenue	1,196	1,332	115,594	5,740
Royalty and other revenue	3,990	2,838	21,076	8,205
Total revenues	10,708	9,997	250,734	44,674
Operating expenses:				
Research and development	137,094	161,821	587,956	582,413
Selling, general and administrative	76,974	76,202	286,896	271,362
Cost of royalty and other revenue	1,499	1,073	5,396	2,978
Change in fair value of contingent consideration	(877)	1,435	(6,468)	2,747
Total operating expenses	214,690	240,531	873,780	859,500
Loss from operations	(203,982)	(230,534)	(623,046)	(814,826)
Interest income, net	1,281	6,855	11,539	34,761
Other income (expense), net	3,080	535	(6,502)	(10,088)
Loss before income taxes	(199,621)	(223,144)	(618,009)	(790,153)
Income tax (expense) benefit	(253)	(203)	(686)	545
Net loss	$ (199,874)	$ (223,347)	$ (618,695)	$ (789,608)
Net loss per share - basic and diluted:	$ (3.01)	$ (4.04)	$ (9.95)	$ (14.31)
Weighted-average number of common shares used in computing net loss per share - basic and diluted:	66,395	55,344	62,178	55,191

bluebird bio, Inc.
Condensed Consolidated Balance Sheet Data
(in thousands, except per share data)
(unaudited)

	As of December 31, 2020	As of December 31, 2019
Cash, cash equivalents and marketable securities	$ 1,274,142	$ 1,237,966
Total assets	$ 1,781,252	$ 1,727,424
Total liabilities	$ 426,196	$ 442,431
Total stockholders' equity	$ 1,355,056	$ 1,284,993