bluebird bio, Inc.(BLUE)

$97.47 (As of 01/16/20)

Price Target (6-12 Months): **$102.00**

Long Term: 6-12 Months	**Zacks Recommendation:** (Since: 02/18/19) Prior Recommendation: Underperform	**Neutral**
Short Term: 1-3 Months	**Zacks Rank:** (1-5)	**3-Hold**
	Zacks Style Scores:	VGM:F
	Value: F | Growth: F | Momentum: C	

Summary

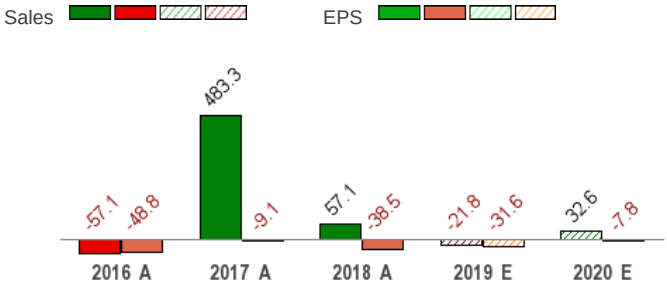
bluebird incurred a wider-than-expected loss and missed sales estimates in the third quarter of 2019. The company has an impressive pipeline of gene therapies for genetic diseases and cancer. The conditional approval of Zynteglo for patients aged 12 years or above with transfusion-dependent ?-thalassemia in Europe is a significant boost for the company. Per the company, Zynteglo is the first gene therapy approved for this indication. We are also positive about bluebird's collaboration with Regeneron, as this provides the former with funds. Additionally, the company is developing CAR T therapies for myeloma in collaboration with Celgene and the successful development of the candidates will benefit the company in the long run. However, competition is stiffening in this space. Shares have underperformed the industry year to date.

Price, Consensus & Surprise

Data Overview

52 Week High-Low	**$163.43 - $71.42**
20 Day Average Volume (sh)	**883,604**
Market Cap	**$5.4 B**
YTD Price Change	**11.1%**
Beta	**2.37**
Dividend / Div Yld	**$0.00 / 0.0%**
Industry	**Medical - Biomedical and Genetics**
Zacks Industry Rank	**Top 35% (90 out of 254)**
Last EPS Surprise	**-5.7%**
Last Sales Surprise	**-15.9%**
EPS F1 Est- 4 week change	**0.0%**
Expected Report Date	**02/20/2020**
Earnings ESP	**0.0%**
P/E TTM	**NA**
P/E F1	**NA**
PEG F1	**NA**
P/S TTM	**100.0**

Sales and EPS Growth Rates (Y/Y %)



Sales Estimates (millions of $)

	Q1	Q2	Q3	Q4	Annual*
2020	10 E	13 E	15 E	20 E	57 E
2019	12 A	13 A	9 A	10 E	43 E
2018	16 A	8 A	12 A	19 A	55 A

EPS Estimates

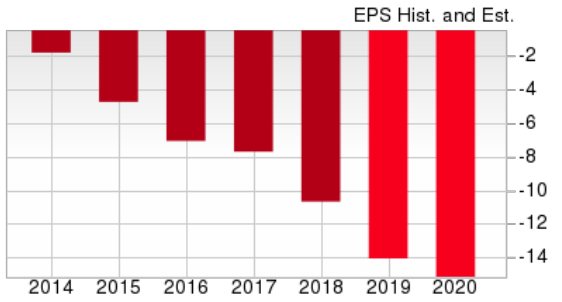
	Q1	Q2	Q3	Q4	Annual*
2020	-$3.69 E	-$3.74 E	-$3.81 E	-$3.66 E	-$15.15 E
2019	-$2.99 A	-$3.55 A	-$3.73 A	-$3.79 E	-$14.06 E
2018	-$2.31 A	-$2.91 A	-$2.73 A	-$2.72 A	-$10.68 A

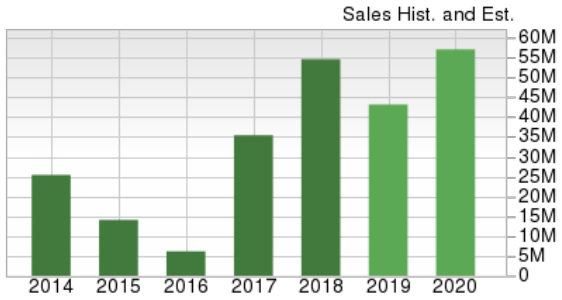
*Quarterly figures may not add up to annual.

The data in the charts and tables, including the Zacks Consensus EPS and Sales estimates, is as of 01/16/2020. The reports text is as of 01/17/2020.

Overview

Cambridge, MA based bluebird bio, Inc. is a clinical-stage biotechnology, which is focused on developing gene therapies for severe genetic diseases and cancer. The company has developed a deep pipeline using its lentiviral-based gene therapies, T cell immunotherapy expertise and gene editing capabilities to treat severe genetic diseases and cancer as well.





The pipeline for severe genetic diseases, include Zynteglo product candidate for the treatment of transfusion-dependent ?-thalassemia (TDT) and severe sickle cell disease (SCD), and Lenti-D product candidate for the treatment of cerebral adrenoleukodystrophy (CALD). Zynteglo (formerly LentiGlobin) was recently approved in Europe for TDT, making it the first gene therapy approved for this indication. The gene therapy has gained a lot of attention of late. The objective of the therapy is to correct the underlying genetic defect, which is the cause of the disease, rather than offering treatments that only address the symptoms. The gene therapy has a huge advantage over other therapies, especially for diseases like SCD and cancer, which have no cure.

The oncology programs are focused on developing novel T cell-based immunotherapies, including chimeric antigen receptor (CAR) and T cell receptor (TCR) T cell therapies. The oncology pipeline includes CAR T cell product candidates — bb2121 and bb21217 — for the treatment of multiple myeloma. The company is co-developing and co-promoting bb2121 in the United States with Celgene Corporation. Celgene also owns the development and commercialization rights for bb2121 product candidate in the United States, while bluebird has an option to elect to co-develop and co-promote bb21217 within the United States.

With no approved products in its kitty, the company does not generate any revenues from the sale of products. However, the company derived revenues from collaboration arrangements, out-licensing arrangements including royalties on net sales of products to licensees or sublicensees, research fees, and grant revenues.

Revenues in 2018 came in at $54.6 million, up from $35.4 million in 2017.

Reasons To Buy:

> bluebird's efforts to develop its pipeline is impressive. The collaboration with Celgene is a big positive for the company.

- **Potential of Gene Therapy**: The gene therapy has gained a lot of attention of late. The objective of the therapy is to correct the underlying genetic defect, which is the cause of the disease, rather than offering treatments that only address the symptoms. The gene therapy has a huge advantage over other therapies, especially for diseases like SCD and cancer, which have no cure.

  The company acquired Washington based Pregenen, a privately-held biotechnology company in June 2014, thereby obtaining rights to Pregenen's gene editing technology platform and cell-signaling technology. bluebird has integrated these technologies and research team, and expanded its research efforts. The company is focused on utilizing homing endonuclease and megaTAL gene editing technologies in a variety of potential applications and disease areas, including for oncology and hematology.

- **Zynteglo Products Hold Promise**: bluebird's efforts to develop its pipeline is impressive. The company is developing its Zynteglo product candidate for different genotypes of TDT, and SCD. The company is currently conducting five clinical studies for its Zynteglo product candidate: first, a phase I/II study (Northstar Study (HGB-204)) in the United States, Australia, and Thailand for the treatment of subjects with TDT; second, a multi-site, international, phase III study (Northstar-2 Study (HGB-207)) for the treatment of patients with TDT and non-?0/?0 genotypes; third, a multi-site, international, phase III study (Northstar 3 Study (HGB-212)) for the treatment of subjects with TDT and a ?0/?0 genotype; fourth, a single-center phase I/II study in France for the treatment of subjects with TDT or severe SCD (HGB-205); and finally, a multi-site phase I study in the United States for the treatment of subjects with severe SCD (HGB-206). The company presented positive new data from Northstar (HGB-204) and Northstar-2 (HGB-207) studies. In December 2019, bluebird announced new data from ongoing studies of LentiGlobin gene therapy for ?-thalassemia in pediatric, adolescent and adult patients who have TDT, including results from the phase III Northstar-3 (HGB-212) study in patients with a ?0/?0 genotype or IVS-I-110 mutation, and the phase III Northstar-2 (HGB-207) study in patients who do not have a ?0/?0 genotype. The company also announced new data from its ongoing phase I/II HGB-206 study.

  In June 2019, the European Commission (EC) granted conditional marketing authorization to Zynteglo (autologous CD34+ cells encoding ?A-T87Q-globin gene) in patients aged 12 years or older with transfusion-dependent ?-thalassemia (TDT), who do not have a ?0/?0 genotype and for whom hematopoietic stem cell (HSC) transplantation is appropriate but a human leukocyte antigen (HLA)-matched related HSC donor is not available. The candidate is expected to treat the first commercial patient in early 2020. In janury 2020, the company launhed Zynteglo in Germany.

  The company expects to initiate a rolling biologics licensing application submission to the FDA for Zynteglo in patients with TDT and non-?0/?0 genotypes by the end of 2019.

  The company is also developing its Lenti-D product candidate for CALD, a rare, hereditary neurological disorder, which can be often fatal. A multi-site, international phase II/III study, Starbeam Study (ALD-102), is ongoing for the same. The initial cohort of the study treated seventeen subjects with Lenti-D product candidate and the company is also enrolling up to thirteen additional subjects in an expansion cohort of this study for a total target enrollment of thirty subjects. An observational study of subjects with CALD, treated by allogeneic hematopoietic stem-cell transplant referred to as the ALD-103 study, is also being conducted. Assuming successful completion, the company expects that the results from the Starbeam study could potentially form the basis of a Biologics License Application (BLA) and a Marketing Authorization Application (MAA) submission in the United States and European Union, respectively, shortly.

  The company also initiated a phase III HGB-210 study of LentoGlobin in patients with SCD.

  The FDA granted Breakthrough Therapy designation to Lenti-D for the treatment of patients with CALD which should expedite the development and review of the therapy. Lenti-D also enjoys orphan drug status in the Unites States and Europe. It was also granted Rare Pediatric Disease designation by the FDA for the treatment of adrenoleukodystrophy (ALD).

- **Collaboration With Celgene a Big Positive**: bluebird's collaboration agreement with Celgene is a big positive for the company, given the latter's expertise in the biotech space. Most importantly, the agreement provides bluebird with funds as upfront payments. In 2013, both the companies entered into a collaboration agreement, whereby Celgene will discover, develop and commercialize potentially disease-altering gene therapies in oncology. The collaboration is primarily focused on applying gene therapy technology to genetically modify a patient's own T cells, known as chimeric antigen receptor or CAR T cells, to target and destroy cancer cells. Both the companies also entered into a licensing deal, whereby bluebird obtained a sublicense to certain intellectual property from Celgene, originating under Celgene's license from Baylor College of Medicine, for use in the collaboration. Per the terms, bluebird obtained an upfront payment of $75.0 million.

  The collaboration agreement was amended in June 2015. Per the amended terms, both the companies have narrowed the focus of the collaboration exclusively to anti- B-cell maturation antigen (BCMA) product candidates for a new three-year term ending in June 2018. bluebird received an upfront, one-time, non-refundable, non-creditable payment of $25.0 million upon the amendment.

  In February 2016, Celgene exercised its option to obtain an exclusive worldwide license to develop and commercialize bb2121, the first product candidate under the amended agreement. Celgene is responsible for development and related funding of bb2121, after the substantial completion of the on-going trial. Thereafter, in March 2018, bluebird elected to co-develop and co-promote bb2121 within the United States. bluebird will have an equal share in all profits and losses, relating to developing, commercializing and manufacturing bb2121 within the country. The company may receive up to $70.0 million in development milestone payments for the first approved indication along with tiered royalty payments.

- **Anti - BCMA Candidates Hold Potential**: The two anti-BCMA candidates, bb2121 and bb21217, hold promise. bb2121 is the lead candidate in this program. The company is conducting a multi-site phase I study in the United States on bb2121, for the treatment of subjects with relapsed/refractory multiple myeloma (CRB-401). The FDA has granted Breakthrough Therapy designation and the EMA has granted PRIME eligibility to bb2121 for relapsed/refractory multiple myeloma.

In November 2018, bluebird and Celgene completed enrollment for the KarMMa pivotal study of bb2121, the lead investigational anti-BCMA CAR T cell therapy candidate, to treat patients with relapsed/refractory multiple myeloma. The open-label, single-arm, multi-center phase II study is evaluating the efficacy and safety of bb2121 in patients with relapsed/refractory multiple myeloma. Celgene and bluebird anticipate potential approval of bb2121 for the same indication in the second half of 2020.

bluebird and partner Celgene announced the completion of enrollment in the KarMMa pivotal study on the companies' lead investigational anti-B-cell maturation antigen (BCMA) chimeric antigen receptor (CAR) T cell therapy candidate, bb2121 for patients with relapsed and refractory multiple myeloma. KarMMa is a phase II study evaluating the efficacy and safety of bb2121 in patients with relapsed and refractory multiple myeloma.

The company also initiated a phase I study on bb21217, the second anti-BCMA product candidate in September 2017. Celgene has exercised its option to obtain an exclusive worldwide license to develop and commercialize bb21217. Hence, bluebird plans to exercise its option to co-develop and co-promote bb21217 within the United States. The candidate also enjoys orphan drug status in the United States.

▲ **Collaborations With Other Big Pharma Companies Bode Well**: Apart from Celgene, bluebird has collaborations with other big companies as well, which provide it with funds as upfront payments. In April 2017, the company entered into a license agreement with GlaxoSmithKline Intellectual Property Development Limited, whereby Glaxo licensed certain patent rights of the company, related to lentiviral vector technology to develop and commercialize gene therapies for two rare genetic diseases — Wiscott-Aldrich syndrome and metachromatic leukodystrophy. In exchange, the company received an upfront payment and is entitled to milestone payments as well. Concurrently, the company also entered into a license agreement with Novartis Pharma, whereby the latter licensed certain patent rights of bluebird, related to lentiviral vector technology to develop and commercialize CAR T therapies for oncology, including Novartis' approved CAR-T therapy, Kymriah, in lieu of an upfront payment and potential milestone payments. Regeneron will also make a $100 million investment in the company's common stock at a price of $238.10 per share. Both companies have collaborated on applying their respective technology platforms to the discovery, development and commercialization of novel immune cell therapies for cancer. Regeneron will leverage its VelociSuite platform technologies for the discovery and characterization of fully human antibodies as well as T cell receptors (TCRs) directed against tumor-specific proteins and peptides while bluebird bio will contribute its field-leading expertise in gene transfer and cell therapy. Regeneron and bluebird have jointly selected six initial targets and will equally share the costs of research and development up to the point of submitting an Investigational New Drug (IND) application. Both companies might select additional targets over the five-year research collaboration term. When an IND is submitted for a potential cell therapy product, Regeneron will have the right to opt-in to a co-development/co-commercialization arrangement for certain collaboration targets, with 50/50 cost and profit sharing. If Regeneron decides not to opt-in, it will be entitled to receive payments and royalties from bluebird bio on any potential resulting products.

In January 2019, bluebird and Inhibrx, Inc. entered into an exclusive license agreement to research, develop and commercialize CAR T cell therapies using Inhibrx's proprietary single domain antibody (sdAb) platform for multiple cancer targets.

In October 2019, bluebird bio and Novo Nordisk announced a research collaboration to jointly develop next-generation vivo genome editing treatments for genetic diseases, including hemophilia. During the three-year research collaboration, bluebird and Novo Nordisk will focus on identifying a development gene therapy candidate with the ambition of offering people with hemophilia A a lifetime free of factor replacement therapy.

Reasons To Sell:

bluebird is highly dependent on its partners for funding requirements. R&D spend is expected to increase significantly.

- **Share Price Performance**: bluebird's shares have underperformed the industry in the year so far.
- **Intense Competition**: The company faces potential competition from many larger and better-funded pharmaceutical and biotechnology companies. Chronic blood transfusions are the current standard of care in the ?-thalassemia market but iron chelation therapy is often required by these patients. Novartis and ApoPharma Inc. provide the leading iron chelation therapy and are striving to further innovate the existing therapies. Moreover, Acceleron Pharma, Inc., in collaboration with Celgene, has developed Reblozyl (luspatercept–aamt) which received approval by the FDA in November 2019, for the treatment of anemia (lack of red blood cells) in adult patients with beta thalassemia who require regular red blood cell (RBC) transfusions. For sickle cell disease, Emmaus Life Sciences, Inc. recently received FDA approval for Endari (L-glutamine) and has launched the same. In November 2019, Novartis received approval for Adakveo (crizanlizumab) to reduce the frequency of vaso-occlusive crises (VOCs), or pain crises, in adult and pediatric patients aged 16 years and older with SCD. In the relapsed/refractory multiple myeloma space, an anti-BCMA CAR T cell therapy is currently in a single-center phase I study by the University of Pennsylvania, in collaboration with Novartis. Gilead Sciences and Celgene also have such candidates in their pipeline.
- **Dependence on Partners and Funding Requirement**: The company is highly dependent on Celgene for the development of its candidates. Termination of the agreement with Celgene will have a negative impact on the company's growth prospects. Moreover, R&D spend is expected to increase significantly as it advances its pipeline candidates to late-stage studies.
- **Pipeline Candidates Still Far from Commercialization**: Although the company is progressing well with its pipeline, there is still a lot of time for any of the candidates to get regulatory approval. Unfavorable outcome from any of the development programs could adversely affect the company's prospects as well as the stock.

Last Earnings Report

bluebird's Loss Wider Than Expected, Revenues Miss

Quarter Ending	09/2019
Report Date	**Oct 31, 2019**
Sales Surprise	**-15.94%**
EPS Surprise	**-5.67%**
Quarterly EPS	**-3.73**
Annual EPS (TTM)	**-12.99**

bluebird reported a loss of $3.73 per share in the third quarter of 2019, wider than the Zacks Consensus Estimate of a loss of $3.53 and the year-ago quarter's loss of $2.73.

Revenues of $8.91 million missed the Zacks Consensus Estimate of $11 million. Revenues were down from $11.5 million in the year-ago quarter. The decrease was mainly attributable to lower collaboration revenues under the company's arrangement with Celgene Corp.

Quarter in Detail

R&D expenses escalated to $151.4 million in the third quarter of 2019 from $116.7 million a year ago, due to costs incurred by the company to advance and expand the pipeline.

G&A expenses of $66.3 million were up 48.8% from the year-ago quarter,due to the overall growth of the pipeline and commercial-readiness activities.

Pipeline Development

In October, bluebird announced that the European Medicines Agency (EMA) approved the refined commercial drug product manufacturing specifications for Zynteglo, a one-time gene therapy for patients 12 years or older with transfusion-dependent ?-thalassemia (TDT), who do not have a ?0/?0 genotype and for whom hematopoietic stem cell (HSC) transplantation is appropriate but a human leukocyte antigen (HLA)-matched related HSC donor is not available. With this update, apceth is in the final stages of preparations to manufacture Zynteglo for commercial use. The company continues to proceed with discussions on value-based payment agreements and Qualified Treatment Center contracts, and expects to treat the first commercial patient in early 2020.

In August, Fred Hutchinson Cancer Research Center infused the first patient in their proof-of-concept phase I/II single-arm study evaluating Merkel Cell Polyomavirus (MCPyV) TCR-engineered autologous T cells in combination with avelumab (anti-PDL1) for the treatment of MCC.

In October, bluebird and Novo Nordisk announced a research collaboration to jointly develop next-generation vivo genome editing treatments for genetic diseases, including hemophilia. During the three-year research collaboration, bluebird and Novo Nordisk will focus on identifying a development gene therapy candidate with the ambition of offering people with hemophilia A a lifetime free of factor replacement therapy.

Recent News

Announces Zynteglo Launch in Germany –Jan 13

bluebird announced the launch in Germany of Zynteglo (autologous CD34+ cells encoding ?A-T87Q-globin gene), a one-time gene therapy for patients 12 years and older with transfusion-dependent ?-thalassemia (TDT) who do not have a ?0/?0 genotype, for whom hematopoietic stem cell (HSC) transplantation is appropriate but a human leukocyte antigen (HLA)-matched related HSC donor is not available. This is the first time Zynteglo is commercially available.

Further, bluebird also entered into value-based payment agreements with multiple statutory health insurances in Germany to help ensure patients and their healthcare providers have access to Zynteglo and that payers only pay if the therapy delivers on its promise. bluebird's proposed innovative model is limited to five payments made in equal installments. An initial payment is made at the time of infusion. The four additional annual payments are only made if no transfusions for TDT are required for the patient.

bluebird bio and Bristol-Myers Squibb Present Updated Data from CAR T Cell Therapy bb21217-Dec 9

bluebird and Bristol-Myers Squibb announced updated safety and efficacy results from the ongoing phase I study (CRB-402) of bb21217, an investigational BCMA-targeted chimeric antigen receptor (CAR) T cell therapy being studied in patients with relapsed/refractory multiple myeloma (R/RMM).

The data showed safety profile was consistent with known toxicities of CAR T therapies.

CAR T persistence observed in 8/10 evaluable responders at Month 6 and 2/2 evaluable responders at month 18.

Presents New Data Showing Long-Term Transfusion Independence and Safety for LentiGlobin Gene Therapy-Dec 9

bluebird announced new data from ongoing studies of LentiGlobin gene therapy for ?-thalassemia (betibeglogene autotemcel) in pediatric, adolescent and adult patients who have transfusion-dependent ?-thalassemia (TDT), including results from the phase III Northstar-3 (HGB-212) study in patients with a ?0/?0 genotype or IVS-I-110 mutation, and the phase III Northstar-2 (HGB-207) study in patients who do not have a ?0/?0 genotype.

The data showed that more than four years of durable transfusion independence (TI), stable total hemoglobin (Hb) levels and reduced liver iron concentrations in completed phase I/II Northstar (HGB-204) study in patients who do not have a ?0/?0 genotype

Ninety percent of evaluable patients who do not have a ?0/?0 genotype achieved TI, with median average total Hb levels of 12.2 g/dL in phase III Northstar-2 (HGB-207) study

In ongoing phase III Northstar-3 (HGB-212) study in patients with ?0/?0 genotype or IVS-I-110 mutation, the two patients evaluable for TI achieved it with Hb levels of 13.2 g/dL and 10.4 g/dL at last visit

Nine of 11 patients with at least six months of follow-up in HGB-212 have not had a transfusion for at least three months

Presents New Data from Ongoing HGB-206 Study of LentiGlobin Gene Therapy –Dec 7

bluebird bio, announced new data from its ongoing phase I/II HGB-206 study of investigational LentiGlobin gene therapy for sickle cell disease (SCD), including additional patients treated in the study and updated data for those previously reported. These data, as well as results from exploratory assays designed to assess the relationship between drug product characteristics and red blood cell physiology.

Treatment with LentiGlobin for SCD reduced key markers of hemolysis, including reticulocyte counts, lactate dehydrogenase (LDH) levels and total bilirubin concentration, which suggests that treatment is improving biological markers of the disease.

Among the nine patients with at least six months of follow-up who had four or more vaso-occlusive crises (VOC) or acute chest syndrome (ACS) events in the two years prior to treatment, there was a 99% reduction in annualized rate of VOC and ACS. There were no reports of ACS or serious VOC at up to 21 months post-treatment in these patients. As previously reported, there was one non-serious Grade 2 VOC was observed in a patient approximately 3.5 months post-LentiGlobin for SCD treatment.

Group C patients at six months post-treatment produced consistent median levels of gene therapy-derived anti-sickling hemoglobin (HbAT87Q) ranging from 44 – 59%, reducing the median level of abnormal sickle hemoglobin (HbS).

Continued improvement in key markers of hemolysis in Group C patients demonstrates the potential of LentiGlobin for SCD to modify the underlying pathophysiology of sickle cell disease

Reports Positive Top-Line Data on Myeloma Drug-Dec 6

bluebird and partner Bristol-Myers Squibb Company announced positive top-line results from a phase II study on their lead investigational BCMA-targeted chimeric antigen receptor (CAR) T-cell therapy candidate, idecabtagene vicleucel (ide-cel; bb2121).

The phase II KarMMa study evaluated the efficacy and safety of idecabtagene vicleucel in patients with relapsed and refractory multiple myeloma. The study met its primary endpoint and key secondary endpoint demonstrating deep and durable responses in a heavily pre-treated multiple myeloma patient population.

In the study, all treated patients were exposed to at least three prior therapies, including an immunomodulatory (IMiD) agent, a proteasome inhibitor (PI) and an anti-CD38 antibody, and all were refractory to their last regimen. Of these patients, 94% were refractory to an anti-CD38 antibody and 84% were triple refractory to an IMiD agent, PI, and anti-CD38 antibody.

Overall, the safety results were consistent with those observed in the phase I CRB-401 study, which evaluated the preliminary safety and efficacy of ide-cel.

The company is preparing for the submission of these data to Health Authorities for the proposed initial registration of ide-cel as a first-in-class B-cell maturation antigen (BCMA)-targeted CAR T-cell therapy.

Ide-cel is being developed as part of a co-development, co-promotion and profit share agreement between bluebird and Bristol-Myers.

In November 2017, ide-cel was granted the Breakthrough Therapy designation by the FDA and PRIority Medicines (PRIME) eligibility by the European Medicines Agency based on preliminary clinical data from the phase I CRB-401 study.

Inks Deal With Forty Seven to Study Antibody Conditioning Regimen-Nov 12

bluebird and Forty Seven, Inc. (FTSV) inked a deal to pursue clinical proof-of-concept for the latter's novel antibody-based conditioning regimen, FSI-174 (anti-cKIT antibody) plus magrolimab (anti-CD47 antibody), with bluebird's ex vivo lentiviral vector hematopoietic stem cell (LVV HSC) gene therapy platform. The collaboration will focus on a conditioning approach aimed to reduce toxicity and initially target diseases that have the potential to be corrected with transplantation of autologous gene-modified blood-forming stem cells. If successful, the new conditioning regimen could allow more patients to undergo gene therapy.

bluebird announced that the European Medicines Agency (EMA) approved the refined commercial drug product manufacturing specifications for Zynteglo (autologous CD34+ cells encoding ?$^{A\text{-}T87Q}$-globin gene), a one-time gene therapy for patients 12 years and older with transfusion-dependent ?-thalassemia (TDT), who do not have a ?^{0}/?^{0} genotype and for whom hematopoietic stem cell (HSC) transplantation is appropriate but a human leukocyte antigen (HLA)-matched related HSC donor is not available.

The refined commercial drug product specifications support the efficacy and safety profile of Zynteglo and will give patients the best opportunity for meaningful outcomes consistent with the results that were foundational to the conditional marketing authorization in the European Union.

Inks Genome Editing Research Deal With Novo Nordisk – Oct 9

bluebird announced that it has entered a research collaboration agreement with Denmark's Novo Nordisk A/S for jointly developing next-generation in vivo genome editing therapies for addressing genetic diseases including hemophilia.

The research collaboration will span over a period of three years and focus on developing a gene therapy candidate offering hemophilia A patients with a lifetime free of factor replacement therapy. Financial terms of the deal were not disclosed.

The deal will leverage bluebird's proprietary mRNA-based megaTAL gene editing technology with Novo Nordisk's hemophilia portfolio. It will initially focus on correcting FVIII-clotting factor deficiency with the potential to explore additional therapies.

Presents Updated Data from Phase II/III Study of Lenti-D Gene Therapy-Sep 18

bluebird announced updated results from the clinical development program for its investigational Lenti-D gene therapy in patients with cerebral adrenoleukodystrophy (CALD) at the 13th European Pediatric Neurology Society (EPNS) Congress in Athens, Greece.

The data from phase II/III Starbeam (ALD-102) study showed that all boys with CALD who were treated with Lenti-D and were free of major functional disabilities (MFDs) at 24 months continued to be MFD-free. There were no reports of graft failure or treatment-related mortality, and adverse events were generally consistent with myeloablative conditioning. These results support the potential of Lenti-D as a treatment for CALD.

The company also presented updated data from the ongoing observational study (ALD-103) of allogeneic hematopoietic stem cell transplant (allo-HSCT) in boys 17 years of age and under with CALD. Updated results showed that early treatment with allo-HSCT provides improved overall and MFD-free survival for patients with CALD irrespective of the stage of early disease. In the all early disease cohort at 24 months post-allo-HSCT, 77.2% of patients achieved MFD-free survival and 89.1% achieved overall survival compared to 35% and 52.5%, respectively, in the advanced disease cohort at 24 months post-allo-HSCT.

Valuation

bluebird's shares are down 23.0% over the trailing 12-month period. Over the past year, the Zacks sub-industry is down 2.1% and the sector is up 5.2%.

The S&P 500 index is up 23.8% in the past year.

The stock is currently trading at 3.67X trailing 12-month book per share, which compares to 3.92X for the Zacks sub-industry, 4.58X for the Zacks sector and 4.50X for the S&P 500 index.

Over the past five years, the stock has traded as high as 13.42X and as low as 1.66X, with a 5-year median of 3.94X. Our Neutral recommendation indicates that the stock will perform in-line with the market. Our $102.00 price target reflects 3.84X trailing 12-month book per share.

Industry Analysis **Zacks Industry Rank:** Top 35% (90 out of 254)

Top Peers

Sangamo Therapeutics, Inc. **(SGMO)**	Outperform
Amgen Inc. **(AMGN)**	Neutral
Bellicum Pharmaceuticals, Inc. **(BLCM)**	Neutral
CRISPR Therapeutics AG **(CRSP)**	Neutral
Emmaus Life Sciences, Inc. **(EMMA)**	Neutral
Gilead Sciences, Inc. **(GILD)**	Neutral
Novartis AG **(NVS)**	Neutral
Acceleron Pharma Inc. **(XLRN)**	Neutral

Industry Comparison Industry: Medical - Biomedical And Genetics

Industry Peers

	BLUE Neutral	X Industry	S&P 500	AMGN Neutral	NVS Neutral	SGMO Outperform
VGM Score	F	-	-	B	B	F
Market Cap	5.39 B	189.13 M	24.61 B	142.90 B	217.95 B	1.03 B
# of Analysts	12	3	13	10	5	5
Dividend Yield	0.00%	0.00%	1.74%	2.41%	1.94%	0.00%
Value Score	F	-	-	B	B	D
Cash/Price	0.24	0.23	0.04	0.15	0.04	0.37
EV/EBITDA	-8.15	-3.74	14.24	11.63	10.66	-9.80
PEG Ratio	NA	1.67	2.07	2.08	1.96	NA
Price/Book (P/B)	3.66	4.01	3.38	13.08	4.14	2.44
Price/Cash Flow (P/CF)	NA	13.39	13.75	13.30	11.67	NA
P/E (F1)	NA	29.35	19.09	15.06	16.77	NA
Price/Sales (P/S)	100.02	13.78	2.68	6.11	4.51	13.78
Earnings Yield	-15.54%	-15.32%	5.24%	6.64%	5.96%	-12.77%
Debt/Equity	0.12	0.02	0.72	2.54	0.42	0.10
Cash Flow ($/share)	-9.85	-1.07	6.94	18.08	8.15	-0.70
Growth Score	F	-	-	D	C	F
Hist. EPS Growth (3-5 yrs)	NA%	16.50%	10.56%	11.35%	0.15%	NA
Proj. EPS Growth (F1/F0)	-7.77%	7.26%	7.57%	8.95%	8.41%	6.15%
Curr. Cash Flow Growth	67.17%	20.25%	14.73%	2.84%	6.18%	33.61%
Hist. Cash Flow Growth (3-5 yrs)	NA%	8.03%	9.00%	10.23%	2.20%	NA
Current Ratio	6.22	5.12	1.24	2.89	0.95	4.77
Debt/Capital	10.68%	3.91%	42.99%	71.74%	29.33%	8.83%
Net Margin	-1,326.56%	-197.98%	11.14%	34.48%	24.43%	-159.12%
Return on Equity	-42.29%	-64.11%	17.16%	80.26%	20.86%	-30.33%
Sales/Assets	0.03	0.20	0.55	0.38	0.37	0.12
Proj. Sales Growth (F1/F0)	32.18%	17.19%	4.16%	9.09%	2.86%	71.90%
Momentum Score	C	-	-	A	B	D
Daily Price Chg	4.00%	0.70%	0.89%	-0.50%	0.53%	0.63%
1 Week Price Chg	4.20%	1.77%	0.39%	-0.09%	-0.98%	1.64%
4 Week Price Chg	5.50%	4.91%	2.65%	-0.55%	0.88%	2.14%
12 Week Price Chg	20.56%	18.13%	7.55%	18.44%	9.01%	2.02%
52 Week Price Chg	-22.95%	-4.04%	22.12%	19.11%	7.30%	-21.66%
20 Day Average Volume	883,604	229,523	1,536,375	1,687,787	1,073,944	1,414,894
(F1) EPS Est 1 week change	0.00%	0.00%	0.00%	0.00%	0.00%	0.00%
(F1) EPS Est 4 week change	0.00%	0.00%	0.00%	0.37%	-0.11%	0.00%
(F1) EPS Est 12 week change	-8.83%	0.34%	-0.40%	3.28%	-1.60%	9.60%
(Q1) EPS Est Mthly Chg	0.00%	0.00%	0.00%	-0.21%	NA	0.00%

Zacks Stock Rating System

We offer two rating systems that take into account investors' holding horizons: Zacks Rank and Zacks Recommendation. Each provides valuable insights into the future profitability of the stock and can be used separately or in combination with each other depending on your investment style.

Zacks Recommendation

The Zacks Recommendation aims to predict performance over the next 6 to 12 months. The foundation for the quantitatively determined Zacks Recommendation is trends in the company's estimate revisions and earnings outlook. The Zacks Recommendation is broken down into 3 Levels; Outperform, Neutral and Underperform. Unlike many Wall Street firms, we have an excellent balance between the number of Outperform and Neutral recommendations. Our team of 70 analysts are fully versed in the benefits of earnings estimate revisions and how that is harnessed through the Zacks quantitative rating system. But we have given our analysts the ability to override the Zacks Recommendation for the 1200 stocks that they follow. The reason for the analyst over-rides is that there are often factors such as valuation, industry conditions and management effectiveness that a trained investment professional can spot better than a quantitative model.

Zacks Rank

The Zacks Rank is our short-term rating system that is most effective over the one- to three-month holding horizon. The underlying driver for the quantitatively-determined Zacks Rank is the same as the Zacks Recommendation, and reflects trends in earnings estimate revisions.

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The Zacks Style Score is as a complementary indicator to the Zacks rating system, giving investors a way to focus on the highest rated stocks that best fit their own stock picking preferences.

Academic research has proven that stocks with the best Value, Growth and Momentum characteristics outperform the market. The Zacks Style Scores rate stocks on each of these individual styles and assigns a rating of A, B, C, D and F. We also produce the VGM Score (V for Value, G for Growth and M for Momentum), which combines the weighted average of the individual Style Scores into one score. This is perfectly suited for those who want their stocks to have the best scores across the board.

Value Score	F
Growth Score	F
Momentum Score	C
VGM Score	F

As an investor, you want to buy stocks with the highest probability of success. That means buying stocks with a Zacks Recommendation of Outperform, which also has a Style Score of an A or a B.

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