bluebird bio to Present Clinical Data on Lenti-D in CALD in Plenary Session at AAN 2016 Annual Meeting

Cambridge, Mass. March 3, 2016 – bluebird bio, Inc. (Nasdaq: BLUE), a clinical-stage company committed to developing potentially transformative gene therapies for severe genetic diseases and T cell-based immunotherapies for cancer, today announced that interim data from the ongoing Phase 2/3 Starbeam Study (ALD-102) for the treatment of cerebral adrenoleukodystrophy (CALD) will be presented in an oral presentation during the Clinical Trials plenary session on April 20, 2016 at the American Academy of Neurology (AAN) 2016 Annual Meeting. The meeting is being held April 15 – 21, 2016 in Vancouver, BC, Canada.

“The childhood form of cerebral adrenoleukodystrophy is a devastating neurodegenerative genetic disease that affects boys and is generally fatal if left untreated. Allogeneic hematopoietic stem cell transplant (allo-HSCT) is currently the only available effective therapy, but is potentially associated with serious safety risks, including graft rejection, graft-versus-host disease and transplant-related mortality. The Lenti-D gene therapy product candidate consists of a patient’s own modified hematopoietic stem cells and should avoid the problems of immune-incompatibility that can complicate allo-HSCT,” said David Davidson, M.D., chief medical officer, bluebird bio. “We are excited to report interim data from the Starbeam Study of Lenti-D at this year’s AAN meeting. While these data are still early, we are encouraged by the safety profile and the radiographic and neurologic results reported in the abstract. We look forward to presenting updated data and additional findings in Vancouver next month.”

The Starbeam (ALD-102) Study

The Starbeam Study is assessing the efficacy and safety of an investigational gene therapy approach in boys up to 17 years of age with CALD. It involves transplantation with a patient’s own stem cells, which are modified to contain a functioning copy of the ABCD1 gene. The copy of the gene is intended to allow the body to produce ALDP, a protein critical for the breakdown of very long chain fatty acids (VLCFAs). Buildup of VLCFAs in the central nervous system contributes to neurodegeneration in CALD.

The primary efficacy endpoint for the Starbeam Study is the proportion of subjects with no major functional disabilities (MFDs) at 24 months post infusion. MFDs are six specific components of the Neurological Function Score (NFS) that would have a profound negative impact on patients’ lives: loss of communication, cortical blindness, tube feeding, total incontinence, wheelchair dependence and complete loss of voluntary movement.

Subjects enrolled in the study have:

- Eligibility for allo-HSCT but with no matched sibling donor
• Confirmed early-stage, active CALD as indicated by gadolinium enhancement on MRI (an indicator of active neuroinflammation)
• Loes score (a method for quantifying demyelination and atrophy on brain MRI in patients with ALD) between 0.5 – 9.0
• Neurological Function Score (NFS, a scoring system assessing clinical deficits with 15 functional domains) of one or less

Abstract Highlights

• As of October 2015, 17 subjects with CALD have been treated with Lenti-D drug product with median follow-up time of nine months
• ALDP expression was observed in leukocytes of all subjects
• Reported adverse events were consistent with myeloablative conditioning. One serious adverse event with possible relation to drug product was reported (BK virus cystitis) and resolved with supportive care
• Integration site analyses have demonstrated polyclonal reconstitution in all subjects without evidence of clonal dominance
• In the 12 subjects with at least six months of follow-up:
  o No major functional disabilities and no NFS worsening were reported
  o The median change in Loes score was 1 (range 0-6)
  o All subjects experienced resolution of gadolinium enhancement

The abstract is now available online on the AAN Annual Meeting website. Information contained in the abstract reflects data available as of October 2015.

Title: Interim Results from a Phase 2/3 Study of the Efficacy and Safety of Ex Vivo Gene Therapy with Lentiviral Vector (Lenti-D) for Childhood Cerebral Adrenoleukodystrophy
Abstract Code: PL02.002
Session Name: Clinical Trials Plenary Session
Date: Wednesday, April 20, 2016
Session Time: 9:00 a.m. PT

About CALD

Also known as Lorenzo’s Oil disease, cerebral adrenoleukodystrophy (CALD) is a rare and fatal neurodegenerative disease that primarily affects young boys. CALD involves a breakdown of myelin, the protective sheath of the nerve cells in the brain that are responsible for thinking and muscle control. Symptoms usually occur in early childhood and progress rapidly if untreated.

About bluebird bio, Inc.

With its lentiviral-based gene therapies, T cell immunotherapy expertise and gene editing capabilities, bluebird bio has built an integrated product platform with broad potential application to severe genetic diseases and cancer. bluebird bio’s gene therapy clinical
programs include its Lenti-D™ product candidate, currently in a Phase 2/3 study, called the Starbeam Study, for the treatment of cerebral adrenoleukodystrophy, and its LentiGlobin® BB305 product candidate, currently in three clinical studies for the treatment of transfusion-dependent β-thalassemia, also known as β-thalassemia major, and severe sickle cell disease. bluebird bio’s oncology pipeline is built upon the company’s leadership in lentiviral gene delivery and T cell engineering, with a focus on developing novel T cell-based immunotherapies, including chimeric antigen receptor (CAR T) and T cell receptor (TCR) therapies. bluebird bio’s lead oncology program, bb2121, is an anti-BCMA CAR T program partnered with Celgene. bb2121 is currently being studied in a Phase 1 trial for the treatment of relapsed/refractory multiple myeloma. bluebird bio also has discovery research programs utilizing megaTALs/homing endonuclease gene editing technologies with the potential for use across the company’s pipeline.

bluebird bio has operations in Cambridge, Massachusetts; Seattle, Washington; and Paris, France.

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

**Forward-Looking Statements**

*This release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the clinical and market potential of the Company’s Lenti-D product candidate. Any forward-looking statements are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to, the risk 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 risk of a delay in the enrollment of patients in our clinical studies, and the risk that any one or more of our product candidates will not be successfully developed and 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 annual report on Form 10-K, as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and bluebird bio undertakes no duty to update this information unless required by law.*

**Contact:**
bluebird bio, Inc.
Manisha Pai, 617-245-2107
mpai@bluebirdbio.com
or
Pure Communications, Inc.
Dan Budwick, 973-271-6085
dan@purecommunicationsinc.com