



## **Mustang Bio Announces Presentation of MB-102 (CD123 CAR) Safety and Efficacy Data at AACR Special Conference on Tumor Immunology and Immunotherapy**

*Research partner City of Hope to present initial safety and efficacy Phase 1 data in relapsed or refractory acute myeloid leukemia and blastic plasmacytoid dendritic cell neoplasm*

**New York, NY – November 28, 2018** – Mustang Bio, Inc. (“Mustang”) (NASDAQ: MBIQ), a company focused on the development of novel immunotherapies based on proprietary chimeric antigen receptor engineered T cell (“CAR T”) technology and gene therapies for rare diseases, today announced that additional safety and efficacy Phase 1 data evaluating MB-102 (CD123 CAR) in relapsed or refractory acute myeloid leukemia (AML) and blastic plasmacytoid dendritic cell neoplasm (BPDCN) have been selected for an oral presentation at the AACR Special Conference on Tumor Immunology and Immunotherapy. The presentation will take place on November 30, 2018, in Miami Beach, Florida.

Martina Sersch, M.D., Ph.D., Chief Medical Officer of Mustang, said, “We are excited about the additional data demonstrating MB-102’s (CD123 CAR T) potential to treat patients with AML and BPDCN. Initial safety and tolerability data as well as preliminary data on efficacy are very promising in a population with high unmet medical need. Based on the Phase 1 data, we expect to submit an IND filing for MB-102 and look forward to initiating a multicenter Phase 1/2 clinical trial in 2019 in patients with AML, BPDCN and high-risk myelodysplastic syndrome.”

[Lihua Elizabeth Budde](#), M.D., Ph.D., assistant professor in the Department of Hematology & Hematopoietic Cell Transplantation at City of Hope and principal investigator for the Phase 1 trial, said, “There is increased expression of CD123 on AML blasts, leukemic stem cells and BPDCN cells compared to normal hematopoietic stem cells, and it is therefore a promising target for cellular immunotherapy. We remain encouraged by interim data showing MB-102’s potential to treat BPDCN and AML and continue to evaluate MB-102’s clinical benefits in our ongoing Phase 1 clinical trial.”

Details of the oral presentation are as follows:

**Title:** CD123CAR Displays Clinical Activity in Relapsed/Refractory (R/R) Acute Myeloid Leukemia (AML) and Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN): Safety and Efficacy Results from a Phase 1 Study

**Special Session:** Novel Targets, Pathways and Tools

**Date and Time:** Friday, November 30, 2018; 8:00 AM – 9:30 AM ET

**Location:** Loews Miami Beach Hotel

**Presenter:** Elizabeth Lihua Budde, M.D., Ph.D., City of Hope, Duarte, CA

For more information, please visit:

<https://www.aacr.org/Meetings/Pages/MeetingDetail.aspx?EventItemID=145&ItemID=733&DetailItemID=733>.

### **Key Efficacy and Safety Findings**

This single center, first-in-human Phase 1 dose-escalation clinical trial is evaluating the safety and activity of escalating doses of MB-102 in patients with relapsed or refractory AML (cohort 1) and BPDCN (cohort 2). Patients receive a single dose of MB-102 with an option for a second infusion if they continue to meet safety and eligibility criteria and still have CD123+ disease. To date, 18 patients have been enrolled and nine have been treated (seven with AML, two with BPDCN).

In the AML cohort, all seven patients had at least one prior allogeneic stem cell transplant and median of 4 (range: 4-10) prior lines of therapy. Two patients were treated at dose level 1 (50M CAR+ T). Trial investigators reported that one achieved a morphologic leukemic-free state (MLFS) that lasted 70 days. This patient received a second infusion three months later, with blast reduction from 77.9% to 0.9% (flow cytometry) after 35 days. Five patients received dose level 2 (200M CAR+ T). One patient achieved complete remission (CR) with incomplete count recovery at day 28, and one patient achieved MLFS that improved to CR at day 84. The remaining three patients had stable disease.

In the BPDCN cohort, two patients were treated with 100M CAR+ T and tolerated the treatment well, with no grade 3 or above treatment-related toxicities. One patient achieved CR with no evidence of disease in the bone marrow and skin at day 28.

Investigators found MB-102 infusions of up to 200M CAR T cells were safe. All toxicities observed to date were reversible and manageable. No patient developed grade 3 or above cytokine release syndrome or neurotoxicity. There were no treatment-related dose-limiting toxicities and no treatment-related cytopenias longer than 12 weeks. One patient with prior cutaneous graft-versus-host disease developed grade 1 rash five weeks after CAR T infusion, which resolved after clinical management. Peak of T cell expansion occurred within the first 14 days. Investigators did not observe any CD123-loss leukemic variants.

### **About Acute Myeloid Leukemia**

Acute myeloid leukemia (AML) is a cancer of the myeloid line of blood cells characterized by rapid growth of abnormal white blood cells that accumulate in the bone marrow. Although AML is rare, there are approximately 20,000 new cases in the U.S. each year and 10,000 deaths. Current treatment of relapsed or refractory AML with chemotherapy or hematopoietic stem cell transplantation is associated with low rates of complete response and considerable complications.

CD123 is overexpressed on AML blasts and leukemic stem cell-enriched cell subpopulations compared to normal hematopoietic stem cells and myeloid progenitors, making CD123 an attractive target for T cell-based adoptive immunotherapy.

### **About Blastic Plasmacytoid Dendritic Cell Neoplasm**

Blastic plasmacytoid dendritic cell neoplasm (BPDCN) is a rare and incurable blood cancer with a median survival of less than 18 months and no standard of care. High levels of CD123 expression is one of the diagnostic hallmarks of BPDCN, making CD123 an attractive target for T cell-based adoptive immunotherapy.

### **About MB-102 (CD123 CAR T)**

MB-102 (CD123 CAR T) is a CAR T cell therapy that is produced by engineering patient T cells to recognize and eliminate CD123-expressing tumors. CD123 is widely expressed on bone marrow cells of patients with MDS, as well as in hematologic malignancies including AML, B cell acute lymphoblastic leukemia, hairy cell leukemia, BPDCN, chronic myeloid leukemia and Hodgkin's lymphoma.

In the first-in-human clinical trial at City of Hope ([NCT02159495](https://clinicaltrials.gov/ct2/show/study/NCT02159495)), MB-102 has demonstrated complete responses at low doses in AML and BPDCN without dose-limiting toxicities, as reported at the American Society of Hematology annual meeting in December 2017. Dose escalation continues at City of Hope in both indications.

### **About Mustang Bio**

Mustang Bio, Inc. ("Mustang") is a clinical-stage biopharmaceutical company focused on the development and commercialization of a broad range of proprietary chimeric antigen receptor engineered T cell (CAR T) immunotherapies and gene therapies in areas of unmet need. Mustang aims to acquire rights to these technologies by licensing or otherwise acquiring an ownership interest, to fund research and development, and to outlicense or bring the technologies to market. Mustang has partnered with top medical institutions to advance the development of CAR T and CRISPR/Cas9-enhanced CAR T therapies across multiple cancers, as well as a lentiviral gene therapy for X-SCID. Mustang is registered under the Securities Exchange Act of 1934, as amended, and files periodic reports with the U.S. Securities and Exchange Commission. For more information, visit [www.mustangbio.com](http://www.mustangbio.com).

## **About City of Hope**

City of Hope is an independent research and treatment center for cancer, diabetes and other life-threatening diseases. Designated as one of only 49 comprehensive cancer centers, the highest recognition bestowed by the National Cancer Institute, City of Hope is also a founding member of the National Comprehensive Cancer Network, with research and treatment protocols that advance care throughout the world. City of Hope is located in Duarte, California, just northeast of Los Angeles, with [locations](#) throughout Southern California. It is ranked as one of "America's Best Hospitals" in cancer by *U.S. News & World Report*. Founded in 1913, City of Hope is a pioneer in the fields of [bone marrow transplantation](#), [diabetes](#) and [numerous breakthrough cancer drugs](#) based on technology developed at the institution. For more information about [City of Hope](#), follow us on [Facebook](#), [Twitter](#), [YouTube](#) or [Instagram](#).

## **Forward-Looking Statements**

This press release may contain "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934, each as amended. Such statements include, but are not limited to, any statements relating to our growth strategy and product development programs and any other statements that are not historical facts. Forward-looking statements are based on management's current expectations and are subject to risks and uncertainties that could negatively affect our business, operating results, financial condition and stock value. Factors that could cause actual results to differ materially from those currently anticipated include: risks relating to our growth strategy; our ability to obtain, perform under and maintain financing and strategic agreements and relationships; risks relating to the results of research and development activities; risks relating to the timing of starting and completing clinical trials; uncertainties relating to preclinical and clinical testing; our dependence on third-party suppliers; our ability to attract, integrate and retain key personnel; the early stage of products under development; our need for substantial additional funds; government regulation; patent and intellectual property matters; competition; as well as other risks described in our SEC filings. We expressly disclaim any obligation or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein to reflect any change in our expectations or any changes in events, conditions or circumstances on which any such statement is based, except as required by law.

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