



Updated Data from Phase 1/2 Open-Label Study of BCMA-Directed CAR-T Cell Therapy LCAR-B38M Show Tolerable Safety Profile, High Overall Response and MRD Negative Rate in Treatment of Patients with Advanced Relapsed or Refractory Multiple Myeloma

San Diego, CA, December 3, 2018 – Legend Biotech reported updated data on the LEGEND-2 Phase 1/2 open-label study, which evaluated the investigational chimeric antigen receptor T-cell (CAR-T) therapy LCAR-B38M in the treatment of patients with advanced relapsed or refractory (R/R) multiple myeloma. The findings, featured in an oral presentation at the 2018 American Society of Hematology (ASH) Annual Meeting (Abstract #955), build upon the data from one of four clinical sites, the Second Affiliated Hospital of Xi'an Jiaotong University, which were initially presented at the 2017 American Society of Clinical Oncology (ASCO) Annual Meeting and 2017 European Hematology Association (EHA) Meeting. These updated results showed that the B-cell maturation antigen (BCMA) directed CAR-T cell therapy LCAR-B38M achieved deep and durable responses, with a manageable and tolerable safety profile, in patients who failed a median of three prior therapies.

The Second Affiliated Hospital of Xi'an Jiaotong University was the treatment center responsible for enrolling the majority of patients for the LEGEND-2 study (n=57). Three additional sites participating in the study (Shanghai Ruijin Hospital, Shanghai Changzheng Hospital and Jiangsu Province People's Hospital) enrolled 17 patients. Data from these three sites will be submitted separately by the clinical investigators for future publication.

In December 2017, Legend Biotech, USA Inc, and Legend Biotech Ireland Limited ("Legend"), subsidiaries of GenScript Biotech Corporation entered into a worldwide collaboration and license agreement with Janssen Biotech, Inc. (Janssen), to jointly develop and commercialize LCAR-B38M in multiple myeloma. LCAR-B38M is a CAR-T cell therapy directed against two distinct BCMA epitopes, which confers high avidity and affinity binding of the compound to the BCMA-expressing cells. In China, a Phase 2 confirmatory trial registered with the Center for Drug Evaluation (CTR20181007) is currently being planned to further evaluate LCAR-B38M in patients with advanced R/R multiple myeloma. Globally, Legend, together with Janssen, is advancing a Phase 1b/2 trial

([NCT03548207](#)) of JNJ-68284528 to evaluate its efficacy and safety in adults with advanced R/R multiple myeloma. The study is currently enrolling patients following US Food and Drug Administration clearance of an Investigational New Drug application as [announced](#) in May 2018.

LCAR-B38M identifies the investigational product being studied in China and JNJ-68284528 identifies the investigational product being studied in the US/EU, both of which are representative of the same CAR-T therapy.

“There have been limited treatment options available to patients with R/R multiple myeloma, highlighting a need for new options beyond currently available therapies”, said study author Wanhong Zhao, MD, PhD, an Associate Director of Hematology at The Second Affiliated Hospital of Xi’an Jiaotong University in Xi’an, China. “BCMA is an important target in multiple myeloma. We are pleased to share updated data from our clinical trial investigating LCAR-B38M during the ASH Annual Meeting. LCAR-B38M is an investigational CAR-T cell therapy targeting BCMA and could provide a potential meaningful therapeutic option that may achieve and maintain deep and durable responses with a generally tolerable safety profile”.

In this study update, 57 patients with advanced multiple myeloma received LCAR-B38M CAR-T cell therapy. The median age of the patients was 54 years (range, 27–72); median number of prior therapies was three (range, 1–9); and 74 percent of patients had Stage 3 disease by Durie-Salmon staging. According to study findings, there was an 88 percent overall response rate (ORR) (95 percent confidence interval (CI): 76-95). Complete response (CR) was achieved by 74 percent of patients (95 percent CI: 60-85); very good partial response (VGPR) was achieved by 4 percent of patients and partial response was achieved by 11 percent of patients. Notably, among 42 patients with CR, 39 patients (68 percent) were minimal residual disease (MRD) negative in the bone marrow as measured by 8-color flow cytometry. With a median follow-up of 12 months, the median duration of response (DOR) was 16 months (95 percent CI: 12-not reached [NR]) and a median progression-free survival (PFS) of 15 months for all patients was observed. Among the patients who achieved an MRD negative CR, the median PFS was 24 months.

The most common adverse events (AEs) were pyrexia (91 percent), cytokine release syndrome (CRS) (90 percent), thrombocytopenia (49 percent), and leukopenia (47 percent). In patients who experienced Grade 3/4 AEs (65 percent), the most common were leukopenia (30 percent), thrombocytopenia (23 percent) and increased aspartate aminotransferase (21 percent). CRS was mostly low grade, which included Grade 1 (47 percent), Grade 2 (35 percent) and Grade 3 (7 percent). The median time to onset of CRS was nine days (range, 1–19), with a

median duration of nine days (range, 3–57). Neurotoxicity was observed in one patient who had Grade 1 aphasia, agitation and seizure-like activity. Overall, 17 patients died during the study and follow-up period; causes of death were progressive disease (PD; n=14), suicide after PD (n=1), esophagitis (n=1), and pulmonary embolism and acute coronary syndrome (n=1).

“BCMA is an outstanding target for the treatment of multiple myeloma and we are specifically targeting it with our LCAR-B38M program,” said Yuan Xu, CEO of Legend Biotech. “We are very encouraged to see deepening and durable responses with the LCAR-B38M Phase I study. In addition to this Phase 1 study, a new Global Phase Ib/II study is actively enrolling patients in the US today. Data from these clinical trials will continue to shape our understanding of the evolving treatment landscape in R/R multiple myeloma, where available treatment options have been limited for patients.”

About LEGEND-2

LEGEND-2 ([NCT03090659](https://clinicaltrials.gov/ct2/show/study/NCT03090659)) is an ongoing Phase 1/2, single-arm, open-label program in China comprised of four independent institutional studies being conducted at participating hospitals evaluating the efficacy and safety of LCAR-B38M for the treatment of patients with R/R multiple myeloma.

About CAR-T and BCMA

CAR-T cells are an innovative approach to eradicating cancer cells by harnessing the power of a patient's own immune system. BCMA is a protein that is highly expressed on myeloma cells. By targeting BCMA, CAR-T therapies may have the potential to redefine the treatment paradigm for multiple myeloma and potentially advance towards cures for patients with the disease.

About Multiple Myeloma

Multiple myeloma is an incurable blood cancer that occurs when malignant plasma cells grow uncontrollably in the bone marrow.^{1,2} Refractory cancer occurs when a patient's disease is resistant to treatment or in the case of multiple myeloma, patients progress within 60 days of their last therapy.^{3,4} Relapsed cancer means the disease has returned after a period of initial, partial or complete remission.⁵ In 2018, it is estimated that 30,700 people will be diagnosed and 12,770 will die from the disease in the United States.⁶ Most patients are diagnosed due to symptoms, which can include bone fracture or pain, low red blood counts, fatigue, calcium elevation, kidney problems or infections.⁷

About Legend Biotech

Legend Biotech is a clinical stage biopharmaceutical company engaged in the discovery and development of novel Chimeric Antigen Receptor T-cell (CAR-T) therapies targeting various oncology indications. Legend is a subsidiary of GenScript Biotech Corporation (HKEx: 1548), which operates in USA, Hong Kong, mainland China and Ireland. Learn more at www.LegendBiotech.com.

About GenScript Biotech Corporation

GenScript Biotech Corporation (HKEx: 1548) provides reagents services for researchers in basic life sciences, translational and biomedical fields, as well as pre-clinical antibody drug development, through its global operating entities located in the United States, Hong Kong, Ireland, the Netherlands, Japan and China. The diverse portfolio of GenScript encompasses extensive services in gene synthesis and molecular biology, peptide synthesis, protein expression and engineering, custom antibody development and engineering, in vitro/in vivo pharmacology as well as variety of catalogue products for research. Two subsidiaries of GenScript, under the brand name of Bestzyme Biotech and Legend Biotech engaged in Industrial Enzymes and CAR-T as well as other forms of specific cell Immunotherapies respectively, have both made rapid progress and breakthrough in their business development. Learn more at www.GenScript.com.

Cautions Concerning Forward-Looking Statements

This information constitutes forward-looking statements relating to the business of Legend Biotech USA Inc. ("Legend"), including express or implied discussions regarding potential new products, potential new indications, or regarding potential future revenues from any such products. Such forward-looking statements reflect the current views of Legend's management regarding future events, and involve known and unknown risks, uncertainties and other factors that may cause actual results to be materially different from any future results, performance or achievements expressed or implied by such statements.

The safety and efficacy of the agents and/or uses under investigation have not been established. There is no guarantee that the agents will receive health authority approval or become commercially available in any country for the uses being investigated or that such agents as products will achieve any particular revenue levels.

This information was factually accurate on the date it was published. Legend assumes no duty to update the information to reflect subsequent developments. Readers should not rely upon the information on this page as current or accurate after its publication date.

In particular, Legend's expectations could be affected by, among other things, uncertainties involved in the development of new pharmaceutical products; unexpected clinical trial results, including additional analysis of existing clinical data or unexpected new clinical data; unexpected regulatory actions or delays or government regulation generally; Legend's ability to obtain or maintain patent or other proprietary intellectual property protection, including the uncertainties involved in the US litigation process; competition in general; government, industry, and general public pricing and other political pressures. Should one or more of these risks or

uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those described herein as anticipated, believed, estimated or expected.

¹ Kumar, SK et al. Leukemia. 2012 Jan; 26(1):149-57.

² American Cancer Society. "What Is Multiple Myeloma?." Available at:

<http://www.cancer.org/cancer/multiplemyeloma/detailedguide/multiple-myeloma-what-is-multiple-myeloma>. Accessed March 2018.

³ National Cancer Institute. "NCI Dictionary of Cancer Terms: Refractory." Available at:

<https://www.cancer.gov/publications/dictionaries/cancer-terms?CdrID=350245>. Accessed March 2018.

⁴ Richardson, et al. "The Treatment of Relapsed and Refractory Multiple Myeloma." ASH Education Book. January 1, 2007 vol. 2007 no. 1 317-323.

⁵ National Cancer Institute. "NCI Dictionary of Cancer Terms: Relapsed." Available at:

<https://www.cancer.gov/publications/dictionaries/cancer-terms?CdrID=45866>. Accessed March 2018.

⁶ American Cancer Society. "Key Statistics for Multiple Myeloma." Available at: <https://www.cancer.org/cancer/multiple-myeloma/about/key-statistics.html>. Accessed March 2018.

⁷ American Cancer Society. "Diagnosing Multiple Myeloma From Test Results." Available at:

<http://www.cancer.org/cancer/multiplemyeloma/detailedguide/multiple-myeloma-diagnosis>. Accessed March 2018.

For investor-related inquiries, please contact:

Robin Meng (Vice President of Investor Relations):

robin.meng@genscript.com

For media inquiries, please contact:

media@legendbiotech.com