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**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, December 3, 2018 – The Janssen Pharmaceutical Companies of Johnson & Johnson reported today updated results from Legend Biotech Inc.'s 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 60<sup>th</sup> American Society of Hematology (ASH) Annual Meeting (Abstract #[955](#)), build upon the data from one of four independent institutional studies, 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.

In December 2017, Janssen entered into a worldwide collaboration and license agreement with Legend Biotech, USA Inc. and Legend Biotech Ireland Limited ("Legend"), subsidiaries of GenScript Biotech Corporation, 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, Janssen, together with Legend, 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 the U.S. 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 U.S./EU, both of which are representative of the same CAR-T therapy.

“CAR-T science has led to the approval of much-needed therapeutic interventions for certain blood cancers, and it is our hope that the results we are seeing in multiple myeloma will yield another much needed option for patients,” said Wan-Hong Zhao, M.D., Ph.D., Associate Director of Hematology at the Second Affiliated Hospital of Xi’an Jiaotong University in Xi’an, China, and lead study investigator. “We are excited about these data and the fact that they demonstrated notable responses in heavily pretreated patients with multiple myeloma, a population that traditionally has been difficult to treat.”

In this study update, 57 patients with advanced R/R 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 four 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 Grade 1 (47 percent) and 2 (35 percent). However, four patients (seven percent) experienced Grade 3 CRS. The median time to onset of CRS was nine days (range, 1–19). All but one of the CRS events resolved, 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).

“These updated data show the potential of this investigational CAR-T therapy in the treatment of patients with advanced multiple myeloma,” said Sen Zhuang, M.D., Ph.D., Vice President, Oncology Clinical Development, Janssen Research & Development, LLC. “Through the ongoing global development program, we aim to further define and characterize the safety and efficacy profile of this BCMA-targeted immunotherapy in the hope of bringing forward a new treatment option for patients with multiple myeloma in the future.”

### **About LEGEND-2**

LEGEND-2 ([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 via a CAR-T approach, 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.<sup>1,2</sup> 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.<sup>3,4</sup> Relapsed cancer means the disease has returned after a period of initial, partial or

complete remission.<sup>5</sup>In 2018, it is estimated that 30,700 people will be diagnosed and 12,770 will die from the disease in the United States.<sup>6</sup>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.<sup>7</sup>

### **About the Janssen Pharmaceutical Companies of Johnson & Johnson**

At the Janssen Pharmaceutical Companies of Johnson & Johnson, we are working to create a world without disease. Transforming lives by finding new and better ways to prevent, intercept, treat and cure disease inspires us. We bring together the best minds and pursue the most promising science. We are Janssen. We collaborate with the world for the health of everyone in it. Learn more at [www.janssen.com](http://www.janssen.com). Follow us at [@JanssenGlobal](https://twitter.com/JanssenGlobal) and [@JanssenUS](https://twitter.com/JanssenUS). Janssen Research & Development, LLC is one of the Janssen Pharmaceutical Companies of Johnson & Johnson.

### **Cautions Concerning Forward-Looking Statements**

*This press release contains "forward-looking statements" as defined in the Private Securities Litigation Reform Act of 1995 regarding product development and the potential benefits and treatment impact of LCAR-B38M and JNJ-68284528. The reader is cautioned not to rely on these forward-looking statements. These statements are based on current expectations of future events. If underlying assumptions prove inaccurate or known or unknown risks or uncertainties materialize, actual results could vary materially from the expectations and projections of Janssen Biotech, Inc., Janssen Research & Development, LLC, any of the other Janssen Pharmaceutical Companies of Johnson & Johnson and/or Johnson & Johnson. Risks and uncertainties include, but are not limited to: the potential that the expected benefits and opportunities related to the collaboration may not be realized or may take longer to realize than expected; challenges inherent in new product development, including the uncertainty of clinical success and obtaining regulatory approvals; competition, including technological advances, new products and patents attained by competitors; uncertainty of commercial success for new products; the ability of the company to successfully execute strategic plans; impact of business combinations and divestitures; challenges to patents; changes in behavior and spending patterns or financial distress of purchasers of health care products and services; and global health care reforms and trends toward health care cost containment. A further list and descriptions of these risks, uncertainties and other factors can be found in Johnson & Johnson's Annual Report on Form 10-K for the fiscal year ended December 31, 2017, including in the sections captioned "Cautionary Note Regarding Forward-Looking Statements" and "Item 1A. Risk Factors," in the company's most recently filed Quarterly Report on Form 10-Q, and the company's subsequent filings with the Securities and Exchange Commission. Copies of*

*these filings are available online at [www.sec.gov](http://www.sec.gov), [www.jnj.com](http://www.jnj.com) or on request from Johnson & Johnson. Neither the Janssen Pharmaceutical Companies nor Johnson & Johnson undertakes to update any forward-looking statement as a result of new information or future events or developments.*

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

<sup>2</sup> American Cancer Society. "What Is Multiple Myeloma?." Available at: <http://www.cancer.org/cancer/multiplemyeloma/detailedguide/multiple-myeloma-what-is-multiple-myeloma>. Accessed December 2018.

<sup>3</sup> National Cancer Institute. "NCI Dictionary of Cancer Terms: Refractory." Available at: <https://www.cancer.gov/publications/dictionaries/cancer-terms?CdrID=350245>. Accessed December 2018.

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

<sup>5</sup> National Cancer Institute. "NCI Dictionary of Cancer Terms: Relapsed." Available at: <https://www.cancer.gov/publications/dictionaries/cancer-terms?CdrID=45866>. Accessed December 2018.

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

<sup>7</sup> American Cancer Society. "Diagnosing Multiple Myeloma From Test Results." Available at: <http://www.cancer.org/cancer/multiplemyeloma/detailedguide/multiple-myeloma-diagnosis>. Accessed December 2018.