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Janssen's BCMA CAR-T Therapy JNJ-4528 Showed Early, Deep and Durable Responses in Heavily Pretreated Patients with Multiple Myeloma

Longer-term follow-up data from Phase 1b/2 CARTITUDE-1 study demonstrate 100% overall response rate, 86% stringent complete response rate at a median of 11.5 months and 86% progression-free survival at 9 months

RARITAN, NJ, May 13, 2020 – The Janssen Pharmaceutical Companies of Johnson & Johnson announced today updated results from the Phase 1b/2 CARTITUDE-1 study ([NCT03548207](#)) evaluating the efficacy and safety of JNJ-4528, an investigational B-cell maturation antigen (BCMA)-directed chimeric antigen receptor T cell (CAR-T) therapy in the treatment of patients with relapsed or refractory multiple myeloma. Longer-term follow-up results from the Phase 1b portion of the study (n=29), to be shared in an oral presentation at the American Society of Clinical Oncology (ASCO) Virtual Scientific Program ([Abstract #8505](#)), show that all patients responded to treatment and that the responses were deep and durable with 86 percent of patients achieving stringent complete response at a median follow-up of 11.5 months and 86 percent of patients being alive and progression free at 9 months.¹

The 100 percent overall response rate (ORR) included 97 percent of patients achieving a very good partial response or better and three percent achieving a partial response. Responses were observed

among heavily pretreated patients (n=29) at a low dose of CAR-T cells (median administered dose 0.72×10^6 CAR+ viable T cells/kg). Patients evaluated had received a median of five (range, 3-18) prior treatment regimens; 86 percent were triple-refractory and 28 percent were penta-refractory. The median time to first response was one month (range, 1-3), and 81 percent of evaluable patients (n=16) achieved minimal residual disease (MRD)-negative disease status at 10^{-5} or 10^{-6} at the time of first suspected complete response.¹

“The longer-term results for JNJ-4528, as demonstrated through the latest findings from the CARTITUDE-1 study, show the continued treatment effect for heavily pretreated patients who faced a dismal prognosis,” said Jesus G Berdeja, M.D., Director of Myeloma Research, Sarah Cannon Research Institute, and principal study investigator. “We’re encouraged by not only the relatively high rate of stringent complete responses, but also the progression-free survival seen in these patients.”

The most common adverse events (AEs) observed in CARTITUDE-1 were neutropenia (100 percent) and cytokine release syndrome (CRS, 93 percent). In patients who experienced Grade 3 and above AEs, the most common were neutropenia (100 percent), thrombocytopenia (69 percent) and leukopenia (66 percent). The median time of onset of CRS was seven days (range, 2-12) post-infusion, with a majority of patients experiencing Grade 1-2 CRS and two patients experiencing Grade 3 or greater CRS. Neurotoxicity consistent with immune effector cell-associated neurotoxicity syndrome (ICANS) was observed in 3 patients (10 percent), including one patient (3 percent) with Grade 3 or greater toxicity. Three deaths were reported during the Phase 1b study: one due to CRS, one due to acute myeloid leukemia (not treatment-related) and one due to progressive disease.¹

“These recently updated data from the CARTITUDE-1 study suggest a durable response and tolerable safety profile for JNJ-4528,” said Sen Zhuang, M.D., Ph.D., Vice President, Oncology Clinical Development, Janssen Research & Development, LLC. “We continue to advance the investigation of this novel CAR-T treatment with the goal of bringing a differentiated immunotherapy to patients with multiple myeloma, many of whom have exhausted all potential prior treatment options.”

About CARTITUDE-1

CARTITUDE-1 ([NCT03548207](https://clinicaltrials.gov/ct2/show/study/NCT03548207)) is an ongoing Phase 1b/2, open-label, multicenter study evaluating the safety and efficacy of JNJ-4528 in adults with relapsed or refractory multiple myeloma, 97 percent of whom were refractory to the last line of treatment; 86 percent of whom were triple-class

refractory, meaning their cancer did not, or no longer responds to an immunomodulatory agent (IMiD), a proteasome inhibitor (PI) and an anti-CD38 antibody.

The primary objective of the Phase 1b portion of the study was to characterize the safety and confirm the dose of JNJ-4528, informed by the first-in-human study with LCAR-B38M CAR-T cells (LEGEND-2). Based on the safety profile observed in this portion of the study, outpatient dosing will be evaluated in additional CARTITUDE studies. The Phase 2 portion of the study will evaluate the efficacy of JNJ-4528 with overall response as the primary endpoint.

About JNJ-4528 (LCAR-B38M)

JNJ-4528 (LCAR-B38M) is an investigational chimeric antigen receptor T cell (CAR-T) therapy for the treatment of patients with relapsed or refractory multiple myeloma. The design comprises a structurally differentiated CAR-T with two BCMA-targeting single domain antibodies. 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.

In December 2017, Janssen [entered](#) into an exclusive worldwide license and collaboration agreement with Legend Biotech to develop and commercialize JNJ-4528 (LCAR-B38M). In May 2018, Janssen [initiated](#) a Phase 1b/2 trial ([NCT03548207](#)) to evaluate the efficacy and safety of JNJ-4528 in adults with relapsed or refractory multiple myeloma, informed by the LEGEND-2 study results.

In December 2019, Janssen [announced](#) receipt of a Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA) for JNJ-4528, which is granted to expedite the development and regulatory review of an investigational medicine that is intended to treat a serious or life-threatening condition. In February 2019, the FDA granted Janssen an orphan drug designation for JNJ-4528, and in February 2020, the European Commission granted Janssen an orphan designation for JNJ-4528. In April 2019, JNJ-4528 was [granted](#) PRIME (PRIority MEDicines) designation by the European Medicines Agency (EMA). PRIME offers enhanced interaction and early dialogue to optimize drug development plans and speed up evaluation of cutting-edge, scientific advances that target a high unmet medical need.²

About Multiple Myeloma

Multiple myeloma is an incurable blood cancer that affects a type of white blood cell called plasma cells, which are found in the bone marrow.^{3,4} When damaged, these plasma cells rapidly spread and

replace normal cells with tumors in the bone marrow. In 2020, it is estimated that 32,270 people will be diagnosed and 12,830 will die from the disease in the U.S.^{2,4} While some patients with multiple myeloma have no symptoms, most patients are diagnosed due to symptoms, which can include bone fracture or pain, low red blood cell counts, tiredness, high calcium levels, kidney problems or infections.⁵

About the Janssen Pharmaceutical Companies of Johnson & Johnson

At Janssen, we're creating a future where disease is a thing of the past. We're the Pharmaceutical Companies of Johnson & Johnson, working tirelessly to make that future a reality for patients everywhere by fighting sickness with science, improving access with ingenuity, and healing hopelessness with heart. We focus on areas of medicine where we can make the biggest difference: Cardiovascular & Metabolism, Immunology, Infectious Diseases & Vaccines, Neuroscience, Oncology, and Pulmonary Hypertension.

Learn more at www.janssen.com. Follow us at www.twitter.com/JanssenGlobal and www.twitter.com/JanssenUS. Janssen Research & Development, LLC is one of the Janssen Pharmaceutical Companies of Johnson & Johnson.

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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 JNJ-4528. 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 Research & Development, LLC or any of the other Janssen Pharmaceutical Companies and/or Johnson & Johnson. Risks and uncertainties include, but are not limited to: challenges and uncertainties inherent in product research and development, including the uncertainty of clinical success and of obtaining regulatory approvals; uncertainty of commercial success; manufacturing difficulties and delays; competition, including technological advances, new products and patents attained by competitors; challenges to patents; product efficacy or safety concerns resulting in product recalls or regulatory action; changes in behavior and spending patterns of purchasers of health care products and services; changes to applicable laws and regulations, including 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 29, 2019, including in the sections captioned "Cautionary Note Regarding Forward-Looking Statements" and "Item 1A. Risk Factors," and 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, 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.

¹ Berdeja, J. et. al. Update of CARTITUDE-1: A phase 1b/2 study of JNJ-4528, a B-cell maturation antigen (BCMA)-directed CAR-T cell therapy, in relapsed/refractory multiple myeloma. Abstract #8505 [Oral]. To be presented at American Society of Clinical Oncology Virtual Scientific Program 2020.

² European Medicines Agency. PRIME Factsheet. Available at: <https://www.ema.europa.eu/en/human-regulatory/research-development/prime-priority-medicines> Accessed April 2020.

³ Kumar, SK et al. Risk of progression and survival in multiple myeloma relapsing after therapy with IMiDs and bortezomib: a multicenter international myeloma working group study. *Leukemia*. 2012 Jan; 26(1):149-57.

⁴ American Cancer Society. "What Is Multiple Myeloma?" Available at: <http://www.cancer.org/cancer/multiplemyeloma/detailedguide/multiple-myeloma-what-is-multiple-myeloma>. Accessed May 2020.

⁵ American Cancer Society. "Key Statistics About Multiple Myeloma." Available at: <https://www.cancer.org/cancer/multiple-myeloma/about/key-statistics.html>. Accessed May 2020.