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Early, Deep, Durable Responses of Ciltacabtagene Autoleucel (cilta-cel) Observed in Phase 1b/2 CARTITUDE-1 Study Show Potential of BCMA CAR-T in Treatment of Heavily Pretreated Patients with Multiple Myeloma

Combined results from Phase 1b/2 CARTITUDE-1 study presented at ASH 2020 show 97 percent overall response rate at median follow-up of 12.4 months

December 5, 2020 (RARITAN, N.J.) – The Janssen Pharmaceutical Companies of Johnson & Johnson announced today longer-term results from the combined Phase 1b/2 CARTITUDE-1 study (NCT03548207) evaluating the efficacy and safety of ciltacabtagene autoleucel (cilta-cel), an investigational B cell maturation antigen (BCMA)-directed chimeric antigen receptor T cell (CAR-T) therapy, for the treatment of patients with relapsed and/or refractory multiple myeloma. These data, presented as an oral presentation at the American Society of Hematology (ASH) 2020 Annual Meeting (Abstract #177), continued to demonstrate a very high overall response rate of 97 percent, which deepened over time with 67 percent of patients achieving a stringent complete response. With a median follow-up of 12.4 months, median duration of response and progression-free survival (PFS) were not reached.[1]

"Unfortunately, for patients with multiple myeloma for whom at least three established treatment regimens have stopped working, the prognosis is often not good," said Deepu Madduri, M.D., Assistant Professor of Medicine, Hematology and Medical Oncology, The Tisch Cancer Institute at Mount Sinai, New York, and principal study investigator. "In the CARTITUDE-1 study, heavily

pretreated patients, including those who were triple-class refractory, achieved an impressive response following a single infusion of ciltacabtagene autoleucel. These data continue to show consistent efficacy of this novel CAR-T in the treatment of this highly refractory patient population."

Median time to first response was one month (range, 0.9-8.5), with responses observed at a low dose of CAR-T cells (median administered dose 0.71 x 10^6 CAR+ viable T cells/kg) and were ongoing in 72 percent (n=70) of patients.[1] Additionally, 93 percent of evaluable patients (n=53) achieved minimal residual disease (MRD) negative disease status at 10^{-5}.[1] The trial included heavily pretreated patients, with evaluated patients having received a median of six prior treatment regimens (range, 3-18); 88 percent (n=85) were triple-refractory, 42 percent (n=41) were penta-refractory, and 99 percent (n=96) were refractory to the last line of therapy.[1] The 12-month PFS rate was 77 percent (95 percent confidence interval [CI], 66-84).[1] The 12-month overall survival (OS) rate was 89 percent (95 percent CI, 80-94) and manufacturing of cilta-cel was successful for all patients.[1]

"The combined Phase 1b/2 data from the CARTITUDE-1 study include a larger patient population than previously reported in the initial Phase 1b results, and we are encouraged that patients treated with cilta-cel continued to achieve impressive, deep responses," said Sen Zhuang, M.D., Ph.D., Vice President, Clinical Research Development, Janssen Research & Development, LLC. "The responses also appeared to be durable as indicated by the estimate that 89 percent of patients remained alive and 77 percent of patients remained progression-free after one year of follow up."

In these combined results, the most common hematologic adverse events (AEs) observed in the CARTITUDE-1 study were neutropenia (96 percent); anemia (81 percent); thrombocytopenia (79 percent); leukopenia (62 percent); and lymphopenia (53 percent).[1] Cytokine release syndrome (CRS) of any grade was observed in 95 percent of patients, with a median duration of four days (range, 1-97), and 99 percent of which were resolved within 14 days of onset. Of the 92 patients with CRS, 95 percent (n=87) were Grade 1/2, three percent (n=3) were Grade 3, one percent (n=1) was Grade 4 and one percent (n=1) was Grade 5.[1] The median onset of CRS was at seven days (range, 1-12) post-infusion, with 89 percent (n=82) of patients experiencing CRS onset at day four or later, which indicates potential for outpatient administration for cilta-cel.[1] Neurotoxicity of any grade was observed in 21 percent (n=20) of patients, with Grade 3 or higher neurotoxicity observed in 10 percent (n=10) of patients.[1] Of these, Immune effector Cell-Associated Neurotoxicity Syndrome (ICANS) was observed in 16 patients and generally occurred concurrently with CRS; other neurotoxicities were observed in 12 patients and generally occurred after resolution of CRS

and/or ICANS (eight patients experienced both ICANS and other neurotoxicities).[1] ICANS events were resolved in all patients with a median time to recovery of four days (range, 1-12).[1] Other neurotoxicities were resolved in six patients with a median time of 75 days (range, 2-160) and were not resolved in six patients (one with ongoing toxicity, one died from neurotoxicity and four died due to other causes).[1] Fourteen deaths were reported during the study: five due to disease progression, three due to adverse events unrelated to treatment (acute myelogenous leukemia (n=2), pneumonia (n=1)) and six due to adverse events related to treatment (sepsis and/or septic shock (n=2), CRS/ hemophagocytic lymphohistiocytosis (n=1), lung abscess (n=1), respiratory failure (n=1) and neurotoxicity (n=1)).[1]

About CARTITUDE-1

CARTITUDE-1 (NCT03548207) is an ongoing Phase 1b/2, open-label, multicenter study evaluating the safety and efficacy of ciltacabtagene autoleucel in adults with relapsed and/or refractory multiple myeloma, 99 percent of whom were refractory to the last line of treatment; 88 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, involving 29 patients, was to characterize the safety and confirm the dose of ciltacabtagene autoleucel, 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 is being evaluated in additional CARTITUDE studies. The Phase 2 portion of the study, involving 68 additional patients, is evaluating the efficacy of ciltacabtagene autoleucel with overall response as the primary endpoint.

About Cilta-cel

Cilta-cel is an investigational chimeric antigen receptor T cell (CAR-T) therapy for the treatment of patients with multiple myeloma. The design comprises a structurally differentiated CAR-T with two B cell maturation antigen-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 cilta-cel. In May 2018, Janssen initiated a Phase 1b/2 trial (NCT03548207) to evaluate the efficacy and safety of cilta-cel in adults with relapsed and/or refractory multiple myeloma, informed by the LEGEND-2 study results.

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In December 2019, Janssen announced receipt of a Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA) for cilta-cel, 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 cilta-cel, and in February 2020, the European Commission granted Janssen an orphan designation for cilta-cel. In April 2019, cilta-cel 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.[4] In 2020, it is estimated that 32,270 people will be diagnosed and 12,830 will die from the disease in the U.S.[5] 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.

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Cautions Concerning Forward-Looking Statements

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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 ciltacabtagene autoleucel. 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.

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[1] Madduri, D et al. Cartitude-1: Phase 1b/2 Study of Ciltacabtagene Autoleucel, a B-Cell Maturation Antigen–Directed Chimeric Antigen Receptor T Cell Therapy, in Relapsed/Refractory Multiple Myeloma. Abstract #177. To be presented at 2020 American Society of Hematology Annual Meeting.

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

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