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Janssen Initiates Rolling Submission of a Biologics License Application to U.S. FDA for BCMA CAR-T Therapy Ciltacabtagene Autoleucel (cilta-cel) for the Treatment of Relapsed and/or Refractory Multiple Myeloma

December 21, 2020 (RARITAN, N.J.) – The Janssen Pharmaceutical Companies of Johnson & Johnson announced today the initiation of a rolling submission of its Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for ciltacabtagene autoleucel (cilta-cel), an investigational B-cell maturation antigen (BCMA)-directed chimeric antigen receptor T cell (CAR-T) therapy, for the treatment of adults with relapsed and/or refractory multiple myeloma.

“We are committed to innovation in cell therapy and advancing the science of multiple myeloma to improve patients’ lives,” said Peter Lebowitz, M.D., Ph.D., Global Therapeutic Area Head, Oncology, Janssen Research & Development, LLC. “Today’s milestone is the culmination of a remarkable clinical development effort and collaboration with Legend Biotech. We look forward to working with the FDA in their review of cilta-cel with the goal of bringing a highly-active, dual-binding BCMA CAR-T therapy to patients with relapsed and/or refractory multiple myeloma who are in need of new treatment options.”

The FDA previously [granted](#) Breakthrough Therapy Designation (BTD) for cilta-cel and has agreed to a rolling review of the BLA in which completed portions of the application will be submitted and

reviewed on an ongoing basis. Data for cilta-cel were recently presented (Abstract #177) at the 62nd American Society of Hematology (ASH) Annual Meeting.

“Our immuno-oncology capabilities combined with our dedicated Janssen R&D team, who continue to work in close collaboration with Legend Biotech, have enabled the expeditious investigational advancement of cilta-cel,” said Mathai Mammen, M.D., Ph.D., Global Head, Janssen Research & Development, Johnson & Johnson. “Today’s submission marks Janssen’s first cell therapy application, but more importantly, brings cilta-cel one step closer to our goal of making new medical options available to patients with multiple myeloma.”

About CARTITUDE-1

CARTITUDE-1 ([NCT03548207](#)) is an ongoing Phase 1b/2, open-label, multi-center study evaluating the safety and efficacy of cilta-cel in adults with relapsed and/or refractory multiple myeloma, 99 percent of whom were refractory to the last line of treatment and 88 percent of whom were triple-class refractory, meaning their cancer did not respond, 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 cilta-cel, 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 CARTITUDE-1 study, the Phase 2 portion further evaluated the efficacy of cilta-cel at the recommended Phase 2 dose with overall response as the primary endpoint.

About ciltacabtagene autoleucel (cilta-cel)

Cilta-cel is an investigational CAR-T therapy being studied in a comprehensive clinical development program for the treatment of patients with relapsed and/or refractory multiple myeloma and in earlier lines of treatment. Cilta-cel is a unique, structurally differentiated CAR-T cell therapy containing a 4-1BB co-stimulatory domain and two BCMA-targeting single-domain antibodies with a preferential CD8+ T-cell expansion. BCMA is a protein that is highly expressed on myeloma cells.¹ CAR-T cells are an innovative approach to eradicating cancer cells by harnessing the power of a patient’s own immune system. The safety profile observed to date for cilta-cel supports the potential for outpatient dosing, which will be evaluated in ongoing clinical studies.

In December 2017, Janssen Biotech, Inc. [entered](#) into an exclusive worldwide license and collaboration agreement with Legend Biotech USA Inc. to develop and commercialize cilta-cel.

In addition to a U.S. BTD [granted](#) in December 2019, cilta-cel [received](#) a PRIority MEdicines (PRiME) designation from the European Commission in April 2019, and a BTD in China in August 2020. Janssen also received Orphan Drug Designation for cilta-cel from the FDA in February 2019 and from the European Commission in February 2020.

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.^{2,3} When damaged, these plasma cells rapidly spread and replace normal cells with tumors in the bone marrow. **Error! Bookmark not defined.** In 2020, it is estimated that 32,270 people will be diagnosed and 12,830 will die from the disease in the U.S.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. **Error! Bookmark not defined.**

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 and Janssen Biotech, Inc. are part 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 cilta-cel. 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 Tai YT, et al. Targeting B-cell maturation antigen in multiple myeloma.

<https://www.ncbi.nlm.nih.gov/pubmed/26370838>. Accessed December 2020.

² 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.

³ 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.

⁴ American Cancer Society. "Key Statistics About Multiple Myeloma." Available at:

<https://www.cancer.org/cancer/multiple-myeloma/about/key-statistics.html>. Accessed December 2020.