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News Release

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Janssen Submits Marketing Authorisation Application to the European Medicines Agency Seeking Approval of BCMA CAR-T Therapy Ciltacabtagene Autoleucel (cilta-cel) for the Treatment of Relapsed and/or Refractory Multiple Myeloma

Application based on positive Phase 1b/2 data from the CARTITUDE-1 study and follows confirmation of accelerated assessment by the Committee for Medicinal Products for Human Use of the European Medicines Agency

BEERSE, BELGIUM, 30 April 2021 – The Janssen Pharmaceutical Companies of Johnson & Johnson announced today that they have submitted a Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) seeking approval of 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.

The application is supported by positive results from the ongoing Phase 1b/2 CARTITUDE-1 study, investigating the safety and efficacy of cilta-cel.^{1,2} The latest results were presented at the American Society of Hematology (ASH) 2020 Annual Meeting. Clinical development is ongoing with patients enrolled globally in various studies, including sites in Europe, the United States of America, China and Japan.²

“Despite advances in the treatment of multiple myeloma, there remains a high unmet need, especially for patients whose disease continues to progress,” said Peter Lebowitz, M.D., Ph.D., Global Therapeutic Area Head, Oncology, Janssen Research & Development, LLC. “Through our collaboration with Legend Biotech, we continue to expedite the development of cilta-cel with a focus and priority on the patients who may benefit from this novel immunotherapy in the future.”

CAR-T therapy is a highly personalised treatment platform where a patient’s own T-cells are re-programmed to recognise and attack cancer cells.³ In early 2021, the EMA granted accelerated assessment for cilta-cel.⁴ Accelerated assessment is granted when a medicinal product is expected to be of major public health interest and a therapeutic innovation, and can significantly reduce the review timelines to evaluate an MAA.⁵

“Janssen has been advancing the science of oncology for more than 30 years, and we see great opportunity in the area of cell therapy and through our innovative platforms,” says Mathai Mammen, M.D., Ph.D., Global Head, Janssen Research & Development, Johnson & Johnson. “We are continuing to harness our deep scientific expertise in multiple myeloma as we look to advance therapeutic options, deepen clinical responses, and drive towards improved patient outcomes.”

“Today’s submission to the EMA epitomises how we strive to make a meaningful impact in the multiple myeloma landscape through advancing innovative treatments for patients,” says Saskia De Haes, Vice President, EMEA Regulatory Affairs, Janssen R&D BE. “We look forward to working in partnership with health authorities, as part of the accelerated assessment process, to support these patients by ensuring timely access to the latest therapeutic options.”

A Biologics License Application seeking approval of cilta-cel for the treatment of relapsed and/or refractory multiple myeloma is currently under review by the United States Food and Drug Administration.⁶

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About CARTITUDE-1

CARTITUDE-1 ([NCT03548207](#)) is an ongoing Phase 1b/2, open-label, multicentre study evaluating the safety and efficacy of JNJ-68284528 (JNJ-4528) in adults with relapsed or refractory multiple myeloma who have received at least three prior lines of therapy or are double refractory to a proteasome inhibitor (PI) and an immunomodulatory drug (IMiD); have received a PI, IMiD and an anti-CD38 antibody.² The primary objective of the Phase 1b portion of the study is to characterise the safety and confirm the dose of JNJ-68284528 (JNJ-4528), which was informed by the first-in-human study with LCAR-B38M CAR-T cells (LEGEND-2).² The primary objective for the Phase 2 portion of the study is to evaluate the efficacy of JNJ-4528 (primary endpoint: overall response rate as defined by the International Myeloma Working Group response criteria).²

About Ciltacabtagene Autoleucel (cilta-cel)

Cilta-cel is an investigational chimeric antigen receptor T cell (CAR-T) therapy for the treatment of patients with multiple myeloma. Cilta-cel is a differentiated CAR-T therapy with two BCMA-targeting single domain antibodies.¹ CAR-T cells are an innovative approach to targeting cancer cells by harnessing the power of a patient's own immune system.⁷ BCMA is a protein that is highly expressed on myeloma cells.⁸

In December 2017, Janssen Biotech, Inc. (Janssen) entered into an exclusive worldwide license and collaboration agreement with Legend Biotech to develop and commercialise cilta-cel.⁹ In May 2018, Janssen initiated a Phase 1b/2 CARTITUDE-1 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.^{2,10}

In 2019, cilta-cel was granted [PRIME \(PRIority MEDicines\) designation](#) by the European Medicines Agency (EMA).¹¹ PRIME offers enhanced interaction and early dialogue with developers of promising medicines, to optimise drug development plans and speed up evaluation of cutting-edge, scientific advances that target a high unmet medical need.¹² In 2020, the European Commission granted orphan designation for cilta-cel.¹³

About Multiple Myeloma

Multiple myeloma (MM) is an incurable blood cancer that starts in the bone marrow and is characterised by an excessive proliferation of plasma cells.¹⁴ In Europe, 50,918 people were diagnosed with MM in 2020, and more than 32,400 patients died.¹⁵ Around 50 percent of newly diagnosed patients do not reach five-year survival,¹⁶ and approximately 10 percent of patients with multiple myeloma will die within one year of diagnosis.¹⁷

Although treatment may result in remission, unfortunately, patients will most likely relapse as there is currently no cure.¹⁸ Refractory MM is when a patient's disease progresses within 60 days of their last therapy.¹⁹ Relapsed cancer is when the disease has returned after a period of initial, partial or complete remission.²⁰ While some patients with MM have no symptoms at all, others are diagnosed due to symptoms that can include bone problems, low blood counts, calcium elevation, kidney problems or infections.²¹ Patients who relapse after treatment with standard therapies, including protease inhibitors and immunomodulatory agents, have poor prognoses and require new therapies for continued disease control.²²

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/emea. Follow us at www.twitter.com/janssenEMEA for our latest news. Janssen Pharmaceutica NV, Janssen R&D BE, and Janssen Research & Development, LLC 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 ciltacabtagene autoleucl (ciltacel). 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 Pharmaceutica NV, 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 behaviour 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 January 3, 2021, 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. None of 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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