NEWS RELEASE

# U.S. FDA Grants Priority Review to Janssen for Daratumumab as a Treatment for Multiple Myeloma

9/4/2015

RARITAN, N.J., Sept. 4, 2015 /PRNewswire/ -- Janssen Research & Development, LLC (Janssen) announced today the U.S. Food and Drug Administration (FDA) has accepted for Priority Review the Biologics License Application (BLA) for daratumumab as a treatment for patients with multiple myeloma who are refractory to both a proteasome inhibitor (PI) and an immunomodulatory agent (IMiD), or who have received three or more prior lines of therapy, including a PI and an IMiD. This is referred to as "double refractory" multiple myeloma, which occurs when a patient's disease has become resistant to at least two of the most commonly utilized and active classes of anti-myeloma agents. Daratumumab, an investigational human anti-CD38 monoclonal antibody, received Breakthrough Therapy Designation from the FDA for this patient population in **May 2013**.

The FDA grants Priority Review to investigational therapies that if approved, may offer significant improvements in the treatment, prevention or diagnosis of a serious condition.[1] This designation shortens the review period to six months compared to 10 months for Standard Review. With today's announcement, the FDA has assigned a Prescription Drug User Fee Act (PDUFA) target date of March 9, 2016 to render a decision on the daratumumab application.

"Daratumumab has the potential to be the first anti-CD38 monoclonal antibody approved to treat multiple myeloma, offering these patients an important new treatment pathway," said Craig Tendler, M.D., Vice President, Late Development and Global Medical Affairs, Oncology, Janssen. "The FDA's acceptance of our application for daratumumab marks an important step for people affected by multiple myeloma. We look forward to working with the FDA during this Priority Review."

The regulatory submission for daratumumab, which was initiated on **June 5, 2015**, is primarily supported by data

from the Phase 2 MMY2002 (SIRIUS) monotherapy study, which were presented in **May/June 2015** at the 51st Annual Meeting of the American Society of Clinical Oncology (ASCO). Additional data from four other studies, including the Phase 1/2 GEN501 monotherapy study, recently published in **The New England Journal of Medicine**, also support the submission. Daratumumab is the second medicine in the Janssen oncology portfolio to receive Breakthrough Therapy Designation, which is intended to expedite the development and review time for a potential new medicine.

In **August 2012**, Janssen Biotech, Inc. and Genmab A/S entered a worldwide agreement that granted Janssen an exclusive license to develop, manufacture and commercialize daratumumab. Janssen is the global sponsor of all but one clinical studies for daratumumab. If approved, daratumumab would be commercialized in the U.S. by Janssen Biotech, Inc.

## About Multiple Myeloma

Multiple myeloma is an incurable blood cancer that starts in the bone marrow and is characterized by an excess proliferation of plasma cells.[2] Multiple myeloma is the third most common blood cancer in the U.S., behind only leukemia and lymphoma.[3] Approximately 26,850 new patients will be diagnosed with multiple myeloma, and approximately 11,240 people will die from the disease in the U.S. in 2015.[4] Globally, it is estimated that 124,225 people will be diagnosed and 87,084 will die from the disease in 2015.[5,6] While some patients with multiple myeloma have no symptoms at all, most patients are diagnosed due to symptoms which can include bone problems, low blood counts, calcium elevation, kidney problems or infections.[7] Patients who relapse after treatment with standard therapies, including PIs or IMiDs, have poor prognoses and few treatment options.[8]

## About Daratumumab

Daratumumab is an investigational human monoclonal antibody (mAb) that binds with high affinity to the CD38 molecule, which is highly expressed on the surface of multiple myeloma cells. It is believed to induce rapid tumor cell death through multiple immune-mediated mechanisms,[9] including complement-dependent cytotoxicity,[10] antibody-dependent cellular phagocytosis[11] and antibody-dependent cellular cytotoxicity,[12] as well as via induction of apoptosis.[13] Five Phase 3 clinical studies with daratumumab in relapsed and frontline settings are currently ongoing. Additional studies are ongoing or planned to assess its potential in other malignant and pre-malignant diseases on which CD38 is expressed, such as smoldering myeloma and non-Hodgkin lymphoma.

## About Janssen Research & Development, LLC

At Janssen, we are dedicated to addressing and solving some of the most important unmet medical needs of our time in oncology, immunology, neuroscience, infectious diseases and vaccines, and cardiovascular and metabolic diseases. Driven by our commitment to patients, we develop innovative products, services and healthcare solutions to help people throughout the world. Janssen Research & Development, LLC and Janssen Biotech, Inc. are part of

the Janssen Pharmaceutical Companies of Johnson & Johnson. Please visit **www.janssenrnd.com** for more information.

## Janssen in Oncology

In oncology, our goal is to fundamentally alter the way cancer is understood, diagnosed and managed, reinforcing our commitment to the patients who inspire us. In looking to find innovative ways to address the cancer challenge, our primary efforts focus on several treatment and prevention solutions. These include a focus on hematologic malignancies, prostate cancer and lung cancer; cancer interception with the goal of developing products that interrupt the carcinogenic process; biomarkers that may help guide targeted, individualized use of our therapies; as well as safe and effective identification and treatment of early changes in the tumor microenvironment. Please visit **oncology.janssenrnd.com**.

This press release contains "forward-looking statements" as defined in the Private Securities Litigation Reform Act of 1995 regarding product development. 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 and/or Johnson & Johnson. Risks and uncertainties include, but are not limited to: challenges and uncertainties inherent in new product development, including the uncertainty of clinical success and of obtaining regulatory approvals; competition, including technological advances, new products and patents attained by competitors; challenges to patents; changes to applicable laws and regulations, including global health care reforms; and trends toward health care cost containment. A further list and description 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 28, 2014, including in Exhibit 99 thereto, 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 or Johnson & Johnson undertakes to update any forward-looking statement as a result of new information or future events or developments.

[1]U.S. Food and Drug Administration. "Priority Review." Available at **http://www.fda.gov/forpatients/approvals/fast/ucm405405.htm**. Accessed September 2015.
[2]American Cancer Society. "Multiple Myeloma Overview." Available at **http://www.cancer.org/cancer/multiplemyeloma/detailedguide/multiple-myeloma-what-is-multiple-myeloma**. Accessed September 2015.
[3]National Cancer Institute. "A Snapshot of Myeloma." Available at **www.cancer.gov/research/progress/snapshots/myeloma**. Accessed September 2015.
[4]American Cancer Society. "What are the key statistics about multiple myeloma?"

**http://www.cancer.org/cancer/multiplemyeloma/detailedguide/multiple-myeloma-key-statistics**. Accessed September 2015.

[5] GLOBOCAN 2012: Estimated Cancer Incidence, Mortality and Prevalence Worldwide: Number of New Cancers in 2015. Available at: **http://globocan.iarc.fr/old/burden.asp?selection_pop=224900&Text-p=World&selection_cancer=17270&Text-c=Multiple+myeloma&pYear=3&type=0&window=1&submit=%C2%A0Execute**. Accessed August 2015.

[6] GLOBOCAN 2012: Estimated Cancer Incidence, Mortality and Prevalence Worldwide: Number of Cancer Deaths in 2015. Available at **http://globocan.iarc.fr/old/burden.asp?selection_pop=224900&Text-p=World&selection_cancer=17270&Text-c=Multiple+myeloma&pYear=3&type=1&window=1&submit=%C2%A0Execute**. Accessed August 2015.

[7]American Cancer Society. "How is Multiple Myeloma Diagnosed?" **http://www.cancer.org/cancer/multiplemyeloma/detailedguide/multiple-myeloma-diagnosis**. Accessed September 2015.

[8] Kumar, SK et al. Leukemia. 2012 Jan;26(1):149-57.

[9]Michael de Weers et al. Daratumumab, a Novel Therapeutic Human CD38 Monoclonal Antibody, Induces Killing of Multiple Myeloma and Other Hematological Tumors. The Journal of Immunology. February 1, 2011. Vol. 186, No. 3 1840-1848.

[10]Michael de Weers et al. Daratumumab, a Novel Therapeutic Human CD38 Monoclonal Antibody, Induces Killing of Multiple Myeloma and Other Hematological Tumors. The Journal of Immunology. February 1, 2011. Vol. 186, No. 3 1840-1848.

[11]Yulian Khagi and Tomer M Mark. Potential role of daratumumab in the treatment of multiple myeloma. Onco Targets Ther. 2014; 7: 1095-1100.

[12]Michael de Weers et al. Daratumumab, a Novel Therapeutic Human CD38 Monoclonal Antibody, Induces Killing of Multiple Myeloma and Other Hematological Tumors. The Journal of Immunology. February 1, 2011. Vol. 186 no. 3 1840-1848.

[13] Jansen JH, et al. Blood . 2012; 120.2974.

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