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News Release

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Janssen Presents Initial Results from the Phase 2 RAGNAR Study of Erdafitinib in Patients with Advanced Solid Tumours with FGFR Alterations

Data from RAGNAR, the largest tumour-agnostic study reported for a targeted therapy and the first to evaluate fibroblast growth factor receptor (FGFR)-driven malignancies, featured in oral presentation at the 2022 ASCO Annual Meeting[1]

June 7, 2022 (BEERSE, BELGIUM) – The Janssen Pharmaceutical Companies of Johnson & Johnson today announced initial results from the pivotal Phase 2 RAGNAR study evaluating the investigational use of erdafitinib, a fibroblast growth factor receptor (FGFR) kinase inhibitor, in patients with advanced solid tumours with prespecified FGFR alterations. At a planned interim analysis, responses were observed across a variety of FGFR-driven solid tumours for patients who had exhausted all standard of care options with meaningful clinical benefit or who could not tolerate available treatment options prior to being treated with erdafitinib.[1] These results will be featured in an oral presentation (Abstract #3007) today at the 2022 American Society of Clinical Oncology Annual Meeting.

RAGNAR (NCT04083976) is a Phase 2 clinical study designed to evaluate the efficacy and safety of erdafitinib in patients with advanced or metastatic solid tumours and prespecified FGFR gene alterations, regardless of tumour location or histology (tumour-agnostic).[1] The interim analysis was based on 178 patients with 32 distinct solid tumour histologies.[1] Patients in the study were prospectively identified by local molecular testing or central next-generation sequencing (NGS); the most common tumour types were cholangiocarcinoma

(bile duct cancer) (n=31), glioblastoma (tumour of the brain or spinal cord) (n=29), breast cancer (n=14), pancreatic cancer (n=13) and squamous non-small cell lung cancer (n=11).[1] The study also included tumours that occur less frequently, such as salivary gland and parathyroid carcinomas (rare endocrine malignancies), as well as tumours of unknown primary origin.[1] Study participants were heavily pre-treated, with 74.7 percent (n=133) having received two or more prior lines of therapy.[1]

The primary endpoint of the RAGNAR study is the overall response rate (ORR) as assessed by an independent review committee (IRC). At the interim analysis data cut off, IRC-assessed an ORR of 29.2 percent (95 percent confidence interval [CI], 22.7-36.5) and a disease control rate (DCR) of 72.5 percent (95 percent CI, 65.3-78.9) for the overall tumour-agnostic patient population.[1] Investigators observed responses in 14 distinct tumour types. This included responses in hard-to-treat malignancies such as salivary gland cancer (100 percent ORR; treated n=5, responders n=5), pancreatic cancer (30.8 percent ORR; treated n=13, responders n=4) and glioblastoma (20.7 percent ORR; treated n=29, responders=6).[1] Investigators also observed an overall 7.1-month median duration of response (DOR) (95 percent CI, 5.5-9.3). At the data cut off, 51.1 percent (n=24) of patients who had responded to treatment continued to show a response.[1] The primary analysis for all patients treated in this RAGNAR cohort, known as the broad panel cohort, is anticipated later this year.

"Diagnostic advances in the identification of FGFR gene alterations have opened the door to targeted, tumour-agnostic treatment approaches for patients," said Yohann Loriot, M.D., Ph.D., Institut Gustave Roussy and University of Paris-Saclay, and principal study investigator.[‡] "Results from the RAGNAR study show that through the targeted inhibition of FGFR receptors, we may be able to tailor treatment for patients with advanced FGFR-driven cancers, regardless of tumour location or histology."

The safety profile of erdafitinib observed in RAGNAR was consistent with the known safety profile of erdafitinib in metastatic urothelial carcinoma (mUC). Across tumour types, 44.9 percent of patients experienced drug-related treatment emergent adverse events of grade three or above. Adverse events were generally manageable with supportive care and treatment interruptions or reductions, when necessary.[1] The discontinuation rate due to drug-related adverse events was 7.3 percent.[1]

"The RAGNAR Phase 2 study is an important milestone for Janssen Oncology, as our first tumour-agnostic investigation. Here, we have seen initial results suggesting that erdafitinib may improve outcomes for a broader patient population who harbour specific FGFR genetic biomarkers. Through this research, we are advancing scientific understanding of the biology of disease and identifying new treatment pathways in an area of significant unmet need," said Martin Vogel, MD, EMEA Therapeutic Area Lead Oncology, Janssen-Cilag GmbH.

In 2019, erdafitinib was granted accelerated approval by the U.S. Food and Drug Administration (FDA) as a targeted therapy for adult patients with locally advanced or mUC with susceptible FGFR2 or FGFR3 alterations and who have progressed during or following at least one line of prior platinum-containing chemotherapy, including within 12 months of neoadjuvant or adjuvant platinum-containing chemotherapy.[2]

About FGFR Alterations

Fibroblast growth factor receptors are a family of receptor tyrosine kinases that help cells grow, survive and multiply; FGFRs play a key role in several biological processes including tissue repair, inflammatory response and metabolism.[3,4,5] Fusions or mutations in the genes that control FGFR (known as FGFR1–4 alterations) may lead to the development and progression of certain cancers by increasing tumour cell growth and survival.[4] Patients with advanced, FGFR-driven solid tumours who have exhausted standard treatment options typically face a poor prognosis.[6,7,8]

About the RAGNAR Study

RAGNAR (NCT04083976) is a Phase 2 clinical trial evaluating the safety and efficacy of erdafitinib in patients with advanced solid tumours, regardless of cancer type or tumour location (tumour-agnostic), driven by FGFR1–4 alterations. Patients in the trial have progressed on or after at least one line of systemic therapy and have no alternative standard treatment options. Following screening by local molecular testing or central NGS, patients are enrolled in four separate cohorts: a broad panel cohort of patients with pathogenic FGFR mutations or gene fusions (tumour histology included but was not limited to, cholangiocarcinoma (bile duct cancer), high- and low-grade glioma (tumour of the brain or spinal cord), breast, pancreatic, squamous and non-squamous non-small cell lung cancer, colorectal, endometrial, oesophageal, salivary gland, ovarian, duodenal (cancer occurring in

the first part of the small intestine), thyroid and cancer of unknown primary origin); an exploratory cohort of patients with other FGFR mutations; a cholangiocarcinoma expansion cohort; and a paediatric cohort of patients ages 6 to 17 with FGFR alterations.[1,9]

The primary endpoint of RAGNAR is IRC-assessed ORR. Key secondary endpoints include investigator-assessed ORR, DOR, DCR, clinical benefit rate, progression free survival, overall survival and incidence and severity of adverse events.[1]

About Erdafitinib

Erdafitinib is a once-daily oral pan-fibroblast growth factor receptor (FGFR) tyrosine kinase inhibitor being evaluated by Janssen Research & Development in Phase 2 and 3 clinical trials in patients with advanced urothelial cancer.[10,11]

In addition to RAGNAR, erdafitinib is being studied in clinical trials including the Phase 3 THOR (NCT03390504) study evaluating erdafitinib versus standard of care, consisting of chemotherapy (docetaxel or vinflunine) or anti-PD-1 agent pembrolizumab, in participants with advanced urothelial cancer and selected FGFR aberrations with disease progression following one or two prior lines of therapy; and the randomised Phase 2 THOR-2 study (NCT04172675) study examining erdafitinib versus investigator choice of intravesical chemotherapy in participants who received Bacillus Calmette-Guérin and recurred with high risk non-muscle-invasive bladder cancer.[12,13]

In 2008, Janssen Pharmaceutica NV entered into an exclusive worldwide license and collaboration agreement with Astex Pharmaceuticals to develop and commercialise erdafitinib.[11,14]

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 & Retina; Immunology; Infectious Diseases & Vaccines; Neuroscience; Oncology; and Pulmonary Hypertension.

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‡Dr. Loriot has served as a consultant to Janssen; he has not been paid for any media work.

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 erdafitinib. 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 materialise, actual results could vary materially from the expectations and projections of Janssen Research & Development, LLC, Janssen Pharmaceutica N.V., 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 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 2, 2022, including in the sections captioned "Cautionary Note Regarding Forward-Looking Statements" and "Item 1A. Risk Factors," and in Johnson & Johnson's subsequent Quarterly Reports on Form 10-Q and other filings with the Securities and Exchange Commission. Copies of these filings are available online at, 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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