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Janssen Receives Health Canada Approval of BALVERSA™ (erdafitinib), the First FGFR Kinase Inhibitor for the Treatment of Patients with Locally Advanced or Metastatic Urothelial Carcinoma with Certain FGFR Genetic Alterations

Diagnostic testing is available to identify which patients are most likely to benefit from BALVERSA™, offering a personalized treatment approach in bladder cancer

Toronto, ON, February 24, 2020 – The Janssen Pharmaceutical Companies of Johnson & Johnson recently received Health Canada Notice of Compliance with conditions (NOC/c), approving BALVERSA™ (erdafitinib) for the treatment of adults with locally advanced or metastatic urothelial carcinoma (UC) which has susceptible fibroblast growth factor receptor (FGFR)3 or FGFR2 genetic alterations and who have progressed during or following at least one line of prior chemotherapy, including within 12 months of neoadjuvant or adjuvant chemotherapy.¹ This approval marks the first oral FGFR kinase inhibitor approved by Health Canada for patients with locally advanced or metastatic urothelial carcinoma (bladder cancer).

Health Canada approved the therapy on the condition that Janssen Inc. carries out a confirmatory trial to verify the clinical benefit of BALVERSA.² The NOC/c policy provides access to promising new drugs for patients diagnosed with serious, life-threatening or severely debilitating diseases, or conditions for which no drug is currently marketed in

Canada, or for which a significant increase in efficacy or significant decrease in risk is demonstrated in relation to existing drugs marketed in Canada.³

"Diagnostic testing for patients with bladder cancer allows healthcare professionals to offer patients more personalized treatment approaches. This is an important step forward when determining the right therapy for the right patient," said Dr. Scott North,* Professor, Department of Oncology, University of Alberta. "This subset of patients with urothelial carcinoma have had limited treatment options up until now, so this approval is welcome news."

BALVERSA™, a once-daily oral FGFR kinase inhibitor, was issued an NOC/c based on results from a Phase 2 clinical trial (BLC2001, [NCT02365597](#)), a multicenter, open-label, single-arm study, of 87 patients with disease that had progressed on or after at least one prior chemotherapy and that had at least one of the following genetic alterations: FGFR3 gene mutations (R248C, S249C, G370C, Y373C) or FGFR gene fusions (FGFR3-TACC3, FGFR3-BAIAP2L1, FGFR2-BICC1, FGFR2-CASP7), as determined by a clinical trial assay performed at a central laboratory.⁴ The median duration of therapy was 5.3 months, and the median duration of efficacy follow-up was 11.3 months.⁵ BALVERSA™ was administered at a starting dose of 8 mg, with an option to increase the dose to 9 mg daily for patients whose serum phosphate levels were below the target level.⁶ The results demonstrated a 40.2 per cent objective response rate (ORR) as assessed by Investigator Assessment [95 per cent CI(29.9, 50.5)].⁷ In the trial, ORR was defined as the percentage of patients with measurable lesions achieving a complete response (CR) [3.4 per cent] or partial response (PR) [36.8 per cent]⁸ to treatment using the Response Evaluation Criteria in Solid Tumors Version 1.1 (RECIST v1.1) criteria, a standard way to measure how well a patient responds to treatment based on whether tumors shrink, stay the same, or get bigger as assessed per investigator.⁹ Results also showed a median duration of response (DoR) of 5.6 months [95 per cent CI(4.2, 7.0)] in patients treated with BALVERSA™.¹⁰ Data from the BLC2001 study were recognized as a "Best of ASCO" selection at the American Society of Clinical Oncology (ASCO) 2018 Annual Meeting and were published in [The New England Journal of Medicine](#).¹¹

The most common treatment-emergent adverse events (TEAEs) ≥20 per cent from the clinical trial were hyperphosphatemia (76 per cent), stomatitis (56 per cent), diarrhea (47 per cent), dry mouth (45 per cent), decreased appetite (38 per cent), dysgeusia (37 per cent), dry skin (35 per cent), fatigue (33 per cent), constipation (28 per cent), alopecia (26

per cent), palmar-plantar erythrodysesthesia syndrome (26 per cent), asthenia (23 per cent), nausea (21 per cent), onycholysis (20 per cent), dry eye (20 per cent), and anemia (20 per cent).¹² The most common Grade 3-4 adverse reactions (≥ 2 per cent) were: stomatitis, nail dystrophy, palmar-plantar erythrodysesthesia syndrome, paronychia, nail disorder, keratitis, onycholysis, and hyperphosphatemia.¹³

About Urothelial Carcinoma

Urothelial carcinoma starts in the lining of the bladder called the urothelium. It is the most common type of bladder cancer, making up approximately 90 per cent of all bladder cancers.¹⁴ In 2019, there were an estimated 11,800 Canadians diagnosed with bladder cancer.¹⁵ Urothelial carcinomas are often diagnosed at an early stage and have not grown into a deeper muscle layer of the bladder wall.¹⁶ However, up to 25 per cent will have muscle-invasive disease or develop metastatic disease.¹⁷ About one in five patients with muscle-invasive urothelial carcinoma have a FGFR genetic alteration.¹⁸ FGFRs are a family of receptor tyrosine kinases which can be activated by genetic alterations in a variety of tumor types, and these alterations may lead to increased tumor cell growth and survival.¹⁹ BALVERSA™ is approved specifically for the treatment of patients with locally advanced or metastatic urothelial carcinoma harboring FGFR3 or FGFR2 genetic alterations, which can be detected through diagnostic testing, offering a personalized treatment approach.

About BALVERSA™ (erdafitinib)

BALVERSA™ (erdafitinib) is a once-daily, oral fibroblast growth factor receptor (FGFR) kinase inhibitor indicated for the treatment of adults with locally advanced or metastatic urothelial carcinoma (UC) which has susceptible FGFR3 or FGFR2 genetic alterations and who have progressed during or following at least one line of prior chemotherapy, including within 12 months of neoadjuvant or adjuvant chemotherapy.²⁰ Treatment with BALVERSA should be initiated following confirmation of a susceptible FGFR genetic alteration using a validated test.²¹

The pivotal multicenter, open-label Phase 2 BLC2001 (NCT02365597) clinical trial evaluated the efficacy and safety of BALVERSA™ for the treatment of adults with advanced urothelial cancer whose tumors have certain FGFR alterations. In 2008, Janssen entered into an exclusive worldwide license and collaboration agreement with Astex Pharmaceuticals to develop and commercialize BALVERSA™.

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/canada. Follow us at [@JanssenCanada](https://twitter.com/JanssenCanada). Janssen Inc. is part of the Janssen Pharmaceutical Companies of Johnson & Johnson.

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**Dr. North was not compensated for any media work. He has been compensated as a consultant.*

Cautions Regarding Forward-Looking Statements

This press release contains "forward-looking statements" as defined in the Private Securities Litigation Reform Act of 1995 regarding BALVERSA™ (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 materialize, actual results could vary materially from the expectations and projections of Janssen Inc., 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 30, 2018, 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.jnjcanada.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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- ¹ BALVERSA™ (erdafitinib) Product Monograph, Janssen Inc. October 25, 2019.
- ² BALVERSA™ (erdafitinib) Product Monograph, Janssen Inc. October 25, 2019.
- ³ Government of Canada. "Guidance Document: Notice of Compliance with Conditions (NOC/c)." Available from: <https://www.canada.ca/en/health-canada/services/drugs-health-products/drug-products/applications-submissions/guidance-documents/notice-compliance-conditions.html#a1.1>. Accessed January 2020.
- ⁴ BALVERSA™ (erdafitinib) Product Monograph, Janssen Inc. October 25, 2019.
- ⁵ BALVERSA™ (erdafitinib) Product Monograph, Janssen Inc. October 25, 2019.
- ⁶ BALVERSA™ (erdafitinib) Product Monograph, Janssen Inc. October 25, 2019.
- ⁷ BALVERSA™ (erdafitinib) Product Monograph, Janssen Inc. October 25, 2019.
- ⁸ BALVERSA™ (erdafitinib) Product Monograph, Janssen Inc. October 25, 2019.
- ⁹ National Cancer Institute. NCI Dictionary of Cancer Terms. Available from: <https://www.cancer.gov/publications/dictionaries/cancer-terms/def/recist>. Accessed November 2019.
- ¹⁰ BALVERSA™ (erdafitinib) Product Monograph, Janssen Inc. October 25, 2019.
- ¹¹ Lorient Y, Necchi A, Park SE. "Erdafitinib in Locally Advanced or Metastatic Urothelial Carcinoma." *N Engl J Med* 2019; 381:338-348. July 25, 2019.
- ¹² BALVERSA™ (erdafitinib) Product Monograph, Janssen Inc. October 25, 2019.
- ¹³ BALVERSA™ (erdafitinib) Product Monograph, Janssen Inc. October 25, 2019.
- ¹⁴ Cancer Care Ontario. "Bladder Cancer." Available from: <https://www.cancercareontario.ca/en/types-of-cancer/bladder>. Accessed November 2019.
- ¹⁵ Canadian Cancer Society. "Bladder Cancer Statistics." Available from: <https://www.cancer.ca/en/cancer-information/cancer-type/bladder/statistics/?region=bc>. Accessed November 2019.
- ¹⁶ Canadian Cancer Society. "What is bladder cancer?" Available from: <https://www.cancer.ca/en/cancer-information/cancer-type/bladder/bladder-cancer/?region=sk>. Accessed November 2019.
- ¹⁷ Sridhar S S. Evolving Treatment of Advanced Urothelial Cancer. *Journal of Oncology Practice* 13, no. 5 (May 01, 2017) 309-315. Available from: <https://ascopubs.org/doi/full/10.1200/JOP.2017.022137>. Accessed January 2020.
- ¹⁸ Kim Y S, Kim K, Kwon G-Y, et al. Fibroblast growth factor receptor 3 (FGFR3) aberrations in muscle-invasive urothelial carcinoma. *BMC Urol*. 2018, Jul 31; 18: 68. Available from: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6069868/>. Accessed January 2020.
- ¹⁹ Dienstmann R, Rodon J, Prat A, et al. Genomic aberrations in the FGFR pathway: Opportunities for targeted therapies in solid tumors. *Ann Oncol*. 2014;25:552–563.
- ²⁰ BALVERSA™ (erdafitinib) Product Monograph, Janssen Inc. October 25, 2019.
- ²¹ BALVERSA™ (erdafitinib) Product Monograph, Janssen Inc. October 25, 2019.