Balversa® (erdafitinib) – Updated indication, accelerated approval converted to full approval

- On January 19, 2024, Johnson & Johnson announced the full FDA approval of Balversa (erdafitinib), for the treatment of adult patients with locally advanced or metastatic urothelial carcinoma (mUC) with susceptible FGFR3 genetic alterations whose disease has progressed on or after at least one line of prior systemic therapy.
    - Balversa is not recommended for the treatment of patients who are eligible for and have not received prior PD-1 or PD-L1 inhibitor therapy.
- This FDA action converts the April 2019 accelerated approval of Balversa to a full approval and updates the indication.
    - The accelerated approval was for the treatment of adult patients with locally advanced or mUC, that has: (1) susceptible FGFR3 or FGFR2 genetic alterations, and (2) progressed during or following at least one line of prior platinum-containing chemotherapy, including within 12 months of neoadjuvant or adjuvant platinum-containing chemotherapy.
- The full approval of Balversa for this indication was based on BLC3001 Cohort 1, a randomized, open-label study in 266 patients with advanced UC harboring selected FGFR3 alterations. All patients needed to have had disease progression after 1 or 2 prior treatments, at least 1 of which included a PD-1 or PD-L1 inhibitor. Patients were randomized to receive Balversa vs. chemotherapy until unacceptable toxicity or progression. The major outcome measures were overall survival (OS), progression-free survival (PFS), and objective response rate (ORR).
    - Median OS was 12.1 months for Balversa vs. 7.8 months for chemotherapy (hazard ratio [HR] 0.64, 95% CI: 0.47, 0.88; p = 0.0050).
    - Median PFS was 5.6 months for Balversa vs. 2.7 months for chemotherapy (HR 0.58, 95% CI: 0.44, 0.78; p = 0.0002).
    - The ORR was 35.3% (95% CI: 27.3, 43.9) for Balversa vs. 8.5% (95% CI: 4.3, 14.6) for chemotherapy (p < 0.001).
- Study BLC3001 also included Cohort 2, which was an open-label, randomized study in 351 patients with locally advanced or mUC with selected FGFR3 alterations who received 1 prior line of systemic therapy and no prior PD-1 or PD-L1 inhibitor. Patients were randomized to receive Balversa or Keytruda® (pembrolizumab).
    - The study did not meet its major outcome measure for superiority of OS at the pre-specified final analysis. The OS HR was 1.18 (95% CI: 0.92, 1.51; p = 0.18), median 10.9 (95% CI: 9.2, 12.6) months for Balversa vs. 11.1 (95% CI: 9.7, 13.6) months for Keytruda.
- The recommended starting dose of Balversa is 8 mg (two 4 mg tablets) orally once daily, with a dose increase to 9 mg (three 3 mg tablets) once daily based on tolerability, including hyperphosphatemia, at 14 to 21 days. Treatment should continue until disease progression or unacceptable toxicity occurs.

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— Patients should be selected for treatment based on the presence of susceptible FGFR3 genetic alterations in tumor specimens as detected by an FDA-approved companion diagnostic. Information on FDA-approved tests for the detection of FGFR3 genetic alterations in urothelial cancer is available at: http://www.fda.gov/CompanionDiagnostics.

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