Interim analysis of Phase 2 results for cemiplimab in patients (pts) with metastatic basal cell carcinoma (mBCC) who progressed on or are intolerant to hedgehog inhibitors (HHIs)


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Synopsis: HHIs are approved to treat pts w/mBCC or locally advanced (la) BCC who are not candidates for surgery/radiation; there is no approved option for pts after disease progression on/intolerance to HHIs. Cemiplimab is a programmed cell death-1 monoclonal antibody approved to treat pts w/metastatic or la cutaneous squamous cell carcinoma who are not candidates for curative surgery/radiation.

Objective/Purpose: We present this prespecified interim analysis of mBCC pts from the pivotal Phase 2, non-randomized, multi-center study of cemiplimab in pts w/advanced BCC who discontinued HHI therapy (NCT03132636).

Methods: mBCC pts received cemiplimab 350 mg intravenously Q3W; interim analysis included pts who could be followed for ~57 weeks. Primary endpoint: objective response rate (ORR) per independent central review (ICR). Secondary objectives: assessment of safety and tolerability, duration of response (DOR), progression-free survival (PFS), and overall survival (OS).

Results: Of 28 pts (82.1% males; median age: 65.5 years [range 38−90]), 6 pts had partial response. ORR per ICR: 21.4% (95% CI, 8.3, 41.0). ORR per investigator assessment: 28.6% (95% CI, 13.2, 48.7). Observed DOR among responders: 9−23 months. Median time to response per ICR: 3.2 months (range, 2.1−10.5). Median Kaplan–Meier estimation of PFS and OS: 8.3 and 25.7 months, respectively. Median DOR had not been reached. Disease control rate: 67.9% (95% CI, 47.6, 84.1). Most common treatment-emergent adverse events (TEAEs) regardless of attribution: fatigue (50.0%), diarrhea (35.7%), pruritus (25.0%), and constipation (25.0%). Hypertension (n=2) was the only Grade ≥3 TEAE regardless of attribution occurring in ≥2 pts. TEAEs leading to death occurred in 1 (3.6%) pt who died from staphylococcal pneumonia, considered unrelated to study treatment.

Conclusion: Cemiplimab is the first agent to provide clinically meaningful anti-tumor activity in pts w/mBCC after disease progression on/intolerance to HHIs.

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