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High-Specific-Activity Iodine-131-Meta-Iodo-Benzylguanidine for the Treatment of Advanced Pheochromocytoma and Paraganglioma: A Real-World Study

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BACKGROUND

Metastatic pheochromocytomas and paragangliomas are rare neuroendocrine tumors with limited treatment options. We studied the efficacy and safety of off-label High-Specific-Activity I-131-meta-iodobenzylguanidine (HSA-I-131-MIBG) in routine clinical practice.

METHODS

This is a retrospective cohort study. The primary endpoint is objective response rate (ORR) as per RECIST v1.1. Secondary endpoints are blood pressure control, safety, overall and progression free survival rates, duration of response, and correlations with genetic background.

RESULTS

25 patients were studied. 62% were men. Median age at the time of treatment was 43 years (range 18-82). 68% had hormonally active tumors.52% previously received antineoplastic treatment. 60% received two doses of HSA-I-131-MIBG. Median duration of follow up was 19 months (2-61). 24 patients were evaluable for radiographic response. The ORR was 33%, including two patients with complete response (CR). The disease control rate (DCR) was 83%. Median time to response was 12.5 months (95% CI, 4.6 to 25.1). Twelve patients had sporadic disease; ORR was 25% and DCR was 92%. Twelve patients had hereditary disease (SDHB, VHL, RET); ORR was 42%, DCR was 75%. Two SDHB carriers achieved CR. Plasma metanephrines normalized in 27%, improved by at least 50% in 45%. 16 patients had hypertension; blood pressure normalized leading to discontinuation of antihypertensive therapy in 56% of patients with hormonally active tumors and hypertension.

The most common adverse events were grade I/II nausea/vomiting and transient bone marrow suppression. One patient developed premature ovarian failure. Grade III/IV myelosuppression was seen in 28% of patients. One patient had a fatal pneumonitis (probably related) and 1 patient had grade 5 gastrointestinal bleeding (not related).

CONCLUSIONS

HSA 131-I-MIBG is associated with a high DCR regardless of underlying genetic mutation. Severe adverse events are mainly transient and correctable bone marrow insufficiencies.

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