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MIBG and DOTATATE Therapy for Pheochromocytoma and Paraganglioma: A Single Institution Experience

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BACKGROUND

Since the FDA-approval of Lu177-DOTATATE and I131-MIBG radiopharmaceutical therapies in 2018, there is emerging real-world experience with their use. Only a few institutions in the US have both therapies available. Here, we describe our experience with these agents for patients with pheochromocytoma and paraganglioma (PPGL).

METHODS

This is a retrospective evaluation of all patients with progressive, metastatic, PPGL referred for radiopharmaceutical therapy at our institution since 2018. Parameters evaluated include therapy eligibility, side effects and toxicity, and outcomes to date. At our institutions, the choice of treatment is first guided by the degree of uptake on functional imaging, followed by the FDA-label for the therapy.

RESULTS

A total of 17 PPGL patients have been referred to date (all but two with paraganglioma). Five were not treated with a radiopharmaceutical due to a variety of factors such as stable or limited disease, rapid progression, insufficient uptake on imaging, or patient choice. Six were treated with Lu177-DOTATATE (average age 60 (range 30-80)) due to higher SSTR-expression compared to MIBG uptake. All had paraganglioma (three with SDHx mutations, others unknown). Five completed 4 cycles of therapy with minimal side effects and transient cytopenias. Of those five, three have stable disease or partial response, and two had progression within 6 months. Two had marked improvement in quality of life and/or decrease in hypertensive medications. The last patient is currently receiving active therapy. Five were treated with I-131 MIBG (average age 56 (range 31-68)). All had similar SSTR-expression and MIBG uptake, thus were treated with the FDA-approved therapy. Four had paraganglioma, and 2 had pheochromocytoma (3 SDHx mutations, others unknown). Four completed 2 cycles, and two only 1 cycle. Four patients had transient cytopenias, and two had clinically significant thrombocytopenia. One had a complete response, two with partial response, one with stable disease, and 2 with progression within 6 months. Three patients had an improvement in quality of life and/or decrease in hypertensive medications.

CONCLUSIONS

Real-world data show that both DOTATATE and MIBG therapies have a role in the systemic therapy of patients with progressive, metastatic PPGL, with similar outcomes with regard to efficacy and toxicity. Pre-therapy functional imaging can be used to guide the therapeutic choice. Additional data is needed for confirmation of the findings from this small cohort.

ABSTRACT ID 21473