C-34

Outcomes of Peptide Receptor Radionuclide Therapy in Patients with Pheochromocytoma and Paraganglioma: A Center of Excellence Experience

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BACKGROUND

Pheochromocytomas (PCs) and paragangliomas (PGs) are rare neuroendocrine tumors with variable 5-year survival outcomes and limited treatment options. Peptide Receptor Radionuclide Therapy (PRRT) is a relatively novel treatment option that can be administered systemically to treat patients with advanced somatostatin-positive PCs and PGs. This study aims to evaluate the safety and efficacy of PRRT in patients with metastatic/inoperable PCs and PGs.

METHODS

This is a single-center retrospective case series. Records of patients with metastatic or inoperable PCs or PGs treated with PRRT were selected based on participation in the Iowa NET registry. Patient characteristics, adverse events, biochemical response, blood pressure response, and imaging response based on RECIST 1.1 criteria were abstracted. Statistical analyses were performed using RStudio. P-values were calculated using Fisher's exact test for categorical variables and t-test for continuous variables.

RESULTS

A total of 11 patients received PRRT, including 2/11 (18.2%) with PC and 9/11 (81.8%) with PG. Among patients on first restaging imaging, 3/11 (27%) demonstrated partial response (PR), 6/11 (55%) had stable disease (SD), and 2/11 (18%) had progressive disease (PD). Both PC patients exhibited SD on restaging. Among patients with PG, 3/9 (33%) demonstrated PR, 4/9 (44%) with SD, and 2/9 (22%) with PD. Of the patients with non-functional tumors, 1/4 (25%) had PD and 3/4 (75%) had SD. Among functional tumor cases, 3/7 (43%) achieved PR, 3/7 (43%) SD, and 1/7 (14%) PD. No significant difference in likelihood of partial response was observed between functional and non-functional tumor groups ($p = 0.24$). Median progression-free survival (PFS) and overall survival (OS) were not reached at a median follow-up of 11 months. PRRT was well tolerated overall, with the most common adverse effect following PRRT being nausea (2/11, 18.2%). No grade 3 or 4 toxicities were reported. Changes in systolic blood pressure (SBP) were -4.5 mm Hg in patients with PC and +5.9 mm Hg in those with PG ($p=0.40$). Diastolic blood pressure (DBP) changes were -8.0 mm Hg and +0.7 mm Hg, respectively ($p=0.21$). The median change in chromogranin A (CgA) after PRRT was -8.5 ($n=6$, IQR: -84.75 to 298; $p = 0.41$).

CONCLUSIONS

PRRT demonstrated tolerability and efficacy for the treatment of patients with advanced PCs and PGs. Our findings are consistent with the existing literature. Future prospective randomized controlled studies are needed to further assess the efficacy of PRRT in treating this challenging patient group.

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