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Quality of Life Assessments for Advanced Pheochromocytoma and Paraganglioma Patients that Received High-Specific-Activity I-131 MIBG: Results from a Pivotal Phase 2 Clinical Trial

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BACKGROUND

High-specific activity iodine-131 metaiodobenzylguanidine (HSA I-131 MIBG; AZEDRA®) is the only FDA approved systemic treatment for locally advanced or metastatic pheochromocytoma or paraganglioma (PPGL). We have previously described pivotal study efficacy data that served as the basis for HSA I-131 MIBG approval demonstrating improvements in blood pressure control, objective tumor responses, and biomarker responses. Here we provide the results from a European Organization for the Research and Treatment of Cancer Quality of Life Questionnaire (EORTC QLQ-C30 v3) developed and validated to assess the quality of life (QoL) of cancer patients.

METHODS

Patients with iobenguane-avid PPGL who were ineligible for surgery, failed prior therapy or not candidates for chemotherapy were eligible to receive treatment. Patients received up to two therapeutic doses, each at ~500 mCi (18.5 GBq), administered ~90 days apart. QoL assessments, designed to measure cancer patients' physical, psychological, and social functions, were measured by patient reporting of the EORTC QLQ-C30 v3. Questionnaires were administered at screening/baseline before the first therapeutic dose, at Weeks 3, 6, 10, 12, 15, 18, and 22, and monthly at months 6 to 12 following the first therapeutic dose. Best response within 12 months post-therapeutic dose 1 were determined. A high score (scale of 0 to 100) for overall global health status/QoL represents a high QoL. A high score (scale of 0 to 100) for the functional scales represents a high/healthy level of functioning. A high score (scale of 0 to 100) for a symptom scale/item represents a high level of symptomatology/problems.

RESULTS

An improvement in mean±SD and median Global Health Status/QoL from baseline (n=57) compared with best response was observed (59.8±19.8 vs. 77.8±17.7 (+18.0); and 58.3 vs. 83.3 (+25.0), respectively). For each parameter of the five functional scales, mean scores after baseline (n=58) suggest an improvement in function that was sustained for at least 12 months: Role (64.3±29.9 vs. 86.2±19.8 (+21.9)); Social (67.7±29.2 vs. 90.2±20.0 (+22.5)); Physical (72.3±20.7 vs. 88.7±14.1 (+16.4)); Emotional (73.7±21.7 vs. 93.1±11.7 (+19.4)); and Cognitive (80.8±21.9 vs. 96.8±8.39 (+16.0)). For symptom scales (fatigue, pain, financial difficulties, insomnia, dyspnea, constipation, appetite loss, nausea and vomiting, and diarrhea), the best response mean scores after baseline suggest an improvement for all symptoms ranging from -26.5 (pain) to -6.2 (diarrhea).

CONCLUSIONS

In a pivotal clinical study, advanced PPGL patients' physical, psychological, and social functions were all improved over baseline consistent with improvements in reported efficacy outcomes when treated with HSA I-131 MIBG.

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