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Abstract

A phase II trial of imatinib in patients with advanced carcinoid tumor

K. Carr, J. C. Yao, A. Rashid, S.-C. Yeung, J. Szklaruk, J. Baker, J.-N. Vauthey, S. Curley, L. Ellis and J. A. Ajani

University of Texas M. D. Anderson Cancer Center, Houston, TX

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Background: Effective systemic therapy options for carcinoid tumors are lacking. Carcinoid tumors co-express PDGF and PDGFR, a receptor/ligand system important in cell growth and survival. A phase II trial was conducted to assess the response rate and safety profile of PDGFR inhibitor, imatinib (Gleevec ^{TM}), at a dose of 400 mg PO BID in patients with advanced carcinoid tumors. **Methods:** Previously treated or untreated patients with advanced carcinoid tumors with adequate organ and bone marrow function were included. Response was evaluated every 12 weeks by CT or MRI. **Results:** 15 men and 12 women with a median age of 60 (22–74) enrolled. 21 patients received concurrent octreotide. 10, 6, 3 and 2 patients received prior chemotherapy, hepatic artery embolization, interferon, and radiation, respectively. Median number of weeks on study was 16 (range, 12–78+). By RECIST criteria, 1 patient, receiving concurrent octreotide, had a radiologic PR (remains on study after 1.5 years), 17 had SD, and 9 with PD. Among 14 patients with PD at entry, 8 patients remained progression free for at least 12 weeks (range, 18–52+). 4 patients had > 50% reduction in markers (chromogranin, 5-HIAA, pancreatic polypeptide). Median PFS duration is 24 weeks (range, 12–68+). PFS duration was significantly better in patients receiving concurrent octreotide (14 weeks vs 38 weeks; P=0.05). 1-year OS rate is 88%. There was no correlation between plasma VEGF and bFGF, CT flow studies and patient outcome. Grade 3–4 toxicities included fatigue (7), hypophosphatemia (5), diarrhea (3), fluid retention (3), nausea (2), granulocytopenia (2), hypokalemia (2), rash (1), anorexia (1), thrombocytopenia (1), hyperglycemia (1), and hyperbilirubinemia (1). **Conclusions:** Our data suggest a modest level of biologic effect of imatinib in carcinoid, therefore, further development of imatinib in conjunction with other agents may be warranted. Supported by a grant from Novartis.