

BASELINE DISEASE CHARACTERISTICS AND SYMPTOMS IN PATIENTS WITH METASTATIC MEDULLARY THYROID CANCER (MTC) ENROLLED IN THE PHASE 3 EXAM TRIAL

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Background/Purpose: Symptomatology and disease burden in progressive, metastatic MTC are not well characterized. Patients in the EXAM trial placebo arm had short median progression-free survival (4.0 months). We analyzed baseline characteristics in EXAM to define the study population, and characterize disease burden and symptomatology in patients with progressive MTC as a reference for future research. **Methods:** EXAM randomized 330 patients with MTC and radiographic evidence of disease progression (≤ 14 months between reference and screening scans) by mRECIST to cabozantinib or placebo. Patient baseline symptoms (CTCAE v3.0) and disease characteristics were collected and analyzed descriptively. **Results:** At baseline, 312 (94.6%) patients had measurable disease (independent radiology review), and most (87.0%) had metastatic disease at ≥ 2 sites/organs, including lymph nodes (79.1%), liver (66.4%), lungs (54.5%), and bone (50.9%). Median age 55 years (20-86), mean bodyweight 73.4 kg (± 18.5), male (67.0%), white (89.4%), and thyroidectomized (92.4%). Median interval between initial MTC diagnosis and randomization was 3.9 years (0.07-48.4); median interval between metastatic disease diagnosis and randomization was 2.0 years (0.04-33.7). Overall, 39% had prior systemic anticancer therapy and 20.6% had prior tyrosine kinase inhibitor therapy (10.3% vandetanib). Frequent patient-reported baseline symptoms were mainly grade 1-2, and included pain (46.1%), diarrhea (39.7%), fatigue (25.8%), dyspnea (16.1%), cough (12.1%), dysphagia (9.1%), anorexia (7%), weight loss (5.5%), and flushing (4.2%). **Discussion & Conclusion:** Although MTC is generally regarded as an indolent disease, patients with radiographic evidence of progressive MTC have a substantial disease burden and high incidence of disease-related symptoms.