Vandetanib for the Treatment of Advanced Medullary Thyroid Cancer Outside a Clinical Trial: Results from a French Cohort

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Titre Vandetanib for the Treatment of Advanced Medullary Thyroid Cancer Outside a Clinical Trial: Results from a French Cohort

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BACKGROUND:
A randomized phase III trial demonstrated that vandetanib treatment is effective in patients with metastatic medullary thyroid cancer (MTC), leading to regulatory approval, but its use may be associated with toxicities that require specific monitoring and management. The objective of the present study performed in France was to describe the toxicity profile and efficacy of vandetanib treatment when given outside any trial.

METHODS:
Sixty-eight patients were treated with vandetanib in the frame of a temporary use authorization (ATU) in France from August 2010 to February 2012, when the drug was available on request for patients with locally advanced or metastatic MTC. Patients were registered by the French health authorities, and characteristics, treatment parameters, toxicity profile, and efficacy were retrospectively reviewed. Eight patients were excluded from the analysis because vandetanib treatment was not administered (n=3), had been given in a trial before ATU (n=3), or was given for a non-MTC cancer (n=2).

RESULTS:
Data from the 60 MTC patients were analyzed. Mean age was 58 years (range 11-83 years), 39 patients were male, and six had hereditary MTC. Fifty-six (93%) had metastatic disease in the mediastinum (82%), bones (65%), liver (53%), or lung (53%), and four had only locally advanced disease. At the time of study evaluation, with a median follow-up of 20 months and a median duration of treatment of 9.7 months (range 0.3-36 months), 15 patients were continuing vandetanib treatment (range 18-36 months). Median progression-free survival was 16.1 months. Twenty-five patients discontinued treatment for disease progression (range 0.3-29 months). Best tumor response was a complete response in one patient, a partial response in 12 (20%), stable disease in 33 (55%), and progression in seven patients (12%). All patients had at least one adverse event (AE) during treatment. The main AEs were skin toxicity, diarrhea, and asthenia. Sixteen patients (27%) discontinued treatment for toxicity, and one patient died from vandetanib-induced cardiac toxicity.

CONCLUSIONS:
Vandetanib is an effective option for patients with advanced MTC. AEs should be monitored carefully and should be minimized by educating both patients and care providers and by applying symptomatic treatment and dose reduction.

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