RESEARCH HIGHLIGHTS

EXPERIMENTAL THERAPIES

Vandetanib—a new treatment for MTC?

At present, there is no effective treatment available for patients with metastatic medullary thyroid cancer (MTC). However, Samuel Wells and colleagues now present evidence that vandetanib therapy might succeed in filling this void in therapeutic options. "Preclinical data from Massimo Santoro's group indicated that this small molecule might have clinical efficacy in patients with advanced MTC," explains Wells. "We worked with the parent company, AstraZeneca, to develop the clinical trial."

In an open-label, single-arm, phase II trial, the investigators treated 30 patients with unresectable locally advanced or metastatic hereditary MTC with 300 mg vandetanib. The drug, which was given until disease progression, unacceptable toxicity or patient withdrawal occurred, is a selective oral inhibitor of 'rearranged during transformation' (RET)-dependent signaling—mutations in the *RET* protooncogene are known to be associated with MTC. The researchers found that

six participants achieved an objective partial response, according to Response Evaluation Criteria In Solid Tumors (RECIST) criteria. Furthermore, a measurable reduction in tumor size was observed in 25 individuals and disease control (stable disease ≥24 weeks) was achieved in 22 patients.

"The incidence of partial responses, the prolonged duration of disease control, and the manageable adverse event profile suggest that vandetanib may provide an effective therapeutic option in patients with locally advanced or hereditary MTC," conclude the authors. Indeed, three subsequent clinical trials have been initiated to further evaluate the efficacy of vandetanib in the treatment of MTC.

Rowan Higgs

Original article Wells, S. A. Jr *et al.* Vandetanib for the treatment of patients with locally advanced or metastatic hereditary medullary thyroid cancer. *J. Clin. Oncol.* **28**, 767–772 (2010)