

Targeted agent selumetinib shows promise in biliary cancer

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The experimental agent selumetinib has shown promising results in people with advanced biliary cancer, according to a multi-institutional clinical trial led by cancer researchers at The Ohio State University.

Selumetinib, also known as AZD6244 (ARRY-142886), blocks a protein called MEK, which [cancer cells](#) need to proliferate and survive.

Biliary cancer is a malignancy of cells lining the [bile ducts](#) and gall bladder. About 100,000 patients are diagnosed annually worldwide with the disease, representing 15-20 percent of all liver-cancer cases. Most patients present at later stages of the disease, which has a universally poor outcome.

The findings of this 28-patient, multi-institutional phase II study are reported online in the [Journal of Clinical Oncology](#).

"This malignancy has no good standard of care," says principal investigator Dr. Tanios Bekaii-Saab, medical director of gastrointestinal oncology and a medical oncologist at Ohio State's Comprehensive Cancer Center-James Cancer Hospital and Solove Research Institute.

"Our study provides a strong rationale for developing this agent further in larger trials, probably in combination with other drugs, which we hope will enable us to establish a new standard of care for biliary cancers in the near future," adds Bekaii-Saab.

In addition to Ohio State, trial participants included the University of North Carolina, Vanderbilt University and Emory University.

Selumetinib belongs to a class of drugs called protein-kinase inhibitors. The agent selectively inhibits the protein kinases MEK1 and MEK2. It is part of a signaling pathway that is often damaged in biliary cancer cells. The trial showed the following:

- One patient responded completely – the tumor shrunk until it was undetectable – and two patients showed partial tumor shrinkage;
- In 17 patients, the tumor stopped growing. This condition of stable disease lasted up to 16 weeks in the majority of cases;
- Patients experienced no cancer progression for a promising 3.7 months on average, despite the fact that nearly 40 percent of patients had prior therapy before receiving selumetinib (such tumors tend to be more resistant to treatment);
- Patients taking the drug regained weight – about 9 pounds on average. This included patients with tumors that may not have responded to the drug, Bekaii-Saab notes.

Patients who lacked a target protein called pERK did not seem to respond to the drug, suggesting that the drug may not work if the protein is missing in the [cancer](#) cells. "This finding suggests that in the future we may be able to identify which patients are most likely to respond to the drug," he adds.

Provided by Ohio State University Medical Center

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