Pre-surgical Combination Therapy Boosts Survival of Patients With Rare Thyroid Cancer

Adding immunotherapy to targeted therapy regimen before surgery increased overall survival from an average of 13 to 21 months.

June 2, 2025 By MD Anderson Cancer Center

A pre-surgical combination therapy including pembrolizumab [Keytruda] plus dabrafenib [Tafinlar] and trametinib [Mekinist] (DTP) significantly improved survival in patients with rare BRAF V600E-mutated anaplastic thyroid cancer (BRAFm-ATC) compared with historical controls, according to new research from The University of Texas MD Anderson Cancer Center.

Results from the Phase II single arm multicenter trial, presented today at the 2025 American Society of Clinical Oncology (ASCO) Annual Meeting, reveal that adding the immunotherapy drug to the targeted therapies enabled tumor resection in the majority of in patients (74%), compared to just 5% historically. Experts also found the combination given before surgery increased overall survival from an average of 13 to 21 months.

“Anaplastic thyroid cancer is one of the most aggressive cancers, and patients often come in with tumors that can’t be surgically removed,” said presenter and lead investigator Mark Zafereo, MD, professor of Head and Neck Surgery. "In previous studies, standard treatment only gave patients a few extra months but, by adding pembrolizumab, we’ve seen signs that we can control the cancer locally without needing a more radical surgical approach.”

Dabrafenib and trametinib are targeted therapies designed to block both the mutant BRAF protein and related proteins that become activated in BRAF-mutated cancers. This combination has been approved to treat multiple types of cancer with BRAF mutations, including ATC, but patients ultimately develop resistance.

Between September 2021 and January 2025, this study enrolled 42 patients with BRAFm-ATC, 40 of whom are included in the current analysis. Patients received a median of four DTP cycles before surgery, and 30 underwent surgery to have their tumors removed.

Researchers also found that the combination therapy increased the amount of time the cancer did not progress, known as progression-free survival, from an average of 6.7 months to over one year.

“This treatment plan has shown that not only are more patients able to undergo surgery, but they’re also living longer without their cancer coming back,“ Zafereo said. ”Based on our results, this approach should now be considered a standard of care for patients with this rare cancer that have this specific gene mutation."

Side effects noted during the study included a kidney injury, sepsis and a tear near the small intestine, after surgery.

Limitations of the study may include the small sample size, the lack of a control arm and short follow-up phase for some patients.

This study was funded by This study was funded by Merck (known as MSD outside of the US and Canada) and Gateway for Cancer Research. A full list of collaborating authors and their disclosures can be found here.

This news release was published by the American Cancer Society on May 31, 2025.

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