Endoxifen Uses Window-of-Opportunity to Reduce Tumor Activity in Breast Cancer

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In the opening between breast cancer diagnosis and surgery, endoxifen may be effective for reducing tumor cell proliferation, leading to Ki-67 change, according to interim analysis results from a phase II clinical trial, announced by Atossa Therapeutics, Inc.

Treatment with endoxifen reportedly led to an overall 74% reduction in tumor cell proliferation, as measured by biomarkers like Ki-67, over a median of 22 day dosing period (range, 16-40). All 6 patients who were included in the analysis achieved a significant reduction in Ki-67 of more than 50%.

“I have been very impressed with the rapid impact Endoxifen has had on tumor proliferation in these patients in the neoadjuvant setting and also the absence of certain side effects, like hot flashes,” commented Vinod Ganju, MBBS, FRACP, principal investigator and adjunct clinical associate professor, Center for Cancer Research, Hudson Institute. “It is my pleasure to be playing a role in developing a much-needed therapy that could improve outcomes for breast cancer patients. Given the results to date, we are interested in expanding the use of Endoxifen to other clinical settings, for example, to metastatic breast cancer patients.

Following treatment with endoxifen, all patients had a Ki-67 below 25%. This decrease held statistical significance (P =.031). Endoxifen therapy did not raise any safety or tolerability concerns with patients in the study. Toxicities that are typically observed with the similar drug, tamoxifen, like hot flashes and night sweats, were not observed in patients who received endoxifen.

The goal of the open-label study was to allow an interim analysis of Ki-67 change in at least 2 of the patients enrolled. The prospective enrollment number is 25 patients with newly-diagnosed patients with ER+ and HER2 negative stage 1 or 2 invasive breast cancer, that requires surgery. The primary end point of the study is the reduction of anti-tumor activity, as measured by Ki-67. As secondary end points, the study is evaluating the safety/tolerability and the drug’s impact on estrogen and progesterone receptor expression levels. The trial is also exploring the impact of endoxifen on additional markers of activity.

Low Ki-67 is significant for patients with breast cancer because it is associated with a lower risk of death , according to prior research in which Ki-67 was identified as a prognostic biomarker for prediction of overall survival in patients with ER-positive disease.

The positive phase II interim data have led Atossa Therapeutics to continue recruiting the remaining 19 patients. Their next action is to seek regulatory approval to continue on to a phase III study.

References:

Atossa Therapeutics announces positive interim phase 2 data from endoxifen breast cancer treatment [news release]. Seattle, Washington: Atossa Therapeutics, Inc; May 7, 2020. https://bit.ly/2Lbx9Xr. Accessed May 7, 2020.