Atossa Therapeutics Provides Five-Year (Z)-Endoxifen Treatment Update on FDA-Approved "Expanded Access" Program for a U.S. Breast Cancer Patient

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SEATTLE, March 19, 2024 (GLOBE NEWSWIRE) -- Atossa Therapeutics, Inc. (Nasdaq: ATOS) (“Atossa” or the “Company”), today announced that the pre-menopausal, Estrogen Receptor positive (ER+) / Human Epidermal Growth Factor Receptor 2 negative (HER2-), breast cancer patient who received neoadjuvant and adjuvant (Z)-endoxifen therapy under an FDA-approved "expanded access" program has completed five years of treatment. As of the date of this press release, the patient remains cancer-free and has reported no significant safety or tolerability issues over the course of her treatment. Atossa is a clinical stage biopharmaceutical company developing innovative medicines in areas of significant unmet medical need in oncology with a focus on breast cancer.

"We believe this patient, and her successful treatment journey, is representative of the opportunity (Z)-endoxifen has to change the treatment paradigm for the approximately 250,000 women diagnosed with estrogen receptor positive breast cancer each year in the U.S.,” said Steven Quay, M.D., Ph.D., Atossa’s President and Chief Executive Officer. "The current standard of care is sub-optimal as it often requires ovarian suppression in premenopausal women and aromatase inhibitors in postmenopausal women, both of which are associated with potential short-term and long-term side effects that diminish adherence. Over five years of adjuvant treatment, her daily (Z)-endoxifen treatment has been well tolerated, and there were no vasomotor symptoms commonly associated with standard of care adjuvant pharmaceuticals. She remains cancer-free today and we are grateful to have been able to help her with a difficult treatment problem.”

Patient / Treatment Background

Diagnosis. In late 2018, a 51-year-old premenopausal woman was diagnosed with a moderately differentiated invasive ductal breast carcinoma with the most frequently reported subtype: ER+, PR+, and HER2-. The initial diagnostic biopsy demonstrated that 90% of the tumor cells were ER+ and 20% expressed Ki-67, a tumor proliferation antigen. Pharmacogenomics analysis showed a CYP2D6 genotype of CYP2D6*4/*4, a nonfunctioning variant, suggesting tamoxifen would be a poor treatment option.

Other than breast cancer, the patient had no comorbidities and took no medications. Her family history was strong for osteoporosis, a contraindication for aromatase inhibitor treatment.

Neoadjuvant treatment. Neoadjuvant treatment is given in the window of time between the diagnosis and the primary treatment, which in the case of locally advanced breast cancer is surgery. The intent of neoadjuvant therapy is to slow the growth of the cancer or even shrink the cancer prior to surgery. The goal of this treatment is to help increase the effectiveness of surgery, and it has also been shown to reduce the likelihood that the cancer returns.

The FDA authorized a single-patient study under its Expanded Access or compassionate use program for this woman to receive 4 mg/day oral (Z)-endoxifen for 20 days before surgery.

Surgery. The patient had a unilateral mastectomy. After (Z)-endoxifen neoadjuvant treatment, the Ki-67 expression in the tumor was reduced 50%, from 20% to 10%. Both the relative response, a 50% reduction, and the absolute value at the time of surgery have been found in large trials to be an indicator of favorable long-term outcomes.

Adjuvant treatment. Following surgery, her physician recommended adjuvant therapy but, like many women, she was not a candidate for tamoxifen due to her low liver enzyme (CYP2D6) activity. She also did not want to take drugs to suppress her ovarian function and go into early menopause, which meant aromatase inhibitors were not an option. Given the strong and rapid response to (Z)-endoxifen, it was recommended that she continue taking (Z)-endoxifen. This led to an additional compassionate use authorization by the FDA to allow this patient to continue taking 4 mg/day (Z)-endoxifen in the adjuvant setting.

She has now completed five years of daily (Z)-endoxifen. As of the date of this press release, she has not had a recurrence of breast cancer or a new cancer in the contralateral breast, as assessed by clinical examination and mammography. Additionally, the treatment has been well tolerated, and there were no vasomotor symptoms commonly associated with tamoxifen (for example, night sweats and hot flashes).

Under the FDA Expanded Access IND program, the use of Atossa's proprietary (Z)-endoxifen is restricted to this patient only.

About FDA Expanded Access
Sometimes called "compassionate use," expanded access is a potential pathway for a patient with an immediately life-threatening condition or serious disease or condition to gain access to an investigational medical product (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available.

Expanded access may be appropriate when all the following apply: the patient has a serious disease or condition, or whose life is immediately threatened by their disease or condition; there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; patient enrollment in a clinical trial is not possible; potential patient benefit justifies the potential risks of treatment; providing the investigational medical product does not interfere with investigational trials that could support a medical product's development or marketing approval for the treatment indication; alternative investigational drugs, biologics or medical devices have not yet been approved or cleared by FDA and FDA has not found these products to be safe and effective for their specific use; and alternative investigational medical product may, or may not, be effective in the treatment of the condition, and use of the product may cause unexpected serious side effects.

For more information, see the FDA website: FDA Expanded Use Website.

About (Z)-Endoxifen
(Z)-endoxifen is the most active metabolite of the FDA approved Selective Estrogen Receptor Modulator (SERM), tamoxifen. Studies have demonstrated that the therapeutic effects of tamoxifen are driven in a concentration-dependent manner by (Z)-endoxifen. In addition to its potent anti-estrogen effects, (Z)-endoxifen at higher concentrations has been shown to target PKCβ1, a known oncogenic protein. (Z)-endoxifen also appears to deliver similar or even greater bone agonistic effects while resulting in little or no endometrial proliferative effects compared with tamoxifen.

Atossa is developing a proprietary oral formulation of (Z)-endoxifen that does not require liver metabolism to achieve therapeutic concentrations and is encapsulated to bypass the stomach as acidic conditions in the stomach convert a greater proportion of (Z)-endoxifen to the inactive (E)-endoxifen. Atossa's (Z)-endoxifen has been shown to be well tolerated in Phase 1 studies and in a small Phase 2 study of women with breast cancer. (Z)-endoxifen is currently being studied in four Phase 2 trials: one in healthy women with measurable breast density, one in women diagnosed with ductal carcinoma in situ, and two other studies including the EVANGELINE study in women with ER+/HER2- breast cancer. Atossa's (Z)-endoxifen is protected by three issued U.S. patents and numerous pending patent applications.

About Atossa Therapeutics
Atossa Therapeutics, Inc. is a clinical-stage biopharmaceutical company developing innovative medicines in areas of significant unmet medical need in oncology with a focus on using (Z)-endoxifen to prevent and treat breast cancer. For more information, please visit www.atossatherapeutics.com.

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FORWARD LOOKING STATEMENTS
This press release contains certain information that may constitute forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. We may identify these forward-looking statements by the use of words such as "expect," "potential," "continue," "may," "will," "should," "could," "would," "seek," "intend," "plan," "estimate," "anticipate," "believe," "future," or other comparable words. Forward-looking statements in this press release are subject to risks and uncertainties that may cause actual results, outcomes, or the timing of actual results or outcomes, including the timing of data related to the (Z)-endoxifen program, the potential of (Z)-endoxifen as a breast cancer prevention and treatment agent, and the potential safety and tolerability profile of (Z)-endoxifen, to differ materially from those projected or anticipated, including risks and uncertainties associated with: macroeconomic conditions and increasing geopolitical instability; the expected timing of releasing data; any variation between interim and final clinical results; actions and inactions by the FDA and foreign regulatory bodies; the outcome or timing of regulatory approvals needed by Atossa, including those needed to continue our planned (Z)-endoxifen trials; our ability to satisfy regulatory requirements; our ability to comply with the continued listing requirements of the Nasdaq Stock Market; our ability to successfully develop and commercialize new therapeutics; the success, costs and timing of our development activities, including our ability to successfully initiate or complete our clinical trials, including our (Z)-endoxifen trials; our anticipated rate of patient enrollment; our ability to contract with third-parties and their ability to perform adequately; our estimates on the size and characteristics of our potential markets; our ability to successfully defend litigation and other similar complaints and to establish and maintain intellectual property rights covering our products; whether we can successfully complete our clinical trial of oral (Z)-endoxifen in women with mammographic breast density and our trials of (Z)-endoxifen in women with breast cancer, and whether the studies will meet their objectives; our expectations as to future financial performance, expense levels and capital sources, including our ability to raise capital; our ability to attract and retain key personnel; our anticipated working capital needs and expectations around the sufficiency of our cash reserves; and other risks and uncertainties detailed from time to time in Atossa's filings with the Securities and Exchange Commission, including without limitation its Annual Reports on Form 10-K and Quarterly Reports on 10-Q. Forward-looking statements are presented as of the date of this press release. Except as required by law, we do not intend to update any forward-looking statements, whether as a result of new information, future events or circumstances or otherwise.