Use of Acetalax for Treatment of Triple Negative Breast Cancer

Summary (1024-character limit)
The National Cancer Institute (NCI) seeks research co-development and/or potential licensees for a potential novel treatment for triple-negative breast cancer (TNBC) with acetalax (oxyphenisatin acetate). Acetalax is a previously FDA approved drug that has been used as a topical laxative but is being repurposed here as an onco-therapy because of its cytotoxic effects on a number of TNBC and other cancer cell lines.

NIH Reference Number
E-041-2018

Product Type
• Therapeutics

Keywords
• Triple Negative Breast Cancer, TNBC, Acetalax, Oxyphenisatin Acetate, Uterine Cancer, Pommier

Collaboration Opportunity
This invention is available for licensing and co-development.

Description of Technology
Triple negative (progesterone receptor (PR)-, estrogen receptor (ER)-, human epidermal growth receptor 2 (HER2)-) breast cancer (TNBC) is an aggressive subtype that affects 15-20% of the 1.7 million cases of breast cancer occurring annually. Currently, standard treatments of TNBC include cytotoxic chemotherapies, surgery, and radiation. However, TNBC readily becomes resistant to chemotherapy, and those with TNBC are more likely to have a recurrence or die within five years compared to those with other breast cancer types. Therefore, there is a need for safer and more effective TNBC treatments to improve patient outcomes.

Investigators from the National Cancer Institute (NCI) and collaborating institutions have identified the compound acetalax (oxyphenisatin acetate) as a promising potential therapy for TNBC. Strikingly, acetalax’s cytotoxic effect checked first against ~178 FDA-approved or clinical trial oncology drugs on the three TNBC against the NCI-60 panel of cancer cell lines – subsequently followed up using the 22 different TNBC cell lines checked against 15 oncology drugs – were significantly most cytotoxic. Acetalax’s efficacy in vivo has been investigated using a TNBC patient-derived xenograft (PDX) mouse model. Untreated PDX mice exhibited a tumor volume doubling rate of approximately 7-8 days whereas acetalax-treated mice tumor volumes decreased to undetectable levels in approximately 13 days.

NCI seeks research co-development and/or potential licensees for a potential novel treatment for triple-negative breast cancer (TNBC) with acetalax (oxyphenisatin acetate).

Potential Commercial Applications

NCI Technology Transfer Center
• A therapeutic for Triple Negative Breast Cancer
• Potential therapeutic for recalcitrant or estrogen-dependent cancers
• Potential applicability to breast (non-Triple Negative), ovarian, pancreatic, sarcoma, and uterine cancers

**Competitive Advantages**
• Repurposed, previously approved FDA drug for a novel use as a therapeutic for TNBC and other cancers
• Repurposed, previously approved drugs can have an expedited approval process due in part to pre-existing safety data

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**Development Stage**
• Pre-clinical (in vivo)

**Publications**

**Patent Status**
• **U.S. Provisional:** U.S. Provisional Patent Application Number 62/627,926, Filed 08 Feb 2018
• **Foreign Filed:** - Patent Application PCT/US2019/017239, Filed 08 Feb 2019
• **Foreign Filed:** - Patent Application 19707221.8, Filed 08 Feb 2019
• **U.S. Patent Filed:** U.S. Patent Application Number 16/967,472, Filed 05 Aug 2020

**Therapeutic Area**
• Cancer/Neoplasm