EO-3001 Program

First -in-Class "Targeted Therapy for ARID1A Mutated Cancers"

Overview:
EO-3001 is a small-molecule anticancer drug in clinical development for solid tumors harboring an **ARID1A mutation**. The initial target indication will be Ovarian Clear Cell Carcinoma (OCCC), an aggressive subtype of epithelial ovarian cancer with poor prognosis and limited treatment options.

Scientific Rationale:

- **ARID1A** encodes a key subunit of the SWI/SNF chromatin remodeling complex (BAF250a) and ARID1A mutation disrupts the SWI/SNF complex rendering the cell dependent on OXPHOS for energy production.
- Loss of **ARID1A** function also leads to epigenetic dysregulation, defective DNA repair, and immune evasion, contributing to tumor progression and resistance to therapy.

Mechanism of Action EO-3001:

- **EO-3001** is a mitochondrial-targeting small molecule.
- It exploits OXPHOS dependency in **ARID1A**-deficient cells by inhibiting mitochondrial proteins critical for oxidative phosphorylation. **EO-3001** induces a cellular energy crisis and apoptosis, selectively in **ARID1A-mutant** cells.

Clinical Development History

- **EO-3001** was previously studied in multiple clinical trials involving more than 1,000 patients.
- **Safety Profile: EO-3001** was well tolerated with no single-agent dose-limiting toxicity observed.
- **Efficacy Signal:** Response to treatment (CR or PR) was observed in multiple solid tumors.

Preclinical Profile:

- **EO-3001** Demonstrated high selectivity for **ARID1A mutant** ovarian cancer cells at low nanomolar concentrations.
- Spares wild-type cells, reducing potential toxicity.

Market Opportunity:

ARID1A mutations are observed in up to 10% of all solid tumors.

OCCC - Unmet Need:

- OCCC accounts for ~10%% of epithelial ovarian cancers in Western populations and up to 35% in patients of East Asian descent.
- These tumors are less responsive to platinum-based chemotherapy and radiation.

- ~1,000–1,800 new U.S. ovarian cancer cases annually may involve **ARID1A mutations**.
- As of April 2025, NCCN guidelines do not provide specific treatment recommendations for **ARID1A-mutant** OCCC, highlighting a significant unmet need.

Development Status (as of April 2025):

- GMP manufacturing of **EO-3001** drug product is complete.
- IND-enabling toxicity/finding study completed.
- Bridging study (20 patients) to validate PK and safety planned for H2-2025.
- Pivotal registration study (90 patients) in **ARID1A-mutant** OCCC to start in 2026.