FOR IMMEDIATE RELEASE

Cancer Prevention Pharmaceuticals Submits New Drug Application to the FDA for CPP-1X/sul for Treatment of Familial Adenomatous Polyposis

Company seeks accelerated approval for cancer drug

TUCSON, Arizona, June 29, 2020 – Cancer Prevention Pharmaceuticals, Inc. (CPP), a private biotech company developing novel therapeutics to prevent cancer and other diseases, announced that it has submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) seeking accelerated approval for CPP-1X/sul for treatment of adults with familial adenomatous polyposis (FAP).

FAP is a rare genetic disease that if left untreated progresses to colorectal cancer in nearly 100% of patients. The clinical development of CPP-1X/sul was designed to establish this fixed dose combination product as a potential pharmaco-preventive drug treatment specifically for FAP patients.

“The NDA submission for our lead drug candidate, CPP-1X/sul, represents a significant milestone for FAP patients and their families,” said CPP CEO Jeff Jacob. “For most FAP patients, current medical practice involves a lifetime of periodic monitoring as well as highly invasive surgical procedures. If approved, CPP-1X/sul could provide an alternative to surgery for many patients, significantly improving their quality of life.”

The FDA’s accelerated approval process allows the agency to approve drugs which address a serious or life-threatening condition based on an intermediate or surrogate endpoint that is likely to predict a clinical long-term benefit. The FDA takes into account such factors as the severity, rarity, or the lack of effective current treatments. The designation has been frequently applied to cancer drugs.

In addition, CPP-1X/sul received an orphan drug designation from the FDA. Among the benefits are eligibility for seven years of market exclusivity upon approval of a drug and tax credits for various costs of clinical testing.

CPP also recently submitted a Marketing Authorization Application (MAA) with the European Union (EU) for CPP-1X/sul for the same indication. The drug received an orphan medicinal product designation for FAP following a favorable assessment provided by the European Medicines Agency’s Committee for Orphan Medicinal Products.

About CPP-1X/sul
CPP-1X/sul is a combination of CPP-1X (eflornithine) and sulindac (CPP-1X/sul). In a clinical trial in patients with large bowel polyps, the CPP-1X/sul combination prevented > 90% subsequent pre-cancerous sporadic adenomas versus placebo. Based on the close biologic similarities with FAP, a Phase 3 pivotal trial compared this same combination to each drug alone (CPP FAP-310).

"With up to four years of treatment, the combination appears to greatly delay the need for major surgeries in the colon, rectum or surgical pouch. We hope that this new drug regimen will soon be available as an adjunct in the management of FAP patients facing large bowel surgery," said Dr. Alfred Cohen, Chief Medical Officer of CPP.

The CPP FAP-310 trial enrolled 171 FAP patients at 17 research institutes in the U.S., Canada, and Europe. It was the largest ever prospective, controlled study performed in FAP and treated patients for up to 48 months, much longer than any other clinical trial in this population. The study was designed to determine if CPP-1X/sul is superior to sulindac or eflornithine as single agents in delaying time to the first occurrence of any FAP-related event, such as surgical removal of the colon, rectum, surgical pouch, duodenum and/or high-risk adenomas. The trial design included FAP patients with varying lower and upper GI disease. CPP-1X/sul did not demonstrate statistical significance in outperforming the single agents in the overall population; however, further analysis of the data showed key differential effects of the agents in the lower vs upper GI anatomy. Focusing on lower GI anatomy (patients with an intact colon, retained rectum or surgical pouch), the data showed statistically significant benefit for CPP-1X/sul vs both single agents ($p \leq 0.02$) in delaying surgical events in the lower GI for up to four years. Also, the safety profile of the combination did not significantly differ from that already known for the single agents to support the overall safety assessment of the fixed combination product for long-term therapeutic use.

About Cancer Prevention Pharmaceuticals, Inc.
Cancer Prevention Pharmaceuticals, Inc. (CPP) is developing therapeutics designed to reduce the risk of cancer and other diseases. CPP's pharmaco-prevention approach has been used with success in other disease categories such as cardiovascular, neurovascular, and infectious disease. In addition to the CPP FAP-310 trial, CPP is co-sponsoring with the National Cancer Institute (NCI) and SWOG a large Phase 3 trial in colon cancer survivors. CPP is also working collaboratively with nonprofit groups to support their clinical trials in neuroblastoma, gastric cancer, and early-onset type 1 diabetes. CPP is located in Tucson, Arizona. For more information, please visit www.canprevent.com.

This press release contains forward looking statements subject to risks and uncertainties that could cause actual results to differ materially from those projected. Forward looking statements include statements regarding FDA approval of CPP-1X, CPP-1X/sul providing an alternative to surgery for many patients, significantly improving their quality of life. These forward looking statements represent the company's judgment as of the date of this release. The company disclaims, however any intent or obligation to update these forward looking statements.

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