FOR IMMEDIATE RELEASE

Cancer Prevention Pharmaceuticals (CPP) Reports FAP Phase 3 Clinical Trial Results at Digestive Disease Week Conference

Based on unmet medical need and encouraging data company will explore regulatory pathways

TUCSON, Arizona, June 20, 2019 – Cancer Prevention Pharmaceuticals, Inc. (CPP) recently reported results from its pivotal Phase 3 trial of CPP-1X/sul for adults with familial adenomatous polyposis (FAP) at the Digestive Disease Week annual conference in San Diego.

FAP is a rare genetic disease that if left untreated progresses to colorectal cancer in nearly 100% of patients. FAP patients have no approved pharmaceutical therapies.

The Phase 3 trial compared the performance of the combination of CPP-1X (eflornithine) in combination with sulindac (CPP-1X/sul), to each single agent alone in delaying progression to an FAP-related event, such as surgery. The trial design included FAP patients with varying lower and upper GI disease. The combination 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. Also, the safety profile of the combination did not significantly differ from that demonstrated by the single agents and did not pose any concerns for long-term use of the product.

“The data suggests that in adults with FAP, CPP-1X/sulindac may delay colorectal surgery,” said Carol Burke, MD, Principal Investigator and staff member in the Department of Gastroenterology, Hepatology and Nutrition, at Cleveland Clinic, Cleveland who presented the data at DDW.

"The reversal or prevention of FAP-associated polyp growth remains the greatest unmet medical need facing the FAP community. As such, we at the Hereditary Colon Cancer Foundation have been watching Cancer Prevention Pharmaceuticals' (CPP) recent CPP FAP-310 clinical trial closely and are encouraged by the positive results of this trial. We are very hopeful that CPP will successfully develop an alternative to surgery for FAP patients and look forward to supporting them as they work with regulatory agencies to capitalize on these promising results,” said Travis H. Bray, PhD, Executive Director of the Hereditary Colon Cancer Foundation.

The Phase 3 trial, which enrolled 171 patients at 17 research institutes in the United States, Canada and Europe, 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.

“An effective treatment for FAP remains a complex and unmet medical need as indicated by fast track designation granted by the FDA for CPP-1X/sul,” Jacob added. “The positive data in the lower GI will be the basis of our approach to pursue regulatory approval in the US and EU to give these patients a pharmaceutical option to delay surgical procedures” he added.

FAP occurs in about 1 in 10,000 people in the United States. For most FAP patients, current medical practice recommends a lifetime of periodic monitoring as well as surgical procedures (FAP-related events). These FAP-related events include surgical removal of the colon, rectum, surgical pouch, duodenum and/or high-risk adenomas.

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 that the data suggests that in adult FAP patients with intact colons, retained rectums, or surgical pouches, CPP-1X/sulindac may provide a significant delay in polyposis progression to allow surgery to be performed at a time more convenient for the patient. 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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