FOR IMMEDIATE RELEASE

Cancer Prevention Pharmaceuticals Announces *NEJM* Publication of Landmark Phase 3 Clinical Trial for Treatment of Familial Adenomatous Polyposis

Potential benefits seen for patients with intact colons

TUCSON, Arizona, September 11, 2020 – Cancer Prevention Pharmaceuticals, Inc. (CPP), a private biotech company developing novel therapeutics to prevent cancer and other diseases, today announced *The New England Journal of Medicine (NEJM)* has published results from its landmark FAP-310 Phase 3 clinical trial of CPP-1X/sul as a pharmaco-preventive treatment for 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. Standard of care is surgery as there are no approved drugs for FAP.

The FAP-310 Phase 3 trial compared the performance of the combination of CPP-1X (eflornithine) and sulindac (CPP-1X/sul), to each single agent alone in delaying progression to an FAP-related clinical event, such as surgery. The trial enrolled 171 patients and was conducted over many years where patients received daily treatment for 2-4 years. It was the largest prospective controlled study ever performed in FAP, and the only clinical event driven trial ever conducted.

Although the trial did not demonstrate that overall disease progression was significantly lower with CPP-1X/sul compared to eflornithine or sulindac alone, the data showed that in a subgroup of patients with intact colons - representing the vast majority of young FAP patients - there was a 70% decreased risk of disease progression with CPP-1X/sul.

"The results of the Phase 3 trial demonstrate CPP-1X/sul may be a potential pharmaco-preventive option for FAP patients in delaying surgical procedures," said Alfred Cohen, M.D., Chief Medical Officer of CPP and a co-author of the NEJM article. "These surgeries are life-altering and we are encouraged by this positive data for pre-colectomy FAP patients."

In a video released by the Mayo Clinic, Niloy Jewel Samadder, M.D., a gastroenterologist at the Mayo Clinic in Arizona and a co-author of the NEJM article, quoted, "No patient with an intact colon who received combination therapy underwent surgical intervention. Additionally, the combination therapy was well tolerated over long-term treatment of 2-4 years."

Based on the results of the trial and observed benefits in a broad group of FAP patients, CPP intends to pursue regulatory approval from the U.S. Food and Drug Administration (FDA) and a

Marketing Authorization Application (MAA) with the European Medicines Agency for approval of CPP-1X/sul to delay or prevent surgeries in patients with an intact colon, retained rectum, or surgical pouch.

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 some 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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