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FOR IMMEDIATE RELEASE

Phase III Familial Adenomatous Polyposis (FAP) Trial Now Open for Enrollment

Tucson, Arizona, December 5, 2013—Cancer Prevention Pharmaceuticals, Inc. (CPP) announced today the launch of a double-blind, randomized, Phase III trial of the efficacy and safety of CPP-1X/sulindac (eflornithine/sulindac) compared with CPP-1X and sulindac as single agents in 150 patients with Familial Adenomatous Polyposis. Each patient will receive daily treatment for two years to minimize the occurrence and/or recurrence of problematic polyps and tumors associated with this debilitating disease. The trial is being conducted at 8 North American and 4 European clinical trial sites.

"The design of this trial is particularly exciting" noted Alfred Cohen, MD and Chief Medical Officer of CPP. "Rather than count polyps, which has been the past indicator of clinical success in pharmacoprevention trials in FAP, we have designed our trial to assess meaningful clinical outcomes in these patients. With a successful outcome in this study, we will have real evidence of patient benefit." Jeff Jacob, Chief Executive Officer of CPP, added "the initiation of this trial is a huge milestone for our Company and for patients with FAP. Taking specific guidance from both the Food and Drug Administration (FDA) and European Medicines Agency (EMA) on trial design, we and our expert advisors believe the

drug could change the treatment paradigm in FAP by delaying or eliminating surgical interventions and/or cancer."

About Familial Adenomatous Polyposis (FAP)

Familial adenomatous polyposis (FAP) is an inherited disorder characterized by cancer of the large intestine (colon) and rectum. People with the classic type of FAP may begin to develop multiple noncancerous (benign) growths (polyps) in the colon as early as their teenage years. Currently, there are no approved pharmacologic treatments for FAP. Unless the colon is removed – the only real treatment option – these polyps will become malignant (cancerous). The average age at which an individual develops colon cancer in classic FAP is 39 years. Some people have a variant of the disorder, called attenuated FAP, in which polyp growth is delayed. The average age of colorectal cancer onset for attenuated FAP is 55 years.

In people with classic FAP, the number of polyps increases with age, and over time, hundreds to thousands of polyps carpet the colon. Also of particular significance are noncancerous growths called desmoid tumors. These fibrous tumors usually occur in the tissue covering the intestines and may be provoked by surgery to remove the colon. Desmoid tumors tend to recur after they are surgically removed. In both classic FAP and its attenuated variant, benign and malignant tumors are sometimes found in other places in the body, including the duodenum (a section of the small intestine), stomach, bones, skin, and other tissues. If left untreated, FAP leads to colorectal cancer and death.

About Cancer Prevention Pharmaceuticals, Inc. (CPP)

CPP is developing therapeutics that reduce the risk of cancer. CPP's approach has been used with great success in other disease categories such as cardiovascular, neurovascular and infectious disease. Agents that target pre-disease states have helped reduce death rates from these conditions by 50%-

70% over the past 30 years. Just as these other prevention therapies represent the largest-selling drug classes on the market today, CPP believes there is even more potential for therapies that reduce the risk of cancer.

In addition to this Phase III trial, CPP, in collaboration with the National Cancer Institute (NCI) and the SWOG cancer research cooperative group, is sponsoring a large 1,340 patient Phase III trial in colon cancer survivors. CPP is also working collaboratively with non-profit groups to support trials in neuroblastoma—treating and preventing—with three active clinical trials. Additional information is available at www.canprevent.com.

Forward-Looking Statements

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 about the planned Phase III trial of the CPP-1X/sul combination therapy as well as the company's focus, collaborative partners, and independent and partnered product candidates. 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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