



Cancer Prevention Pharmaceuticals Completes Target Enrollment in Pivotal Phase 3 Clinical Trial Evaluating CPP-1X/sulindac in Patients with Familial Adenomatous Polyposis

TUCSON, Ariz., April 4, 2016 – Cancer Prevention Pharmaceuticals, Inc. (CPP), a private biotech company developing novel therapeutics to prevent cancer and other diseases, announced today that target enrollment has been achieved for the company’s pivotal Phase 3 clinical trial, CPP FAP-310, evaluating CPP-1X/sulindac in adults with familial adenomatous polyposis (FAP). FAP is a rare genetic disease that, if untreated, progresses to colorectal cancer in almost all cases. The trial has enrolled 166 patients at 21 research institutes in the United States, Canada and Europe.

Cancer Prevention Pharmaceuticals is developing CPP-1X/sulindac as a pharmacoprevention therapy designed to minimize the progression of polyps and tumors associated with FAP. The purpose of CPP FAP-310, a randomized, double-blind, Phase 3 clinical trial is to determine in FAP patients if the combination of CPP-1X and sulindac delays the time to disease progression and subsequent FAP-related events, including the need for surgical and endoscopic procedures. Patients in the clinical trial receive two years of daily treatments of CPP-1X/sulindac, and time to the first occurrence of any FAP-related event is evaluated during this two-year time frame.

“The completion of target enrollment in this pivotal Phase 3 clinical trial is an important milestone for the non-invasive treatment of FAP, and we look forward to completing the trial and bringing to market a first-in-class pharmacoprevention therapeutic,” said Mr. Jeffrey Jacob, CEO of Cancer Prevention Pharmaceuticals. “For this clinical trial, we have worked closely with many groups, including the U.S. Food and Drug Administration and the European Medicines Agency to develop clinically-relevant trial endpoints, with top clinical researchers and medical centers in the design and implementation of the trial, and with patient advocacy organizations to support awareness of the trial in the FAP community.”

“We are honored to be working with Cancer Prevention Pharmaceuticals on this very important clinical trial,” said Travis Bray, founder and executive director of the Hereditary Colon Cancer Foundation (<http://www.hcctakesguts.org>). “It is our intent that, together, a successful treatment will be available for patients to help reduce their polyp burden and increase their quality of life with FAP.”

Patients with the classic presentation of FAP share a genetic mutation that conveys an extremely high risk of developing colon cancer, and they begin to develop multiple benign polyps in the colon in their early teens. Eventually, the colon becomes carpeted with hundreds to thousands of polyps, some of which will become cancerous if left untreated. For most FAP patients, current medical practice recommends the surgical removal of the



colon and sometimes the rectum followed by a lifetime of monitoring of the upper GI, retained rectum or ileal pouch, additional operations and a reduced quality of life.

“FAP patients face a lifetime of complex endoscopic and invasive surgical procedures, including colectomy, proctectomy and duodenectomy, which begin in the teenage years and progressively remove the colon, rectum and duodenum as the disease evolves,” said Alfred Cohen, M.D., chief medical officer of Cancer Prevention Pharmaceuticals. “The intent of this clinical trial is to provide FAP patients an approved, once-daily dosing to intervene in the disease’s progression and defer major medical interventions.”

For more information on the clinical trial (CPP FAP-310), please visit: <https://clinicaltrials.gov/>.

About Cancer Prevention Pharmaceuticals, Inc.

Cancer Prevention Pharmaceuticals, Inc. (CPP) is developing therapeutics designed to reduce the risk of cancer. CPP’s pharmaco-prevention approach has been used with success in other disease categories such as cardiovascular, neurovascular and infectious disease. CPP is co-sponsoring a large Phase 3 trial in colon cancer survivors with NCI and SWOG and a Phase 3 clinical trial in patients with familial adenomatous polyposis (FAP). 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 on our current expectations and projections about future events. In some cases, forward-looking statements can be identified by terminology such as “may,” “should,” “potential,” “continue,” “expects,” “anticipates,” “intends,” “plans,” “believes,” and similar expressions. These statements are based upon our current beliefs, expectations and assumptions and are subject to a number of risks and uncertainties, many of which are difficult to predict and include statements regarding completion of the Phase 3 trial, bringing to market a first-in-class pharmaco-prevention therapeutic, our intent that a successful treatment will be available for patients to help reduce their disease burden and increase their quality of life with FAP and that our clinical trials can provide FAP patients with an approved, once-daily dosing to intervene in the disease’s progression and to defer major medical interventions. These forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially from those set forth or implied by any forward-looking statements. Important factors that could cause actual results to differ materially from those reflected in our forward-looking statements include, among others, our ability to obtain the necessary regulatory approvals for commercialization of our therapeutics, our ability to commence or complete our clinical trials and those of our investigators on time or to achieve desired results and benefits, our ability to continue enrollment of our clinical trials as expected or receive anticipated funding, our ability to successfully develop, market or sell our products, our ability to maintain our material licensing agreements, or our strategic partners’ ability to successfully market, sell and commercialize our products. The information in this release is provided only as



of the date of this release, and we undertake no obligation to update any forward-looking statements contained in this release on account of new information, future events, or otherwise, except as required by law.

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