February 23, 2016

OPKO Health Announces Dosing of First Patient in Phase 2a Clinical Study of a Long-Acting Factor VIIa for the Treatment of Hemophilia

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NYSE:OPK) today announced dosing of the first patient in a Phase 2a study evaluating the safety of a long-acting Factor VIIa in hemophilia patients. The Phase 2a study is a dose escalation study to determine safety and explore efficacy endpoints in patients with OPKO's long-acting version of coagulation Factor VIIa (Factor VIIa-CTP) for the treatment of bleeding episodes in hemophilia A or B patients with inhibitors to Factor VIII or Factor IX. The study is intended to enroll 24 patients in the United States.

“This is a significant step in the development of another drug candidate utilizing our CTP technology,” commented Phillip Frost, MD, Chairman and Chief Executive Officer of OPKO. “Later this year, we expect to initiate a second Phase 2a trial for Factor VIIa-CTP in a subcutaneous formulation that, if successful, could provide prophylactic therapy to prevent bleeding episodes and thereby change the treatment paradigm for patients with hemophilia.”

Currently, Factor VIIa therapy is available only as an intravenous (IV) formulation which, due to Factor VIIa's short half-life, requires multiple infusions to treat a bleeding episode in hemophilia A or B patients with inhibitors. In addition, frequent infusions are onerous when used as prophylactic therapy, especially for children. Pre-clinical studies of intravenous and subcutaneous formulations of our product in hemophilic animal models demonstrated its duration of action and significantly increased survival.

About Factor VIIa-CTP

Factor VIIa-CTP is a novel, long-acting recombinant Factor VIIa utilizing OPKO's proprietary technology to extend its circulatory half-life without the use of polymers, encapsulation techniques, or nanoparticles. The technology is based on a naturally occurring peptide, the C-terminal peptide (CTP) of the beta chain of human chorionic gonadotropin. The CTP technology is also used in OPKO's hGH-CTP, its long-acting recombinant human growth hormone product which is being evaluated in Phase 3 clinical trials for adults and Phase 2 trials for children with growth hormone deficiencies. OPKO recently announced a global agreement with Pfizer for the development and commercialization of hGH-CTP.

Factor VIIa-CTP has been granted orphan drug designation in the U.S. and Europe.

About OPKO Health, Inc.

OPKO Health, Inc. is a diversified healthcare company that seeks to establish industry-leading positions in large, rapidly growing markets. Our diagnostics business includes Bio-

Reference Laboratories, the nation's third-largest clinical laboratory with a core genetic testing business and a 420-person sales force to drive growth and leverage new products, including the 4Kscore® prostate cancer test and the Claros®1 in-office immunoassay platform. Our pharmaceutical business features Rayaldee™, a treatment for SHPT in stage 3-4 CKD patients with vitamin D insufficiency (March 29, 2016 PDUFA date) and VARUBI™ for chemotherapy-induced nausea and vomiting (oral formulation launched by partner Tesaro, IV formulation in Phase 3). Our biologics business includes hGH-CTP, a once-weekly human growth hormone injection (in Phase 3 and partnered with Pfizer), and a long-acting Factor VIIa drug for hemophilia (entering Phase 2a). We also have production and distribution assets worldwide, multiple strategic investments and an active business development strategy. More information is available at www.opko.com.

This press release contains "forward-looking statements," as that term is defined under the Private Securities Litigation Reform Act of 1995 (PSLRA), regarding product development efforts and other non-historical facts about our expectations, beliefs or intentions regarding our business, technologies and products, financial condition, strategies or prospects, including statements regarding our expectations about our long-acting version of clotting Factor VIIa (Factor VIIa-CTP) for the treatment of bleeding episodes in patients with hemophilia A or B with inhibitors to Factor VIII or Factor IX, whether Factor VIIa-CTP has the potential for substantial improvement of the quality of life of patients, via both IV and subcutaneous (SC) administration, whether Factor VIIa-CTP will be able to be administered subcutaneously using a simple injection and allow children and adults with hemophilia to easily self-administer the drug at home on a prophylactic basis, whether we will be in a position to be first to market with a longer acting Factor VIIa product, expectations about market potential for Factor VIIa-CTP and its ability to provide prophylactic therapy to prevent bleeding episodes and thereby change the treatment paradigm for patients with hemophilia, whether we will be able to successfully develop, obtain approval for and launch sales of the Factor VIIa-CTP, and the expected completion dates for our trials. Many factors could cause our actual activities or results to differ materially from the activities and results anticipated in forward-looking statements. These factors include those described in our filings with the Securities and Exchange Commission, as well as risks inherent in funding, developing and obtaining regulatory approvals of new, commercially-viable and competitive products and treatments, including the risks that clinical trials for Factor VIIa-CTP may not be successful or achieve the expected results or effectiveness, and may not generate data that would support the approval or marketing of this product for the indications being studied, that others may develop products which are superior to Factor VIIa-CTP, and that Factor VIIa-CTP may not have advantages or prove to be superior over presently marketed products. In addition, forward-looking statements may also be adversely affected by general market factors, competitive product development, product availability, federal and state regulations and legislation, the regulatory process for new products and indications, manufacturing issues that may arise, patent positions and litigation, among other factors. The forward-looking statements contained in this press release speak only as of the date the statements were made and we do not undertake any obligation to update forward-looking statements. We intend that all forward-looking statements be subject to the safe-harbor provisions of the PSLRA.OPKO Health, Inc.

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OPKO Health, Inc.
Tara Mackay, 305-575-4100
Investor Relations
or
Media
Rooney & Associates
Terry Rooney, 212-223-0689
trooney@rooneyco.com
or
Marion Janic, 212-223-4017
mjanic@rooneyco.com
or
Investors
LHA
Anne Marie Fields, 212-838-3777
afields@lhai.com
or
Bruce Voss, 310-691-7100
bvoss@lhai.com

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