



March 24, 2016

OPKO To Present 18-24 Month Clinical Data on its Long-acting Human Growth Hormone (hGH-CTP) from Open Label Extension (OLE) Phase 2 Pediatric Growth Hormone Deficiency Clinical Study in Oral Presentation at ENDO 2016

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NYSE:OPK) today announced it will present 18-24 month clinical data from its ongoing open label extension study for hGH-CTP in an oral presentation at the Endocrine Society's 98th Annual Meeting to be held April 1-4, 2016, in Boston, MA.

The open label extension study is a continuation of our phase 2 one-year dose-finding study in which 53 naïve growth hormone deficient children received one of three doses of hGH-CTP once weekly (0.25, 0.48 and 0.66 mg/kg/week) or daily Genotropin® as a comparator arm (34 µg/kg/day). The published twelve-month data from the phase 2 study confirmed comparable response of hGH-CTP to daily Genotropin® as reflected by the twelve-month safety, efficacy and pharmacodynamic profile. Patients were rolled into the open label extension portion of the study while the daily arm patients were randomized to one of the hGH-CTP doses. For more information on the phase 2 study, please refer to <https://clinicaltrials.gov/ct2/show/NCT01592500>.

The oral presentation is scheduled for Sunday, April 3, 2016 at 11:45 AM - 1:15 PM ET, Oral Session Number: OR31-6.

Based on the promising phase 2 clinical data, OPKO confirms its plan to initiate a global pivotal phase 3 study in pre-pubertal growth hormone-deficient children later this year evaluating a weekly single dose of hGH-CTP versus daily injections of growth hormone. OPKO has a world-wide collaboration agreement with Pfizer Inc. for the development and commercialization of hGH-CTP.

OPKO poster presentations on hGH-CTP include the following:

Poster title	Poster ID + link
Safety and Tolerability of Once-Weekly Administration of CTP-Modified Human Growth Hormone (MOD-4023): Phase 2 Study in Children with Growth Hormone Deficiency	SAT028
Evaluation of Pharmacokinetics and Pharmacodynamics of Long-Acting Human Growth Hormone (MOD-4023) in Healthy Caucasian and Japanese Adults	FRI 537
Pharmacokinetic and Pharmacodynamic Modeling of Long-Acting Human Growth Hormone (MOD-4023) in Growth Hormone Deficient Children	SAT 029
When Should IGF-1 be Monitored during Weekly Administration of a Long-Acting Human Growth Hormone (MOD-4023)?	SAT 039
Batch-to-Batch Consistency of a Highly O-Glycosylated Long-Acting Human Growth Hormone (MOD-4023)	FRI 513
Robust Viral Clearance Capacity of CTP-Modified Long-Acting Growth Hormone (MOD-4023) Downstream Production Process	SAT 109

In addition, the following poster presentations will include non-clinical data characterizing Oxyntomodulin (MOD-6031), a novel long acting dual GLP-1/Glucagon agonist for the induction of weight management.

In Vitro Characterization of MOD-6031- a Novel Long Acting Dual GLP-1/Glucagon Agonist for the Induction of Weight Management	SUN 622
Improved Glycemic Profile and Anti-Obesity Effects Alongside Significant Elongated Pharmacokinetic Profile Following Administration of Mod-6031 - a Novel Long-Acting Dual GLP-1/Glucagon Agonist	SUN 621

Earlier this month, OPKO announced dosing of the first subject in a phase 1 single dose escalation study evaluating the safety and pharmacokinetics of a long-acting Oxyntomodulin in healthy, overweight or obese subjects. The study is intended to enroll 40 subjects in Israel.

About hGH-CTP

hGH-CTP is a novel, long-acting recombinant human growth hormone analog being developed by OPKO for the treatment of children with growth failure due to inadequate endogenous growth hormone secretion, and adults with growth hormone deficiency (GHD) of either childhood or adult-onset etiology. hGH-CTP is intended to reduce the burden of daily injection therapy.

OPKO's proprietary technology enables elongation of a therapeutic protein's half-life without the use of polymers, encapsulation techniques, or nanoparticles. This technology is based on a natural peptide, the C-terminal peptide (CTP) of the beta chain of human chorionic gonadotropin (hCG). hGH-CTP has been granted orphan drug designation in the U.S. and Europe for both adults and children with growth hormone deficiency.

About Oxyntomodulin

Oxyntomodulin is a peptide hormone that acts as a dual GLP-1/Glucagon receptor agonist, with the potential to promote weight loss while improving glycemic control. Oxyntomodulin has been shown to increase energy expenditure, while reducing food intake and body weight, although its clinical utility is limited by its short circulating half-life. OPKO's MOD-6031 has been designed, using a proprietary bi-functional hydrolysable linker, as a long-acting version of Oxyntomodulin for the treatment of Type II diabetes and obesity, and is intended to reduce the required dosage frequency by prolonging the half-life, while improving the hormone's pharmacokinetics and pharmacodynamics. A MOD-6031 phase 1 study was recently initiated.

About OPKO Health

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 and NDA filed for IV formulation). Our biologics business includes hGH-CTP, a once-weekly human growth hormone injection (in Phase 3 and partnered with Pfizer), a long-acting Factor VIIa drug for hemophilia (entering Phase 2a), and Oxyntomodulin for obesity and diabetes (Phase 1). 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.

SAFE HARBOR STATEMENT

This press release contains "forward-looking statements," as that term is defined under the Private Securities Litigation Reform Act of 1995 (PSLRA), which statements may be identified by words such as "expects," "plans," "projects," "will," "may," "anticipates," "believes," "should," "intends," "estimates," and other words of similar meaning, including statements regarding expected timing of study completion, OPKO's ability to commence its phase 3 pediatric study for hGH-CTP later this year, whether OPKO's clinical trials for hGH-CTP in adult and pediatric growth hormone deficiency and Oxyntomodulin for diabetes and obesity will be successful or generate data to support marketing approval, whether study results will demonstrate hGH-CTP is non-inferior compared to daily hGH, whether hGH-CTP or Oxyntomodulin will be successfully developed or commercialized, expectations regarding the products and their market potential, as well as other non-historical statements about our expectations, beliefs or intentions regarding our business, technologies and products, financial condition, strategies or prospects. 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 the risks inherent in funding, developing and obtaining regulatory approvals of new, commercially-viable and competitive products and treatments. 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, litigation, and the success of our collaboration on hGH-CTP with Pfizer, Inc. 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.

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