June 23, 2014

OPKO Announces Positive Interim Six-Month Lagova™ (hGH-CTP) Phase 2 Data in Pediatric Growth Hormone Deficiency Disorder

Interim efficacy results show that a single weekly injection of Lagova (hGH-CTP) can replace seven consecutive daily injections of currently marketed human growth hormone (hGH)

Conference Call and Slide Presentation Webcast Scheduled Tuesday at 8:30am ET / 7:30am CT

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NYSE:OPK), a multinational biopharmaceutical and diagnostics company, today announced 6 month results of a Phase 2 dose-finding study evaluating the safety and efficacy of its novel long-acting human growth hormone product (Lagova) to treat pediatric growth hormone deficiency disorder (GHD).

All three Lagova once-weekly doses demonstrate strong catch-up growth during the six months treatment. The annualized growth rates are above 12 cm in all three doses. The results are supported by excellent dose dependent pharmacokinetics (PK) and pharmacodynamics (PD) profiles. Lagova shows a promising safety profile with no serious adverse events reported. Glucose and lipid metabolism markers are within the normal ranges. No lipoatrophy was observed in any patients dosed, and no clinically significant local tolerability issues were identified.

“The interim results further affirm that a once-weekly administration of Lagova can replace daily injections of marketed hGH in pediatric GHD patients. The results enable dose selection for the company’s upcoming Phase 3 pediatric trial,” said Dr. Ron Rosenfeld, clinical advisor on the study and professor of Pediatrics (emeritus), Stanford University and professor of Pediatrics at Oregon Health and Science University (emeritus). “Because Lagova consists of native human growth hormone attached to a C-Terminal Peptide of endogenous hormone, one would anticipate low immunogenicity,” Rosenfeld noted.

“Based on these encouraging safety and efficacy results, OPKO plans to move aggressively into a single confirmatory pivotal Phase 3 study for pediatric GHD patients. We hope to make Lagova available to pediatric GHD patients as soon as possible,” said CEO, Phillip Frost, M.D. “Lagova is one of a family of important products being developed at OPKO Biologics designed to improve compliance and offer ease of administration to patients.”

Study Design

The randomized, comparator-controlled Phase 2 study was conducted in up to 56 pre-pubertal, naïve GHD children receiving one of three Lagova doses as once-weekly regimen (0.25, 0.48, 0.66mg/Kg/week; equivalent of 0.18, 0.35, 0.48 mg/Kg/week of hGH) or daily

hGH (34µg/Kg/day) subcutaneously. In order to introduce naïve patients to the allocated Lagova dose in a gradual manner, a stepwise dose increase approach was implemented. Once patients reached the targeted doses, Lagova, GH, IGF-1 and IGF-BP3 concentrations were measured and PK-PD analysis was conducted utilizing a population based approach.

Study Results

An interim analysis of 6 months data demonstrated that all doses of Lagova used in the study provided strong catch-up growth response better than historical controls of daily growth hormone therapy.

The baseline characteristics of all patients were comparable among all groups. Interim analysis of the PK profile following administration of Lagova demonstrates a significantly extended half-life as reflected by the T1/2 and AUC respectively. A dose dependent PD (IGF-1) response was observed between Lagova cohorts, reaching steady state with no accumulation or excessive levels. All cohorts demonstrated promising “catch-up” growth, in line with reported age and GHD severity-matched data. The annualized height velocities are more than 12 cm, which correlates with the PK/PD profile in those patients.

Conference Call and Webcast

OPKO will hold a conference call and live webcast on Tuesday, June 24, 2014 at 8:30 a.m. EDT (7:30 a.m. CDT). The dial-in numbers are 1-877-407-0789 for domestic callers and 1-201-689-8562 for international callers. To join the live webcast of the presentation, please register for ‘OPKO Health: ENDO Conference’ on June 24, 2014, at 8:30 a.m. EDT (7:30 a.m. CDT) at:

http://public.viavid.com/index.php?id=109688

After the webcast, the call will remain available on the OPKO website, www.opko.com, for 30 days.

About Lagova (hGH-CTP)

In June 2013, OPKO initiated a pivotal Phase 3 clinical trial in adults for its proprietary long-acting version of hGH-CTP (Lagova). Lagova has been awarded orphan drug designation in the U.S. and Europe for both adults and children with growth hormone deficiency.

ABOUT OPKO HEALTH

OPKO is a multinational biopharmaceutical and diagnostics company that seeks to establish industry-leading positions in large, rapidly growing markets by leveraging its discovery, development and commercialization expertise and novel and proprietary technologies. For more information, visit http://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 results and benefits of Lagova, including its safety and efficacy, whether OPKO's clinical trials for adult and pediatric growth hormone deficiency will generate data to support marketing approval, whether a single injection of Lagova can replace seven consecutive daily injections of currently marketed hGH, whether Lagova will have low immunogenecity, the expected commencement date for the Phase 3 clinical trial for Lagova in pediatric patients, whether Lagova will be successfully developed or commercialized, expectations regarding the product and its 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, including the risks that the Phase 3 clinical trials for the Lagova product 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 Lagova, and that Lagova may not have advantages or prove to be superior over presently marketed products or products introduced in the future. 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.

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