October 7, 2015

OPKO Presents Clinical Data on its Long-acting Human Growth Hormone (hGH-CTP) in Two Oral Presentations at the 54th Annual Meeting of the European Society for Pediatric Endocrinology (ESPE)

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NYSE: OPK) presented clinical data from its completed 12-month hGH-CTP Phase 2 pediatric growth hormone deficiency clinical study in two oral presentations at the 54th Annual Meeting of the European Society for Paediatric Endocrinology on October 3, 2015, in Barcelona, Spain. The presentations included pharmacokinetic, pharmacodynamic, safety and efficacy data of OPKO's hGH-CTP in comparison to a daily Genotropin® arm.

This phase 2 trial was a 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 (34µg/kg/day). The twelve month data confirmed comparable response of hGH-CTP to daily Genotropin® as reflected by the twelve month pharmacodynamic, efficacy and safety profile.

Following hGH-CTP administration, IGF-1 SDS profile was maintained within the normal range with no accumulation or excessive levels (> +2SDS) during the 12 months of treatment.

Among patients treated with hGH-CTP, the average annual height velocity (HV) ranged between 10.5 to 12.5 cm, a comparable response to the daily hGH arm.

In this study, the safety profile of hGH-CTP was also shown to be comparable to daily hGH.

Highlights from the top-line analysis include:

- there were no patient withdrawals from the study;
- there were no reports of drug-related serious or unexpected adverse events;
- there were no clinically significant local tolerability issues; and
- there were no hGH-CTP neutralizing antibodies.

For all subjects postprandial glucose, insulin and HbA1c (%) levels remained within the normal range throughout the study.

OPKO has a world-wide collaboration agreement with Pfizer Inc. for the development and commercialization of hGH-CTP. Based on the promising phase 2 clinical data, the company confirms its plan to initiate a global pivotal phase 3 study in pre-pubertal GHD children next year following supply of the product by Pfizer in a pen device evaluating a single dose of

hGH-CTP versus daily injections of growth hormone.

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 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 timing of study completion and the commencement date for the phase 3 study, whether OPKO's clinical trials for adult and pediatric growth hormone deficiency will generate data to support marketing approval, whether study results will demonstrate non-inferiority compared to daily hGH, whether hGH-CTP 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. 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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