June 25, 2014

Strong Data from Lagova™ Phase II Clinical Study Presented during Webcast

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NYSE:OPK), a multinational biopharmaceutical and diagnostics company, yesterday hosted a webcast presenting outstanding interim six-month results from a Phase 2 study of its long-acting human growth hormone product Lagova to treat pediatric growth hormone deficiency disorder (GHD).

During the webcast, Dr. Ron Rosenfeld, Chairman of OPKO Biologic's Scientific Advisory Board and professor emeritus of Pediatrics at both Stanford University and Oregon Health and Science University, highlighted the superior interim results from the study. In particular, Dr. Rosenfeld, a pioneer in the recombinant human growth hormone field, noted that the mean height velocity achieved with each of the three different doses of Lagova administered in the study was "actually quite dramatic, ranging from 12.25 centimeters to almost 15.5 centimeters. Every child enrolled in the study experienced a significant improvement in height velocity from weekly administration of Lagova," he noted.

"OPKO believes there is a significant market opportunity with Lagova, as compliance with daily injections is problematic. Non-compliance leads to a significant decrease in growth response in both adults and children, which is well evidenced by current clinical literature," said OPKO's CEO, Phillip Frost, M.D. "The outstanding interim Phase II results allow for dose selection for a Phase III study which is anticipated to start in 2015."

Unlike other pediatric long-acting growth hormone deficiency clinical trials, the Lagova Phase II trials also included a direct comparator arm at the same population with similar baseline values. Children were given daily administration of growth hormone at a dosage of 0.24 mg/kg per week, but administered daily in a traditional manner. There was no statistically significant difference among any of the three dosage strengths of Lagova used in the study versus the comparator arm.

As any Phase III trial for a long-acting recombinant human growth hormone will likely require proof of non-inferiority against a comparator arm of daily administered growth hormone, this is an important distinction that gives OPKO great confidence in designing its non-inferiority pivotal Phase III study for Lagova. Had OPKO utilized historical database comparisons as utilized in other pediatric GHD clinical trials versus an actual comparator arm, annualized height velocity for Lagova at all three dosage strengths would have been notably superior, exceeding comparable age-matched historical controls, as published by Bakker (2008) and Ranke (2010) for the same GHD patient population by approximately 30%.

Lagova uses OPKO Biologics' novel CTP technology which attaches a short naturally occurring peptide to the natural human growth hormone. Approximately 75% of the injected protein in Lagova is the actual native growth hormone, altogether resulting in a highly water soluble compound that can be delivered in high concentrations using a very thin 31 gauge needle to patients of a wide range of body weights and ages by a single, weekly injection. As

expected, the safety profile from the six-month data was excellent. No serious adverse events were reported. There were no episodes of either lipoatrophy or lipodystrophy and there were no injection site tolerability issues. More importantly, long term safety is supported by the pharmacokinetic and pharmacodynamics profiles; there was no accumulation and no over-exposure of IGF-1.

A replay of the webcast is available on OPKO's website, www.opko.com, for the next 30 days.

About Lagova (hGH-CTP)

In June 2013, OPKO initiated a multi-center worldwide 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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