August 29, 2019

OPKO Completes Pivotal Phase 3 Study for Somatrogon (hGH-CTP), an Investigational Long-Acting Growth Hormone Being Studied for the Treatment of Children With Growth Hormone Deficiency

MIAMI, Aug. 29, 2019 (GLOBE NEWSWIRE) -- **OPKO Health, Inc. (NASDAQ: OPK)** today announced that the last patient has completed the final visit in the Company's pivotal Phase 3 study evaluating the safety and efficacy of the once weekly somatrogon (hGH-CTP) for treatment of growth hormone deficiency in children. The study enrolled a total of 224 treatment naïve patients from 21 countries who were randomized 1:1 into two arms: once-weekly somatrogon vs. once-daily Genotropin® (somatropin). The primary endpoint of the trial is height velocity at 52 weeks. Secondary endpoints are safety and pharmacodynamic endpoints. The clinical safety and efficacy data are currently in the process of being compiled and validated prior to data base lock. Top line results are expected to be announced in the fourth quarter of 2019.

Patients who completed the Phase 3 study had the option to enter an open label extension study to evaluate the long-term safety of somatrogon. Approximately 95% of the patients switched into the open label extension study and receive somatrogon treatment.

"We are pleased to report the completion of the somatrogon pivotal study, an important milestone in the development of once weekly somatrogon. Completion of this study brings us a step closer to potentially providing children with growth hormone deficiency a once weekly treatment," stated Phillip Frost, M.D., Chairman and Chief Executive Officer of OPKO. "We believe that a once weekly treatment could offer a convenient dosing regimen, which by reducing the number of injections could enhance a patient's adherence to treatment and quality of life."

In 2014, Pfizer Inc. and OPKO entered into a worldwide agreement for the development and commercialization of somatrogon for the treatment of growth hormone deficiency. Under the agreement, OPKO is responsible for conducting the clinical program and Pfizer is responsible for registering and commercializing the product.

About OPKO Health, Inc.

OPKO Health is a diversified healthcare company. In diagnostics, its BioReference Laboratories is the nation's third largest clinical laboratory; GeneDx is a rapidly growing genetic testing business; the 4Kscore® prostate cancer test is used to confirm an elevated PSA to help decide about next steps such as prostate biopsy; Claros® 1 is a point-of-care

diagnostics platform with a total PSA test approved by the FDA and testosterone as the most advanced test in development. In our pharmaceutical pipeline, RAYALDEE is our first pharmaceutical product to be marketed. OPK88003, a once-weekly oxyntomodulin for type 2 diabetes and obesity recently reported positive data from a Phase 2 clinical trial. It's among a new class of GLP-1/glucagon receptor dual agonists. OPK88004, a SARM (selective androgen receptor modulator) is currently being studied for various potential indications. The company's most advanced product utilizing its CTP technology, a once-weekly human growth hormone for injection, is in Phase 3 trials and is partnered with Pfizer. OPKO also has research, development, production and distribution facilities abroad. More information is available at www.opko.com

Cautionary Statement Regarding Forward-Looking Statements

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," "could," "may," "anticipates," "believes," "should," "intends," "estimates," and other words of similar meaning, including product development efforts and the expected benefits of our products, including hGH-CTP, whether the drug will demonstrate long term safety and efficacy and meet the primary or secondary endpoints, whether the Phase 3 study will be successfully completed and support a dosing change from daily to weekly administration, improve compliance, and positively impact the quality of life for children, and the timeline for announcing topline results, 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 Annual Reports on Form 10-K filed and to be filed with the Securities and Exchange Commission and in our other filings with the Securities and Exchange Commission, as well as liquidity issues and the risks inherent in funding, developing and obtaining regulatory approvals of new, commercially-viable and competitive products and treatments, that earlier clinical results of effectiveness and safety may not be reproducible or indicative of future results, that currently available over-the-counter and prescription products, as well as products under development by others, may prove to be as or more effective than our products for the indications being studied. In addition, forward-looking statements may also be adversely affected by the success of our relationship with Pfizer, 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.

CONTACTS:
Investors
LHA Investor Relations
Miriam Weber Miller, 212-838-3777
MMiller@lhai.com
or
Bruce Voss, 310-691-7100

bvoss@lhai.com

Source: OPKO Health, Inc.