

## **OPKO Health Completes Enrollment in Global Phase 3 Study of Somatrogen (hGH-CTP) in Growth Hormone Deficient Children**

**MIAMI (August 3, 2018) – OPKO Health, Inc.** (NASDAQ: OPK) has completed enrollment in the Company's global pivotal Phase 3 study in growth hormone deficient children, evaluating a single weekly injection of its investigational long-acting human growth hormone product, somatrogen, delivered in a multi-dose disposable pen.

The somatrogen Phase 3 trial is a randomized, open-label, active-controlled study taking place in over 30 countries. This study enrolled approximately 225 treatment-naïve children with growth hormone deficiency (GHD) who were randomized 1:1 into two arms: once-weekly somatrogen vs once-daily Genotropin®. The primary endpoint of the trial is height velocity at 52 weeks. Secondary endpoints are safety and pharmacodynamic endpoints. Children completing this study may enroll in an open-label long-term extension, in which they will receive somatrogen.

Somatrogen (hGH-CTP) is a new molecular entity that maintains the natural sequence of growth hormone, fused with a C-terminus peptide to extend its half-life. In a Phase 2 pediatric trial in which growth hormone deficient children were treated with once-weekly injections of somatrogen, somatrogen produced pharmacodynamic effects similar to daily growth hormone replacement therapy with comparable efficacy and safety. The long-term safety of somatrogen and absence of neutralizing antibodies is confirmed in the ongoing extension, as 43 of the Phase 2 pediatric patients are in their fourth and fifth year of treatment. Somatrogen has received Orphan Drug designation in the U.S. and the EU for the treatment of children and adults with GHD.

"We are pleased to have completed enrollment for this study, as the pediatric segment represents approximately 80% of the market for the treatment of GHD," stated Phillip Frost, M.D., Chairman and Chief Executive Officer of OPKO Health. "We are hopeful that the outcome of this study will support a dosing change from daily to weekly administration and positively impact the quality of life for children with GHD."

OPKO has a worldwide collaboration and license agreement with Pfizer Inc. for the development and commercialization of somatrogen. 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 PSA and testosterone as the most advanced 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 in Phase 2 clinical trials, is among a new class of GLP-1 – glucagon receptor dual agonists. OPK88004, a SARM (Selective Androgen Receptor Modulator) for treating BPH (Benign Prostatic Hypertrophy), urinary incontinence, and other conditions, is in clinical trials. 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 has research, development, production and distribution facilities abroad. More information is available at [www.opko.com](http://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 absence of neutralizing antibodies, whether the Phase 3 study will be successfully completed and support a dosing change from daily to weekly administration and positively impact the quality of life for children, 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.*

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