



## **OPKO Provides Update to Topline Data of Phase 3 Clinical Study of hGH-CTP in Growth Hormone Deficient Adults**

**MIAMI (June 15, 2017) –OPKO Health, Inc. (NASDAQ:OPK)** announces an update to the topline data analysis of the Phase 3, double-blind, placebo-controlled study of its investigational long-acting human growth hormone product (hGH-CTP) in adults with growth hormone deficiency (GHD). In December 2016 OPKO reported that the primary endpoint of change in trunk fat mass from baseline to 26 weeks did not demonstrate a statistical difference between the hGH-CTP treated group and placebo. At the time, one or more outliers were identified that may have affected the outcome on the primary endpoint.

OPKO has now completed post-hoc sensitivity analyses to evaluate the influence of outliers on the primary endpoint results using multiple statistical approaches. Analyses that excluded outliers showed a statistically significant difference between hGH-CTP and placebo on the change in trunk fat mass. Additional analyses that did not exclude outliers showed mixed results. Post-hoc analyses do not carry the same weight of evidence as a pre-specified primary analysis.

“We are encouraged by the results of these analyses,” stated Phillip Frost, M.D., OPKO Chairman and Chief Executive Officer.

OPKO has initiated a global Phase 3 trial, utilizing a multi-dose disposable pre-filled pen, to evaluate hGH-CTP in pediatric GHD patients. OPKO also plans to utilize this multi-dose pen for patients who continue in the open label extension phase of the adult Phase 3 and the pediatric Phase 2 GHD studies. In addition, preparation for the pivotal pediatric GHD trial in Japan is progressing and site selection is near completion.

### **About OPKO Health, Inc.**

OPKO Health is a diversified healthcare company that seeks to establish industry leading positions in large, rapidly growing markets. Our diagnostics business includes BioReference Laboratories, the nation's third largest clinical laboratory with a core genetic testing business and a 400 person sales and marketing team to drive growth and leverage new products, including the 4Kscore® prostate cancer test and the Claros® 1 in office immunoassay platform. Our pharmaceutical business features RAYALDEE, an FDA approved treatment for SHPT in stage 3-4 CKD patients with vitamin D insufficiency (launched in November 2016), VARUBI™ for chemotherapy induced nausea and vomiting (oral formulation launched by partner TESARO and IV formulation pending FDA approval), OPK88004, a once or twice weekly oxyntomodulin for type 2 diabetes and obesity, in Phase 2 clinical trials, among the new class of GLP-1 glucagon receptor dual agonists, and OPK88003, a selective androgen receptor modulator for benign prostatic hyperplasia (Phase 2). Our biologics business includes hGH-CTP, a once weekly human growth hormone in Phase 3 and partnered with Pfizer; and a long-acting Factor VIIa drug for hemophilia in Phase 2a. More information available at [www.opko.com](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 expectations about hGH-CTP and the results of the sensitivity analyses, expectations regarding our ongoing and expected clinical studies for hGH-CTP, whether the studies will be successfully completed and on a timely basis, and whether data from our studies for hGH-CTP, including the post-hoc sensitivity analyses, will support submission of a Biologics License Application and approval for hGH-CTP, 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 the risks inherent in funding, developing and obtaining regulatory approvals of new, commercially-viable and competitive products and treatments. 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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