OPKO PROVIDES UPDATE ON THE DEVELOPMENT OF OPK-88004, A SELECTIVE ANDROGEN RECEPTOR MODULATOR

MIAMI (January 31, 2019) – OPKO Health, Inc. (NASDAQ: OPK) today announced an update on the ongoing development plans of OPK-88004, a selective androgen receptor modulator (SARM).

OPK-88004 has the ability to serve as an antagonist to androgen receptors in the prostate, resulting in decreased prostate specific antigen (PSA) levels, and as an agonist resulting in increased anabolic effects, such as increased lean body mass and physical function and decreased fat mass. Promising clinical data from prior studies suggest that OPK-88004 can be used for the treatment of a number of symptoms such as frailty, physical function and quality of life parameters for various indications.

OPKO is currently conducting a Phase 2 clinical trial that evaluates the effect of 15mg and 25mg doses of OPK-88004 on prostate volume in patients with Benign Prostatic Hyperplasia. In reviewing the blinded data, it has been noticed that to measure one of the study's primary endpoints (decreases in prostate volume), the trans-rectal ultrasound method proved to be too imprecise to reliably determine the drug effect. Also, although most of the laboratory changes were consistent with this class of drugs, increases in liver enzymes were observed in several men at these higher doses. The increases were transient in patients that completed the trial and returned to normal once the drug was withdrawn. Importantly, no increases in liver enzymes were observed in a previous large Phase 2 study of 420 men taking lower doses of OPK-88004 (1mg and 5mg). OPKO plans to suspend the current trial but continue to analyze data relating to the other primary endpoint, the effect of OPK-88004 on serum PSA levels, and the secondary endpoints, changes in lean body mass (LBM) and fat mass. The results of this data analysis are expected in Q2 2019.

OPKO is planning additional exploratory Phase 2 clinical studies to assess the feasibility of OPK-88004 in other indications. One of the trials is designed to study the safety and efficacy of OPK-88004 in prostate cancer patients treated with Androgen Deprivation Therapy (ADT). ADT results in the reduction of testosterone to castration levels in men with prostate cancer. However, ADT is associated with side effects such as decreased LBM, physical function, bone quality and increases in body fat, frailty and hot flashes. The diminished quality of life results in approximately one-third of men treated with ADT stopping treatment within six months of initiation. OPKO's trial data in aging men indicate that OPK-88004 increases LBM, decreases fat and increases physical function. Because OPK-88004 decreases PSA levels due to its antagonistic effects on the prostate, it may be well-suited to treat men on ADT therapy. The contemplated Phase 2 study is designed to show that OPK-88004 improves ADT-associated symptoms and quality of life of prostate cancer patients. Approximately one million men are receiving ADT therapy in the U.S.

Because of OPK-88004's significant increase of muscle mass (LBM) and strength, and because of OPKO's strong interest in the therapy of chronic kidney disease (CKD) patients, another Phase 2 trial is planned to treat kidney dialysis patients who have low testosterone levels and commonly suffer from muscle weakness and general frailty. Approximately 500,000 CKD patients are on dialysis therapy in the U.S.

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) currently being studied for benign prostatic hyperplasia but for which we are exploring other 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," "may," "anticipates," "believes," "should," "intends," "estimates," and other words of similar meaning, including statements about expectations for the SARM and our ability to develop it for ADT and other indications, as well as other non-historical statements about our expectations, beliefs or intentions. 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. 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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