OPKO Initiates RAYALDEE Phase 2 Trial in Dialysis Patients

MIAMI (September 4, 2018) – OPKO Health, Inc. (NASDAQ: OPK) has initiated a Phase 2 clinical trial to study the safety and efficacy of RAYALDEE® as a new treatment for secondary hyperparathyroidism (SHPT) in adults with vitamin D insufficiency and stage 5 chronic kidney disease (CKD) requiring hemodialysis. The trial will be conducted at multiple dialysis centers in the U.S. in two sequential cohorts.

The first cohort of approximately 44 patients will be treated for 26 weeks in a randomized, open-label fashion with either RAYALDEE or placebo to identify the appropriate dosing to be studied in the second cohort. Data readout for this first cohort is expected in 2019.

The second cohort of more than 200 patients will be treated for 26 weeks in a randomized, double-blind fashion with one of three different doses of RAYALDEE or placebo. The primary efficacy endpoint will be correction of vitamin D insufficiency and control of SHPT. Patients will then be treated with RAYALDEE for another 26 weeks in an open-label extension.

“We are pleased to announce the start of this important trial as it represents a major step in our coordinated plan to develop RAYALDEE for the growing global population of dialysis patients,” commented Phillip Frost, M.D., Chairman and Chief Executive Officer of OPKO Health. “Currently, there are approximately 500,000 stage 5 CKD patients receiving dialysis in the U.S., most with SHPT and vitamin D insufficiency.”

RAYALDEE is an extended-release prohormone of calcitriol, the active form of vitamin D_3. The product is the only such therapy approved by the U.S. Food and Drug Administration (FDA) that raises serum 25-hydroxyvitamin D and lowers blood levels of parathyroid hormone. RAYALDEE is approved in the U.S. to treat SHPT in adults with stage 3 or 4 CKD and vitamin D insufficiency. It is not yet approved for patients with stage 5 CKD on dialysis therapy. OPKO Health launched RAYALDEE in the U.S. in November 2016.

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.

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 RAYALDEE, whether we will successfully develop RAYALDEE as a new treatment for SHPT in adults with vitamin D insufficiency and stage 5 chronic kidney disease requiring hemodialysis, whether we will successfully complete Cohort 1 and Cohort 2 of the clinical study contemplated for RAYALDEE in hemodialysis patients and the timing for doing so, the timing for the data readout from Cohort 1 of the study, and our ability to develop and commercialize RAYALDEE for the growing global population of dialysis patients, 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, the success of our relationship with our commercial partners for RAYALDEE, that earlier clinical results of effectiveness and safety may not be reproducible or indicative of future results, and 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 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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