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OPKO Completes Patient Enrollment in Third Phase 3 Trial of RAYALDEE™

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NYSE: OPK), has completed patient enrollment in the third phase 3 trial of *RAYALDEE*. This trial is designed to evaluate the product's long-term safety and efficacy in treating secondary hyperparathyroidism (SHPT) in patients with stage 3 or 4 chronic kidney disease (CKD) and vitamin D insufficiency.

This third phase 3 trial is a 6-month open-label extension of two ongoing and identical randomized, double-blind, placebo-controlled, multi-site pivotal phase 3 studies for *RAYALDEE* intended to support marketing approval in the United States (US). Together, the two pivotal trials involve approximately 430 patients recruited at approximately 90 US sites who are receiving six months of treatment with either *RAYALDEE* or placebo. Both trials will end in July 2014 and the Company expects to announce top-line data during the third quarter of 2014. In the open-label extension study, patients either continue *RAYALDEE* treatment or switch to *RAYALDEE* from placebo treatment. Additional patients will be allowed to enroll in the open-label extension study through July as they exit the blinded pivotal trials. The endpoints of all three phase 3 studies include vitamin D status and changes in plasma intact parathyroid hormone (PTH), serum calcium and serum phosphorus.

Recently, OPKO also completed a pharmacokinetic study of *RAYALDEE* in approximately 50 healthy US volunteers. This study, designed to evaluate the effect of food on the intestinal absorption of *RAYALDEE*, is the last clinical trial (aside from the above-mentioned phase 3 trials) needed to support the forthcoming New Drug Application (NDA). OPKO expects that an NDA for *RAYALDEE* will be filed with the US Food and Drug Administration in the first quarter of 2015.

"The development of *RAYALDEE* is proceeding fully as we expected: on schedule and on budget," remarked Phillip Frost, M.D., OPKO's CEO and Chairman. "We believe our proprietary first-in-class vitamin D product will be a valuable new treatment for chronic kidney disease patients and we look forward to reporting final data from the ongoing pivotal trials promptly."

About *RAYALDEE*

RAYALDEE is a first-in-class oral vitamin D prohormone treatment being developed for SHPT in patients with stage 3 or 4 CKD and vitamin D insufficiency. It has a proprietary modified-release formulation designed to gradually and reliably raise serum total 25-hydroxyvitamin D (prohormone) concentrations to targeted levels (at least 30 ng/mL) while avoiding upregulation of CYP24, a cytochrome P-450 enzyme that reduces the PTH-lowering potency of current vitamin D supplements. Activation of calcifediol, the active ingredient in *RAYALDEE*, by the kidney is tightly regulated, preventing excessive elevation of serum calcium and related side effects which limit the value of current vitamin D hormone therapies

by promoting vascular and renal calcification. *RAYALDEE* is expected to address the approximately 4 million patients in the US, and many more elsewhere, with stage 3 or 4 CKD, SHPT and vitamin D insufficiency.

About Chronic Kidney Disease

CKD is a condition characterized by a progressive decline in kidney function. The kidney is normally responsible for excreting waste and excess water from the body, and for regulating various hormones. CKD is classified in five different stages — mild (stage 1) to severe (stage 5) disease — as measured by the kidney's glomerular filtration rate. According to the National Kidney Foundation, CKD afflicts over 26 million people in the US, including more than eight million patients with moderate (stages 3 or 4) and severe (stage 5) forms of CKD. In stage 5 CKD, kidney function is minimal to absent and patients require regular dialysis or a kidney transplant for survival.

About Vitamin D Insufficiency

Vitamin D insufficiency is a condition in which the body has low vitamin D stores, characterized by inadequate blood levels of vitamin D prohormone, known as 25-hydroxyvitamin D. An estimated 70-90% of CKD patients have vitamin D insufficiency, which can lead to SHPT and resultant debilitating bone diseases.

About Secondary Hyperparathyroidism (SHPT)

SHPT is a condition commonly associated with CKD in which the parathyroid glands secrete excessive amounts of PTH. SHPT arises as a result of vitamin D insufficiency or impaired kidney function that prevents sufficient production of vitamin D hormone to properly regulate calcium and phosphorus metabolism, and PTH secretion. Prolonged elevation of blood PTH causes excessive calcium and phosphorus to be released from bone, leading to elevated serum calcium and phosphorus, softening of the bones (osteomalacia) and calcification of vascular and renal tissues. SHPT affects 40-60% of patients with moderate CKD and approximately 90% of patients with severe CKD.

About OPKO

OPKO is a multinational biopharmaceutical and diagnostics company that seeks to establish industry leading positions in large, rapidly growing markets by leveraging its discovery, development and commercialization expertise and novel and proprietary technologies.

This press release contains "forward-looking statements," as that term is defined under the Private Securities Litigation Reform Act of 1995 (PSLRA), regarding product development efforts and other non-historical facts about our expectations, beliefs or intentions regarding our business, technologies and products, financial condition, strategies or prospects, including statements regarding our ability to successfully launch and commercialize proprietary renal disease products, expectations about RAYALDEE, its market potential, that it will address the approximately 4 million patients in the US, and many more elsewhere, with stage 3 or 4 CKD, SHPT and vitamin D insufficiency, that RAYALDEE will be a valuable new treatment for CKD patients, the expected timing for completion of our clinical trials for RAYALDEE, announcement of top line results, and the submission of an NDA, whether the trials for RAYALDEE will continue to proceed on schedule and on budget, and that we will be

able to successfully develop, obtain approval for and launch sales of RAYALDEE. 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 filings with the Securities and Exchange Commission, as well as risks inherent in funding, developing and obtaining regulatory approvals of new, commercially-viable and competitive products and treatments, including the risks that the phase 3 clinical trials for RAYALDEE may not be successful or achieve the expected results or effectiveness, and may not generate data that would support the approval or marketing of this product for the indications being studied, that others may develop products which are superior to RAYALDEE, and that RAYALDEE may not have advantages or prove to be superior over presently marketed products, including the currently used high monthly doses of prescription vitamin D_2, activated vitamin D hormone and over-the-counter vitamin D supplements. 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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Source: OPKO Health, Inc.