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OPKO Completes Patient Recruitment in Second Phase 3 Trial of RayaldyTM

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NYSE: OPK), has completed patient recruitment in the second phase 3 trial of *Rayaldy*™ to treat patients with secondary hyperparathyroidism (SHPT), stage 3 or 4 chronic kidney disease (CKD) and vitamin D insufficiency. This trial is the second of two identical randomized, double-blind, placebo-controlled, multi-site studies intended to establish the safety and efficacy of *Rayaldy*™ as a new treatment for SHPT in the targeted population. The endpoints of both studies, which are being conducted in parallel, include vitamin D status and changes in serum calcium, serum phosphorus and plasma intact parathyroid hormone (PTH).

Each of the two pivotal phase 3 trials involves approximately 210 patients recruited at approximately 40 sites in the United States (U.S.). Patients are being stratified by CKD stage and randomized in a 2:1 fashion to receive six months of treatment with either *Rayaldy*™ or placebo. Top-line data from both trials are expected in mid-2014.

The two pivotal trials are being followed by an open-label extension study in which patients are treated, at their election, for an additional 6 months with *Rayaldy*™. More than 50% of the required patients have enrolled in this open-label study and some have already completed treatment.

About *Rayaldy*™

Rayaldy™ is a first-in-class oral vitamin D prohormone treatment being developed for SHPT in stage 3 and 4 CKD patients with 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 Rayaldy™, by the kidney is tightly regulated, preventing excessive elevation of serum calcium and related side effects which encumber current vitamin D hormone therapies and promote vascular and renal calcification. Once approved, *Rayaldy*™ is expected to address the approximately 4 million CKD stage 3 and 4 patients in the U.S. and many more, elsewhere, with 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 U.S., including

more than eight million patients with moderate (stages 3 and 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 Health, Inc.

OPKO is a multi-national biopharmaceutical and diagnostics company that seeks to establish industry-leading positions in large and rapidly growing medical markets by leveraging our discovery, development and commercialization expertise and our novel and proprietary technologies. For more information, visit our website at http://www.opko.com.

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 expectations about Rayaldy™, its market potential, and that it will address the approximately 4 million CKD stage 3 and 4 patients in the U.S. and many more elsewhere, with SHPT and vitamin D insufficiency, that we will be able to successfully develop, obtain approval for and launch sales of Rayaldy™, the expected completion dates for our trials and that top-line data will be available in mid-2014. 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 Rayaldy™ 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 Rayaldy™, and that Rayaldy™ 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.