OPKO Health Initiates Clinical Trial of RAYALDEE in COVID-19 Patients

MIAMI (September 15, 2020) – OPKO Health, Inc. (NASDAQ: OPK) announces the initiation of a Phase 2 trial with RAYALDEE® as a treatment for mild-to-moderate COVID-19. The trial, "A Randomized, Double-Blind Placebo-Controlled Study to Evaluate the Safety and Efficacy of **R**AYALDEE (calcifediol) **E**xtended-releas**e** **C**apsu**l**es to Treat Symptomatic Patients Infected with SARS-CoV-2 (**REsCue**)," is expected to enroll approximately 160 subjects, many with stage 3 or 4 chronic kidney disease (CKD) who are at higher risk for developing more severe illness.

The trial will be conducted at multiple COVID-19 outpatient clinics in the U.S. The initial sites are located primarily in South Florida, the Central Gulf coast, the Midwest and the Southwest. The first subjects are expected to be enrolled within the next few weeks.

The REsCue trial will randomize COVID-19 outpatients in a 1:1 ratio to 4 weeks of treatment with RAYALDEE or placebo and 2 weeks of follow-up. Dosing with RAYALDEE will commence with 300 mcg per day on Days 1, 2 and 3 followed by 60 mcg per day on Days 4 through 27. Primary efficacy endpoints are raising and maintaining serum total 25-hydroxyvitamin D (25D) within the range of 50-100 ng/mL and time to resolution of COVID-19 symptoms. Secondary endpoints include incidence of emergency room or urgent care visits, oxygen saturation below 94%, need for and duration of hospitalizations, requirement for mechanical ventilation, mortality rate, and severity and duration of illness evidenced by quality-of-life measures. More information about this trial will soon become available on https://clinicaltrials.gov/.

Numerous independent studies have reported a correlation between vitamin D status and COVID-19 risk and severity. OPKO expects to report topline results from this Phase 2 trial before year-end.

About RAYALDEE

RAYALDEE is an extended-release oral formulation of calcifediol, a prohormone of calcitriol, the active form of vitamin D_3. The product is the first and only medicine approved by the U.S. Food and Drug Administration (FDA) for raising serum total 25D and lowering blood levels of intact parathyroid hormone (iPTH). RAYALDEE, approved to treat secondary hyperparathyroidism (SHPT) in adults with stage 3 or 4 CKD and vitamin D insufficiency, was launched in November 2016.

About OPKO Health, Inc.

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. For more information, visit 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 RAYALDEE, whether and when we will initiate and complete the clinical studies contemplated for RAYALDEE and whether final study data will be positive, our ability to develop and commercialize RAYALDEE for COVID-19 patients, whether RAYALDEE is capable of treating patients with COVID-19 including whether RAYALDEE could impact the SARS-CoV-2 virus or cytokine storm, or have any impact on the severity of the disease or that it will effectively raise and maintain serum total 25D consistently at or above 50ng/mL, 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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