June 1, 2020

FDA Authorizes OPKO Health Clinical Trial Evaluating RAYALDEE in COVID-19 Patients

MIAMI, June 01, 2020 (GLOBE NEWSWIRE) -- **OPKO Health, Inc.** (NASDAQ: OPK) today announced that the U.S. Food and Drug Administration (FDA) has authorized OPKO to undertake a Phase 2 trial with RAYALDEE® as a treatment for patients with mild-to-moderate COVID-19. The trial, entitled "A Randomized, Double-Blind Placebo-Controlled Study to Evaluate the Safety and Efficacy of **R**AYALDEE (calcifediol) **E**xtended-relea**s**e **C**aps**u**l**e**s to Treat Symptomatic Patients Infected with SARS-CoV-2 (**REsCue**)," is estimated to enroll 166 subjects, including many with stage 3 or 4 chronic kidney disease (CKD).

The REsCue trial will have 4 weeks of treatment with RAYALDEE or placebo and 2 weeks of follow-up. The objective is to raise and maintain serum total 25-hydroxyvitamin D (25D) within the range of 50-100 ng/mL in order to mitigate COVID-19 severity.

"Raising serum 25D enables macrophages, a type of white blood cell of the immune system, to secrete potent antiviral proteins that can destroy SARS-CoV-2, the virus that causes COVID-19," explained Charles W. Bishop, PhD, CEO of OPKO's Renal Division. "It also can suppress the cytokine storm triggered by viral infection."

COVID-19 disproportionately afflicts patients with obesity, older age, darker skin or CKD, all of which are risk factors for reduced serum 25D. Raising 25D sufficiently with supplements is difficult.

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. 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, impacting 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.

CONTACTS:
Investors
LHA Investor Relations
Yvonne Briggs, 310-691-7100
ybriggs@lhai.com
or
Bruce Voss, 310-691-7100
bvoss@lhai.com

Source: OPKO Health, Inc.