

OPKO ANNOUNCES POSITIVE TOPLINE RESULTS IN PHASE 2 DIABETES AND OBESITY TRIAL

OPKO Health's Oxyntomodulin, OPK88003, Shows Significant Reduction in HbA1c and Body Weight in Patients with Type 2 Diabetes

MIAMI (March 21, 2019) – OPKO Health, Inc. (NASDAQ: OPK) today announced positive topline results from a Phase 2 dose escalation trial of OPK88003 to treat type 2 diabetes and obesity. OPK88003 is a once-weekly injectable oxyntomodulin compound with glucagon-like-peptide 1 (GLP-1) and glucagon dual agonist activity. Based on data from a previous 420 patient Phase 2 study, an optimized dosing regimen for OPK88003 was evaluated to improve glucose control and increase weight loss. Topline analysis of results of the study demonstrated that OPK88003 met the primary objective with a statistically significant lowering of hemoglobin A1c (HbA1c) after 30 weeks of treatment versus placebo as well as an important secondary endpoint, statistically significant weight loss versus placebo.

This Phase 2b trial evaluated the effects of a dose escalation regimen of OPK88003 on HbA1c, weight loss and safety over 30 weeks in adult type 2 diabetes patients with inadequate glucose control with metformin and/or diet and exercise. One hundred and thirteen type 2 diabetes patients were enrolled and randomized into two arms, OPK88003 or placebo, at a ratio of 1.75:1, with a volume matched placebo arm and an OPK88003 treated arm, starting with 20 mg for 4 weeks, then 40 mg for 4 weeks, and finally, a target dose of 70 mg for 22 weeks.

The data were analyzed for the modified intent to treat patient population (mITT; 108 of 113 patients) using the mixed model repeated measures (MMRM). The mITT population includes all patients that received at least one dose of drug and had one post baseline evaluation.

OPK88003 showed a strong, clinically meaningful reduction in HbA1c at 30 weeks (-1.30% versus placebo, -0.09% mean absolute reduction, $p < 0.0001$). Additionally, 50% of OPK88003 treated patients achieved HbA1c $\leq 6.5\%$ versus 13.8% of placebo treated subjects ($p = 0.0008$).

Patients treated with OPK88003 achieved a significant weight loss at 30 weeks (-4.4 kg, compared to placebo -1.8 kg, $p = 0.01$). Approximately 38% of treated patients achieved a 5% or greater body weight loss compared to 13% of placebo treated patients ($p = 0.008$).

OPK88003 treated patients showed significant blood triglyceride decreases from baseline. The decrease in triglycerides in the OPK88003 treatment group was -31.2 mg/dL ($p = 0.005$) compared to -11.6 mg/dL for placebo ($p = 0.44$). OPK88003 treatment showed a safety and tolerability profile expected for the GLP-1 receptor agonist class. The most frequent adverse events were nausea, vomiting and diarrhea. These were mostly mild and occurred predominantly during the titration period and resolved over time. No serious adverse events were observed.

Based on this trial data, OPKO is planning to further evaluate OPK88003 for a Phase 3 clinical program in type 2 diabetes and obesity and potentially for other promising indications such as NASH.

"We are pleased with the results of this trial, which show that OPK88003 has the potential to compete favorably with other drugs on the market or under development to treat type 2 diabetes, obesity and related conditions. We will now carefully evaluate strategic options for the design of Phase 3 trials and later, if successful, commercialization," said Dr. Phillip Frost, Chairman and Chief Executive Officer of OPKO Health.

The Company plans to present these results at the upcoming American Diabetes Association (ADA) meeting (San Francisco, June 7-11, 2019).

According to the ADA, approximately 30 million people in the U.S. have type 2 diabetes and 1.5 million Americans are diagnosed each year. The ADA estimates the annual cost of care for diabetic patients to be \$327 billion, including direct medical costs and reduced productivity.

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 a PSA test approved by the FDA and testosterone as the most advanced test 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) has been studied for benign prostatic hyperplasia but we are exploring other potential indications. 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 also has research, development, production and distribution facilities abroad. More information is available at www.opko.com.

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," "may," "anticipates," "believes," "should," "intends," "estimates," and other words of similar meaning, including statements about expectations for OPK88003 and our ability to develop it for diabetes, obesity and other indications, plans for the product and whether it has the potential to compete with other drugs on the market or under development to treat type 2 diabetes, obesity and related conditions, whether we will be able to successfully develop, complete studies for, or commercialize OPK88003, as well as other non-historical statements about our expectations, beliefs or intentions. 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. 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:

LHA Investor Relations

Miriam Weber Miller, 212-838-3777

MMiller@lhai.com

or

Bruce Voss, 310-691-7100

bvoss@lhai.com

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