

## OPKO REPORTS ADDITIONAL OXYNTOMODULIN, OPK-88003, RESULTS FROM PHASE 2 DIABETES AND OBESITY TRIAL

**MIAMI (April 12, 2019)** – **OPKO Health, Inc. (NASDAQ: OPK)** today reported additional results from the per protocol patient population from the Phase 2 dose escalation trial of OPK-88003 to treat type 2 diabetes and obesity.

Topline data were previously reported on March 21, 2019 for the modified intent to treat (mITT) patient population using the mixed model repeated measures (MMRM). The mITT population included all patients that received at least one dose of drug and had one post-baseline evaluation. In the mITT population OPK-88003 showed a strong, clinically meaningful reduction in HbA1c at 30 weeks of -1.30% in the treated group from baseline (p-value <0.0001), and -0.09% in the placebo group (p-value <0.60). Patients treated with OPK-88003 achieved a significant weight loss from baseline at 30 weeks, -4.4 kg (p-value <0.0001), compared to placebo, -1.8 kg, (p-value <0.05).

### Analysis of HbA1c and Body Weight Changes from Baseline to Week 30

Parameter (Unit) Statistics	mITT Population		Per Protocol Population	
	OPK-88003 (n=69)	Placebo (n=39)	OPK-88003 (n=45)	Placebo (n=28)
HbA1c (%)				
LS Mean (SE)	-1.30 (0.137)	-0.09 (0.181)	-1.47 (0.168)	-0.25 (0.209)
95% Confidence Interval	[-1.57, -1.02]	[-0.45, 0.27]	[-1.81, -1.14]	[-0.67, 0.17]
p-value	<0.0001	<0.60	<0.0001	<0.24
Body Weight (kg)				
LS Mean (SE)	-4.4 (0.633)	-1.8 (0.890)	-5.5 (0.682)	-1.9 (0.902)
95% Confidence Interval	[-5.64, -3.13]	[-3.52, 0.01]	[-6.90, -4.17]	[-3.73, -0.13]
p-value	<0.0001	<0.05	<0.0001	<0.04

Following further analyses in the per protocol population, which includes all patients that received treatment with OPK-88003 for at least 26 weeks of the 30-week trial, the decrease in HbA1c and the increase in weight loss were more pronounced compared to the mITT patient population. The reduction in HbA1c at 30 weeks was -1.47% (p-value <0.0001) in the treated group, and -0.25% in the placebo group (p-value <0.24). Additionally, in the per protocol population the weight loss was -5.5 kg (p-value

<0.0001) in the treated group, and -1.9 kg (p-value <0.04) in the placebo group. OPK-88003 treatment was more effective in patients who received drug for at least 26 weeks and were compliant with the protocol for the duration of the trial.

Similarly, other efficacy parameters improved in the per protocol population such as percentage of patients achieving less than 6.5% HbA1c, percentage of patients achieving weight loss greater than 5% and decrease in serum level of triglycerides.

This Phase 2b trial evaluated the effects of a dose-escalation regimen of OPK-88003 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. A total of 113 type 2 diabetics were enrolled and randomized into two arms; OPK-88003 and placebo, at a ratio of 1.75:1. The trial included a volume-matched placebo arm and an OPK-88003 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 Phase 2b dose escalation trial demonstrated that the once weekly OPK-88003 provided competitive clinical data. OPKO is planning to further evaluate OPK-88003 in a Phase 3 clinical program in type 2 diabetes and obesity, as well as in other promising indications such as NASH.

### **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<sup>®</sup> prostate cancer test is used to confirm an elevated PSA to help decide about next steps such as prostate biopsy; Claros<sup>®</sup> 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](http://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," "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, NASH 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.

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