OPKO Health Reports 2019 First Quarter Business Highlights and Financial Results

Conference call begins today at 4:30 p.m. Eastern time

MIAMI (May 7, 2019) – OPKO Health, Inc. (NASDAQ: OPK) reports business highlights and financial results for the three months ended March 31, 2019.

Business Highlights

- **RAYALDEE total prescriptions reported by IQVIA increased 121% in 1Q 2019 compared with 1Q 2018:** Total prescriptions for the first three months of 2019 increased to over 10,400, compared with approximately 4,700 during the comparable period of 2018.

- **RAYALDEE Marketing Authorization Applications filed by Vifor Fresenius accepted for review in several European countries:** These applications request approval to market RAYALDEE for the treatment of secondary hyperparathyroidism (SHPT) in adult non-dialysis patients with chronic kidney disease (CKD).

- ***American Journal of Nephrology* published article and editorial:** A post hoc analysis of RAYALDEE pivotal trials was published in the March edition of the *American Journal of Nephrology* and provides the first prospective data demonstrating that the current clinical practice guideline target for serum total 25-hydroxyvitamin D (30 ng/ml) is too low to decrease elevated serum PTH levels in CKD patients. These data show that the correct target is at least 51 ng/mL, and possibly as high as 93 ng/mL, levels no competitive therapy can reliably attain. This analysis received a favorable review in a co-published editorial by prominent nephrology KOLs.

- **OPK88003 topline results reported from Phase 2 diabetes and obesity trial:** In the mITT patient population, which included all patients who had a single dose of drug and one post-baseline efficacy value, HbA1c decreased by -1.3% (p-value <0.0001) and body weight decreased by -4.4 Kg (p-value <0.0001) from baseline. In the Per Protocol patient population, which included patients who were treated for at least 26 weeks and were compliant with the protocol for the duration of the trial, HbA1c decreased by -1.47% (p-value <0.0001) and body weight decreased by -5.5 Kg (p-value <0.0001) from baseline. The safety profile was similar to that expected for the incretin class of drugs, with GI side effects such as nausea, vomiting and diarrhea mostly mild and occurring during the dose-escalation phase.

- **4Kscore® utilization remained strong during 1Q 2019 with approximately 19,400 tests performed**: With all required documentation nearly complete, the company plans to make a

submission shortly to the FDA for approval or clearance. In addition, OPKO has submitted a request for Medicare coverage reconsideration to its Medicare Administrative Contractor.

- **BioReference and its GeneDx subsidiary expand access to commercially insured lives:** BioReference and GeneDx have been selected for inclusion in the UnitedHealthcare Preferred Lab Network beginning July 1, 2019. This was a comprehensive process that evaluated service standards, turnaround time, quality and accreditation. More than 300 laboratories were invited to apply; only 100 submitted applications due to the complexity of the requirements, and BioReference and GeneDx, along with five other laboratories, earned a place in the Preferred Lab Network. In addition, effective April 1, 2019, BioReference and GeneDx are now in-network providers with Humana, which provides access to 11 million additional lives.

Financial Highlights

- Consistent with financial guidance issued in February 2019, the net loss for the first quarter of 2019 was $80.8 million, compared with a net loss of $43.1 million for the comparable period of 2018.

- Consolidated revenues for the first quarter of 2019 were $222.5 million, compared with $254.9 million for the comparable period of 2018. During the 2019 quarter, revenue from services was $178.9 million, revenue from products was $25.3 million, including RAYALDEE net revenue of $5.8 million, and revenue from licensing and intellectual property was $18.3 million. The decrease in revenue from services principally reflect reimbursement headwinds from higher denial rates in the company's esoteric testing lines of business.

- Operating expenses for the first quarter of 2019 of $297.8 million included continued investment in the company's pharmaceutical pipeline, with R&D expense of $36.5 million, principally for pediatric trials with the hGH-CTP long-acting human growth hormone product.

- In February 2019 OPKO completed the sale of $200 million aggregate principal amount of 4.5% Convertible Senior Notes due 2025, as well as retired and repaid $28.8 million of its 2033 Convertible Notes.

- Cash, cash equivalents and marketable securities were $207.3 million as of March 31, 2019.

CONFERENCE CALL & WEBCAST INFORMATION

OPKO's senior management will provide a business update and discuss results in greater detail in a conference call and live audio webcast at 4:30 p.m. Eastern time today, May 7, 2019. The conference call dial-in and webcast information is as follows:

DOMESTIC DIAL-IN:	866-634-2258
INTERNATIONAL DIAL-IN:	330-863-3454
PASSCODE:	1547027
WEBCAST:	https://edge.media-server.com/m6/p/hhtmqkq9

For those unable to participate in the live conference call or webcast, a replay will be available beginning approximately two hours after the completion of the conference call. To access the replay, dial 855-859-2056 or 404-537-3406. The replay passcode is 1547027. The replay can be accessed for a period of time on OPKO's website at https://edge.media-server.com/m6/p/hhtmqkq9.

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 total 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 recently reported positive data from a Phase 2 clinical trial. It's among a new class of GLP-1/glucagon receptor dual agonists. OPK88004, a SARM (selective androgen receptor modulator) is currently being studied for various 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

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 regarding expected financial performance and expectations regarding the market for and sales of our products, our product development efforts and the expected benefits of our products, including whether our ongoing and future clinical trials will be successfully enrolled or completed on a timely basis or at all and whether the data from any of our trials will support submission or approval, validation and/or reimbursement for our products, whether we will be able to reverse or rescind the Novitas non coverage decision for the 4kscore test for Medicare beneficiaries, statements regarding our planned submission for FDA approval of the 4kscore test and the timeline for the submission, whether we will be able to develop OPK88004 for additional indications, whether OP88003 will be shown to be safe and effective and achieve even greater weight loss, the expected timing for launch of our products in development, the expected timing of commencing and concluding our clinical trials, expected enrollment in clinical trials, the timing of our regulatory submissions, our ability to market and sell any of our products in development, and expectations about developing RAYALDEE for dialysis patients, 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 the continuation and success of our relationship with Pfizer and our other partners, integration challenges for Bio-Reference, and other acquired businesses, liquidity issues and the risks inherent in funding, developing and obtaining regulatory approvals of new, commercially-viable and competitive products and treatments, that earlier clinical results of effectiveness and safety may not be reproducible or indicative of future results, that the 4Kscore, RAYALDEE, hGH-CTP, OPK88003, OPK88004, and/or any of

our compounds or diagnostic products under development may fail, may not achieve the expected results or effectiveness and may not generate data that would support the approval or marketing of products for the indications being studied or for other indications, 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:

LHA Investor Relations
Miriam Weber Miller, 212-838-3777
MMiller@lhai.com
or
Bruce Voss, 310-691-7100
bvoss@lhai.com

—Tables to Follow—

OPKO Health, Inc. and Subsidiaries
Condensed Consolidated Balance Sheets
(in millions)

	As of	
	March 31, 2019	December 31, 2018
Assets:		
Cash, cash equivalents and marketable securities	$ 207.3	$ 96.5
Other current assets	227.9	221.2
Total Current Assets	435.2	317.7
In-process Research and Development and Goodwill	1,334.1	1,335.8
Other assets	806.0	797.6
Total Assets	$ 2,575.3	$ 2,451.1
Liabilities and Equity:		
Current liabilities	$ 396.1	$ 288.3
Convertible Notes	203.7	57.3
Deferred tax liabilities, net	116.0	115.2
Other long-term liabilities, principally deferred revenue, contingent consideration and lines of credit	96.8	199.0
Total Liabilities	812.6	659.8
Equity	1,762.7	1,791.3
Total Liabilities and Equity	$ 2,575.3	$ 2,451.1

OPKO Health, Inc. and Subsidiaries
Condensed Consolidated Statements of Operations
(in millions, except share and per share data)

	For the three months ended March 31,	
	2019	2018
Revenues		
Revenue from services	$ 178.9	$ 211.3
Revenue from products	25.3	27.9
Revenue from transfer of intellectual property	18.3	15.7
Total revenues	222.5	254.9
Costs and expenses		
Cost of revenues	144.0	154.1
Selling, general and administrative	95.2	91.5
Research and development	36.5	32.9
Contingent consideration	4.8	1.7
Amortization of intangible assets	16.6	17.3
Asset impairment charges	0.7	0.0
Total Costs and expenses	297.8	297.5
Operating loss	(75.3)	(42.6)
Other income and (expense), net	(2.8)	1.0
Loss before income taxes and investment losses	(78.1)	(41.6)
Income tax benefit (provision)	(0.8)	1.0
Loss before investment losses	(78.9)	(40.6)
Loss from investments in investees	(1.9)	(2.5)
Net loss	$ (80.8)	$ (43.1)
Loss per share, basic and diluted	$ (0.14)	$ (0.08)

# # #