OPKO Health Reports 2017 Third Quarter Highlights and Financial Results

- ***RAYALDEE*** **total prescriptions increased 66% in Q3 2017 compared with Q2 2017**
- ***4Kscore*** **utilization increased 19% in Q3 2017 compared with Q3 2016**
- ***4Kscore*** **TV ads in the Northeast to begin November 21**
- **Submitted a Premarket Approval (PMA) application with FDA for Claros point-of-care (POC) prostate specific antigen (PSA) test**
- **Exclusive agreement with Japan Tobacco to develop and commercialize *RAYALDEE* in Japan**
- **Enrollment for global pediatric Phase 3 hGH-CTP clinical trial continues; Japanese pediatric registration trial underway**
- **Intravenous VARUBI® approved by US FDA with launch expected November 2017; addresses 90% of the market for chemotherapy induced nausea and vomiting (CINV); OPKO to receive double digit royalties**
- **Plans to initiate four Phase 2 programs in late 2017 or early 2018**
- **Consolidated revenue for the three months ending September 30, 2017 was $263.5 million compared to $298.0 million for the comparable 2016 period**
- **Net loss for the three months ending September 30, 2017, was $46.4 million compared to net loss of $15.0 million for the comparable 2016 period as OPKO invested heavily this quarter in future growth**

MIAMI (November 8, 2017) – OPKO Health, Inc. (NASDAQ:OPK), reports operating and financial results for the three months ended September 30, 2017.

Business Highlights

- ***RAYALDEE* commercial activities continue to progress:** Total prescriptions for *RAYALDEE*, as reported by IMS, increased 66% during the three months ended September 30, 2017 compared to the three months ended June 30, 2017. OPKO expanded its field based sales force from 35 to 71 as of October 1st. The commercial and medical science liaison teams now total more than 80 professionals. In early November 2017, OPKO participated in the American Society of Nephrology meeting, the largest nephrology meeting of the year, and presented four posters highlighting the impact of RAYADEE in Stage 3 and Stage 4 chronic kidney disease patients.
- **Exclusive Agreement with Japan Tobacco (JT) to Develop and Commercialize *RAYALDEE* in Japan:** Under the terms of the agreement, JT made an upfront payment to OPKO of $6 million with another $6 million payment to be made upon initiation of OPKO's planned phase 2 study of *RAYALDEE* in U.S. dialysis patients. In addition, OPKO will be eligible to receive up to an additional $31 million in development and regulatory milestones and $75 million in sales based milestones. JT will also pay OPKO tiered, double-digit royalties on net product sales. JT will be responsible for all regulatory approvals and commercial activities pertaining to *RAYALDEE* in Japan. According to JT, an estimated 13.3 million people in Japan have CKD and more than 300,000 are undergoing dialysis, with both patient populations increasing due to the aging population.
- ***4Kscore®* utilization increased 19% in Q3 2017 compared with Q3 2016:** OPKO has undertaken a number of initiatives to drive utilization of the *4Kscore* test, the Company's blood test that gives a man with elevated PSA levels a personalized prediction of his chance of having or developing an aggressive form of prostate cancer. In addition to developing a small urology-focused sales force to complement BioReference Laboratories' efforts, OPKO will launch regional television ads in the Northeast for the *4Kscore* test beginning on November 21, 2017.
- **PMA filing for Claros PSA test submitted November 6, 2017; Claros POC testosterone test trials and 510(k) filing expected to follow in 2018:** OPKO completed its analytic and clinical validation studies and submitted a PMA for a PSA test utilizing the Claros 1 immunoassay analyzer, a novel diagnostic instrument that can provide rapid, quantitative blood test results in 10 minutes in the physician's office with only a finger stick drop of whole blood. OPKO expects to begin an additional multi-center study of its POC testosterone test in 2018 followed by a 510(k) submission to the FDA.
- **Global pediatric Phase 3 hGH-CTP in 220 growth hormone deficient children is underway and we continue enrolling patients:** This is a pivotal, non-inferiority study comparing a single weekly dose of hGH-CTP with daily injections of a currently marketed growth hormone. A registration trial in pediatric patients is also underway in Japan. These studies are using the to-be-marketed pen device and formulation that will be launched commercially upon approval. The pediatric segment represents approximately 80% of the commercial market for treatment of hGH deficiency.
- **Phase 2a trial for intravenously administered Factor VII-CTP and Phase 1 trial for subcutaneously administered Factor VII-CTP ongoing:** These long acting forms of Factor VII utilizing OPKO's CTP technology are expected to better support prophylaxis to prevent bleeding episodes, provide easier administration and decrease dosing frequency for hemophilia patients.

- **Initiation of four Phase 2 clinical trials anticipated in late 2017 and early 2018**
    - ***RAYALDEE* line extension in dialysis patients with secondary hyperparathyroidism (SHPT):** Together with its partner, Vifor Fresenius, OPKO is developing *RAYALDEE* for Stage 5 chronic kidney disease (CKD) patients with SHPT undergoing dialysis and anticipates initiating a Phase 2 trial shortly in dialysis centers around the country and abroad.
    - **OPK88004, orally administered selective androgen receptor modulator (SARM):** OPKO plans to initiate a Phase 2b dose ranging study in Q4 2017 to evaluate its use to treat men with benign prostatic hypertrophy (BPH). It is expected to improve symptoms of BPH by reducing prostate size and, on the basis of data from a previous trial in 350 men, increase muscle mass and bone strength and decrease fat mass. BPH affects approximately 50 million men in the U.S.
    - **OPK88003, once weekly oxyntomodulin dual GLP1-Glucagon agonist for type 2 diabetes and obesity:** In a 420 patient phase 2 diabetes trial, OPK88003 treatment reduced HbA1c levels to a similar extent as Exenetide Extended Release (Ex ER). The drug also showed statistically significantly greater weight loss, and lowering of cholesterol and triglycerides compared to once weekly Ex ER. The drug has a good safety profile and is expected to enter a phase 2b dose escalation study in early 2018.
    - **OPK88002, NK-1 antagonist to treat pruritus (itching) in Stage 5 CKD patients undergoing dialysis:** Approximately 50% of renal dialysis patients experience difficult to control pruritus. An IND was approved and plans are now being finalized to begin a single dose Phase 2a trial of OPK88002.

Financial Highlights

- Consolidated revenues for the three months ended September 30, 2017 were $263.5 million compared to $298.0 million for the comparable period of 2016.
- During the three months ended September 30, 2017, operating expenses included significant investment in the commercial activities supporting the launch of *RAYALDEE* of $8.3 million, as well as continued investment in the Company's pharmaceutical pipeline with R&D expense increasing to $32.3 million.
- Cash, cash equivalents and marketable securities were $100.3 million as of September 30, 2017.

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.

The conference call dial in information is listed below. To access the webcast, please log on to the OPKO website at www.opko.com.

WHEN: Wednesday, November 8, 2017 at 4:30 p.m. Eastern time.
DOMESTIC DIAL-IN: (866) 634-2258
INTERNATIONAL DIAL-IN: (330) 863-3454
PASSCODE: 8399217
WEBCAST: http://investor.opko.com/events.cfm

The replay can also be accessed for a period of time on OPKO's website at www.opko.com.

About OPKO Health, Inc.

OPKO Health is a diversified healthcare company that seeks to establish industry-leading positions in large, rapidly growing markets. Our diagnostics business includes BioReference Laboratories, the nation's third-largest clinical laboratory with a core genetic testing business and a 400-person sales and marketing team to drive growth and leverage new products, including the *4Kscore* prostate cancer test and the Claros® 1 in-office immunoassay platform. Our pharmaceutical business features *RAYALDEE*, an FDA-approved treatment for SHPT in stage 3-4 CKD patients with vitamin D insufficiency (launched in November 2016), VARUBI® for chemotherapy-induced nausea and vomiting (oral formulation launched by partner TESARO and IV formulation recently approved by the FDA), OPK88003, a once-weekly oxyntomodulin for type 2 diabetes and obesity that is a clinically advanced drug candidate among the new class of GLP-1 glucagon receptor dual agonists, and OPK88004, a selective androgen receptor modulator being developed for benign prostatic hypertrophy and other urologic and metabolic conditions. Our biologics business includes hGH-CTP, a once-weekly human growth hormone injection (in Phase 3 and partnered with Pfizer), and a long-acting Factor VIIa drug for hemophilia in Phase 2a. We also have various production and distribution assets abroad, multiple strategic investments and an active business development strategy. 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, whether 4Kscore test utilization and prescriptions for RAYALDEE will continue to increase, our product development efforts and the expected benefits of our products, including whether our ongoing and future clinical trials will be successfully 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, the expected timing for launch of our products in development, including Varubi®, the expected timing of commencing and concluding our clinical trials, including studies for the testosterone POC test and whether we will commence four Phase 2 clinical programs in 2017 or early 2018, enrollment in clinical trials, and disclosure of results for the trials, the timing of our regulatory submissions, our ability to market and sell any of our products in development, expectations about developing RAYALDEE for dialysis patients, and whether we will receive milestone and royalty payments from JT, 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 integration

challenges for Bio-Reference, EirGen, Transition, and other acquired businesses, 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, Varubi®, hGH-CTP, OPKO88003, 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.

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—Tables to Follow—

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

	As of	
	September 30, 2017	December 31, 2016
Assets:		
Cash, cash equivalents and marketable securities	$ 100.3	$ 168.7
Other current assets	330.2	314.9
Total Current Assets	430.5	483.6
In-process Research and Development and Goodwill	1,364.0	1,349.3
Other assets	927.5	933.7
Total Assets	$ 2,722.0	$ 2,766.6
Liabilities and Equity:		
Current liabilities	$ 259.0	$ 263.3
2033 Senior Notes, net of discount	28.6	43.7
Deferred tax liabilities	118.8	165.3
Other long-term liabilities, principally deferred revenue and contingent consideration	226.6	202.5
Total Liabilities	633.0	674.8
Equity	2,089.0	2,091.8
Total Liabilities and Equity	$ 2,722.0	$ 2,766.6

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

	For the three months ended September 30,		For the nine months ended September 30,	
	2017	2016	2017	2016
Revenues				
Revenue from services	$ 229.0	$ 259.0	$ 741.0	$ 777.6
Revenue from products	22.8	20.6	74.0	63.3
Revenue from transfer of intellectual property	11.7	18.4	58.8	105.3
Total revenues	263.5	298.0	873.8	946.2
Costs and expenses				
Cost of revenues	151.3	151.2	463.5	452.2
Selling, general and administrative	131.4	124.9	396.3	370.3
Research and development	32.3	24.4	91.0	83.6
Contingent consideration	(11.2)	3.1	(4.5)	15.6
Amortization of intangible assets	18.0	18.1	53.9	47.3
Total Costs and expenses	321.8	321.7	1,000.2	969.0
Operating loss	(58.3)	(23.7)	(126.4)	(22.8)
Other income and (expense), net	(8.5)	(10.5)	0.9	(8.1)
Loss before income taxes and investment losses	(66.8)	(34.2)	(125.5)	(30.9)
Income tax benefit	24.4	20.0	42.3	24.6
Loss before investment losses	(42.4)	(14.2)	(83.2)	(6.3)
Loss from investments in investees	(4.0))	(0.8)	(11.8)	(5.1)
Net loss	$ (46.4)	$ (15.0)	$ (95.0)	$ (11.4)
Loss per share, basic and diluted:				
Loss per share	$ (0.08)	$ (0.03)	$ (0.17)	$ (0.02)
Weighted average common shares outstanding, basic and diluted	559,405,309	552,229,266	559,065,232	548,550,641

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