



OPKO Health Reports 2017 Fourth Quarter Business Highlights and Financial Results

Conference Call begins today at 4:30 p.m. Eastern Time

MIAMI (March 1, 2018) – OPKO Health, Inc. (NASDAQ: OPK) reports business highlights and financial results for the three months ended December 31, 2017.

Business Highlights

- **RAYALDEE total prescriptions reported by IMS increased 47% in Q4 2017 compared with Q3 2017:** Total prescriptions were more than 3,900 during the three months ended December 31, 2017. As of January 1, 2018, more than 79% of patients have access to RAYALDEE under their insurance plans.
- **4Kscore® utilization increased 15% in Q4 2017 compared with Q4 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 prostate specific androgen (PSA) levels a personalized prediction of his chance of having or developing an aggressive form of prostate cancer. OPKO launched regional television ads in the Northeast for the 4Kscore test on November 21, 2017 and expanded the television ads to Florida in February 2018.
- **Premarket Approval (PMA) application for Claros® point-of-care (POC) PSA test submitted to FDA:** On November 6, 2017, OPKO 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. A second product on the same platform for testosterone is advancing toward a 510(k) submission to the FDA later this year.
- **Global and Japan Phase 3 and registration studies for hGH-CTP in pediatric growth hormone deficient children are making good progress in enrolling patients:** The global pediatric study is a pivotal, non-inferiority design comparing a single weekly administration of hGH-CTP with daily injections of a currently marketed growth hormone product. This study is expected to complete enrollment this year. The global and Japanese pediatric studies utilize the pen device and formulation that will be launched commercially upon approval. The pediatric segment represents more than 80% of the commercial market for treatment of hGH deficiency.

- **Initiated a Phase 2b clinical trial for OPK88004, an orally administered selective androgen receptor modulator (SARM):** In November 2017, OPKO initiated a Phase 2b dose-ranging study for the treatment of men with benign prostatic hypertrophy (BPH), or enlarged prostate. OPK88004 is expected to improve the 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.
- **Initiation of four Phase 2 clinical trials anticipated in 2018:**
 - **RAYALDEE line extension in dialysis patients with secondary hyperparathyroidism (SHPT):** Together with its partners Vifor Fresenius and Japan Tobacco, OPKO is developing RAYALDEE for Stage 5 chronic kidney disease (CKD) patients with SHPT undergoing dialysis and anticipates initiating a global Phase 2 trial in dialysis centers in the second quarter of this year.
 - **OPK88003, a once-weekly oxyntomodulin dual GLP1-Glucagon agonist for type 2 diabetes and obesity:** OPKO expects to initiate a Phase 2b dose-escalation study with OPK88003 in the second quarter of this year. In a 420-patient Phase 2 trial in patients with type 2 diabetes, OPK88003 reduced HbA1c levels similar to exenatide extended-release (Ex ER). The drug also showed statistically significantly greater weight loss and lowering of total cholesterol and triglycerides compared to once-weekly Ex ER, with a good safety profile.
 - **OPK88002, an NK-1 antagonist to treat pruritus (itching) in Stage 5 CKD patients undergoing dialysis:** An Investigational New Drug application was submitted to the FDA and plans are being finalized to begin a single-dose Phase 2a trial of OPK88002 in dialysis patients to treat severe itching. Approximately 50% of renal dialysis patients experience difficult to control pruritus.
 - **OPK88001 for the treatment of Dravet Syndrome:** Three clinical research centers in the United States are expected to participate in this first in human clinical study of the AntagoNAT for treatment of Dravet Syndrome.

Financial Highlights

- Net loss of \$213.9 during the three months ended December 31, 2017 included \$147.7 million of non-recurring or non-cash items consisting of:
 - \$73.3 million of revenue adjustments
 - \$13.2 million of intangible impairment related to VARUBI™
 - \$61.2 million Income tax provision
- Consolidated revenues for the three months ended December 31, 2017 were \$193.7 million compared to \$275.5 million for the comparable period of 2016. During the three months ended December 31, 2017, revenue from services were negatively impacted by non-recurring reimbursement adjustments from commercial and federal payor programs of \$73.3 million and by reduced sample volume of \$9.3 million. Revenue from products included \$9.1 million of revenue from RAYALDEE, including \$6.2 million related to revenue previously deferred through September 30, 2017.
- During the three months ended December 31, 2017, operating expenses included investment in the commercial activities supporting the launch of RAYALDEE of \$7.9 million, as well as continued investment in the Company's pharmaceutical pipeline, with R&D expense increasing

to \$34.2 million. In addition, fourth quarter 2017 operating expenses included a \$13.2 million impairment related to our VARUBI intangible assets as a result of our licensee's discontinuation of the IV formulation.

- During the three months ended December 31, 2017, a \$61.2 million income tax provision was recorded, principally as a result of the Tax Cuts and Jobs Act (\$31.8 million) as well as recording a valuation allowance against our U.S. based deferred tax assets. The comparable period of 2016 includes an income tax benefit of \$31.5 million.
- Cash, cash equivalents and marketable securities were \$91.5 million as of December 31, 2017.
- OPKO strengthened its balance sheet with a \$55 million private placement of convertible notes issued on February 27, 2018.

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 and webcast information is as follows:

DOMESTIC DIAL-IN:	866-634-2258
INTERNATIONAL DIAL-IN:	330-863-3454
PASSCODE:	1973978
WEBCAST:	http://investor.opko.com/events.cfm

For those unable to participate in the live conference call or webcast, a replay will be available beginning March 1, 2018 approximately two hours after the close of the conference call. To access the replay, dial 855-859-2056 or 404-537-3406. The replay passcode is 1973978. The replay can be accessed for a period of time on OPKO's website at <http://investor.opko.com/events.cfm>.

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 (launched by partner TESARO), 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 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, the expected timing for launch of our products in development, the expected timing of commencing and concluding our clinical trials, including studies for the testosterone POC test, OPK88002, OPK88003, and OPK88004, expected 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, 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 integration challenges for Bio-Reference, EirGen, Transition, 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.

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—Tables to Follow—

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

	As of	
	December 31, 2017	December 31, 2016
Assets:		
Cash, cash equivalents and marketable securities	\$ 91.5	\$ 168.7
Other current assets	252.0	314.9
Total Current Assets	343.5	483.6
In-process Research and Development and Goodwill	1,364.4	1,349.3
Other assets	876.7	933.7
Total Assets	\$ 2,584.6	\$ 2,766.6
 Liabilities and Equity:		
Current liabilities	\$ 301.3	\$ 263.3
2033 Senior Notes, net of discount	29.2	43.7
Deferred tax liabilities	148.7	165.3
Other long-term liabilities, principally deferred revenue, contingent consideration and lines of credit	220.0	202.5
 Total Liabilities	 699.2	 674.8
Equity	1,885.4	2,091.8
Total Liabilities and Equity	\$ 2,584.6	\$ 2,766.6

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

	For the three months ended December 31,		For the twelve months ended December 31,	
	2017	2016	2017	2016
Revenues				
Revenue from services	\$ 148.1	\$ 234.6	\$ 889.1	\$ 1,012.1
Revenue from products	33.8	20.2	107.7	83.5
Revenue from transfer of intellectual property	11.8	20.7	70.7	126.1
Total revenues	193.7	275.5	1,067.5	1,221.7
Costs and expenses				
Cost of revenues	156.6	159.3	620.1	611.5
Selling, general and administrative	124.6	120.5	521.0	490.9
Research and development	34.2	27.6	125.2	111.2
Contingent consideration	1.1	1.4	(3.4)	17.0
Amortization of intangible assets	30.8	17.1	84.7	64.4
Total Costs and expenses	347.3	325.9	1,347.6	1,295.0
Operating loss	(153.6)	(50.4)	(280.1)	(73.3)
Other income and (expense), net	3.6	7.7	4.6	(0.2)
Loss before income taxes and investment losses	(150.0)	(42.7)	(275.5)	(73.5)
Income tax benefit (provision)	(61.2)	31.5	(18.9)	56.1
Loss before investment losses	(211.2)	(11.2)	(294.4)	(17.4)
Loss from investments in investees	(2.7)	(2.5)	(14.5)	(7.7)
Net loss	\$ (213.9)	\$ (13.7)	\$ (308.9)	\$ (25.1)
Basic loss per share	\$ (0.38)	\$ (0.02)	\$ (0.55)	\$ (0.05)
Diluted loss per share	\$ (0.38)	\$ (0.04)	\$ (0.55)	\$ (0.05)

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