



OPKO Health Reports 2017 First Quarter Business and Financial Results

- U.S. commercial launch of *RAYALDEE* underway
- *4Kscore* test utilization continues to grow; Level 1 CPT code and CMS pricing in place
- Pediatric global phase 3 initiated and Japanese registration trial for hGH-CTP commencing shortly
- Adult study trial data analysis near completion; preparation for Biologics License Application (BLA) submission underway
- Clinical trials of Claros point of care (POC) PSA test began in January 2017; PMA filing anticipated upon completion; Claros POC testosterone test clinical trials and 510(k) filing to follow
- Initiation of four Phase 2 trials anticipated in 2H 2017 and early 2018
 - *RAYALDEE* for dialysis patients with secondary hyperparathyroidism (SHPT)
 - OPK88003, an orally administered selective androgen receptor modulator (SARM) for BPH (Benign Prostatic Hypertrophy)
 - OPK88004, a once weekly oxyntomodulin dual GLP1-Glucagon agonist for type 2 diabetes and obesity
 - OPK88002, an NK-1 inhibitor to treat pruritus (itching) in dialysis patients
- OPK8801, Orphan Drug Status received in the U.S. and EU for new Oligonucleotide to treat Dravet Syndrome; IND planned for 2H 2017
- Clinical trials ongoing for long acting Factor VII-CTP

- Consolidated revenue for the quarter ended March 31, 2017 increased to \$296.1 million from \$291.0 million for the comparable 2016 period
- For the three months ended March 31, 2017, net loss was \$31.0 million compared to net loss of \$12.0 million for the comparable 2016 period
- Financial results reflect significant investments in our pipeline development and marketing and sales expenses related to the introduction of *RAYALDEE*

MIAMI (May 9, 2017) – OPKO Health, Inc. (NASDAQ:OPK), reports operating and financial results for the three months ended March 31, 2017.

Business Highlights

- **U.S. commercial launch of *RAYALDEE* underway:** *RAYALDEE* was launched by our experienced commercial marketing and sales teams and substantial progress has been made in obtaining formulary access for *RAYALDEE*, with more than 60% of potential patient lives now covered under insurance plans. Obtaining broad insurance coverage is a critical step in the adoption of the use of *RAYALDEE*.
- **Forthcoming revisions to the Kidney Disease Improving Global Outcomes (or KDIGO) Clinical Practice Guidelines for Chronic Kidney Disease – Mineral and Bone Disorder CKD-MBD) are expected to recommend against the routine use of vitamin D receptor activators (VDRA) for the treatment of SHPT in this population.** This guideline is also expected to highlight the unproven effectiveness of nutritional vitamin D as a treatment for SHPT and is anticipated to greatly enhance the adoption of *RAYALDEE* therapy by physicians and patients.
- **Dr. Akhtar Ashfaq, a board certified nephrologist with significant industry experience, has joined OPKO as Senior Vice President, Clinical Research & Development and Medical Affairs.** Dr. Ashfaq will support commercial and scientific development strategies for *RAYALDEE*.
- **4Kscore test utilization continues to grow; Level 1 CPT code and CMS pricing in place, negotiations with payors continue.** Level 1 CPT code and CMS pricing became effective on January 1, 2017, and the Company is actively working to secure coverage with additional payors. During the quarter ended March 31, 2017 approximately 18,600 4Kscore tests were ordered which represents growth of more than 100% from the first three months of 2016.
- **Phase 3 pediatric trial for hGH-CTP initiated; Analyses of data from long acting hGH-CTP trial in adults continue and BLA preparation for FDA submission underway:** OPKO will complete a statistical outlier analysis of data from its Phase 3 trial study in adults and, together with Pfizer, is preparing a BLA for FDA submission.
- **Phase 2a trial for intravenously administered Factor VII-CTP and Phase 1 trial for subcutaneously administered Factor VII-CTP are ongoing.** These long acting forms of Factor VII utilizing OPKO's CTP platform technology are expected to better support prophylaxis, provide for easier administration and decrease the frequency of dosing.
- **Analytical and clinical validation trials for Claros point of care (POC) PSA test began in January 2017; PMA filing anticipated upon completion; Claros POC testosterone test trials and 510(k) filing to follow.** OPKO has begun analytic and clinical validation studies of its POC diagnostic test

for prostate specific antigen (PSA) utilizing its proprietary diagnostic platform and intends to submit its PMA application to the FDA for approval in 2017. OPKO expects to begin an additional multi-center study of its POC testosterone test in 2017 followed by a 510(k) submission.

- **Initiation of four Phase 2 clinical trials anticipated in 2H 2017 and early 2018**
 - **RAYALDEE line extension in dialysis patients with SHPT:** Together with its partner, Vifor Fresenius, OPKO is developing RAYALDEE for Stage 5 CKD patients with SHPT undergoing dialysis and anticipates initiating a Phase 2 trial during the second half of 2017.
 - **OPK88003, an orally administered selective androgen receptor modulator (SARM):** The Company plans to initiate a Phase 2b dose ranging study in the second half of 2017 to evaluate the use of OPK88003 to treat men with benign prostate hypertrophy (BPH) (enlarged prostate). It is expected to ameliorate symptoms of BPH by reducing prostate size and, on the basis of data from a previous trial in 350 men, provide other benefits such as increase in muscle mass and bone strength and decreased fat mass.
 - **OPK88004, a once weekly oxyntomodulin dual GLP1-Glucagon agonist for type 2 diabetes and obesity:** OPKO plans to initiate a Phase 2b study in early 2018 to determine optimal dosing. The drug has been shown to be safe and effective in a previous 400 patient Phase 2a trial.
 - **OPK88002, an NK-1 inhibitor 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 recently filed for a Phase 2a trial of OPK88002, obtained as part of the transaction with Schering Plough in which OPKO also obtained Rolapitant, licensed to TESARO, and now on the market.
- **Orphan Drug Status received in the U.S. and EU for its new Oligonucleotide to treat Dravet Syndrome IND planned for 2H 2017;** OPKO's oligonucleotide based AntagoNAT (OPK88001) for the treatment of Dravet Syndrome has received orphan drug designation in the US and EU and OPKO plans to initiate clinical trials of OPK88001 for treatment of Dravet Syndrome later this year. Currently, there is no globally approved treatment for Dravet Syndrome. AntagoNAT, anti-Natural Antisense Transcripts, is an OPKO platform technology in which single strand oligonucleotide molecules are designed to interfere with regulatory gene expression in order to enhance production of endogenous functional proteins.
- **The combination of our expanding and maturing pipeline of products in clinical development and the expected increase in sales of existing products currently in the market positions the company well for significant growth**

Financial Highlights

- Consolidated revenues for the three months ended March 31, 2017 of \$296.1 million improved incrementally over the comparable period of 2016 of \$291.0 million.
- During the first three months of 2017, operating expenses included significant investment in the commercial activities supporting the launch of RAYALDEE, as well as continued investment in advancement of the Company's pharmaceutical pipeline.
- The three month period ended March 31, 2016 benefited from a non-recurring income tax rate change in Israel, which was the principal result of the \$20.5 million income tax benefit.
- Cash, cash equivalents and marketable securities were \$131.1 million as of March 31, 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: Tuesday, May 9, 2017, 4:30 p.m. Eastern time.

DOMESTIC DIAL-IN: (866) 634-2258

INTERNATIONAL DIAL-IN: (330) 863-3454

PASSCODE: 17226044

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 May 9, 2017 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: 17226044. The replay can be accessed for a period of time on OPKO's website at <http://investor.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 Bio-Reference 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 pending FDA approval), OPK88004, a once or twice weekly oxyntomodulin for type 2 diabetes and obesity which is a clinically advanced drug candidate among the new class of GLP-1 glucagon receptor dual agonists, and OPK88003, an androgen receptor modulator for androgen deficiency indications. 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 production and distribution assets worldwide, multiple strategic investments and an active business development strategy. More information 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 sales of our products, whether 4Kscore test utilization will continue to grow, 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 approval, validation and/or reimbursement for our products, the expected timing for launch of our products in development, whether the data for the hGH-CTP study in adults will support approval of a BLA, the expected timing of commencing and concluding our clinical trials, 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, our ability to obtain broad reimbursement coverage for the 4Kscore test, increased adoption rates for the 4Kscore, the recommendations expected for the KDIGO guidelines, 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, government investigations, 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.

Company

OPKO Health, Inc.

Tara Mackay, 305-575-4100

Investor Relations

or

Media

Rooney & Partners

Terry Rooney, 212-223-0689

trooney@rooneyco.com

or

Marion Janic, 212-223-4017

mjanic@rooneyco.com

or

Investors

LHA

Anne Marie Fields, 212-838-3777

afields@lhai.com

or

Bruce Voss, 310-691-7100

bvoss@lhai.com

- Tables to Follow-

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

	March 31, 2017	As of December 31, 2016
Assets:		
Cash, cash equivalents and marketable securities	\$ 131.1	\$ 168.7
Other current assets	328.8	314.9
Total Current Assets	459.9	483.6
In-process Research and Development and Goodwill	1,351.0	1,349.3
Other assets	949.2	933.7
Total Assets	<u>\$ 2,760.1</u>	<u>\$ 2,766.6</u>
Liabilities and Equity:		
Current liabilities	\$ 264.9	\$ 263.3
2033 Senior Notes, net	39.3	43.7
Deferred tax liabilities	159.3	165.3
Other long-term liabilities, principally deferred revenue and contingent consideration	189.6	202.5
Total Liabilities	653.1	674.8
Equity	2,107.0	2,091.8
Total Liabilities and Equity	<u>\$ 2,760.1</u>	<u>\$ 2,766.6</u>

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

For the three months ended March 31,

	2017	2016
Revenues		
Revenue from services	\$ 255.3	\$ 252.5
Revenue from products	22.2	19.9
Revenue from transfer of intellectual property	18.6	18.6
Total revenues	<u>296.1</u>	<u>291.0</u>
Costs and expenses		
Cost of revenues	154.8	147.5
Selling, general and administrative	136.7	128.0
Research and development	26.0	27.8
Contingent consideration	2.4	1.8
Amortization of intangible assets	17.9	13.4
Total Costs and expenses	<u>337.8</u>	<u>318.5</u>
Operating (loss) income	<u>(41.7)</u>	<u>(27.5)</u>
Other income and (expense), net	5.9	(2.6)
(Loss) income before income taxes and investment losses	<u>(35.8)</u>	<u>(30.1)</u>
(Provision for) benefit from income taxes	6.9	20.5
(Loss) income before investment losses	<u>(28.9)</u>	<u>(9.6)</u>
Loss from investments in investees	(2.1)	(2.4)
Net (loss) income	<u>(31.0)</u>	<u>(12.0)</u>
Basic and diluted (loss) per share	<u>\$ (0.06)</u>	<u>\$ (0.02)</u>

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