May 9, 2016

OPKO Health Reports First Quarter Financial and Operating Results

- **Consolidated Revenue Increased to $291.0 Million From $30.1 Million for the First Quarter of 2016 as compared to the 2015 period**
- **Vifor Fresenius Medical Care Renal Pharma (VFMCRP) and OPKO Health Entered into Agreement for OPKO's *RAYALDEE*®; OPKO to Receive up to $282 Million in Upfront and Milestone Payments, Plus Tiered Double-Digit Royalties; Vifor Fresenius also Acquired an Option for Rights to the U.S. Dialysis Market for a New Dosage Form of RAYALDEE Which Provides for Payment to OPKO of up to $555 Million in Additional Milestone Payments Plus Double-Digit Royalties if Exercised**
- ***RAYALDEE* PDUFA Date Set for October 22, 2016**
- **New Leadership at Bio Reference Laboratories and Senior Vice President of Pharmaceutical Sales for RAYALDEE launch appointed**
- **4Kscore® Recommended in 2016 European Association of Urology Prostate Cancer Guidelines**
- **First Patient Dosed in Phase 2a Clinical Trial of Long Acting Factor VIIa**
- **First Patient Dosed in Phase 1 Clinical Trial of Long-Acting Subcutaneous Oxyntomodulin for Obesity and Diabetes**

MIAMI--(BUSINESS WIRE)-- **OPKO Health, Inc. (NYSE:OPK)**, a multinational biopharmaceutical and diagnostics company, today reported financial and operating results for the three months ended March 31, 2016.

Business Highlights

- **Vifor Fresenius Medical Care Renal Pharma and OPKO Health Enter into Agreement for OPKO's *RAYALDEE*:** VFMCRP, a common company of Galenica and Fresenius Medical Care, and OPKO Health, have entered into a collaboration and license agreement for the development and commercialization of *RAYALDEE* in Europe, Canada, Mexico, Australia, South Korea and certain other markets for the treatment of secondary hyperparathyroidism (SHPT) in patients with chronic kidney disease (CKD) and vitamin D insufficiency. Under the terms of the agreement, VFMCRP will make an upfront payment to OPKO of $50 million, plus up to an additional $52 million in regulatory and launch milestones, and $180 million in sales-based milestones. In addition, VFMCRP will pay OPKO tiered, double-digit royalties on sales of the product. The parties will also collaborate to develop and commercialize a new dosage form of *RAYALDEE* for the treatment of SHPT in dialysis patients, and OPKO has granted VFMCRP an option to acquire rights to the US market for dialysis patients. If VFMCRP exercises its option for rights to the US dialysis market for the new dosage form, VFMCRP will pay OPKO up to $550 million in additional milestones, as well as double-digit royalties.
- ***RAYALDEE* New PDUFA Date Set for October 22, 2016:** OPKO resubmitted the

New Drug Application (NDA) for *RAYALDEE* following receipt of a complete response letter (CRL) from the U.S. Food and Drug Administration on March 29, 2016, in which the FDA indicated the NDA could not be approved due to deficiencies observed during a facility inspection of OPKO's third party manufacturer. The observations were not specific to *RAYALDEE* manufacturing, and the CRL did not cite any safety, efficacy or labeling issues with regard to *RAYALDEE*, nor did it request any additional studies to be conducted prior to FDA approval.

- **Key Executive Additions - New Leadership at Bio Reference Laboratories and Senior Vice President of Pharmaceutical Sales:** Gregory Henderson, M.D., Ph.D. was appointed President, Bio Reference Laboratories; James Demarco was appointed Senior Vice President of Pharmaceutical Sales; Ronald Trust, Ph.D. was named Vice President of Regulatory Affairs – OPKO expects to make key additional commercial hires in 2Q 2016.
- ***4Kscore* Recommended in 2016 European Association of Urology Prostate Cancer Guidelines:** The European Association of Urology (EAU) Prostate Cancer Guidelines Panel included the *4Kscore* in the 2016 EAU Guidelines for Prostate Cancer. The panel concluded that the *4Kscore*, as a blood test with greater specificity over the PSA test, is indicated for use prior to a first prostate biopsy or after a negative biopsy to assist patients and physicians in further defining the probability of high grade cancer.
- **Topline Phase 3 Results for hGH-CTP in Adults Expected 2H 2016; Pediatric Phase 3 Initiation Anticipated in 2H 2016:** OPKO expects to report top line results from its Phase 3 trial evaluating the safety and efficacy of once weekly injections of hGH-CTP with a primary endpoint of superiority compared with placebo in decreasing fat mass in adults with growth hormone deficiency (GHD) in the second half of 2016. The trial is a randomized, double-blind, placebo controlled, multicenter, global study in adults with GHD. The study is divided into two treatment periods: a 26 week, double blind, placebo controlled period, followed by a 26 week, open label extension period. A Phase 3 trial in pediatric patients is anticipated to commence in the second half of 2016.
- **First Patient Dosed in Phase 2a Clinical Trial of Long Acting Factor VIIa for the Treatment of Hemophilia:** In February 2016, the first patient was dosed in OPKO's Phase 2a clinical trial for its long acting Factor VIIa. The study is a dose escalation study to determine safety and explore efficacy endpoints of OPKO's long-acting version of coagulation Factor VIIa (Factor VIIa-CTP) for the treatment of bleeding episodes in hemophilia A or B patients with inhibitors to Factor VIII or Factor IX. The study is intended to enroll 24 patients in the United States.
- **First Patient Dosed in Clinical Study for Long Acting Oxyntomodulin for Obesity and Diabetes:** In March 2016 the first patient was enrolled in OPKO's Phase 1 single dose escalation study evaluating the safety and pharmacokinetics of a long acting oxyntomodulin (MOD-6031) in healthy, overweight or obese subjects. The study is intended to enroll 40 subjects. Oxyntomodulin is a peptide hormone that acts as a dual GLP-1/glucagon receptor agonist, with the potential to promote weight loss while improving glycemic control. Oxyntomodulin has been shown to increase energy expenditure, while reducing food intake and body weight, although its clinical utility is limited by its short circulating half life. OPKO's MOD-6031 has been designed, using a proprietary bifunctional hydrolysable linker, as a long acting version of oxyntomodulin for the treatment of Type 2 Diabetes and obesity, and is intended to reduce the required dosing frequency by prolonging the half life, while improving the hormone's

pharmacokinetic and pharmacodynamic profiles.

“We started 2016 with strong results from our diagnostics business driven by an increase in patient volume at Bio-Reference Laboratories, including our GeneDx business, and continued growth in the utilization of our innovative *4Kscore* Test. On the pharmaceutical side, we are looking forward to the launch of *RAYALDEE* and our collaboration with a leader in the chronic kidney disease field that will allow us to expand the reach of this product to patients outside the US and expand development of the product for patients undergoing dialysis. We are steadily building our commercial team as we work with the FDA to finalize regulatory approval for *RAYALDEE*. We also made progress advancing our earlier stage development programs including the next program utilizing our CTP technology, with the first patients being administered our long acting Factor VIIa-CTP, and the initiation of a Phase 1 clinical trial for long acting oxyntomodulin,” stated Phillip Frost, M.D., Chairman and Chief Executive Officer of OPKO.

Financial Highlights

- Consolidated revenues for the three months ended March 31, 2016 increased to $291.0 million from $30.1 million for the three months ended March 31, 2015. The 2016 period include revenue from Bio-Reference Laboratories and EirGen which were acquired in August and May 2015, respectively.
- Net loss for the three months ended March 31, 2016 was $12.0 million compared with net loss of $117.1 for the 2015 period. Net loss during the three month periods include significant non-recurring and/or non-cash activities, including:
    - $20.5 million of income tax benefit, primarily reflecting a change in the statutory tax rate in Israel during 2016;
    - $17.2 million of severance expense related to the resignation of certain Bio-Reference executives during the first quarter of 2016, which is included in selling, general and administrative expense. Of this expense, $8.9 million is a non-cash expense related to the acceleration of stock options;
    - The first three months of 2015 include $25.9 million of non-recurring operating expense related to the repayment of a grant to the Office of the Chief Scientist in Israel related to the Pfizer transaction; and,
    - Other income and (expense) was ($2.6) million and ($53.9) million in the 2016 and 2015 periods, respectively, primarily related to the change in fair value of derivative instruments. The change in fair value is principally related to an embedded derivative in OPKO’s January 2013 convertible senior notes due in 2033.
- Cash, cash equivalents and marketable securities were $175.0 million as of March 31, 2016.

CONFERENCE CALL & WEBCAST INFORMATION:

OPKO’s senior management will provide a business update and discuss its 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: Monday, May 9, 2016, 4:30 p.m. ET

DOMESTIC DIAL-IN: (866) 634-2258
INTERNATIONAL DIAL-IN: (330) 863-3454
PASSCODE: 2590322

For those unable to participate in the conference call or webcast, a replay will be available beginning May 9, 2016 at 7:30 p.m. ET until May 15, 2016 at 11:59 p.m. ET. To access the replay, dial (855) 859-2056 or (404) 537-3406. The replay passcode is: 2590322.

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, Inc. 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 420-person sales force 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*, a treatment for SHPT in stage 3-4 CKD patients with vitamin D insufficiency (PDUFA date is October 22, 2016) and VARUBI™ for chemotherapy-induced nausea and vomiting (oral formulation launched by partner Tesaro, IV formulation in Phase 3). Our biologics business includes hGH-CTP, a once-weekly human growth hormone injection (in Phase 3 and partnered with Pfizer), a long-acting Factor VIIa drug for hemophilia (in Phase 2a) and a long acting oxyntomodulin for diabetes and obesity (in Phase 1). We also have production and distribution assets worldwide, 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 revenue growth, whether we have sufficient liquidity to fund development of our product candidates and operations, our product development efforts and the expected benefits of our products, including whether our ongoing and future clinical trials will be 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, including RAYALDEE and hGH-CTP, the expected timing of our clinical trials, enrollment in clinical trials, and disclosure of results for the trials, our ability to market and sell any of our products in development, including RAYALDEE, the 4Kscore, and hGH-CTP, expectations about potential milestone payments from our partners, our ability to obtain broad reimbursement coverage for the 4Kscore test, increased adoption rates for the 4Kscore by Urologists in the U.S. and abroad, expectations about the RAYALDEE PDUFA date, 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 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, 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.

OPKO Health, Inc. and Subsidiaries

Condensed Consolidated Balance Sheets

(unaudited)

(in millions)

	As of March 31, 2016	December 31, 2015
Assets:		
Cash and cash equivalents	$ 175.0	$ 193.6
Other current assets	278.5	260.5
Total Current Assets	453.5	454.1
In-process Research and Development and Goodwill	1,540.6	1,535.6
Other assets	799.1	809.5
Total Assets	$ 2,793.2	$ 2,799.2
Liabilities and Equity:		
Current liabilities	$ 267.2	$ 251.9
2033 Senior Notes, net	49.6	49.0
Deferred tax liabilities	202.7	226.0

Other long-term liabilities, principally deferred revenue and contingent consideration	275.8	292.5
Total Liabilities	795.3	819.4
Equity	1,997.9	1,979.8
Total Liabilities and Equity	$ 2,793.2	$ 2,799.2

OPKO Health, Inc. and Subsidiaries

Condensed Consolidated Statements of Operations

(unaudited)

(in millions, except per share data)

	For the three months ended March 31,	
	2016	2015
Revenues	$ 291.0	$ 30.1
Costs and expenses		
Cost of revenues	147.5	10.3
Selling, general and administrative	128.0	17.4
Research and development	27.8	25.5
Contingent consideration	1.8	5.2
Amortization of intangible assets	13.4	2.7
Grant repayment	-	25.9
Total Costs and expenses	318.5	87.0
Operating loss	(27.5)	(56.9)
Other income and (expense), net	(2.6)	(53.9)
Income (loss) before income taxes and investment losses	(30.1)	(110.8)
Benefit from (provision for) income taxes	20.5	(5.5)
Income (loss) before investment losses	(9.6)	(116.3)
Loss from investments in investees	(2.4)	(1.7)
Net income (loss)	(12.0)	(118.0)
Less: Net loss attributable to non-controlling interests	-	(0.9)
Net income (loss) attributable to common shareholders	$ (12.0)	$ (117.1)
Basic income (loss) per share	$ (0.02)	$ (0.26)
Diluted income (loss) per share	$ (0.02)	$ (0.26)

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Source: OPKO Health, Inc.