May 9, 2014

OPKO Announces First Quarter Operating Results

- **4Kscore™ Test successfully completed clinical validation study and launched on March 31, 2014**
- **All RAYALDEE™ Phase 3 clinical trials have completed enrollment on schedule – top line data expected for release mid-2014**
- **Cash and cash equivalents totaled $156.4 million providing sufficient liquidity to fund development programs**

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NYSE:OPK), a multi-national biopharmaceutical and diagnostics company, today reported operating and financial results for its first quarter ended March 31, 2014.

Business Highlights

- **4KscoreTest Launch:** During the first quarter 2014, OPKO successfully completed the 4Kscore Test clinical trial in the U.S. and on March 31, 2014, launched the 4Kscore Test in the U.S. through its CLIA-accredited OPKO Lab in Nashville, TN. OPKO expects to begin offering the 4Kscore Test through its Spanish subsidiary in late 2014 and through its other subsidiaries shortly thereafter. The laboratory-developed test is designed to enhance the prostate biopsy decision making process that, in the U.S., leads to approximately 1 million biopsies being performed annually, with 80% of the results indicating no cancer or a low-grade cancer. The 4Kscore Test will help to reduce unnecessary prostate biopsies by providing information on the risk (probability) of having high-grade prostate cancer.
- **4Kscore Data Presentations:** OPKO will present data from its recently completed U.S. clinical validation study at two upcoming conferences: the American Urological Association (AUA) in Orlando, which selected “The 4Kscore Test as a Predictor of High-Grade Prostate Cancer on Biopsy” as a Late-Breaking Abstract for presentation in Plenary I on Sunday, May 18, 2014; and, the 83rd Annual Meeting of the New England Section of the AUA in October 2014 in Newport.
- **Completed Patient Enrollment in the Third Phase 3 Trial of*RAYALDEE*:** This trial is a 6-month open-label extension of two ongoing identical randomized, double-blind, placebo-controlled, multi-site pivotal phase 3 studies for *RAYALDEE* intended to support marketing approval in the U.S. This third study is designed to evaluate the product’s long-term safety and efficacy in treating secondary hyperparathyroidism (SHPT) in subjects with stage 3 or 4 chronic kidney disease (CKD) and vitamin D insufficiency. OPKO is on schedule for releasing top-line pivotal data in the third quarter of 2014 and filing a New Drug Application (NDA) with the FDA in the first quarter of 2015.
- **Inspiro Acquisition:** In April, OPKO entered into a definitive agreement to acquire Inspiro Medical Ltd., an Israeli medical device company with a new platform to deliver small molecule drugs such as corticosteroids and beta agonists, as well as its own new drug working with a novel mechanism of action, to treat respiratory diseases. Inspiro's Inspiromatic™ is a "smart" easy-to-use dry powder inhaler with several advantages over existing devices.
- **Rolapitant Continues on Schedule For a Mid-Year NDA Filing:** OPKO’s partner, TESARO announced it is on track for its NDA filing for rolapitant mid-2014 and anticipates completing a dose study for the intravenous (IV) formulation during the second quarter of 2014. The IV formulation is expected to be launched approximately one year after the oral product becomes available.
- **Key Management Positions Added:** OPKO made a number of important additions to its management team: Greg Stanley joined OPKO as Vice President of Sales and Marketing for the Global Diagnostics Business Unit, and Scott Toner joined OPKO as Vice President, U.S. Marketing and Sales for the Renal Division.
- **Establishment of a Global Supply Chain Infrastructure and Holding Company Based in Dublin, Ireland:** OPKO is expanding its presence by establishing a global supply chain operation and holding company in Ireland. OPKO is recruiting employees to support the ongoing launch of the 4Kscore diagnostic test and in anticipation of the commercial launch of *RAYALDEE*. The Irish operation is also expected to manage the global supply of other products over the next several years and to serve as a holding company for many of OPKO's non-U.S. subsidiaries.
- In January, OPKO completed the acquisition of Laboratorio Arama de Uruguay Limitada (“Arama Uruguay”), a privately-owned company located in Montevideo, Uruguay. Arama Uruguay will expand OPKO’s presence in Latin America and complement the business activities of its operations in Chile and Mexico, as well as permit commercialization of OPKO’s products currently commercialized and those in development.

“We made significant progress during the first quarter of 2014,” stated Dr. Phillip Frost, Chairman and CEO. “The

completion of our validation study for the 4Kscore and subsequent launch were significant milestones which we expect will provide substantial benefits to both patients and healthcare professionals by informing them of the risk of a patient having high-grade prostate cancer and helping clarify the decision process surrounding prostate biopsy. Our *RAYALDEE* phase 3 clinical trials continue on track for release of top line data later this summer and our hGH-CTP clinical trials continue on schedule. In addition to these important project developments, we expanded the global footprint of our commercial platform with the entry into the Uruguayan market. With our planned acquisition of Inspiro, we will also add an innovative next generation platform drug delivery system."

Financial Highlights

At March 31, 2014, OPKO had cash and cash equivalents of $156.4 million providing OPKO with strong liquidity and the ability to continue the development of its product candidates. During the three months ended March 31, 2014, OPKO continued to invest in its research and development programs and as a result, utilized cash in operations of $29.0 million for the three months ended March 31, 2014. Cash used in operations include the final deferred acquisition payments for OPKO Spain and FineTech totaling $7.9 million. The remaining cash used in operations reflects OPKO's continued investment in research and development activities including its ongoing phase 3 clinical trials for *RAYALDEE* and hGH-CTP and the clinical validation study for 4Kscore Test which was completed in March 2014.

Product revenue for the quarter increased approximately 28% to $19.8 million compared to $15.5 million for the 2013 period. The increase in product revenue was principally the result of increased revenue from OPKO Spain and FineTech. Total revenue for the three months ended March 31, 2014 was $22.3 million compared to $31.3 million for the 2013 period. Total revenue for the three months ended March 31, 2013 included non-cash, non recurring revenue of $12.5 million related to OPKO's transaction with RXi, which was partially offset by increased product revenue.

Net loss attributable to common shareholders increased to $45.1 million for the three months ended March 31, 2014 compared to $34.6 million in the comparable period of 2013 partially as a result of the 2013 period including $12.5 million of non-cash income related to the RXi transaction. In addition, OPKO continued to increase its investment in research and development activities related to its ongoing phase 3 programs for *RAYALDEE* and hGH-CTP as well as costs associated with the clinical validation study for the 4Kscore. As a result, OPKO's spending on research and development increased $11.0 million to $21.0 million for the three months ended March 31, 2014 from $9.9 million for the three months ended March 31, 2013. Net loss for the three months ended March 31, 2014 included $10.5 million of non-cash expense related to the mark to market of certain derivative instruments related to our convertible debt instruments compared to $23.5 million for the three months ended March 31, 2013.

About OPKO Health, Inc.

We are a multi-national biopharmaceutical and diagnostics company that seeks to establish industry-leading positions in large and rapidly growing medical markets by leveraging our discovery, development and commercialization expertise and our novel and proprietary technologies.

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, continued revenue growth and our ability to build a profitable business, whether we have sufficient liquidity to fund our research and development and operations, our product development effort and the expected benefits of our products, including whether the phase 3 clinical trials for RAYALDEE, hGH-CTP, and rolapitant will be completed on a timely basis or at all and whether the data 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, our ability to launch sales of the 4Kscore Test in Spain and through our other subsidiaries, the timing for submission of an NDA by us for RAYALDEE and by TESARO for rolapitant, whether TESARO will complete a dose study for its IV formulation and the timing thereof, whether the 4Kscore will provide substantial benefits to patients and doctors by informing them of the risk of a patient having a high-grade cancer and clarify the decision making process, whether the 4Kscore will reduce unnecessary biopsies, whether the Irish operation will manage, oversee and contribute to the manufacturing and sale of the 4Kscore, RAYALDEE, and other products in development, 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 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, rolapitant, 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

(in millions)

	As of	
	March 31, 2014	December 31, 2013
Assets:		
Cash and cash equivalents	$ 156.4	$ 185.8
Other current assets	48.8	56.9
	205.2	242.7
In-process Research and Development and Goodwill	1,091.3	1,019.7
Other assets	59.1	129.1
Total Assets	$ 1,355.6	$ 1,391.5
Liabilities, Series D Preferred Stock and Equity:		
Current liabilities	$ 84.5	$ 91.8
2033 Senior Notes, net	224.8	211.9
Other long-term liabilities	215.1	214.8
Total Liabilities	524.4	518.5
Series D Preferred Stock and Equity	831.2	873.0
Total Liabilities, Series D Preferred Stock and Equity	$ 1,355.6	$ 1,391.5

OPKO Health, Inc. and Subsidiaries

Condensed Consolidated Statements of Operations

(in millions, except per share data)

	For the three months ended March 31,	
	2014	2013
Revenues	$ 22.3	$ 31.3
Costs and expenses	52.6	38.1
Operating loss	(30.3)	(6.8)
Other income and (expense), net	(12.1)	(24.1)
Loss before income taxes and investment losses	(42.4)	(30.8)

Benefit from (provision for) income taxes	(0.6)	(0.1)
Loss before investment losses	(43.0)	(30.9)
Loss from investments in investees	(2.1)	(3.9)
Net loss	(45.1)	(34.8)
Less: Net loss attributable to non-controlling interests	(0.5)	(0.5)
Preferred stock dividend	-	(0.4)
Net loss attributable to common shareholders	$ (44.6)	$ (34.6)
Basic and diluted loss per share	$ (0.11)	$ (0.11)

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Source: OPKO Health, Inc.