February 27, 2015

OPKO Announces Fourth Quarter Operating and Financial Results

- **Pfizer Collaboration Agreement for Long Acting Human Growth Hormone Closed in January 2015; OPKO Received $295 Million of $570 Million Total Potential Up Front and Milestone Payments**
- **Rayaldee™ New Drug Application (NDA) Submission Planned for Q1 2015**
- **Positive Rayaldee Phase 3 Clinical Trial Results Presented at American Society of Nephrologists Meeting**
- **Clinical Trial for Rayaldee as Adjunctive Cancer Therapy Began in Q4 2014**
- **Marketing for 4Kscore® Blood Test to Identify Risk of Aggressive Prostate Cancer in the US and Europe Began During 2014 and in Mexico in January 2015**
- **Two Papers Supporting 4Kscore Blood Test in *European Urology* Published**
- **Rolapitant™ NDA Submitted by OPKO Licensee, TESARO; Accepted for Review by FDA with a PDUFA date of September 5, 2015; OPKO Received $5 Million of $121 Million Total Potential Up Front and Milestone Payments**
- **Investigational New Drug (IND) Application for Long Acting Factor VIIa-CTP for Hemophilia Filed and Accepted in Q1 2015**
- **Clinical Studies for Long Acting Oxyntomodulin for Obesity and Diabetes Expected to Begin During 2015**

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

Business Highlights

- **Pfizer Collaboration Agreement for Long Acting Human Growth Hormone Closed in January 2015; OPKO Received Up-Front Payments totaling $295 million for global commercialization rights to hGH-CTP:** In connection with the collaboration, OPKO received upfront payments of $295 million and will receive an additional $275 million upon achievement of development related milestones. In addition, OPKO will receive initial royalty payments upon the commercialization of hGH-CTP for Adult growth hormone deficiency (GHD). Upon the launch of hGH-CTP for Pediatric GHD, the royalties will transition to gross profit sharing among all indications for both hGH-CTP and Pfizer's Genotropin®. OPKO will lead clinical development and will be responsible for funding the development programs for Adult and Pediatric GHD and growth failure in children born small for gestational age (SGA). Pfizer will be responsible for all development costs for additional indications as well as all post-marketing studies. In addition, Pfizer will fund the commercialization activities for all indications and lead the manufacturing activities covered by the global development plan.
- **End of Phase 2 Meeting for hGH-CTP for Pediatric GHD Scheduled for Q1 2015; Adult Phase 3 Clinical Trial Continues to Advance:** OPKO will present twelve

month data from its ongoing Phase 2 clinical trial for pediatric GHD at the 97th Annual Meeting of the Endocrine Society (ENDO) on March 5th, 2015 in San Diego, California.

- **Rayaldee Met Primary Endpoints in Both Pivotal Phase 3 Trials; NDA Submission planned for Q1 2015:** OPKO announced successful top-line results from both of its pivotal Phase 3 trials with Rayaldee. These trials were identical randomized, double-blind, placebo-controlled, multi-site studies intended to establish the safety and efficacy of Rayaldee as a new treatment for secondary hyperparathyroidism (SHPT) in patients with stage 3 or 4 chronic kidney disease (CKD) and vitamin D insufficiency. OPKO plans to submit a NDA in the first quarter of 2015.
- **Rayaldee Results Presented at American Society of Nephrologists Meeting:** Rayaldee Phase 3 trial data was presented in a late-breaking clinical presentation entitled "Safety and Efficacy of Modified-release Calcifediol for Secondary Hyperparathyroidism in Patients with Stage 3 or 4 CKD and Vitamin D Insufficiency" on November 15, 2014 during the American Society of Nephrology meeting in Philadelphia, PA.
- **Clinical Trial for Rayaldee as Adjunctive Cancer Therapy Initiated in Q4 2014:** OPKO initiated a clinical trial to evaluate Rayaldee as an adjunctive therapy for the prevention of skeletal-related events (SREs) in breast and prostate cancer patients with bone metastases undergoing anti-resorptive therapy during the fourth quarter of 2014.
- **IND for Long Acting Factor VIIa-CTP for Hemophilia Filed and Accepted:** In January 2015, OPKO submitted an IND to Initiate a Phase 2a Trial for its Long-Acting Coagulation Factor VIIa-CTP to Treat Hemophilia. Clinical trials are expected to commence shortly.
- **Clinical Studies for Long Acting Oxyntomodulin for Obesity and Diabetes Expected to Begin During 2015**: OPKO expects to commence studies for its long acting Oxyntomodulin for diabetes and obesity in the second half of 2015.
- **Launched 4Kscore Test in US, Europe and Mexico; Adoption of 4Kscore Test Continues to Grow:** OPKO launched the 4Kscore Test in the US and Europe in 2014, and in Mexico in January 2015. OPKO also expects to launch the 4Kscore Test in additional Latin America markets through its subsidiaries during 2015. OPKO is working to obtain reimbursement for the 4Kscore Test by payers in the U.S. and abroad and expects adoption to rapidly increase once reimbursement is received.
- **Announced Publication of 20 Year Outcome Study for Lethal Prostate Cancer Using Kallikrein Biomarkers in 4Kscore Test:** A team of researchers from Memorial Sloan Kettering Cancer Center and several leading European institutions published results in the journal *European Urology* concluding that the four kallikrein panel of biomarkers utilized in the OPKO 4Kscore Test (Total PSA, Free PSA, Intact PSA and hK2) accurately identify men more likely to develop distant prostate cancer metastases, and men with a low 4Kscore were shown to have a very low risk of developing metastatic prostate cancer in the 5-10 year timeframe a <2% risk in a 20-year follow up period.
- **Rolapitant NDA Filing Submitted in September and Accepted for Review by FDA in November:** OPKO's partner, TESARO, submitted a NDA to the FDA for approval of oral rolapitant, an investigational neurokinin-1 (NK-1) receptor antagonist in development for the prevention of chemotherapy-induced nausea and vomiting (CINV). The NDA is supported by data from four controlled studies covering a spectrum of patients receiving chemotherapy that commonly causes nausea and vomiting. The top-line results of three of the Phase 3 studies were previously announced by TESARO

and were presented in detail at the American Society for Clinical Oncology (ASCO) annual meeting in June 2014. On November 5, 2014, TESARO announced the FDA accepted its NDA filing for rolapitant, with a PDUFA date of September 5, 2015, which triggered a milestone payment of $5 million to OPKO under its license agreement with TESARO.

“We accomplished a number of important objectives during 2014,” said Phillip Frost, M.D., Chairman and CEO. “The completion of the Pfizer transaction rounded out a watershed year for OPKO that saw us report two successful Phase 3 clinical trials for Rayaldee, successful validation of the 4Kscore Test and subsequent launch of the 4Kscore Test in the US and Europe. In addition, we progressed many of our earlier stage programs, particularly our long acting Factor VII and Oxyntomodulin, that have the potential to make significant contributions to the healthcare system and the quality of life of patients,” Dr. Frost continued.

Financial Highlights

We believe that OPKO’s cash and cash equivalents of $96.9 million at December 31, 2014, together with the $295.0 million in upfront payments from Pfizer received in 2015, provide OPKO with adequate liquidity to continue development of its product candidates.

Pharmaceutical product revenue for the three months ended December 31, 2014 increased to $18.5 million compared to $17.5 million for the 2013 period. This increase was principally the result of increased revenue from OPKO’s active pharmaceutical ingredient business at FineTech. Total revenue for the three months ended December 31, 2014 was $25.5 million compared to $20.7 million for the 2013 period. The increase in total revenue was the result of the 2014 period including a $5.0 million payment from TESARO for the acceptance of the NDA for rolapitant.

Net loss for the three months ended December 31, 2014 was $53.0 million, compared to $16.8 million in the comparable period of 2013. During the three months ended December 31, 2014, OPKO recorded increased expense associated with its derivative instruments of $14.4 million, principally related to the derivative liability associated with the increased value of its Senior 2033 Notes. Further, the three months ended December 31, 2013 benefited from the exit from a strategic investment, resulting in an $18.9 million gain in that period. OPKO continued to increase its investment in research and development activities during the three months ended December 31, 2014 related to its ongoing Phase 3 programs for Rayaldee and hGH-CTP. As a result, OPKO’s spending on research and development increased $2.4 million to $25.8 million for the three months ended December 31, 2014 from $23.4 million for the three months ended December 31, 2013.

For the year ended December 31, 2014, pharmaceutical product revenue increased approximately 13% to $77.0 million compared to $68.2 million for the 2013 period. The increase in pharmaceutical product revenue was principally the result of increased revenue from FineTech, OPKO Health Europe and OPKO Mexico. Total revenue for the year ended December 31, 2014 was $91.1 million compared to $96.5 million for the 2013 period. Total revenue for the year ended December 31, 2013 included non-cash, non-recurring revenue of $12.5 million related to OPKO’s transaction with RXi Pharmaceuticals partially offset by increased pharmaceutical product revenue and the milestone payment from TESARO.

Net loss for the year ended December 31, 2014 was $171.7 million compared to $114.8

million for 2013. OPKO's increased investment in research and development activities principally related to its Phase 3 programs for Rayaldee and hGH-CTP, as well as incurred costs associated with the clinical validation study for the 4Kscore, the Claros 1 Analyzer point of care diagnostic platform and earlier stage development programs. As a result, OPKO's investment in research and development increased $29.7 million to $83.6 million for the year ended December 31, 2014 from $53.9 million for the year ended December 31, 2013. As a result of the successful achievement of the primary efficacy and safety endpoints for the Rayaldee Phase 3 clinical trials, the valuation for contingent consideration payable to the sellers of Cytochroma increased significantly during the year ended December 31, 2014 resulting in $17.5 million of increased contingent consideration expense. In addition, net loss for the year ended December 31, 2014 included a non-recurring in-process research and development expense of $12.1 million due to a write-off of in-process research and development expense in connection with the acquisition of Inspiro and a payment to Merck in connection with the NDA filing by TESARO for rolapitant. The year ended December 31, 2013 included $12.5 million of non-cash income related to the RXi transaction and a $29.9 million gain realized from the successful exit of a strategic investment.

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 development of our product candidates and operations, our product development effort and the expected benefits of our products, including whether our ongoing and future Phase 3 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, our ability to launch sales of the 4Kscore Test in Latin America and through our other subsidiaries, increased adoption rates for the 4Kscore by Urologists in the U.S. and abroad, the timing for submission of an NDA by us for Rayaldee, 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, 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

(unaudited)

(in millions)

	As of	
	December 31, 2014	December 31, 2013
Assets:		
Cash and cash equivalents	$ 96.9	$ 185.8
Other current assets	46.0	56.9
Total Current Assets	142.9	242.7
In-process Research and Development and Goodwill	1,017.4	1,019.7
Other assets	107.4	129.1
Total Assets	$ 1,267.7	$ 1,391.5
Liabilities and Equity:		
Current liabilities	$ 83.1	$ 91.8
2033 Senior Notes, net	131.5	211.9
Other long-term liabilities	217.3	214.8
Total Liabilities	431.9	518.5
Equity	835.8	873.0
Total Liabilities and Equity	$ 1,267.7	$ 1,391.5

OPKO Health, Inc. and Subsidiaries

Condensed Consolidated Statements of Operations

(unaudited)

(in millions, except per share data)

	For the three months ended December 31,	
	2014	2013
Revenues	$ 25.5	$ 20.7
Costs and expenses	58.0	56.6
Operating loss	(32.5)	(35.9)
Other income and (expense), net	(20.9)	21.5
Loss before income taxes and investment losses	(53.4)	(14.4)
Benefit from (provision for) income taxes	1.0	0.6
Loss before investment losses	(52.4)	(13.8)
Loss from investments in investees	(1.1)	(3.6)
Net loss	(53.5)	(17.4)
Less: Net loss attributable to non-controlling interests	(0.5)	(0.6)
Preferred stock dividend	-	-
Net loss attributable to common shareholders	$ (53.0)	$ (16.8)
Basic and diluted loss per share	$ (0.12)	$ (0.04)

	For the year ended December 31,	
	2014	2013
Revenues	$ 91.1	$ 96.5
Costs and expenses	236.9	176.1
Operating loss	(145.8)	(79.6)
Other income and (expense), net	(25.2)	(24.6)
Loss before income taxes and investment losses	(171.0)	(104.2)

Benefit from (provision for) income taxes	(0.0)	(1.7)
Loss before investment losses	(171.0)	(105.9)
Loss from investments in investees	(3.6)	(11.4)
Net loss	(174.6)	(117.3)
Less: Net loss attributable to non-controlling interests	(2.9)	(2.9)
Preferred stock dividend	-	(0.4)
Net loss attributable to common shareholders	$ (171.7)	$ (114.8)
Basic and diluted loss per share	$ (0.41)	$ (0.32)

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Source: OPKO Health, Inc.