

March 3, 2014



OPKO Announces Fourth Quarter and Full Year 2013 Results

Fourth Quarter Revenue Increases About 30%; Full Year Revenue More Than Doubled to \$96.5 Million

Company Has Strong Liquidity, Including Cash and Cash Equivalents of \$185.8 million as of December 31, 2013

Launch of 4Kscore™ Planned in Q1 2014

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NYSE:OPK), a multi-national biopharmaceutical and diagnostics company, today reported operating and financial results for its 2013 fourth quarter and full year ended December 31, 2013.

Financial Highlights

- For the fourth quarter of 2013, consolidated revenues increased about 30% to \$20.7 million from \$16.2 million in the prior year period. For the year ended December 31, 2013, consolidated revenues more than doubled to \$96.5 million from \$47.0 million in the prior year. Revenue for the year ended December 31, 2013, included \$12.5 million of revenue resulting from a strategic partnership in the field of RNA interference with RXi Pharmaceuticals Corporation.
- Cash and cash equivalents were \$185.8 million as of December 31, 2013, providing OPKO with liquidity to fund research and development and the Company's operations.
- Cash used in operations was \$58.2 million during the year ended December 31, 2013, as compared with \$25.4 million of cash used in operations during the year ended December 31, 2012. The increase in cash used in operations during 2013 reflects the full impact of our August and March 2013 acquisitions of PROLOR Biotech, Inc. and Cytochroma Inc., respectively. During 2013, we also utilized approximately \$8.8 million for transaction-related expenses.
- Net loss for the 2013 fourth quarter was \$16.8 million compared to a net loss of \$1.1 million for the 2012 period. The increase in net loss for the 2013 fourth quarter was principally related to research and development expenses incurred in connection with our ongoing Phase 3 clinical trials of *Rayaldy*™ and human growth hormone ("hGH-CTP"), and interest expense related to the January 2013 convertible senior notes due in 2033 (the "2033 Senior Notes"), which expenses were partially offset by \$18.9 million in gains recorded on the successful exit from strategic investments. The 2012 fourth quarter results also included \$9.7 million in net tax benefits principally related to the acquisition of our laboratory business in late 2012.
- Net loss for the full 2013 year was \$114.8 million compared to \$31.3 million for the 2012 period. The increase in net loss for the 2013 full year was primarily related to the previously mentioned research and development expenses related to our *Rayaldy*™ and hGH-CTP clinical trials, interest expense on the 2033 Senior Notes, and non-

recurring costs related to strategic and business development activities, as well as the following non-cash charges:

- \$36.5 million related to the change in fair value of derivative instruments, principally related to embedded derivatives that are part of the 2033 Senior Notes;
- \$11.5 million on losses from investments in equity method investees;
- \$8.7 million loss from early conversion of some of the 2033 Senior Notes; and
- \$6.9 million related to the change in fair value of contingent consideration payable in connection with prior acquisitions.

These expenses were partially offset by \$29.9 million in gains recorded on the successful exit from strategic investments during 2013. OPKO's 2012 results also included \$9.6 million in net tax benefits principally related to the acquisition of our laboratory business in late 2012.

Phillip Frost, M.D., OPKO's Chairman and Chief Executive Officer, commented, "From an R&D perspective, all of our programs are progressing. We made significant strides in 2013 with our ongoing Phase 3 trials for *Rayaldy*[™] and hGH-CTP; 2014 will be a pivotal year for our development programs. We look forward to announcing top-line results for *Rayaldy*[™] in mid-2014 and filing a NDA during the first half of 2015. We are also enthusiastic about the planned launch later this month of our *4Kscore*[™] blood test for prostate cancer which we believe will lead to a great improvement in the diagnosis and management of prostate cancer."

"Work has been continuing in our research laboratories on a program to utilize specific oligonucleotides to up regulate protein production. A pre-IND meeting has been scheduled with FDA in connection with the development of the first product to treat Dravet's Syndrome, a congenital condition characterized by chronic seizures."

Dr. Frost added, "During the year, we further strengthened our cash position by exiting certain strategic investments which provided attractive returns. Bolstered by our sound financial position, we look forward to continuing the advance of our robust product development pipeline of promising diagnostics and pharmaceuticals."

Business Highlights

- **Finalizing Steps Toward *4Kscore*[™] Launch:** In February 2014, OPKO announced successful initial results of the clinical validation study of the *4Kscore*[™] test currently underway at 21 large urology centers in the United States. The study, involving more than 1,200 men, is now more than 50% complete and supports the U.S. launch of the *4Kscore*[™] test later this month.
- **Completed Patient Recruitment In The Second Phase 3 Trial of *Rayaldy*[™]:** This trial is the second of two identical randomized, double-blind, placebo-controlled, multi-site studies for *Rayaldy*[™] -- to treat patients with secondary hyperparathyroidism (SHPT), stage 3 or 4 chronic kidney disease (CKD) and vitamin D deficiency. Top-line results from both trials are expected in mid-2014.
- **Positive Pre-Clinical Results on Long-acting Factor VIIIa-CTP Presented at the 7th Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD); Orphan Drug Designation also Granted by FDA.** Preclinical

data presented at EAHAD on February 26-28 in Brussels, showed that OPKO's long-acting Factor VIIa-CTP exhibited a four times longer half-life and a four times improved drug exposure versus Novo Nordisk's \$1.7 billion Factor VIIa product, NovoSeven. Additionally, on February 27, 2014, the FDA granted orphan drug designation to OPKO's long-acting Factor VIIa-CTP for the treatment and prophylaxis of bleeding episodes in patients with hemophilia A or B with inhibitions against Factors VIII or IX.

- **TESARO Achieves Successful Primary Endpoints in Phase 3 Trials of Rolapitant.** TESARO recently announced that two Phase 3 trials of oral rolapitant, one in patients receiving moderately emetogenic chemotherapy (MEC) and one in patients receiving cisplatin-based highly emetogenic chemotherapy (HEC), each met the primary endpoint of complete response (CR) in the delayed (24 to 120 hour) timeframe following chemotherapy. Enrollment in the third and final Phase 3 trial of oral rolapitant, which is being conducted in patients receiving cisplatin-based HEC, is expected to conclude during the first quarter of 2014. TESARO anticipates that results from this study will be available in the second quarter of 2014. Further, the clinical trial of intravenous (IV) rolapitant is well underway, and TESARO anticipates finalizing the dose that will provide comparable exposure to the oral formulation by the end of the first quarter of 2014.
- **Completed Acquisition of Laboratorio Arama de Uruguay Limitada:** OPKO has continued to grow its Latin American presence with the early January 2014 acquisition of Laboratorio Arama de Uruguay Limitada ("Arama"). Arama broadens the global commercial prospects for OPKO's product pipeline by establishing a footprint in Uruguay that may facilitate the Company's future commercial expansion into neighboring Argentina, as well as by providing another platform to commercialize the *4Kscore*[™] product.
- **OPKO Investee, Neovasc, Successfully Completes First Human Implant of Tiara[™] Transcatheter Mitral Valve:** In early February 2014, Neovasc Inc., announced that a human implantation of its Tiara[™] transcatheter mitral valve was successfully performed on January 30th by physicians at St. Paul's Hospital in Vancouver, BC. The transapical procedure resulted in the elimination of mitral regurgitation (MR) and significantly improved heart function in the patient, without the need for cardiac bypass support and with no procedural complications. OPKO's investment in Neovasc continues to appreciate.
- **Exit from Sorrento Therapeutics:** In mid-December 2013, OPKO reported the highly successful exit of its investment in Sorrento Therapeutics, Inc. The sale of Sorrento shares added to OPKO's cash position and represented an approximate ten-fold return of OPKO's 2009 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 our research and development and operations, our product development efforts, including whether the Phase 3 clinical trials for Rayaldy™, hGH-CTP, rolapitant, and our clinical validation study for the 4Kscore™ will be completed on a timely basis or at all and whether the data will support approval, validation and/or reimbursement for our products, our ability to enroll in our 4Kscore™ study more than 1,200 patients referred for a prostate biopsy, the expected timing for launch of our products in development, including the 4Kscore™, Rayaldy™, 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 Rayaldy™, the 4Kscore™, hGH-CTP, and a treatment for Dravet's Syndrome, the timing for submission of a NDA by us for Rayaldy™ and by TESARO for rolapitant, whether TESARO will identify a dose of IV rolapitant that provides comparable exposure to the oral formulation, whether the 4Kscore™ has great potential in the diagnosis and treatment of prostate cancer, expectations regarding the performance of companies in which we have a strategic investment and whether we will monetize and realize a profit from our strategic investments, and whether we will continue to solidify our broad development pipeline across a growing operating platform, 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 Rayaldy™, rolapitant, hGH-CTP, and/or any of our compounds or diagnostic products under development, including our 4Kscore™ test, 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.

TABLE 1

OPKO Health, Inc. and Subsidiaries

CONDENSED CONSOLIDATED BALANCE SHEETS

As of December 31,

(in millions)

	2013	2012
Assets:		
Cash and cash equivalents	\$ 185.8	\$ 27.4
Other current assets	56.9	51.3
	242.7	78.7
In-process R&D and Goodwill	1,019.7	92.0
Other assets	129.1	119.1
Total Assets	\$ 1,391.5	\$ 289.8
Liabilities, Series D Preferred Stock and Equity:		
Current liabilities	\$ 91.8	\$ 52.4
2033 Senior Notes, net	211.9	-
Other long-term liabilities, principally contingent consideration and deferred tax liabilities	214.8	34.1
	518.5	86.5
Series D Preferred Stock and Equity	873.0	203.3
Total Liabilities, Series D Preferred Stock and Equity	\$ 1,391.5	\$ 289.8

TABLE 2

OPKO Health, Inc. and Subsidiaries

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(in millions, except per share data)

	For the Three Months Ended		For the Year Ended	
	December 31,		December 31,	
	2013	2012	2013	2012
Revenues	\$ 20.7	\$ 16.2	\$ 96.5	\$ 47.0
Costs and expenses	(56.5)	(25.8)	(176.1)	(84.3)
Operating loss from continuing operations	(35.8)	\$(9.6)	(79.6)	(37.3)

Other income and (expense), net	21.4	(0.3)	(24.6)	0.1
	(14.4)	(9.9)	(104.2)	(37.2)
Benefit from/(Provision for) income taxes	0.6	9.7	(1.7)	9.6
Loss from continuing operations before investment losses	(13.8)	(0.2)	(105.9)	(27.6)
Loss from investments in investees	(3.5)	(0.6)	(11.4)	(2.1)
Loss from continuing operations	(17.3)	(0.8)	(117.3)	(29.7)
Income from discontinued operations, net of tax	-	0.2	-	0.1
Net Loss	(17.3)	(0.6)	(117.3)	(29.6)
Less: Net loss attributable to noncontrolling interests	0.5	-	2.9	0.5
Preferred stock dividend	-	(0.5)	(0.4)	(2.2)
Net loss attributable to common shareholders	\$(16.8)	\$(1.1)	\$(114.8)	\$(31.3)
Basic and diluted earnings per share	\$(0.04)	\$(0.00)	\$(0.32)	\$(0.11)

OPKO Health, Inc.

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Source: OPKO Health, Inc.