

August 11, 2014



OPKO Announces Second Quarter Operating and Financial Results

- **Royaldeev™ Meets Primary Endpoints in First Pivotal Phase 3 Trial; Top Line Data From 2nd Pivotal Phase 3 Trial to be Available September 2014; NDA Submission on Track for Q4 2014**
- **IND Submitted for Royaldee as Adjunctive Cancer Therapy**
- **Positive Pediatric Phase 2 Data Reported for hGH-CTP Once Weekly Human Growth Hormone**
- **Long Acting Factor VIIa-CTP For Hemophilia Receives Three Orphan Drug Designations in Europe**
- **U.S. Marketing Commenced for 4Kscore™ Blood Test to Diagnose Aggressive Prostate Cancer**
- **Rolapitant™ Meets all Primary and Secondary Endpoints in Final Phase 3 Trial; On Schedule for mid-September NDA Filing**
- **Inspiro Next Generation Inhaler Acquisition Completed**
- **Cash and Cash Equivalents Total \$134.0 Million Providing Adequate 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 second quarter ended June 30, 2014.

Business Highlights

- **Royaldee Meets Primary Endpoints in First Pivotal Phase 3 Trial; NDA Submission on Track for Q4 2014:** OPKO announced successful top-line results from the first pivotal Phase 3 trial of Royaldee. This trial is one of two identical randomized, double-blind, placebo-controlled, multi-site studies intended to establish the safety and efficacy of Royaldee as a new treatment for secondary hyperparathyroidism (SHPT) in patients with stage 3 or 4 chronic kidney disease (CKD) and vitamin D insufficiency. OPKO to announce top line results from the second Phase 3 trial in September 2014 and is on track to submit a New Drug Application (NDA) with the United States (U.S.) Food and Drug Administration (FDA) in the fourth quarter of 2014.
- **Submission of IND for Royaldee as Adjunctive Cancer Therapy:** OPKO submitted an Investigational New Drug (IND) Application to the FDA to evaluate Royaldee as an adjunctive therapy, the first of other possible uses, for the prevention of skeletal-related events (SREs) in patients with bone metastases undergoing anti-resorptive therapy. The clinical trial will commence later in 2014.
- **Positive Pediatric Phase 2 Data Reported for hGH-CTP:** OPKO announced positive 6 month results from its Phase 2 study evaluating the safety and efficacy of hGH-CTP, a long-acting form of human growth hormone, to treat growth hormone deficiency in children. All three hGH-CTP once-weekly doses resulted in strong catch-up growth

during the six month treatment period, with annualized increases in height of more than 12 cm. The results indicated excellent dose dependent pharmacokinetic (PK) and pharmacodynamic (PD) profiles. No serious adverse events were reported.

- **Factor VIIa-CTP Received Positive Opinion For Three Orphan Drug Designations in Europe:** The European Committee for Orphan Medicinal Products (COMP) gave a positive opinion to recommend approval of orphan drug designations for OPKO's long-acting version of clotting Factor VIIa (Factor VIIa-CTP) to treat bleeding episodes in patients with hemophilia A or B who have inhibitors to Factor VIII, Factor IX or congenital Factor VII deficiency. Factor VII-CTP previously was granted orphan status in the U.S. The European designations may provide OPKO the opportunity to have marketing exclusivity for periods of up to 10 years.
- **Adoption of 4Kscore Test is Growing:** OPKO completed a validation study and launched the 4Kscore Test in the U.S. through its CLIA accredited OPKO Lab in late March 2014. Since launch, its adoption by Urologists has steadily increased. OPKO expects to launch the 4Kscore Test in Europe through its Spanish subsidiary in September 2014 and elsewhere shortly thereafter. OPKO is working to obtain reimbursement for the 4Kscore Test by payers. The 4Kscore Test makes the prostate biopsy decision process more rational. In the U.S. approximately 1 million biopsies are performed annually with 80% indicating no cancer or only low grade cancer. With the knowledge of the risk (probability) of having high grade cancer provided by the 4Kscore Test, prostate biopsies are more likely to be performed only in men at higher risk. OPKO presented data from its U.S. clinical validation study in a plenary session at the American Urological Association (AUA) in Orlando, FL. The talk, "The 4Kscore Test as a Predictor of High-Grade Prostate Cancer on Biopsy," was also selected for a plenary presentation at the Spanish National Congress of Urology in June, and will be presented at the 83rd Annual Meeting of the New England Section of the AUA in October 2014 in Newport, RI.
- **Rolapitant Meets all Primary and Secondary Endpoints in Final Phase 3 Trial; On Schedule for a mid-September NDA Filing:** OPKO's partner, TESARO, announced positive results from the third and final Phase 3 trial of Rolapitant, a neurokinin-1 (NK-1) receptor antagonist for the prevention of chemotherapy-induced nausea and vomiting (CINV). The oral formulation of Rolapitant is on track for an NDA filing by mid-September; the intravenous (IV) formulation is expected to be available to patients one year after the oral formulation.
- **Inspiro Acquisition Completed:** OPKO acquired Inspiro Medical Ltd., an Israeli medical device company with a new platform to deliver inhaled drugs. Inspiro's Inspiromatic™ is a "smart", easy-to-use dry powder inhaler with important advantages over existing devices.

"In the second quarter, we continued to make strides in converting our advanced pipeline of diagnostic and pharmaceutical products into commercial successes," said Phillip Frost, M.D., Chairman and CEO. "The 4Kscore Test is being increasingly adopted by Urologists because of its utility for identifying patients at the greatest risk of having high-grade prostate cancer and clinical trials of Rayaldee, hGH-CTP and Rolapitant have all provided the anticipated evidence of safety and efficacy. We look forward to making these important products available as rapidly as possible to patients who will benefit from their use."

Financial Highlights

At June 30, 2014, OPKO had cash and cash equivalents of \$134.0 million providing OPKO with adequate liquidity to continue the development of its product candidates. In addition, OPKO strengthened its balance sheet by retiring \$70.4 million of its convertible senior notes due in 2033 through a privately negotiated transaction. During the six months ended June 30, 2014, OPKO continued to invest in its research and development programs, utilizing \$44.5 million of cash from operations to advance its projects including clinical trials for Rayaldee and hGH-CTP and the clinical validation study for 4Kscore Test which was completed in March 2014.

Pharmaceutical product revenue for the three months ended June 30, 2014 increased approximately 15% to \$21.4 million compared to \$18.6 million for the 2013 period. This increase was principally the result of increased revenue from OPKO's active pharmaceutical ingredient business at FineTech as well as increased pharmaceutical product revenue at OPKO Spain and OPKO Mexico. Total revenue for the three months ended June 30, 2014 was \$23.5 million compared to \$23.8 million for the 2013 period. Total revenue for the three months ended June 30, 2013 included non-recurring revenue of \$2.0 million related to OPKO's transactions with Pharmsynthez.

Net loss for the three months ended June 30, 2014 was \$25.5 million, compared to \$3.4 million in the comparable period of 2013. The increase in net loss was significantly impacted by an \$8.5 million gain related to the successful exit of a strategic investment in the three months ended June 30, 2013. Net loss for the three months ended June 30, 2014 included \$10.1 million of non-recurring expense related to the write-off of acquired in-process research and development expense related to the Inspiro acquisition. OPKO continued to increase its investment in research and development activities during the three months ended June 30, 2014 related to its ongoing Phase 3 programs for Rayaldee and hGH-CTP. As a result, OPKO's spending on research and development increased \$6.7 million to \$16.2 million for the three months ended June 30, 2014 from \$9.6 million for the three months ended June 30, 2013.

For the six months ended June 30, 2014, pharmaceutical product revenue increased approximately 21% to \$41.2 million compared to \$34.1 million for the 2013 period. The increase in pharmaceutical product revenue was principally the result of increased revenue from FineTech, OPKO Spain and OPKO Mexico. Total revenue for the six months ended June 30, 2014 was \$45.8 million compared to \$55.2 million for the 2013 period. Total revenue for the three months ended June 30, 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 for the six months ended June 30, 2014 was \$70.0 million compared to \$38.0 million for the first six months of 2013. 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 \$17.8 million to \$37.2 million for the six months ended June 30, 2014 from \$19.5 million for the six months ended June 30, 2013. In addition, net loss for the six months ended June 30, 2014 included a non-recurring in-process research and development expense of \$10.1 million due to a write-off of in-process research and development expense in connection with the acquisition of Inspiro. The six month period ended June 30, 2013 also included \$12.5 million of non-

cash income related to the RXi transaction and a \$10.8 million gain realized from the successful exit of a strategic investment. These reductions were partially offset during the six months ended June 30, 2014 by our increased pharmaceutical product revenue of \$7.1 million and a decrease in non-cash expense of \$11.4 million from the change in fair value of embedded derivatives principally related to the retirement of our Senior 2033 Notes.

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, increased adoption rates for the 4Kscore by Urologists, the timing for submission of an NDA by us for Rayaldee and by TESARO for Rolapitant, 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	
	June 30, 2014	December 31, 2013
Assets:		
Cash and cash equivalents	\$ 134.0	\$ 185.8
Other current assets	49.0	56.9
Total Current Assets	183.0	242.7
In-process Research and Development and Goodwill	1,019.3	1,019.7
Other assets	124.8	129.1
Total Assets	\$ 1,327.1	\$ 1,391.5
Liabilities, Series D Preferred Stock and Equity:		
Current liabilities	\$ 79.4	\$ 91.8
2033 Senior Notes, net	116.4	211.9
Other long-term liabilities	218.5	214.8
Total Liabilities	414.3	518.5
Series D Preferred Stock and Equity	912.8	873.0
Total Liabilities, Series D Preferred Stock and Equity	\$ 1,327.1	\$ 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

June 30,

	2014		2013	
Revenues	\$ 23.5		\$ 23.8	
Costs and expenses	58.4		41.8	
Operating loss	(34.9)	(18.0)
Other income and (expense), net	9.3		16.9	
Loss before income taxes and investment losses	(25.6)	(1.1)
Benefit from (provision for) income taxes	(0.1)	(0.9)
Loss before investment losses	(25.7)	(2.0)
Loss from investments in investees	(0.4)	(2.4)
Net loss	(26.1)	(4.4)
Less: Net loss attributable to non-controlling interests	(0.6)	(1.0)
Preferred stock dividend	-		-	
Net loss attributable to common shareholders	\$ (25.5)	\$ (3.4)
Basic and diluted loss per share	\$ (0.06)	\$ (0.01)

For the six months ended

June 30,

	2014		2013	
Revenues	\$ 45.8		\$ 55.2	
Costs and expenses	111.0		80.0	
Operating loss	(65.2)	(24.8)
Other income and (expense), net	(2.8)	(7.1)
Loss before income taxes and investment losses	(68.0)	(31.9)
Benefit from (provision for) income taxes	(0.7)	(1.0)
Loss before investment losses	(68.7)	(32.9)
Loss from investments in investees	(2.4)	(6.2)
Net loss	(71.1)	(39.1)
Less: Net loss attributable to non-controlling interests	(1.1)	(1.5)
Preferred stock dividend	-		(0.4)
Net loss attributable to common shareholders	\$ (70.0)	\$ (38.0)

Basic and diluted loss per share

\$ (0.17) \$ (0.12)

OPKO Health, Inc.

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