

November 11, 2013



OPKO Announces Third Quarter 2013 Financial and Operating Highlights

- Revenue Nearly Doubled to \$20.6 million for the Third Quarter 2013; Increased More Than Twofold to \$75.8 million for the First Nine Months of 2013
- Initiation of Pivotal Clinical Validation Study for *4Kscore*™
- Enrollment in Our Pivotal *Rayaldy*™ Clinical Trials Surpassed 75% Completion; Top-Line Data Expected in Mid-2014
- Continuing Progress Toward Completion by TESARO of Rolapitant Phase 3 Clinical Trials; Two of TESARO's Phase 3 trials of Rolapitant Are Now Fully Enrolled, Top-line Data Expected at Year End. TESARO Also Initiated an Open-Label, Dose-Finding and Escalation Study to Evaluate Intravenous (IV) Formulation of Rolapitant
- Final Marketing Authorization for Citicoline Products in Spain
- Completed Acquisition of PROLOR Biotech
- Strategic Investment in Biotechnology Company Engaged in the Development of a New Generation of Antibodies
- Strategic Investment in Clinical Stage Biopharmaceutical Company Focused in the Development of Oncology Therapeutics
- New Listing on Tel Aviv Stock Exchange
- Neovasc, an OPKO Investment Portfolio Company, Reported That the *Neovasc Reducer*™ Achieved Primary Endpoint in COSIRA Trial, Significantly Improving Function in Patients with Refractory Angina
- Cash and Marketable Securities at \$180.8 million as of September 30, 2013

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NYSE:OPK), a multi-national biopharmaceutical and diagnostics company, today reported operating and financial highlights for the third quarter of 2013.

Third Quarter 2013 Financial Highlights

- Cash, cash equivalents and marketable securities were \$180.8 million as of September 30, 2013.
- Consolidated revenues nearly doubled to \$20.6 million during the three months ended September 30, 2013 from \$11.8 million in the prior year period. Consolidated revenue more than doubled to \$75.8 million during the nine months ended September 30, 2013 from \$30.8 million in the prior year period. Revenue for the nine months ended September 30, 2013 included \$12.5 million of revenue resulting from a strategic partnership in the field of RNA interference with RXi Pharmaceuticals Corporation.
- Net loss for the three months ended September 30, 2013 was \$60.0 million, compared to a net loss of \$10.2 million for the 2012 period. The increase in net loss for the three months ended September 30, 2013 was primarily related to increased operating and clinical trial activities, and non-cash charges, such as:
 - \$27.8 million in non-cash charges related to the change in value of embedded

- derivatives which are part of our January 2013 convertible senior notes due in 2033 (the “2033 Senior Notes”). This non-cash charge is principally a result of the increased market price of our common stock since the previous quarter end; and
 - \$8.7 million in non-cash charges related to early conversion of 2033 Senior Notes.
- Net loss for the nine months ended September 30, 2013 was \$98.0 million, compared to a net loss of \$30.2 million for the 2012 period. The increase in net loss for the nine months ended September 30, 2013, was primarily related to increased operating and clinical trial costs, and non-cash charges, such as:
 - \$38.7 million in non-cash charges principally related to the change in value of embedded derivatives which are part of our 2033 Senior Notes, principally as a result of the increase in the market price of our common stock since issuance of such notes; and
 - \$8.7 million in non-cash charges related to early conversion of 2033 Senior Notes.

Net loss for the nine months ended September 30, 2013 also includes \$11.0 million of other income from the sale of securities, partially offset by \$10.1 million of interest expense principally related to our 2033 Senior Notes.

Business Highlights

- On November 4, 2013, we announced the initiation of a multi-center study expected to generate data to support the launch of the *4Kscore*™ test as a laboratory developed test through our Clinical Laboratory Improvement Amendments of 1988 (CLIA)-certified laboratory in Nashville, TN. We are enrolling men at the first of 19 sites across the United States (U.S.) and are planning to enroll more than 1,200 men referred for a prostate biopsy over the next few months. This clinical study is expected to be the last step before our planned commercial launch of the *4Kscore*™ in the first quarter of 2014. The data we generate in this clinical study will not only be used for the required CLIA validation, but will also be used to secure *4Kscore*™ test reimbursement.
- Our two ongoing Phase 3 trials for *Rayaldy*™, our vitamin D prohormone to treat patients with secondary hyperparathyroidism (SHPT), stage 3 or 4 chronic kidney disease (CKD) and vitamin D insufficiency, continues to progress well with aggregate enrollment surpassing 75%. We anticipate top-line data from this pivotal program in mid-2014.
- Each of three Phase 3 trials of rolapitant being conducted by our licensee, TESARO, Inc., for the prevention of chemotherapy-induced nausea and vomiting, continues to progress well towards completion. Two of TESARO's phase 3 trials of oral rolapitant are fully enrolled, with results from the third study becoming available in early 2014. TESARO anticipates announcing top-line data from the two fully enrolled studies at year end. Following completion of Phase 3 trials, TESARO plans to submit a New Drug Application to the U.S. Food and Drug Administration for oral rolapitant in mid-2014. Earlier this year, TESARO also presented results from a pharmacokinetic study of rolapitant at the Multinational Association of Supportive Care in Cancer/International Society of Oral Oncology (MASCC/ISOO) International Symposium in Berlin. These data support concomitant administration of rolapitant with other pharmaceutical products that are metabolized by the liver microsomal enzyme CYP3A4, without a requirement for dose adjustment of the co-administered product. Recently, TESARO

also announced the initiation of an open-label, dose-finding and escalation study to evaluate the pharmacokinetics, safety and tolerability of an IV formulation of rolapitant in healthy volunteers. The clinical trial of intravenous (IV) rolapitant is well underway, and TESARO anticipates identifying a dose of IV rolapitant that provides blood levels similar to those of the oral formulation by year end.

- Our Spanish subsidiary, Pharmadiet, S.L.U., received final marketing authorization for commercialization of its oral and injectable formulations of citicoline which are indicated for the treatment of memory disorders and behavior related to stroke, head injury, chronic disease, as well as degenerative brain disorders. We expect to begin commercialization in Spain of these products, which can be significant contributors to Pharmadiet's sales and earnings, beginning with the first quarter of 2014.
- We completed the acquisition of PROLOR Biotech, Inc., a biopharmaceutical company focused on developing proprietary long-acting forms of presently marketed therapeutic proteins and peptides. In June 2013, PROLOR initiated a pivotal Phase 3 clinical trial in adults of its proprietary long-acting version of human growth hormone, hGH-CTP. The initiation of this Phase 3 clinical trial followed a series of successful Phase 2 trials showing that hGH-CTP can safely reduce the dosing frequency of human growth hormone from the current standard of one injection per day to just one per week. A Phase 2 trial in children with growth hormone deficiency (GHD) is currently ongoing. hGH-CTP has been awarded orphan drug designation in the U.S. and Europe for both adults and children with GHD. PROLOR scientists presented new data at the 95th Annual Meeting of the Endocrine Society in June 2013 regarding MOD-630, our long-acting oxyntomodulin (GLP-1 and Glucagon dual agonist) with potential for treating obese and overweight individuals as well as type 2 diabetes. This pre-clinical study concluded that MOD-630 induced marked weight loss while improving glycemic control and lipid profile in mice with diet-induced obesity after once-weekly administration. PROLOR scientists also presented new data at the XXIV Congress of the International Society of Hemostasis and Thrombosis from preclinical studies of its long-acting clotting factor VIIa (Factor VIIa-CTP), a next generation treatment for hemophilia. These data provided further evidence that Factor VIIa-CTP can be administered by subcutaneous or IV injection, which will facilitate regular prophylactic use. Currently available commercial factor VIIa, which must be administered through IV infusions, limits its prophylactic treatment and can require frequent administrations if patients are treated only "on demand" when a bleed occurs.
- In October 2013, we made a strategic investment in Zebra Biologics, Inc. ("Zebra"), a privately held biotechnology company focused on the discovery and development of biosuperior antibody therapeutics and complex drug targets. Zebra's patented platform is an advanced version of a core technology developed by Richard Lerner, M.D. (also an OPKO director) at The Scripps Research Institute, underlying the commercial success of AbbVie Inc.'s *Humira*™. This new technology allows for the selection of antibodies for function rather than simple binding affinity. Zebra has already developed an early pre-clinical pipeline of candidate antibodies with significant commercial potential in regulating diabetes and obesity, cancer and treatment of neurological disease.
- In October 2013, we made a strategic investment in Arno Therapeutics, Inc. (OTCBQ: ARNI) ("Arno"), a clinical stage biopharmaceutical company focused on the development of oncology drugs. We were attracted to Arno based on the potential of its lead product and cancer therapeutic, onapristone, in addressing the need for new and effective treatment for breast and prostate cancer. In connection with this investment,

we were granted exclusive first rights to negotiate with Arno regarding any potential strategic transactions that Arno elects to pursue.

- OPKO's common stock began trading on the Tel Aviv Stock Exchange ("TASE") in August and is included in the following major TASE indices: Tel Aviv 25, Tel Aviv 100, Tel Aviv Composite, Tel Aviv Biomed, and Tel Aviv BlueTech-50.
- Recently, Neovasc Inc. (NVC.V) ("Neovasc"), a specialty medical device company in which we have a strategic ownership interest and that develops, manufactures and markets products for the rapidly growing global cardiovascular marketplace, reported topline results for its COSIRA trial assessing the efficacy and safety of the Neovasc *Reducer*[™], a novel stent delivered percutaneously to the cardiac sinus for the treatment of refractory angina. The data show that the *Neovasc Reducer*[™] achieved its primary endpoint, significantly improving the symptoms and functioning of patients disabled by previously untreatable refractory angina. The COSIRA trial also confirmed that the *Neovasc Reducer*[™] is safe and well-tolerated, with no reports of device-related serious adverse events.

"We again made great progress during the past quarter across many important business fronts," said Phillip Frost, M.D., OPKO's Chairman and Chief Executive Officer. "Our *4Kscore*[™] has the potential to become a transformational medical tool in the diagnosis and treatment of prostate cancer. We initiated an important pivotal clinical trial to support our planned commercial *4Kscore*[™] launch during the first quarter of 2014. Progress in our two *Rayaldy*[™] clinical trials, now more than 75% enrolled, coupled with the continued development of the clinical-stage products from PROLOR and our recent strategic investments in Zebra and Arno, continue to solidify our broad development pipeline across a growing operating platform," continued Dr. Frost. "Our strategy to make small investments in other health care companies is bearing fruit. We have monetized one of the investments at a significant profit and the value of the remaining portfolio is far in excess of our investment. These results, bolstered by broader shareholder exposure attained through our recent TASE listing, fortify the foundation for an important health care company."

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, 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 over the course of the next few months, the expected timing for launch of our products in development, including the 4Kscore[™], Rayaldy[™], hGH-CTP, and citicoline, 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 citicoline, whether the citicoline products will be significant contributors to Pharmadiet's sales and earnings, the timing for submission of a NDA by TESARO for rolapitant and whether TESARO will identify a dose of IV rolapitant that provides comparable exposure to the oral formulation by year end, whether the 4Kscore™ will become a transformational medical tool 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 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.

OPKO Health, Inc.

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Source: OPKO Health, Inc.