May 11, 2015

OPKO Announces First 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 Pre-Commercialization Payments**
- **Acquired EirGen Pharma, A Growing, Profitable Specialty Pharmaceutical Developer and Manufacturer**
- **Rayaldee™ New Drug Application (NDA) Submission Expected Later in May; Positive Rayaldee Phase 3 Clinical Trial Results Presented at the National Kidney Foundation Spring Clinical Meetings and 2015 ENDO Conference**
- **Investigational New Drug (IND) Application for Long Acting Factor VIIa-CTP for Hemophilia Accepted in Q1 2015; Phase 2a Clinical Trial to Commence in 1H 2015**
- **Phase 1 Clinical Trial for Long Acting Oxyntomodulin for Obesity and Diabetes Expected to Commence During 2H 2015**
- **Marketing for 4Kscore® Blood Test to Identify Risk of Aggressive Prostate Cancer Began in Mexico in January 2015; Additional Latin American Rollout Expected During 2015**
- **Paper Supporting the 4Kscore Blood Test Published in Journal of the National Cancer Institute**
- **Expected Rolapitant™ PDUFA Date of September 5, 2015; OPKO to Receive Up To $110 Million of Milestone Payments Upon Approval and Commercialization**
- **OPKO Consolidated Investee SciVac Entered into an Agreement Pursuant to Which It Will Become a Public Company on the Toronto Stock Exchange with over $19 Million in Working Capital**

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, 2015.

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 up-front 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.
- **hGH-CTP for Pediatric GHD Data Presented at 2015 ENDO; Adult Phase 3 Clinical Trial Continues to Advance:** OPKO presented positive 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.
- **Acquired EirGen Pharma; A Growing, Profitable Specialty Pharmaceutical Developer and Manufacturer:** OPKO acquired EirGen Pharma, Ltd., a growing, profitable and cash flow positive specialty pharmaceutical company focused on the development and commercial supply of high potency, high barrier to entry pharmaceutical products for sale in the U.S., Canada, Japan, Australia, most European countries, and more than 40 others around the world. EirGen, based in Waterford, Ireland, was founded by two former executives of IVAX Pharmaceuticals, Tom Brennan and Patsy Carney. The company, situated in a state of the art high containment research and development and manufacturing facility, is approved by the FDA, EMEA (European Health Authorities) and the PMDA (Japanese Health Authorities). To date, EirGen and its commercial partners have filed 10 product applications with the FDA and 5 each in Europe and Japan. EirGen has a strong research and development portfolio of over 20 niche, high barrier to entry drugs. EirGen will rapidly expand its drug portfolio with access to additional capital from OPKO, together with the benefits of Irish government programs encouraging research and development in Ireland. EirGen also offers significant synergies and benefits to OPKO through its ability to manufacture OPKO's current and future products.
- **Rayaldee Met Primary Endpoints in Both Pivotal Phase 3 Trials; NDA Submission Scheduled Later in May:** OPKO announced successful top-line results from both of its pivotal Phase 3 trials with Rayaldee in September 2014. 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 has scheduled submission of NDA to the FDA later in May.

- **Positive Rayaldee Results Presented at the National Kidney Foundation Spring Clinical Meetings and 2015 ENDO Conference:** The presentations of positive Rayaldee Phase 3 trial data were entitled "Modified-Release Calcifediol Is Effective for SHPT in Both Stages 3 and 4 CKD" (March 2015 in Dallas TX) and "Modified-Release Calcifediol Controls Elevated iPTH, Corrects 25(OH)D Levels and Reduces Bone Markers in CKD Patients" (March 2015 in San Diego, CA).
- **IND for Long Acting Factor VIIa-CTP for Hemophilia Filed and Accepted:** In March 2015, the FDA accepted OPKO's IND application 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 Mexico and Expects Further Latin American Launches in 2015; Adoption of 4Kscore Test Continues to Grow in the U.S. and Europe:** OPKO launched the 4Kscore Test 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 a Study Entitled "Predicting High-Grade Cancer at Ten-Core Prostate Biopsy Using Four Kallikrein Markers Measured in Blood in the ProtecT Study.":** The ProtecT study is a prospective randomized controlled trial conducted in the UK for the purpose of evaluating the cost effectiveness of conventional treatments in PSA-detected, clinically localized prostate cancer. Of the 82,428 men recruited for the trial, a total of 6,129 men with elevated PSA (≥3.0 ng/mL) who underwent prostate biopsy and provided an adequate blood sample were tested for the four kallikreins and their 4Kscore results were determined. The study showed that the four kallikrein panel enhanced aggressive prostate cancer detection compared with PSA and age alone. The area under the curve (AUC) for the 4K model was 0.820 (95% CI = 0.802 to 0.838) while the PSA model was 0.738 (95% CI = 0.716 to 0.761) for high-grade cancer. The ProtecT study is the latest in a series of peer-reviewed publications demonstrating the superior clinical value that testing with four kallikrein markers adds to risk prediction for aggressive prostate cancer.
- **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. Upon approval and following commercialization, OPKO will receive up to $110 Million in additional milestones and tiered, double digit royalties.

"We began 2015 with a number of significant activities including the closing of the Pfizer transaction, the initiation of our clinical development program for Factor VIIa-CTP and the imminent submission of our NDA for Rayaldee," said Phillip Frost, M.D., Chairman and CEO. "The 4Kscore Test continues to gain acceptance in the US, Mexico and Europe and we are pleased with the recently published studies further supporting the long-term outcomes of using this potentially revolutionary blood test. With the EirGen acquisition, we added a growing, profitable, cash flow positive business to our complement of operating companies which we believe will lead to further growth and profitability of our other businesses," Dr. Frost continued.

Financial Highlights

- Cash, cash equivalents and marketable securities were $348.2 million as of March 31, 2015.
  - Reflects receipt of Pfizer upfront payments of $295.0 million, partially offset by a non-recurring $25.9 million payment to the Office of the Chief Scientist in Israel related to the Pfizer transaction.
- Consolidated revenues increased to $30.1 million during the three months ended March 31, 2015 from $22.3 million in the comparable period of 2014. Revenue for the three months ended March 31, 2015 included $12.5 million of revenue resulting from OPKO's collaboration agreement with Pfizer. OPKO is recording revenue in connection with the Pfizer transaction on a straight-line basis over the expected development period.
- Net loss for the three months ended March 31, 2015 included increased operating expenses including a $4.5 million increase in research and development expense to $25.5 million, and non-recurring and non-cash charges, such as:
  - $25.9 million of non-recurring operating expense related to the repayment of a grant to the Office of the

Chief Scientist in Israel related to the Pfizer transaction; and
- $49.8 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 December 31, 2014.

CONFERENCE CALL & WEBCAST INFORMATION:

WHEN: Monday, May 11, 2015, 4:30 p.m. ET
DOMESTIC & CANADA DIAL-IN: (877) 407-0789
INTERNATIONAL DIAL-IN: (201) 689-8562

LIVE WEBCAST LINK: http://public.viavid.com/index.php?id=114458

For those unable to participate in the conference call or webcast, a replay will be available beginning May 11, 2015 at 7:30 p.m. ET until May 18, 2015 at 11:59 p.m. ET. To access the replay, dial (877) 870-5176 or (858) 384-5517. The replay passcode is 13608883.

The replay can be accessed for a limited time on OPKO's website at www.opko.com.

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 efforts 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, expectations about potential milestone payments from our partners, our ability to launch sales of the 4Kscore Test in additional Latin American countries, increased adoption rates for the 4Kscore by Urologists in the U.S. and abroad, the timing for submission of a 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, our ability to manufacture products at EirGen and achieve expected synergies, 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	
	March 31, 2015	December 31, 2014
Assets:		
Cash and cash equivalents	$ 348.2	$ 96.9
Other current assets	45.8	46.0
Total Current Assets	394.0	142.9
In-process Research and Development and Goodwill	1,016.2	1,017.4
Other assets	102.0	107.4
Total Assets	$ 1,512.2	$ 1,267.7
Liabilities and Equity:		
Current liabilities	$ 182.8	$ 83.1
2033 Senior Notes, net	106.7	131.5
Other long-term liabilities	410.2	217.3
Total Liabilities	699.7	431.9
Equity	812.5	835.8
Total Liabilities and Equity	$ 1,512.2	$ 1,267.7

OPKO Health, Inc. and Subsidiaries

Condensed Consolidated Statements of Operations

(unaudited)

(in millions, except per share data)

	For the three months ended March 31,	
	2015	2014
Revenues	$ 30.1	$ 22.3
Costs and expenses	87.0	52.6
Operating loss	(56.9)	(30.3)
Other income and (expense), net	(53.9)	(12.1)
Loss before income taxes and investment losses	(110.8)	(42.4)
Benefit from (provision for) income taxes	(5.5)	(0.6)
Loss before investment losses	(116.3)	(43.0)
Loss from investments in investees	(1.8)	(2.1)
Net loss	(118.1)	(45.1)
Less: Net loss attributable to non-controlling interests	(1.0)	(0.5)
Net loss attributable to common shareholders	$ (117.1)	$ (44.6)
Basic and diluted loss per share	$ (0.26)	$ (0.11)

OPKO Health, Inc.
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Source: OPKO Health, Inc.