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OPKO Licensee TESARO Receives FDA Approval for VARUBI™ (rolapitant)

- **VARUBI™ to Address a Potential U.S. Market Opportunity That Exceeds $1 Billion**
- **U.S. Commercial Launch Planned for Q4 2015; OPKO to Receive up to $110 million of Milestone Payments and Double Digit tiered Royalties**

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NYSE:OPK), today announced that the U.S. Food and Drug Administration (FDA) has approved VARUBI™ (rolapitant) in combination with other antiemetic agents in adults for the prevention of delayed nausea and vomiting associated with initial and repeat courses of emetogenic cancer chemotherapy, including, but not limited to, highly emetogenic chemotherapy.

“We are extremely happy that our partners at TESARO were able to successfully develop and obtain FDA approval for VARUBI. TESARO’s excellent management team is the ideal partner to successfully commercialize and reach VARUBI’s full commercial potential,” commented Phillip Frost, MD, OPKO’s Chairman and Chief Executive Officer.

About Chemotherapy-Induced Nausea and Vomiting (CINV)

Chemotherapy-induced nausea and vomiting is a debilitating, yet often preventable, side effect of chemotherapy.

Up to 50% of patients undergoing highly or moderately emetogenic chemotherapy experience delayed CINV (25 to 120 hours post chemotherapy)—even when prescribed a 5-HT_3 receptor antagonist and corticosteroid.

Blocking both 5-HT_3 and NK-1 receptors has been shown to offer better control of nausea and vomiting than inhibiting 5-HT_3 receptors alone. Adding a single dose of VARUBI to an antiemetic regimen, including a 5-HT_3 receptor antagonist and corticosteroid, further improves prevention of CINV in the delayed Phase following chemotherapy.

About the VARUBI (Rolapitant) Clinical Program

The superior efficacy of VARUBI was established in multiple randomized, well-controlled, blinded clinical trials that enrolled more than 2,500 patients. VARUBI, when administered in combination with a 5-HT_3 receptor antagonist and dexamethasone, was superior to a 5-HT_3 receptor antagonist and dexamethasone in preventing CINV in patients receiving either moderately or highly emetogenic chemotherapy.

The clinical profile of VARUBI in cisplatin-based highly emetogenic chemotherapy (HEC) was confirmed in two identical Phase 3 studies: HEC1 and HEC2. Both trials met their primary endpoint of complete response (CR), and demonstrated statistical superiority of

rolapitant 180 mg compared to active control (5-HT_3 receptor antagonist + dexamethasone) in the delayed Phase (25-120 hours) of CINV. In HEC1, 264 patients received rolapitant 180 mg and 262 received control. The proportion of patients achieving a CR was 72.7% vs. 58.4% (p= < 0.001). In HEC2, 271 patients received rolapitant and 273 received control. The proportion of patients achieving a CR was 70.1% vs. 61.9% (p=0.043). The most common adverse reactions (≥3%) among patients receiving cisplatin-based chemotherapy were neutropenia (9% VARUBI vs. 8% control), hiccups (5% vs. 4%), and abdominal pain (3% vs. 2%).

A Phase 3 trial was also conducted to evaluate rolapitant 180 mg compared to active control in 1,332 patients receiving moderately emetogenic chemotherapy regimens, including anthracycline/cyclophosphamide combinations, carboplatin, irinotecan, pemetrexed, oxaliplatin, and doxorubicin. This trial met its primary endpoint of CR, and demonstrated statistical superiority of rolapitant 180 mg compared to active control (5-HT_3 receptor antagonist + dexamethasone) in the delayed Phase of CINV. The proportion of patients achieving a CR was 71.3% vs 61.6% (p= < 0.001). The most common adverse reactions (≥3%) among patients receiving these chemotherapies were decreased appetite (9% VARUBI vs. 7% control), neutropenia (7% vs. 6%), dizziness (6% vs. 4%), dyspepsia (4% vs. 2%), urinary tract infection (4% vs. 3%), stomatitis (4% vs. 2%), and anemia (3% vs. 2%).

Primary data from the three Phase 3 studies have recently been published online ahead of print in *Lancet Oncology,* the analysis of the non-AC MEC population was presented at the 2015 annual meeting for the Multinational Association for Supportive Care in Cancer, and commentary has been provided in *Nature Reviews Clinical Oncology*.

VARUBI Additional Safety Information

VARUBI is contraindicated in patients receiving thioridazine, a CYP2D6 substrate with a narrow therapeutic index.

Use of VARUBI should be avoided in patients who are receiving pimozide, a CYP2D6 substrate with a narrow therapeutic index. Adverse reactions should be monitored if concomitant use of VARUBI and other CYP2D6 substrates with a narrow therapeutic index cannot be avoided. The inhibitory effect of VARUBI on CYP2D6 lasts for at least 7 days and may last longer after administration of a single dose of VARUBI.

VARUBI is available by prescription only.

About VARUBI

VARUBI is a substance P/neurokinin-1 (NK-1) receptor antagonist indicated in combination with other antiemetic agents in adults for the prevention of delayed nausea and vomiting associated with initial and repeat courses of emetogenic cancer chemotherapy, including, but not limited to, highly emetogenic chemotherapy. NK-1 receptors are highly concentrated in the brain and bind neurokinin substance P. Activation of NK-1 receptors plays a central role in nausea and vomiting induced by emetogenic stimuli, including certain cancer chemotherapies. A Positron Emission Tomography (PET) study with rolapitant in normal, healthy volunteers demonstrated that rolapitant crosses the blood brain barrier and occupies brain NK-1 receptors at high levels for up to 120 hours. VARUBI has a half-life of approximately 7 days, which may contribute to the ability of a single dose of VARUBI to

cover the entire delayed CINV Phase (25-120 hours).

An intravenous formulation of rolapitant is also being developed.

The full prescribing information for VARUBI will be available at www.VarubiRx.com.

About OPKO

OPKO is a multinational biopharmaceutical and diagnostics company that seeks to establish industry-leading positions in large, rapidly growing markets by leveraging its discovery, development and commercialization expertise and novel and proprietary technologies. For more information, visit http://www.opko.com.

Forward Looking Statements

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 benefits of VARUBI, that it will address a U.S. market exceeding $1 billion, that TESARO will launch VARUBI in the fourth quarter, expectations regarding milestones, that it will be successfully commercialized, TESARO's ability to market and sell the product, 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 VARUBI 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.

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