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OPKO's GeneDx Study Demonstrates Whole Exome Sequencing Usefulness In Diagnosing Rare Disorders

Study reveals new disease causing genes

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NYSE:OPK) announced today through its subsidiary GeneDx, results from a retrospective review of molecular diagnostic testing by Whole Exome Sequencing (WES). The peer-reviewed study, "*Clinical Application of Whole Exome Sequencing across Clinical Indications,*" was published in the December 2015 issue of Genetics in Medicine. It is the largest data set of its kind published to date and demonstrates that WES is successful in providing a definitive diagnosis for patients with rare and complex genetic conditions when parents or other affected family members are included in the analysis.

GeneDx, a wholly-owned subsidiary of OPKO, reviewed 3,040 consecutive WES cases over three years to better understand how WES could provide a genetic diagnosis for signs and symptoms in an affected individual. Each patient and his or her parents, when possible, were analyzed, which significantly improved diagnostic yield, especially for genetic disorders caused by a newly occurring (*de novo*) pathogenic variant not present in either parent. By comparing molecular findings in many patients with similar or overlapping presentations, GeneDx was able to observe possible links between groups of patients and genes that, until now, have not been known to cause human disease ('candidate' genes).

"Our research underscores the benefit of testing parents and the patient; demonstrating that WES has the potential to identify pathogenic variants in genes that would not typically have been tested via targeted gene panels and which would have otherwise been missed," said Marc D. Grodman, M.D., CEO at BioReference Laboratories. "Interrogating our large data set for clustering of pathogenic variants with similar clinical presentations in 'candidate' genes allowed us to successfully identify novel genes that cause rare genetic disorders."

"Whole Exome sequencing is one of the most interesting technologies available for the diagnosis of hereditary disease, and holds great promise for providing information and potential management options for patients and families dealing with complex genetic conditions," said Sherri Bale, PhD, FACMG, co-founder and Managing Director of GeneDx.

About OPKO Health, Inc.

OPKO Health, Inc. is a diversified healthcare company that seeks to establish industry-leading positions in large, rapidly growing markets. Our diagnostics business includes Bio-Reference Laboratories, the nation's third-largest clinical laboratory with a core genetic testing business and a 420-person sales force to drive growth and leverage new products, including the 4Kscore® prostate cancer test and the Claros®1 in-office immunoassay platform. Our pharmaceutical business features Rayaldee™, a treatment for SHPT in stage

3-4 CKD patients with vitamin D insufficiency (March 29, 2016 PDUFA date) and VARUBI™ for chemotherapy-induced nausea and vomiting (oral formulation launched by partner Tesaro, IV formulation in Phase 3). Our biologics business includes hGH-CTP, a once-weekly human growth hormone injection (in Phase 3 and partnered with Pfizer), and a long-acting Factor VIIa drug for hemophilia (entering Phase 2a). We also have production and distribution assets worldwide, multiple strategic investments and an active business development strategy. More information is available at www.opko.com.

About GeneDx

GeneDx and its parent company BioReference Laboratories Inc. are members of the OPKO Health, Inc. (NYSE: OPK) group of companies. GeneDx is a world leader in Genomics with an acknowledged expertise in rare and ultra rare genetic disorders, as well as one of the broadest menus of sequencing services available among commercial laboratories. GeneDx performs more clinical Whole Exome Sequencing tests than any other diagnostic lab in the world. The GeneDx mission is to make clinical testing affordable and available to people with rare genetic conditions and their families. GeneDx provides testing to patients and their families in more than 55 countries. To learn more, please visit www.genedx.com.

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 WES and the ability to provide a definitive diagnosis for patients with rare and complex genetic conditions, our ability to successfully identify novel disease genes that cause rare genetic disorders, expectations about the uses of WES, 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. 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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