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OPKO'S GeneDx Announces Research Collaboration with the Wellcome Trust Sanger Institute

MIAMI, Feb. 01, 2017 (GLOBE NEWSWIRE) -- (Nasdaq:OPK) OPKO Health, Inc. announced today that its subsidiary and business unit, GeneDx, is entering into a collaboration with the Deciphering Developmental Disorder (DDD) study led by the Wellcome Trust Sanger Institute. The Wellcome Trust Sanger Institute is a non-profit research institute that played a central role in the human genome project. The DDD study aims to determine the clinical utility of leveraging advanced genomic technologies to diagnose patients with developmental disorders. This will be accomplished in two ways: (i) identifying novel genes and pathways for human genetic diseases and characterizing the associated phenotypes, and (ii) improving informatics and statistical methods to robustly diagnose patients with genetic conditions.

Through the DDD study, scientists at the Wellcome Trust Sanger Institute have worked alongside doctors from 24 Regional Genetics Services throughout the UK and Republic of Ireland over the past 4 years to analyze DNA from 13,500 patients with developmental disorders along with their biological parents. Through this research, so far, the DDD study has led to the discovery of 30 novel genes associated with developmental disorders.[1-3]

GeneDx, an industry leader in whole exome sequencing (WES), launched its WES program in 2011, and over the past 5 years has discovered or contributed to the phenotypic understanding of over 25 genes associated with developmental delay and/or intellectual disability[4] . With GeneDx's collaboration, the joint study cohort will expand by over 12,000 cases* presenting with developmental delay or intellectual disability. "We are thrilled to have the opportunity to collaborate with the Wellcome Trust Sanger Institute's DDD study," said Kyle Retterer, MS, Director of Data Science, GeneDx. "We expect that the combination of our cohorts and our analytical tools will help improve the ability to find statistically significant enrichment of previously undescribed disease genes, thus aiding in the diagnosis of patients from both cohorts and in the general understanding of Mendelian neurodevelopmental disorders."

Matthew Hurles, PhD, Chief Investigator of the DDD study, adds, "Pooling data globally is critical for identifying rare and ultra-rare genetic disorders. We are delighted to be collaborating with GeneDx and look forward to being able to increase substantially the proportion of patients we can diagnose confidently with known genetic disorders through combining data from over 20,000 families."

To learn more about the Wellcome Trust Sanger Institute, please visit www.sanger.ac.uk/about/who-we-are. For GeneDx's complete list of testing options, please visit our website www.genedx.com or email us at genedx@genedx.com. Follow us on Twitter @GeneDx and become a fan on Facebook @GeneDxLab to get real-time updates from us.

*GeneDx will not share individual patient-level data as part of this collaboration. Additionally, if this research results in publication, GeneDx will only include patients where explicit consent for publication has been obtained.

References:

1. Fitzgerald et al. (2015). Large-scale discovery of novel genetic causes of developmental disorders. Nature 519 (7542), 223-8, PMID: 25533962
2. Akawi et al. (2015). Discovery of four recessive developmental disorders using probabilistic genotype and phenotype matching among 4,125 families. Nature Genetics 47 (11), 1363-9, PMID: 26437029
3. McRae et al. (2017). Prevalence and architecture of de novo mutations in developmental disorders. Nature. DOI: 10.1038/nature21062
4. Retterer et al. (2015). Clinical application of whole-exome sequencing across clinical indications. Genetics in Medicine 18(7), 696-704, PMID: 26633542

About GeneDx:
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 provides testing to patients and their families in more than 55 countries. GeneDx is a business unit of BioReference Laboratories, a wholly owned subsidiary of OPKO Health, Inc.. To learn more, please visit www.genedx.com.

About OPKO Health, Inc.
OPKO Health 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, an FDA-approved treatment for SHPT in stage 3-4 CKD patients with vitamin D insufficiency (launched in November 2016), VARUBI™ for chemotherapy-induced nausea and vomiting (oral formulation launched by partner TESARO and IV formulation PDUFA date: January 2017), TT401, a once or twice weekly oxyntomodulin for type 2 diabetes and obesity which is a clinically advanced drug candidate among the new class of GLP-1 glucagon receptor dual agonists, and TT701, an androgen receptor modulator for androgen deficiency indications. Our biologics business includes hGH-CTP, a once-weekly human growth hormone injection (in phase 3 and partnered with Pfizer), a long-acting Factor VIIa drug for hemophilia (in phase 2a) and a long-acting oxyntomodulin for diabetes and obesity (in phase 1). 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.

SAFE HARBOR STATEMENT

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 the collaboration with Wellcome Trust Sanger Institute, our ability to leverage advanced genomic technologies to diagnose patients with developmental disorders by (i) identifying novel genes and pathways for human genetic diseases and characterizing the associated phenotypes, and (ii) improving informatics and statistical methods to robustly diagnose patients with genetic conditions, the ability to find statistically significant enrichment of previously undescribed disease genes, thus aiding in the diagnosis of patients from both cohorts and in the general understanding of Mendelian neurodevelopmental disorders, 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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