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OPKO Receives EU Orphan Drug Status for its New Oligonucleotide to Treat Genetic Neurological Disorder

MIAMI, March 08, 2017 (GLOBE NEWSWIRE) -- **OPKO Health, Inc.** (NASDAQ:OPK) through its subsidiaries, Eirgen Pharma Limited, Ireland and OPKO Pharmaceuticals LLC, has received notification from the European Commission designating OPKO's oligonucleotide-based AntagoNAT (CUR-1916) an orphan medicinal product under Regulation (EC) No 141/2000 for the treatment of Dravet Syndrome. An Orphan Drug Application is under review by the U.S. FDA. There is currently no approved treatment for Dravet Syndrome in the U.S.

AntagoNAT, anti-Natural Antisense Transcripts, is an OPKO platform technology in which single strand oligonucleotide molecules are designed to interfere with regulatory gene expression in order to enhance production of endogenous functional proteins. The AntagoNAT technology was part of CURNA Pharmaceuticals, acquired by OPKO in 2011, and then further developed in OPKO's Miami research laboratories under the direction of Dr. Jane Hsiao, Ph.D., OPKO's Vice Chairman and Chief Technical Officer.

OPKO has studied over 250 genes and confirmed involvement of natural antisense transcripts (NAT) in their regulatory pathways. Of those, 89 genes were demonstrated to be subject to significant upregulation of mRNA in *in vitro* screening, and 7 AntagoNAT oligonucleotides have been validated *in vivo* to date. OPKO plans to initiate a clinical trial of CUR-1916 for treatment of Dravet Syndrome this year.

It is worth noting that several oligonucleotide compounds have been reported to be in late phase clinical development and one has been approved by the FDA in 2016. They work by down regulating transcription (antisense) or by correcting gene defects.

About EMA Orphan Designation

The European Medicines Agency (EMA) grants Orphan Designation to medicines intended to treat, prevent or diagnose life threatening and debilitating diseases, with a prevalence no greater than five in 10,000 in the EU, and for which no satisfactory method of treatment, prevention or diagnosis exists, and the proposed medicine offers significant medical benefit to those with the condition. Following Orphan Designation, sponsors can access a number of incentives including market exclusivity for a ten-year period following approval, protocol assistance, and potential fee reductions.

On November 24, 2016, OPKO requested Scientific Advice for the clinical development strategy of CUR-1916, and Committee for the Medicinal Products for Human Use (CHMP) has since met with OPKO and provided its advice for the CUR-1916 clinical development program. This is an important milestone for initiating a clinical trial of CUR-1916 designed to assess drug safety and significant medical benefits to patients with Dravet Syndrome.

What is Dravet Syndrome?

Dravet Syndrome, also called severe myoclonic epilepsy of infancy (SMEI), is a severe form of epilepsy that affects children and adults. It is caused by defects in the SCN1A genes (voltage gated sodium channel) required for the proper function of brain cells.

In Dravet Syndrome, seizures begin in the first year of life, and are most often associated with elevated body temperature (febrile convulsions). Later, other types of seizures occur, including status epilepticus (seizures lasting at least 5 minutes and requiring emergency medical care). From age 2, the child's development begins to decline or reverse, and results in impaired mental and motor skills, leading to long term disability.

Dravet Syndrome is debilitating and the death rate is reported to be 10-15%.

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 400-person sales and marketing team 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 pending FDA approval), 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 (phase 2), and TT701, an androgen receptor modulator for androgen deficiency indications (phase 2). 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 (in phase 2a). We also have production and distribution assets worldwide, multiple strategic investments and an active business development strategy. More information available at www.opko.com.

Cautionary Statement Regarding 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 expectations about CUR-1916 and its effectiveness in treating Dravet syndrome, whether the drug will receive orphan designation in the U.S., whether we will commence clinical trials for CUR-1916 this year or at all, whether the data from any of our trials will support approval, validation and/or reimbursement for our products, 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. 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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