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

MIAMI, March 22, 2017 (GLOBE NEWSWIRE) -- OPKO Pharmaceuticals LLC, a subsidiary of **OPKO Health, Inc.** (NASDAQ:OPK) announces that the Company has received orphan drug designation from the U.S. Food and Drug Administration (FDA) Office of Orphan Products Development for OPKO's oligonucleotide-based AntagoNAT (CUR-1916) for the treatment of Dravet Syndrome. Currently, there is no approved treatment for Dravet Syndrome in the U.S. On March 7, 2017, OPKO Health received orphan drug designation for CUR-1916 for the treatment of Dravet Syndrome from the European Commission.

Orphan drug designation provides certain marketing exclusivity, tax credits for research and a waiver of the New Drug Application user fee.

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, part of CURNA Pharmaceuticals, acquired by OPKO in 2011, was further developed in OPKO's Miami research laboratories under the direction of 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 seven 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.

Oligonucleotides are synthetic chemical compounds consisting of mixtures of modified DNAs and RNAs. Only five oligonucleotide compounds are approved by FDA for various indications and others have been reported to be in late phase clinical development. The majority of the compounds work by down regulating, or depressing transcription (anti-sense) or by correcting gene defects. CUR-1916 works by upregulating a defective gene to increase the production of functional protein.

About FDA Orphan Drug Designation

Under the Orphan Drug Act (ODA) the FDA grants Orphan Drug status to drugs, vaccines, and diagnostic agents intended to treat a disease affecting less than 200,000 American citizens. Under the ODA, orphan drug sponsors qualify for seven-year FDA administered market Orphan Drug Exclusivity, tax credits of up to 50% of R&D costs, R&D grants, waived FDA fees, protocol assistance and may get clinical trial tax incentives.

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 also qualifies as a Rare Pediatric Disease under Section 529 of Food, Drug, and Cosmetic Act (the FD&C Act).

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

About OPKO Health

OPKO Health is a diversified healthcare company that seeks to establish industry leading positions in large, rapidly growing markets. Our diagnostics business includes BioReference 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, in phase 2 clinical trials, among the new class of GLP-1 glucagon receptor dual agonists, and TT701, a selective androgen receptor modulator for benign prostatic hyperplasia (phase 2). Our biologics business includes hGH-CTP, a once weekly human growth hormone in phase 3 and partnered with Pfizer; and a long-acting Factor VIIa drug for hemophilia in phase 2a. 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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