



Orphagen Pharmaceuticals

Innovative Drugs for Chronic Disease

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Orphagen Pharmaceuticals Awarded \$224,954 to Investigate an Orphan Nuclear Receptor as Novel Drug Discovery Target for Cancer Immunotherapy

San Diego, CA July 1, 2015 – Orphagen Pharmaceuticals, a privately-held pharmaceutical company, announced today that the National Institutes of Health (NIH) National Cancer Institute (NCI) has awarded the company \$224,954 under the Small Business Innovative Research (SBIR) program to identify small molecule antagonist ligands for an novel drug discovery target for cancer immunotherapy.

Despite a steady decline in cancer mortality over the past two decades more than half a million Americans will die of cancer in 2015. Existing therapies are often poorly tolerated and inadequate for most patients. New “cancer immunotherapies”, developed to mobilize the immune system against tumors, have produced striking results in metastatic melanoma, lung and renal cancer. Orphagen proposes to find drug-like molecules to a previously unexplored orphan drug target that normally suppresses the immune response.

“We believe there is an opportunity to identify small molecule ligands to this orphan drug target. The ligands will enhance T cell activation and thus stimulate anti-tumor responses by enhancing the ongoing immune surveillance that already occurs in cancer patients.” said Dr. Haiyan Tao, Scientist and Principal Investigator of the grant. The drug-like molecules identified from this project would create a powerful and potentially safer therapeutic tool to enhance clinical cancer immunotherapy and improve patient survival.

Orphagen is a first-mover in the identification of small molecule compounds to three orphan nuclear receptors. Support from the NIH is a critical part of the Company’s overall strategy to discover new classes of drugs and commercialize them through partnerships.

About Orphagen: Orphagen discovers drug candidates for potential drug targets from the nuclear receptor family for which small molecule ligands--potential drug-like molecules--have yet to be identified. Its goal is to identify, characterize, and position a new class of drug so that pre-clinical and clinical development can be initiated with commercial partners. Orphagen successfully partnered its first program for ROR-gamma with JT Pharma, several years ahead of competitors. Funding from this partnership and other non-dilutive sources, including grants, has advanced several other first-in-class drug discovery programs.

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