



Orphagen Pharmaceuticals

Innovative Drugs for Chronic Disease

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Orphagen Pharmaceuticals Awarded \$223,229 to Investigate Cushing's Syndrome

San Diego, CA September 1, 2014 – Orphagen Pharmaceuticals, a privately-held pharmaceutical company, announced today that the National Institutes of Health (NIH) National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) has awarded the company \$223,229 under the Small Business Innovative Research (SBIR) program to synthesize improved SF-1 antagonists and to establish *in vivo* evidence of their therapeutic potential in the treatment of Cushing's syndrome. Cushing's syndrome is a classic dysfunction of the endocrine system caused by prolonged exposure to inappropriately high levels of the steroid hormone cortisol. The condition is tumor-driven and leads to obesity, diabetes, hypertension and psychiatric dysfunction, symptoms which improve with a reduction in cortisol.

“Orphagen has identified first-in-class SF-1 antagonists that potently suppress cortisol synthesis in adrenal cells in culture,” said Paul Crowe, Director of Biology at Orphagen and Principal Investigator of the grant. “Despite availability of therapies for controlling cortisol there remains a significant unmet need for safe and effective treatment options. SF-1 antagonists discovered at Orphagen have the potential to broadly suppress adrenal steroid synthesis, avoiding side effects frequently seen with existing drugs”.

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 Japan Tobacco, 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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