



Orphagen Pharmaceuticals

Innovative Drugs for Chronic Disease

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Orphagen Pharmaceuticals Awarded Federal Grant for Adrenocortical and Prostate Cancer

San Diego, August 10, 2010 – Orphagen Pharmaceuticals announced today that the National Cancer Institute (NCI) has awarded a Phase I SBIR grant to the Company for development of a new class of small molecule drugs for the treatment of adrenocortical and prostate cancer. The objective of the studies is to demonstrate that the Company's proprietary small molecules are effective at physiological targets that will enhance therapy for these two devastating diseases. Adrenocortical cancer, a rare malignancy, affects several thousand patients globally. Prostate cancer leads to 30,000 deaths each year in the U.S. alone. The award will total \$520,000 over two years.

"Orphagen has had a long-term interest in developing a new class of therapeutic small molecules for treatment of endocrine cancers such as adrenocortical and prostate cancer. We've now reached the point of generating proof-of-principle data with these new compounds, and the NCI funding will be critical to our progress in the area. We're also extremely proud of the fact that the program has survived the rigorous peer review necessary to receive this award," said Scott Thacher, Ph.D., CEO and Founder of Orphagen.

The description of the proposed research reads in part: "Orphagen has identified antagonists to an orphan receptor that regulates the major biosynthetic steps of steroid hormone synthesis in the adrenal cortex and gonads. Safe and effective inhibition of adrenal steroid synthesis has a number of potential applications in oncology, including treatment of two rare and very poorly managed conditions. One is life-threatening, uncontrolled glucocorticoid and/or mineralocorticoid release from metastatic adrenocortical carcinoma (ACC). The other is Cushing's syndrome resulting from unresectable ACTH-secreting tumors that stimulate adrenal steroid synthesis without normal hypothalamic feedback. In addition, these novel orphan receptor antagonists are potentially of

significant value in the treatment of hormone-dependent prostate cancer as chemical castration does not block release of the adrenal androgens, DHEA and DHEA-S, which contribute significantly to the total androgen load in prostate. The novel approach of specifically antagonizing this critical orphan receptor has the potential to be both safer and more effective than mitotane, ketoconazole and aminoglutethimide, broad spectrum P450 inhibitors currently used for this purpose.”

ABOUT ORPHAGEN

Orphagen identifies and characterizes small molecule drug-like compounds to orphan nuclear receptors. Leveraging academic biological research, we use these compounds to validate the receptors as novel drug targets, establishing first-in-class discovery research programs that are highly attractive to pharmaceutical industry partners.

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