



Orphagen Pharmaceuticals

Small Molecule Discovery at Novel Drug Targets

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Orphagen Pharmaceuticals Awarded US Patent for modulators of orphan nuclear receptor ROR-gamma

San Diego, CA March 5, 2013 – Orphagen Pharmaceuticals, a privately-held pharmaceutical company, announced today that it has been awarded US patent 8,389,739 for modulators of retinoid-related orphan receptor gamma (ROR γ). The patent is Orphagen's first in the ROR γ field and describes several small molecule antagonists to ROR γ and their effect on Th-17 cell regulation. Orphagen demonstrated that ROR γ could, in fact, be regulated by small molecule ligands, establishing the receptor as a valuable new drug target for autoimmune disease.

"We are delighted to have this patent awarded in what has become a highly competitive field," said Scott Thacher, Orphagen CEO, and lead inventor on the patent. "The patent is recognition of the early technology that established Orphagen as a leader in the identification of small molecule ligands to orphan nuclear receptors. It is clear validation of Orphagen's business model to de-orphanize nuclear receptors for the development of entirely new therapeutic classes."

ROR γ is a member of the nuclear receptor superfamily of ligand-activated transcription factors, and is considered to be the "master regulator" of Th-17 cells, which were first described in 2005. In 2006, the critical involvement of ROR γ was published. The published patent demonstrates that antagonists to ROR γ , which could be administered orally, have the potential to specifically suppress Th-17 function for the targeted treatment of conditions like psoriasis and multiple sclerosis. Orphagen's ROR γ antagonists are being developed in partnership with Japan Tobacco.

Orphagen has taken an industry-leading position in the identification and characterization of drugs targeting previously unexplored orphan receptors. Orphagen's strategy is to validate new targets in cellular and animal models before securing a partner to complete development and commercialization. Orphagen has now identified the first small molecule ligands to three orphan nuclear receptors including ROR γ .

About Orphagen: Orphagen discovers drug candidates for potential drug targets 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 partners and/or outside sources of funding. These targets come from the nuclear receptor family of drug targets. On a per target basis, the nuclear receptors are one of the most successful target classes known to the pharmaceutical industry. Targets of interest to Orphagen encompass several of the so-called orphan nuclear receptors—potential therapeutic receptors that have yet to be exploited by the pharmaceutical industry.

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