



ORPHAGEN PHARMACEUTICALS

-----*SMALL MOLECULE DISCOVERY AT NOVEL DRUG TARGETS*-----

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Orphagen Pharmaceuticals Awarded Additional US Patent for Modulators of Orphan Nuclear Receptor ROR-gamma

San Diego, June 1, 2018 – Orphagen Pharmaceuticals, a privately held pharmaceutical company, announced today that it has been awarded US patent 9,657,053 for the use of small molecule ligands to inhibit retinoid-related orphan receptor gamma (ROR γ). The patent describes methods for inhibiting ROR γ , now recognized as a potentially important target for treatment of autoimmune disease. Inhibition of RORg has numerous effects, including suppression of a class of T cells known as Th-17 cells that appear to have a role in psoriasis and other autoimmune diseases.

“We are delighted that another patent has been awarded to Orphagen regarding this very important receptor target,” said Scott Thacher, Orphagen’s President and CEO, and lead inventor on the patent. “The patent is based on the pioneering work that Orphagen undertook to explore, understand, and develop small molecules that target ROR γ . This work created a foundation for discovery of new small molecule drug candidates for inflammation and autoimmune disorders.”

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 describes several classes of small molecule antagonists to ROR γ , which could be administered orally for the targeted treatment of conditions including autoimmune and allergic diseases.

About Orphagen: Orphagen has a successful track record of identifying small molecules that modulate unexplored or “orphan” members of the nuclear receptor family. In many cases, including for RORg, Orphagen has been a first mover in ligand identification and characterization in cellular and animal models for these unexplored targets. In addition to this and other collaborations for new drug discovery with pharmaceutical partners, Orphagen is also developing its own breakthrough therapies for cancer and inflammatory diseases.

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