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United Therapeutics Announces Collaboration With Ascendis Pharma To Develop Self-Injectable Treprostinil For Pulmonary Arterial Hypertension

SILVER SPRING, Md., Sept. 20, 2012 /PRNewswire/ -- United Therapeutics Corporation (NASDAQ: UTHR) announced today that it has signed an exclusive agreement with Ascendis Pharma A/S to apply Ascendis Pharma's proprietary TransCon technology platform to United Therapeutics' treprostinil molecule, the active ingredient in Remodulin® (treprostinil) injection. United Therapeutics believes that the TransCon technology platform may enable a controlled, long-acting release of a novel, carrier-linked product, significantly enhancing the delivery profile of treprostinil by establishing a self-injectable alternative for patients who currently use the drug via a continuous infusion pump for the treatment of pulmonary arterial hypertension (PAH).

"We are thrilled to enter into this license agreement with Ascendis Pharma," said Martine Rothblatt, Ph.D., United Therapeutics' Chairman and Chief Executive Officer. "The potential to bring another novel therapeutic option to PAH patients represents an exciting new opportunity for Remodulin delivery as we constantly re-charge our mission to better the lives of patients suffering from PAH."

"Pre-clinical studies in multiple animal models with a lead compound of TransCon-linked treprostinil have already produced encouraging results, demonstrating a sustained release profile supportive of once-daily injection. The application of TransCon technology to treprostinil may also minimize injection site pain and reactions that frequently occur with continuously infused subcutaneous Remodulin," commented Roger Jeffs, Ph.D., United Therapeutics' President and Chief Operating Officer. "We are very excited to be working with Ascendis Pharma to further develop and advance this promising product into the clinic."

Under the terms of the agreement, United Therapeutics will also have exclusive rights to develop prostacyclin, prostacyclin analog and prostacyclin-related products for treatment of PAH using the TransCon technology, and will hold worldwide commercial rights to products resulting from the collaboration.

About United Therapeutics

United Therapeutics Corporation is a biotechnology company focused on the development and commercialization of unique products to address the unmet medical needs of patients with chronic and life-threatening conditions.

About Ascendis Pharma and TransCon

Ascendis Pharma A/S is an emerging specialty pharmaceutical company which creates improved, patentable versions of marketed drugs. The company is headquartered in Palo Alto, California and Copenhagen, Denmark, with research in Heidelberg, Germany.

Ascendis Pharma's prodrug technology platform, TransCon, is a proprietary technology enabling transient conjugation of peptides, proteins and small molecules to various carriers. The technology utilizes Ascendis Pharma's proprietary TransCon Linker families, which are a diverse group of structures with inherent self-cleaving properties, making drug release independent of enzyme activity and tissue conditions. Ascendis Pharma's transient conjugation is designed to release drug in the body in a precise, time-controlled fashion, thus creating a long-acting effect for the original unmodified drug.

About Remodulin (treprostinil) Injection

Indication

Remodulin is a prostacyclin vasodilator indicated in the United States for the treatment of pulmonary arterial hypertension (PAH) (WHO Group 1) to diminish symptoms associated with exercise. Studies establishing effectiveness included patients with NYHA Functional Class II-IV symptoms and etiologies of idiopathic or heritable PAH (58%), PAH associated with congenital systemic-to-pulmonary shunts (23%), or PAH associated with connective tissue diseases (19%). Remodulin may be administered as a continuous subcutaneous infusion or continuous intravenous infusion; however, because of the risks associated with chronic indwelling central venous catheters, including serious blood stream infections, continuous intravenous infusion should be reserved for patients who are intolerant of the subcutaneous route, or in whom these risks are considered warranted.

In patients with PAH requiring transition from Flolan® (epoprostenol sodium), Remodulin is indicated to diminish the rate of clinical deterioration. The risks and benefits of each drug should be carefully considered prior to transition.

Important Safety Information

Chronic intravenous infusions of Remodulin are delivered using an indwelling central venous catheter. This route is associated with the risk of blood stream infections, or BSI, and sepsis, which may be fatal. Therefore, continuous subcutaneous infusion is the preferred mode of administration.

Remodulin should be used only by clinicians experienced in the diagnosis and treatment of PAH. Remodulin is a potent pulmonary and systemic vasodilator. It lowers blood pressure, which may be further lowered by other drugs that also reduce blood pressure. Remodulin inhibits platelet aggregation and therefore, may increase the risk of bleeding, particularly in patients on anticoagulants. Remodulin dosage adjustment may be necessary if inhibitors or inducers of CYP2C8 are added or withdrawn. Initiation of Remodulin must be performed in a setting with adequate personnel and equipment for physiological monitoring and emergency care. Therapy with Remodulin may be used for prolonged periods, and the patient's ability to administer Remodulin and care for an infusion system should be carefully considered.

Remodulin dosage should be increased for lack of improvement in, or worsening of, symptoms and it should be decreased for excessive pharmacologic effects or for unacceptable infusion site symptoms.

Abrupt withdrawal or sudden large reductions in dosage of Remodulin may result in worsening of PAH symptoms and should be avoided. Caution should be used in patients with hepatic or renal insufficiency.

The most common side effects of Remodulin included those related to the method of infusion. For subcutaneous infusion, infusion site pain and infusion site reaction (redness and swelling) occurred in the majority of patients. These symptoms were often severe and could lead to treatment with narcotics or discontinuation of Remodulin. For intravenous infusion, line infections, sepsis, arm swelling, tingling sensations, bruising, and pain were most common. General side effects (> 5% more than placebo) were diarrhea, jaw pain, vasodilatation and edema.

For full prescribing information for Remodulin in the United States, visit <http://www.remodulin.com/images/pdf/PI.pdf>, or call 1-877-864-8437.

Forward-looking Statements

Statements included in this press release that are not historical in nature are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include, among others, statements regarding future commercial and development plans for treprostinil using Ascendis Pharma's TransCon technology and the potential benefits to patients. These forward-looking statements are subject to certain risks and uncertainties and are qualified by the cautionary statements, cautionary language and risk factors set forth in our periodic reports and documents filed with the Securities and Exchange Commission, including our most recent Annual Report on Form 10-K, Quarterly Reports on Form 10-Q and current reports on Form 8-K, which could cause actual results to differ materially from anticipated results. These risks and uncertainties include, among others, the outcome of required pre-clinical and clinical studies. We are providing this information as of September 20, 2012, and assume no obligation to update or revise the information contained in this press release whether as a result of new information, future events or any other reason. [uthr-g]

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