FREEDOM-C(2) Trial of Oral Treprostinil in Pulmonary Arterial Hypertension Does Not Meet Primary Endpoint

Conference Call to be Held at 9:00 a.m. Eastern Time on August 24, 2011

SILVER SPRING, Md., Aug. 24, 2011 /PRNewswire/ -- United Therapeutics Corporation (NASDAQ: UTHR) announced today the completion of its FREEDOM-C(2) Phase 3 trial of treprostinil diethanolamine (oral treprostinil), an investigational sustained release oral formulation of treprostinil, a stable synthetic form of prostacyclin, in patients with pulmonary arterial hypertension (PAH). Preliminary analysis demonstrates that the trial did not achieve statistical significance for the primary endpoint, six-minute walk distance (6MWD) at Week 16.

The FREEDOM-C(2) trial was a randomized, double-blind, placebo-controlled trial of patients with PAH, a chronic, life-threatening illness. The study population consisted of 310 patients who were optimized on an endothelin receptor antagonist, a phosphodiesterase-5 inhibitor, or both. In addition to one of these oral therapy regimens, patients were administered oral treprostinil or placebo twice daily with the dose titrated (increased to tolerability) over the 16-week trial. The majority (~73%) of patients were World Health Organization (WHO) Class III patients of varied etiologies, including idiopathic or familial PAH (~65%), collagen vascular disease associated PAH (~31%), and PAH associated with HIV or other associated conditions (~3%). Mean baseline walk distance was approximately 333 meters.

The primary efficacy endpoint of the trial was the median change in 6MWD at 16 weeks relative to baseline. With regard to the primary efficacy results, the placebo-corrected median change in 6MWD at Week 16 was 10 meters (p=0.089, Hodges-Lehmann estimate and non-parametric analysis of covariance in accordance with the trial's pre-specified statistical analysis plan).

Overall, 132 (84%) oral treprostinil and 138 (90%) placebo patients completed Week 16 with an average dose of 3.0 plus or minus 1.9 mg twice daily in the oral treprostinil group. Discontinuations due to adverse events were 18 (11%) and 5 (3%) in the treprostinil and placebo groups, respectively. Adverse events associated with discontinuations were typically prostacyclin-related effects including headache, nausea and vomiting.

Preliminary analysis of other secondary efficacy measures, including time to clinical worsening, change in combined 6MWD and Borg Dyspnea Score rating (shortness of breath test) and Dyspnea-Fatigue index, WHO functional class, and PAH signs and symptoms, did not differ significantly between oral treprostinil and placebo (p>0.05).

Further review and analysis of the FREEDOM-C(2) preliminary results are ongoing. A summary of the preliminary analysis conducted thus far can be accessed via United Therapeutics' website at http://ir.unither.com/events.cfm beginning at 5:00 a.m. Eastern Time on August 24, 2011. Full data from FREEDOM-C(2) will be presented at an upcoming medical meeting and will also be available through the publication of peer-reviewed journal articles.

"While we did not achieve a statistically significant result for this trial, we believe the positive results from our previously-announced FREEDOM-M study support an NDA filing of oral treprostinil in treatment naive patients," said Roger Jeffs, Ph.D., United Therapeutics' President and Chief Operating Officer. "Given that treprostinil is already approved for use in PAH by subcutaneous, intravenous and inhaled routes of administration, we believe the data obtained from all FREEDOM trials support an NDA for oral treprostinil in this indication," added Dr. Jeffs. "We will now focus on completing the NDA for filing by the first half of 2012."

Conference Call

On Wednesday, August 24, 2011 at 9:00 a.m. United Therapeutics will host a one-hour conference call to answer questions related to FREEDOM-C(2).

The conference call is accessible by dialing 1-877-351-5881, with international callers dialing 1-970-315-0533. A rebroadcast of the conference call will be available for two weeks and can be accessed by dialing 1-855-859-2056, with international callers dialing 1-404-537-3406, and using conference code: 94755163.

This conference call is also being webcast and can be accessed via our website at http://ir.unither.com/events.cfm.

About United Therapeutics Corporation

United Therapeutics 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. [uthr-g]

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, our expectations regarding our pursuit and potential success in obtaining regulatory approvals for oral treprostinil, including our filing for regulatory approval in 2012 and that full data from the trial will be presented at an upcoming medical meeting and will also be available in peer-reviewed journal articles. These forward-looking statements are subject to certain risks and uncertainties, such as those described in our periodic and other reports filed with the Securities and Exchange Commission, that could cause actual results to differ materially from anticipated results. These risks and uncertainties include, among others, the failure of oral treprostinil to receive regulatory approval at all, or on the schedule expected; the possible inaccuracies of our analysis with respect to the FREEDOM-C(2) preliminary trial results and market opportunity; and our or our suppliers' inability to manufacture oral treprostinil in accordance with all applicable regulatory requirements and in sufficient quantity to support patient demand. Consequently, such forward-looking statements 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. We claim the protection of the safe harbor contained in the Private Securities Litigation Reform Act of 1995 for forward-looking statements. We are providing this information as of August 24, 2011, 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.

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