



Osiris Resumes Enrollment in Stem Cell Trial for Crohn's Disease Following Positive Interim Analysis

COLUMBIA, Md., May 05, 2010 (BUSINESS WIRE) -- [Osiris Therapeutics, Inc.](#) (NASDAQ:OSIR) today announced that it is resuming enrollment in its clinical trial evaluating Prochymal for patients with treatment-resistant Crohn's disease. The findings of an interim analysis showed that for the primary endpoint of disease remission, Prochymal is approaching statistical significance in the intent to treat (ITT) population, and has reached significance in the per protocol population. Additionally, the analysis showed that Prochymal continued to demonstrate a benign safety profile with no significant differences in any of the pre-defined safety outcomes compared to placebo.

The Crohn's program consisted of two linked trials - one aimed at inducing remission (Protocol 603) and the other at maintaining response (Protocol 610) in patients who had failed other available treatments for the disease. Enrollment was suspended in 2009 over concerns the trial design would make it difficult to detect a treatment effect. The trial remained blinded, however, to permit an interim analysis of all 207 patients enrolled in the study. The results showed that despite the initial concerns, the effect size, or difference between the Prochymal and placebo response rates, of one dose arm of Prochymal is consistent with the original statistical assumptions of the protocol and is significantly outperforming placebo.

The decision to resume enrollment was made following discussions with the Food and Drug Administration (FDA) about the results of the interim analysis. Enrollment will now continue with the best-performing Prochymal dose arm and the placebo arm, according to the pre-specified adaptive trial design. The trial has been repowered to compensate for the statistical penalty incurred by the interim analysis in the ITT population. The follow-on maintenance trial (Protocol 610) has been discontinued to remove the potential for bias.

"We are encouraged by the robust results for the primary endpoint of disease remission from the 207 patients evaluated thus far," said Douglas Jacobstein, M.D., Pediatric Gastroenterologist and Medical Director of the Crohn's program at Osiris Therapeutics. "We will have a better understanding of the benefits Prochymal may offer to patients with treatment-resistant Crohn's disease after enrollment has been completed and the full data set has been thoroughly analyzed. Our goal is to use the data from this well-controlled trial to appropriately design and power an efficient pivotal program to support product approval for this devastating disease."

The trial is evaluating patients with severe Crohn's disease that is not responsive to treatment with steroids, immunomodulators (azathioprine, 6-mercaptopurine, and methotrexate), and biologic agents (Remicade(R), Humira(R), and Cimzia(R)). The majority of patients enrolled thus far have failed at least two immunomodulators and two biological agents. Patients are required to complete a "washout" period prior to study entry to ensure that residual benefit from prior therapy does not influence their response. There were no differences in average Crohn's Disease Activity Index (CDAI) scores at entry, which exceeded 350 in all arms prior to treatment, indicating the patients had very debilitating disease.

The primary endpoint for Protocol 603 is the proportion of patients experiencing disease remission within 28 days of initiation of therapy with Prochymal, compared to those patients receiving placebo. For a patient to reach the primary end-point of disease remission, their CDAI score must fall to below 150.

About Prochymal

Prochymal is a preparation of mesenchymal stem cells (MSCs) formulated for intravenous infusion. The MSCs utilized in Prochymal are isolated from the bone marrow of healthy young adult donors, avoiding the controversy surrounding embryonic and fetal cell sources. They are grown in culture, permitting large-scale production. Because the cells can be expanded, thousands of doses can be produced from a single donation. Studies suggest MSCs are able to safely facilitate tissue repair through a number of mechanisms. Specifically, these studies indicate that MSCs are able to down-regulate severe inflammation and work at the cellular level to rebuild damaged tissue through the coordinated release of tissue-specific growth factors.

Prochymal is being evaluated in clinical programs for graft versus host disease (GvHD), Crohn's disease, acute myocardial infarction, pulmonary disease and type 1 diabetes. Prochymal has been granted Fast Track status by the FDA for GvHD and Crohn's disease, and is the first stem cell product to receive FDA expanded access approval, making the product available now to patients with life-threatening GvHD. Prochymal also obtained Orphan Drug status for GvHD and type 1 diabetes from the FDA, as well as Orphan Drug status for GvHD from the European Medicines Agency.

About Osiris Therapeutics

Osiris Therapeutics, Inc. is the leading stem cell therapeutic company focused on developing products to treat serious medical conditions in the inflammatory, autoimmune, orthopedic and cardiovascular areas. The Company's pipeline of internally developed biologic drug candidates under evaluation includes Prochymal for inflammatory, autoimmune and cardiovascular indications, as well as Chondrogen for arthritis in the knee. Osiris is a fully integrated company, with capabilities in research, development, manufacturing and distribution of stem cell products. Osiris has developed an extensive intellectual property portfolio to protect the company's technology, including 49 U.S. patents each having one or more foreign counterparts. Osiris, Prochymal and Chondrogen are registered trademarks of Osiris Therapeutics, Inc. More information can be found on the company's website, www.Osiris.com. (OSIR-G)

Osiris and Genzyme formed a strategic alliance for the development and commercialization of Prochymal and Chondrogen. Under the terms of the agreement, Osiris retains commercialization rights to Prochymal and Chondrogen in the United States and Canada. Genzyme holds these rights in all other countries except Japan, where JCR Pharmaceuticals holds rights to Prochymal for the treatment of patients with hematological malignancies.

Remicade(R) is a registered trademark of Centocor, Inc., Cimzia(R) is a registered trademark of the UCB Group of Companies, and Humira(R) is a registered trademark of Abbott Laboratories.

Forward-Looking Statements

This press release contains forward-looking statements. Forward-looking statements include statements about our expectations, beliefs, plans, objectives, intentions, assumptions and other statements that are not historical facts. Words or phrases such as "anticipate," "believe," "continue," "ongoing," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project" or similar words or phrases, or the negatives of those words or phrases, may identify forward-looking statements, but the absence of these words does not necessarily mean that a statement is not forward-looking. Examples of forward-looking statements include, but are not limited to, statements regarding the following: our product development efforts; our clinical trials and anticipated regulatory requirements and the ability to successfully navigate these requirements; the success of our product candidates in development; status of the regulatory process for our biologic drug candidates; implementation of our corporate strategy; our financial performance; our product research and development activities and projected expenditures, including our anticipated timeline and clinical strategy for Prochymal, Chondrogen and our other MSC and biologic drug candidates; our cash needs; patents and proprietary rights; the safety and ability of our potential products to treat disease and the results of our scientific research; our plans for sales and marketing; our plans regarding our facilities; types of regulatory frameworks we expect will be applicable to our potential products; and results of our scientific research. Forward-looking statements are subject to known and unknown risks and uncertainties and are based on potentially inaccurate assumptions that could cause actual results to differ materially from those expected or implied by the forward-looking statements. Risks and uncertainties related to our Collaboration Agreement with Genzyme for the development and commercialization of Prochymal and Chondrogen include, among others: typical business transactional risks; risks related to product development and clinical trial design, performance and completion; uncertainty of the success of Prochymal and Chondrogen in clinical trials and their ability to treat disease; Genzyme's early termination and opt-out rights; the ability of Osiris and Genzyme to successfully navigate regulatory requirements and to manufacture and commercialize products; and the uncertainty as to our ability to successfully perform under the collaborative arrangement and earn milestone and royalty payments thereunder. Our actual results could differ materially from those anticipated in forward-looking statements for many reasons, including the factors described in the section entitled "Risk Factors" in our Annual Report on Form 10-K and other Periodic Reports filed on Form 10-Q, with the United States Securities and Exchange Commission. Accordingly, you should not unduly rely on these forward-looking statements. We undertake no obligation to publicly revise any forward-looking statement to reflect circumstances or events after the date of this press release or to reflect the occurrence of unanticipated events.

SOURCE: Osiris Therapeutics, Inc.

Osiris Therapeutics, Inc.
Erica Elchin, 443-545-1834
OsirisPR@Osiris.com

or

Media Contacts:
Schwartz Communications
Andrew Law/Rachel Gross
781-684-0770
Osiris@schwartz-pr.com

Copyright Business Wire 2010