



Osiris Therapeutics Announces Preliminary Results for Prochymal Phase III GvHD Trials

COLUMBIA, Md., Sep 08, 2009 (BUSINESS WIRE) -- [Osiris Therapeutics, Inc.](http://www.osiris.com) (NASDAQ: OSIR) today announced preliminary results for two phase III trials evaluating Prochymal for the treatment of acute graft versus host disease (GvHD). GvHD, the most common complication of bone marrow transplantation, is a life-threatening disease for which there is currently no approved treatment. Prochymal showed significant improvements in response rates in difficult-to-treat liver and gastrointestinal GvHD, however neither trial reached its primary endpoint.

Key findings from the GvHD trials include:

- There was no statistical difference between Prochymal and placebo on the primary endpoints for either the steroid-refractory (35% vs. 30%, n=260) or the first-line (45% vs. 46%, n=192) GvHD trials.
- The primary endpoint for the steroid-refractory GvHD trial (durable complete response) for the per-protocol population approached statistical significance (40% vs. 28%, p=0.087, n=179).
- In patients with steroid-refractory liver GvHD, treatment with Prochymal significantly improved response (76% vs. 47%, p=0.026, n=61) and durable complete response (29% vs. 5%, p=0.046).
- Prochymal significantly improved response rates in patients with steroid-refractory gastrointestinal GvHD (88% vs. 64%, p=0.018, n=71).
- In pediatric patients, Prochymal showed a strong trend of improvement in response rates (86% vs. 57%, p=0.094, n=28).

"These data are still preliminary and further analysis is needed to gain a full appreciation of the results of these rigorous, double-blind, placebo-controlled trials," said C. Randal Mills, Ph.D., President and Chief Executive Officer of Osiris Therapeutics. "We are encouraged to see Prochymal significantly improve response rates above standard of care in GvHD patients who currently have no good treatment options. We will meet with the FDA as soon as possible to discuss the most appropriate and efficient path forward for Prochymal in this life-threatening indication."

Protocol 265 was designed to evaluate Prochymal as a first-line agent for the treatment of acute GvHD in combination with steroid therapy. The majority of patients in this trial were suffering from skin GvHD, which responded significantly better to steroids than had been previously reported in controlled trials. This high response rate to standard of care diminished the potential for Prochymal to demonstrate an effect.

In the more severe, steroid-refractory GvHD setting (protocol 280), the benefit of adding Prochymal to second-line therapy was evaluated. Prochymal approached statistical significance for the primary endpoint in the per-protocol patient population which is the group of patients that met all of the study protocol requirements, such as inclusion and exclusion criteria. Additionally, Prochymal significantly improved response rates to liver and gastrointestinal GvHD, for which there is currently no known reliable therapy. Notably, the Prochymal cohort had more severe GvHD (27% of Prochymal patients had grade D GvHD, the most severe form, vs. 16% of placebo patients, p=0.05).

Based upon the results of the steroid-refractory GvHD trial, Osiris plans to file an amendment with the FDA to the current expanded access program, broadening the entry criteria to include patients with severe GvHD of the liver.

Webcast and Conference Call

The Company has scheduled a webcast and conference call to discuss the GvHD clinical program today, September 8, 2009, at 9:00 AM ET. To access the webcast, visit the Investor Relations section of the company's website at <http://investor.osiris.com/events.cfm>. Alternatively, callers may participate in the conference call by dialing (888) 352-6798 (U.S. participants) or (719) 325-2376 (international participants).

A replay of the conference call will be available approximately two hours after the completion of the call through September 15, 2009. Callers can access the replay by dialing (888) 203-1112 (U.S. participants) or (719) 457-0820 (international participants). The audio replay passcode is 8379243. To access a replay of the webcast, visit the Investor Relations section of the company's website at <http://investor.osiris.com/events.cfm>.

About the Phase III Acute Steroid-Refractory Trial (Protocol 280)

The Phase III trial evaluated the safety and efficacy of Prochymal in conjunction with standard of care for treatment of patients who had failed to respond to corticosteroid treatment for acute GvHD. The clinical trial is a double-blind, placebo-controlled study. Patients were randomized to either Prochymal or placebo at a 2:1 ratio. GvHD assessments performed according to the International Bone Marrow Transplant Registry (IBMTR) were used in the trial to detect improvements in subjects treated with Prochymal. The primary endpoint for this trial is durable complete response defined as complete resolution of GvHD for a duration of at least 28 days. The trial enrolled 260 patients from 72 leading bone-marrow transplant centers across the United States, Canada, Europe and Australia.

About the Phase III Acute GvHD Trial (Protocol 265)

The Phase III double-blind, placebo controlled trial evaluated the safety and efficacy of Prochymal in conjunction with steroid therapy in patients with newly diagnosed acute GvHD, grades B-D. The primary endpoint of the trial is the proportion of patients surviving at least 90 days that achieve a complete response when Prochymal is added to steroid therapy as compared to those receiving steroids alone. Patients are considered treatment failures if they do not achieve a complete response within 28 days of initiating treatment, if the steroid dose is increased or other immunosuppressive agents are added, or if the patient does not survive 90 days following initial treatment. The trial enrolled 192 patients from 52 leading transplant centers across the United States, Canada and Australia.

About GvHD

GvHD represents a major unmet medical need with no approved treatment. It is the most common complication of allogeneic (using cells from a family member, unrelated donor or cord blood unit) hematopoietic stem cell transplantation (HSCT). GvHD occurs when immune cells from the donated cell population (the graft) attack the transplanted patient's body cells (the host) because the recipient cells are seen as "foreign." Organs that are mainly affected by the immunological attack are the gastrointestinal tract, skin, and liver.

Acute GvHD is a potentially life-threatening complication that arises in approximately 50% of all patients who receive an allogeneic HSCT. Severe acute steroid-refractory GvHD is fatal in up to 90 percent of cases. Current treatments are marginally effective with significant side effects.

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 have indicated 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 three, double-blind, placebo-controlled Phase III studies, including steroid-refractory GvHD, acute GvHD and Crohn's disease. 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 children with life-threatening GvHD. Prochymal also obtained Orphan Drug status by FDA and the European Medicines Agency for GvHD. Prochymal is also being studied in Phase II trials for the treatment of acute myocardial infarction, COPD and type 1 diabetes.

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, 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)

In November 2008, Osiris and Genzyme announced 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, with Genzyme having these rights in all other countries.

Safe Harbor Statement

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.

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