



## **Osiris Therapeutics Receives FDA Orphan Drug Designation for Stem Cell Treatment for Type 1 Diabetes**

COLUMBIA, Md., May 04, 2010 (BUSINESS WIRE) -- [Osiris Therapeutics, Inc.](#) (NASDAQ:OSIR) today announced that it has been granted Orphan Drug designation from the U.S. Food and Drug Administration (FDA) for Prochymal as a treatment for type 1 diabetes mellitus. The FDA instituted the Orphan Drug Act to promote the development of treatments for underserved patient populations. To be eligible for Orphan Drug designation, the treatment must target a disease that affects fewer than 200,000 new patients per year in the United States.

Orphan Drug designation provides market exclusivity for up to seven years following approval, eliminates FDA filing and registration fees, and provides tax incentives for as much as 50 percent of the clinical development costs. Osiris also holds Orphan Drug designation for Prochymal used in the treatment of graft versus host disease (GvHD).

Prochymal, a formulation of adult mesenchymal stem cells (MSC) designed to provide therapeutic benefit by controlling inflammation, promoting tissue regeneration, and preventing scar formation, is currently being evaluated in a double-blind, placebo-controlled Phase II clinical trial for type 1 diabetes. Osiris is developing Prochymal in partnership with the Juvenile Diabetes Research Foundation (JDRF) as a treatment for patients with newly diagnosed type 1 diabetes mellitus.

In type 1 diabetes, the patient's own immune system attacks and destroys insulin-producing islet cells in the pancreas, resulting in the loss of blood-sugar control. Currently, there are no approved treatments for altering the rate of destruction of these critical islet cells, called beta cells. Preclinical studies first conducted by researchers at Genzyme found that MSCs may delay the progression of type 1 diabetes by preserving beta cell function.

### **About the Phase II Type 1 Diabetes Trial**

The Phase II trial is evaluating the safety and efficacy of Prochymal in preserving insulin production in patients 12 to 35 years old recently diagnosed with type 1 diabetes. To be eligible, candidates must have been diagnosed two to twenty weeks prior to participation in the study. The 62 patient, double-blind, placebo-controlled trial is being conducted at 20 leading medical centers across the United States.

### **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 Phase III programs for graft versus host disease (GvHD) and Crohn's disease, as well as Phase II programs for the treatment of 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 Juvenile Diabetes Research Foundation**

JDRF is a leader in setting the agenda for diabetes research worldwide, and is the largest charitable funder and advocate of type 1 research. The mission of JDRF is to find a cure for diabetes and its complications through the support of research. Type 1 diabetes is a disease which strikes children and adults suddenly and requires multiple injections of insulin daily or a continuous infusion of insulin through a pump. Insulin, however, is not a cure for diabetes, nor does it prevent its eventual and devastating complications which may include kidney failure, blindness, heart disease, stroke, and amputation.

Since its founding in 1970 by parents of children with type 1 diabetes, JDRF has awarded more than \$1.3 billion to diabetes research, including more than \$100 million in FY2009.

## 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](http://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.

## 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.

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