



## Osiris and FDA Reach Agreement on Submission of First Marketing Application for a Stem Cell Product

**COLUMBIA, Maryland – January 15, 2009** – Osiris Therapeutics, Inc. (NASDAQ:OSIR) announced today that it has held a successful pre-Biologics License Application (pre-BLA) meeting for Prochymal used in the treatment of graft-versus-host disease (GvHD). A BLA is a comprehensive regulatory submission prepared by a biologic drug's sponsor to obtain full marketing approval from the Food and Drug Administration (FDA). As an outcome of the meeting between Osiris and FDA, the parties finalized the content and timing of what is expected to be the first BLA submission for a stem cell product.

"Agreement on the timing and content of the BLA marks an important milestone in our quest to make Prochymal the world's first fully approved stem cell therapy," said C. Randal Mills, Ph.D., President and Chief Executive Officer of Osiris. "We thank FDA for their significant efforts over the past several years as we have forged a highly productive partnership in this new and exciting area of medicine. Having proactively addressed issues critical to a successful review, we have created a clear understanding of the regulatory path to market approval."

A pre-BLA meeting is a mechanism by which FDA and the sponsor can discuss the application in advance of submission to avoid potential pitfalls in review process. In the Prochymal pre-BLA meeting there were three main areas of discussion. The first was the type and extent of safety and efficacy data that would be included. The second was manufacturing and quality data that would comprise the submission. The third was the process and format of the submission itself. Prior to the meeting, Osiris submitted a comprehensive outline of the proposed content and structure of the BLA.

Key results of the pre-BLA meeting are below:

- FDA agreed with the proposed content and structure of the BLA for Prochymal for the treatment of GvHD.
- The primary endpoint of the Phase III trial that will be reviewed by FDA for approval was confirmed to be GvHD Complete Response (CR). A CR is complete remission of the disease.
- FDA indicated that the statistical analysis plan was appropriate.
- The overall safety database (Integrated Summary of Safety) of Prochymal is sufficient in scope for the indication. No additional clinical data beyond what was presented is anticipated to be necessary for the BLA.
- Agreement was reached on BLA requirements for Chemistry, Manufacturing and Controls (CMC) data.
- The FDA concurred with the proposed product stability and process validation plan for submission and provided guidance on how to present the data for ease of review.
- Agreement was reached on the timing and content of the BLA in rolling submission format. The rolling submission is an FDA provision available to drug candidates that have received Fast Track designation, which allows for completed sections of a BLA to be submitted on an ongoing basis. It can facilitate the process by allowing FDA to complete review of sections as soon as they are available. It is anticipated that the nonclinical sections, such as toxicology, will be the first submitted for review.
- The BLA will be in the electronic Common Technical Document (eCTD) format. The eCTD format facilitates the review of the BLA and allows parallel submission of the dossier in other territories.
- At the time of submission, Osiris will also submit the request for Priority Review of the Prochymal BLA. Investigational drugs with Fast Track designation are eligible for consideration for Priority Review, which provides for an accelerated six month application review by FDA.

Osiris and FDA also discussed the need for an Expanded Access Program (EAP) to make Prochymal available to critically ill steroid-refractory adult GvHD patients in the interim period until a final regulatory decision is reached. Osiris currently has an EAP in place for pediatric GvHD patients in the US and Canada. Osiris committed to work with FDA to determine the details of the new EAP.



In November 2008, Osiris and Genzyme announced a strategic alliance for the development and commercialization of Prochymal. Under the terms of the agreement, Osiris will commercialize Prochymal in the United States and Canada, and Genzyme will commercialize the treatment in all other countries.

### **About Prochymal**

Prochymal is a preparation of mesenchymal stem cells specially formulated for intravenous infusion. The stem cells are obtained from the bone marrow of healthy adult donors. Prochymal is currently being evaluated in Phase III trials for steroid refractory GvHD, acute GvHD, and Crohn's disease. Prochymal has been granted Fast Track status by FDA for all three of these indications. Prochymal also obtained Orphan Drug status by FDA and the European Medicines Agency for GvHD. Prochymal is being studied in Phase II trials for the treatment of COPD, type 1 diabetes, and acute myocardial infarction. Additionally, the Department of Defense recently awarded Osiris a contract to develop Prochymal as a treatment for acute radiation syndrome.

### **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 a partnership with Genzyme Corp. for the development and commercialization of Prochymal and Chondrogen in countries outside the United States and Canada. Osiris has developed an extensive intellectual property portfolio to protect the company's technology including 47 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)

### **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 the Collaboration Agreement with Genzyme 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 the ability of the parties to successfully perform under the collaborative arrangement and for Osiris to 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 Quarterly 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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