# Onyx Pharmaceuticals and Multiple Myeloma Research Foundation Lau Carfilzomib Expanded Access Program

PRNewswire

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SOUTH SAN FRANCISCO, Calif. and NORWALK, Conn., Aug. 3, 2011 /PRNewswire/ -- Onyx Pharmaceuticals, Inc. (Nasdaq:ONXX - News) and the Multiple Myeloma Research Foundation (MMRF) today announced the initiation of an expanded access program for carfilzomib a selective, next-generation proteasome inhibitor. The Carfilzomib Myeloma Access Program (C-MAP) will include eligible patients in the United States (U.S.) with relapsed and refractory multiple myeloma. Expanded access programs make investigational medicines still being evaluated in clinical trials available to patients for whom no satisfactory treatment alternatives are available. The C-MAP protocol has been reviewed by the U.S. Food and Drug Administration (FDA) and the company has received authorization to initiate the study.

"In recognition of the immediate needs of patients with relapsed and refractory multiple myeloma who have limited treatment options available to them, Onyx and the MMRF are offering this expanded access program with the hope of potentially helping patients who may benefit from carfilzomib prior to FDA approval in the U.S.," said N. Anthony Coles, M.D., President and Chief Executive Officer of Onyx Pharmaceuticals. "Onyx plans to submit a New Drug Application (NDA) for accelerated approval of carfilzomib with the goal of making this promising new therapy commercially available as quickly as possible."

"New approaches to treating multiple myeloma are needed since nearly all patients will eventually relapse and no longer respond to currently available therapies," said Kathy Giusti, Founder and Chief Executive Officer of the MMRF and a multiple myeloma patient. "The MMRF has established the clinical platform necessary for this innovative program to support multiple myeloma patients and their healthcare providers at a critical point in their care. Through this collaboration, the MMRF and Onyx have developed the first expanded access program for patients with advanced multiple myeloma in nearly six years. By making carfilzomib available through C-MAP, we believe we are addressing a critical need in the treatment of patients with relapsed and refractory multiple myeloma."

Under C-MAP, approximately 40 medical centers across the U.S. will enroll patients, including institutions and investigators who have previous experience administering carfilzomib in clinical trials, member institutions of the Multiple Myeloma Research Consortium (MMRC) and newly identified institutions. Clinical sites are expected to open beginning this month through the end of 2011. Onyx and MMRF are making every effort to ensure geographic balance of sites to provide as convenient access as possible to patients.

**About the Carfilzomib Myeloma Access Program**

C-MAP is a multi-center study of carfilzomib for patients with relapsed and refractory multiple myeloma. The trial is a single-arm study, meaning carfilzomib is not being compared to another anti-cancer therapy. The study is designed to provide access to patients who have progressive disease, are refractory to at least one prior therapy, have received at least four prior therapies for multiple myeloma and are not eligible for any other company-sponsored carfilzomib trial in the U.S. Refractory disease is defined as progression during therapy or within 60 days after completion of therapy. (i)

Interested patients should ask their physician to call Onyx Medical Information at 1-877-ONYX-1-2-1 (1-877-669-9121) or visit www.onyxtrials.com to learn if they are eligible for the trial.

**About Carfilzomib**

Carfilzomib is a selective, next generation proteasome inhibitor that has shown encouraging results in a broad clinical trial program in multiple myeloma. The carfilzomib clinical development program includes a randomized, international Phase 3 clinical trial, known as the ASPIRE trial, studying the combination of lenalidomide and low dose dexamethasone with or without carfilzomib in patients with relapsed multiple myeloma The company has an agreement with the FDA on a Special Protocol Assessment (SPA) and has received Scientific Advice from the European Medicines Agency (EMA) and on the design and planned analysis of the ASPIRE trial. Carfilzomib is also being evaluated in the Phase 3

FOCUS trial, which is designed to support a European registration filing. In addition, Carfilzomib is being evaluated in a broad investigator sponsored trial program including first-line multiple myeloma, combination studies, lymphoma and other malignancies.

**About Multiple Myeloma**

Multiple myeloma is the second most common hematologic cancer and results from an abnormality of plasma cells, usually in the bone marrow. In the United States, more than 50,000 people are living with multiple myeloma and approximately 20,000 new cases are diagnosed annually. (ii) Worldwide, more than 180,000 people are living with multiple myeloma and approximately 86,000 new cases are diagnosed annually. (iii)

**About Onyx Pharmaceuticals, Inc.**

Based in South San Francisco, California, Onyx Pharmaceuticals, Inc. is a global biopharmaceutical company engaged in the development and commercialization of innovative therapies for improving the lives of people with cancer and other serious diseases. The company is focused on developing novel medicines that target key molecular pathways. For more information about Onyx, visit the company's website at www.onyx-pharm.com.

**About the Multiple Myeloma Research Foundation**

Multiple Myeloma Research Foundation was established in 1998 as a 501(c)3 non-profit organization by twin sisters Karen Andrews and Kathy Giusti, soon after Kathy's diagnosis with multiple myeloma. The mission of the MMRF is to relentlessly pursue innovative means that accelerate the development of next-generation multiple myeloma treatments to extend the lives of patients and lead to a cure. As the world's number-one private funder of multiple myeloma research, the MMRF has raised over $170 million since its inception to fund nearly 120 laboratories worldwide, including 70 new compounds and approaches in clinical trials and pre-clinical studies and has facilitated more than 30 clinical trials through its affiliate organization, the Multiple Myeloma Research Consortium (MMRC). As exceptional stewards of its donor's investments, the MMRF has been consistently recognized for its sound fiscal management. For more information about the MMRF, please visit www.themmrf.org.

**About the Multiple Myeloma Research Consortium**

The Multiple Myeloma Research Consortium (MMRC) is a 509(a)3 non-profit organization that integrates leading academic institutions to accelerate drug development in multiple myeloma. It is led from MMRC offices in Norwalk, Conn., and comprises 16 member institutions. Baylor Charles A. Sammons Cancer Center at Dallas, City of Hope, Dana-Farber Cancer Institute, Emory University's Winship Cancer Institute, the John Theurer Cancer Center at Hackensack University Medical Center, H. Lee Moffitt Cancer Center & Research Institute, Mayo Clinic, Ohio State University, Mount Sinai School of Medicine, Sarah Cannon Research Institute, University Health Network (Princess Margaret Hospital), University of California-San Francisco, University of Chicago, University of Michigan, Virginia Cancer Specialists, and Washington University in St. Louis.

The MMRC was founded in 2004 by Kathy Giusti, a myeloma patient, and with the help of the scientific community. The MMRC is a sister organization to the Multiple Myeloma Research Foundation (MMRF), the world's leading funder of multiple myeloma research. The MMRC is widely recognized as an optimal research model to rapidly address critical challenges in drug development and to explore opportunities in the today's most promising research areas in genomics, compound validation, and clinical trials. The MMRC is the only consortium to join academic institutions through membership agreements, customized IT systems, and an integrated tissue bank. For more information, please visit www.themmrc.org.

**Forward Looking Statements**

*This news release contains "forward-looking statements" of Onyx within the meaning of the federal securities laws. These forward-looking statements include without limitation, statements regarding the progress and results of the clinical development, the expanded access program, safety, regulatory processes, commercialization efforts or commercial potential of carfilzomib. These statements are subject to risks and uncertainties that could cause actual results and events to differ materially from those anticipated, including risks related to the development and commercialization of pharmaceutical products. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Reference should be made to Onyx's Annual Report on Form 10-K for the year ended December 31, 2010, filed with the Securities and Exchange Commission under the heading "Risk Factors" and Onyx's Quarterly Reports on Form 10-Q for a more detailed description of such factors. Readers are cautioned not to place undue reliance on these forward-looking statements that speak only as of the date of this release. Onyx undertakes no obligation to update publicly any forward-looking statements to reflect new information, events, or circumstances after the date of this release except as required by law.*

(i) Anderson et al. Clinically relevant end points and new drug approvals for myeloma. Leukemia. 2008; 22:231

(i) Anderson et al. Clinically relevant end points and new drug approvals for myeloma. Leukemia. 2008; 22:231

(ii) National Cancer Institute, Surveillance Epidemiology and End Results, 2007 Facts and Figures

(iii) International Agency for Research on Cancer, GLOBOCAN 2002 database