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# Actinium Receives Orphan-Drug Designation From FDA for Actimab-A in the Treatment of Newly Diagnosed Acute Myeloid Leukemia in Elderly Patients

## Orphan-Drug Designation Anticipated to Provide Faster Regulatory Review and Financial Incentives

NEW YORK, NY -- (Marketwired) -- 12/01/14 -- [Actinium Pharmaceuticals, Inc.](http://www.actiniumpharma.com) (NYSE MKT: ATNM) ("Actinium" or "the Company"), a biopharmaceutical company developing innovative targeted payload immunotherapeutics for the treatment of advanced cancers, announced today that the US Food and Drug Administration (FDA) has granted orphan-drug designation for Actimab-A, an alpha radiolabeled antibody being developed for newly diagnosed AML in patients over the age of 60. Actimab-A is currently in a multicenter Phase 1/2 trial clinical trial.

On November 6, 2014, Actinium announced positive interim data from the ongoing Phase 1/2 trial of Actimab-A in older patients with newly diagnosed Acute Myeloid Leukemia ("AML"). Most notably, median overall survival ("OS") of the seven secondary AML patients (with prior myelodysplastic syndrome, or MDS) in the study was 9.1 months, which compares favorably to historical norms of 2 to 5 months depending on the treatment modality. Older AML patients are already higher risk, with secondary AML patients considered to have the more severe and less treatable form of AML, and as a consequence have shorter expected survival. The clinical abstract was published and is available online in *Blood*, the official Journal of the American Society of Hematology. Actinium expects additional data to be available from this trial in 2015.

Kaushik J. Dave, Ph.D., President and CEO of Actinium, stated, "The FDA's decision to grant orphan-drug status for Actimab-A is a significant milestone for the Company and recognizes the need for innovative new approaches to treat AML. The designation will provide Actinium access to various development benefits and financial incentives from the Agency, including an exemption from prescription drug user fees for Actimab-A for this indication and, if the drug receives marketing approval, it will enjoy seven years of market exclusivity in the United States."

### ***About Orphan Drug Status***

The FDA, through its Office of Orphan Products Development (OOPD), grants orphan status to drugs and biologic products that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases or disorders that affect fewer than 200,000 people in the U.S. Orphan drug designation provides a drug developer with certain benefits and incentives, including a period of marketing exclusivity if regulatory approval is ultimately

received for the designated indication; potential tax credits on U.S. clinical trials; eligibility for orphan drug grants; and waiver of certain administrative fees.

### **About AML**

Acute myeloid leukemia (AML) is an aggressive cancer of the blood and bone marrow. It is characterized by an uncontrolled proliferation of immature blast cells in the bone marrow. The American Cancer Society estimates there will be approximately 18,860 new cases of AML and approximately 10,460 deaths from AML in the U.S. in 2014, a majority of which will be in adults. Patients over age 60 comprise the majority of those diagnosed with AML, with a median age of a patient diagnosed with AML of about 67 years. Treatment approaches in this population are limited because a majority of these individuals are judged too frail and unable to tolerate standard induction chemotherapy or having forms of disease generally unresponsive to currently available drugs. Elderly, high risk patients ordinarily have a life expectancy of 5 or fewer months if treated with standard chemotherapy, though only about a third of them do receive treatment because of toxicity. The other two-thirds receive best supportive care, with 2 months survival, according to Oran and Weisdorf (*Haematologica* 2012; 1916-24).

### **About Actimab-A**

[Actimab-A](#) is a radiolabeled antibody being developed for newly diagnosed AML in patients over 60, and is currently in a multicenter Phase 1/2 clinical trial. Based on Actinium's alpha-particle immunotherapy (APIT) platform, Actimab-A consists of the CD33 antibody lintuzumab linked to the actinium-225 payload. Actimab-A has attracted support from leading experts at the prestigious and high-volume cancer treatment hospitals due to the potential of its safety and efficacy profile, as well as its potential potency, specificity and ease of use. Clinical trials are being conducted at world-class cancer institutions such as Memorial Sloan Kettering Cancer Center, MD Anderson Cancer Center, Johns Hopkins Medicine, Columbia University Medical Center, University of Pennsylvania Health System, Fred Hutchinson Cancer Research Center, and the Texas Oncology-Baylor Charles A. Sammons Cancer Center. The Company expects additional updates to its Phase 1/2 clinical trial in December 2014. Actimab candidates are in early development for other cancers.

### **About Actinium Pharmaceuticals**

Actinium Pharmaceuticals, Inc. ([www.actiniumpharma.com](http://www.actiniumpharma.com)) is a New York-based biopharmaceutical company developing innovative targeted payload immunotherapeutics for the treatment of advanced cancers. Actinium's targeted radiotherapy products are based on its proprietary delivery platform for the therapeutic utilization of alpha-emitting actinium-225 and bismuth-213 and certain beta emitting radiopharmaceuticals in conjunction with monoclonal antibodies. The Company's lead radiopharmaceutical product candidate lomab-B is designed to be used, upon approval, in preparing patients for hematopoietic stem cell transplant, commonly referred to as bone marrow transplant. The Company plans to conduct a single, pivotal, multicenter Phase 3 clinical study of lomab-B in refractory and relapsed AML patients over the age of 55 with a primary endpoint of durable complete remission. The Company's second product candidate, Actimab-A, is continuing its clinical development in a Phase 1/2 trial for newly diagnosed AML patients over the age of 60 in a single-arm multicenter trial.

### **Forward-Looking Statement for Actinium Pharmaceuticals, Inc.**

This news release contains "forward-looking statements" as defined in the Private Securities

Litigation Reform Act of 1995. These statements are based on management's current expectations and involve risks and uncertainties, which may cause actual results to differ materially from those set forth in such statements. The forward-looking statements may include statements regarding product development, product potential or financial performance. No forward-looking statement can be guaranteed and actual results may differ materially from those projected. Actinium undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events or otherwise.

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