Turning Cancer Against Itself: Major Grant Advances Pioneering Melanoma Therapy at NeoStem

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Company on Leading Edge of Research With Experimental Therapy Targeting Specific Cancer Cells

NEW YORK, May 21, 2015 (GLOBE NEWSWIRE) -- NeoStem, Inc. (Nasdaq:[NBS](http://globenewswire.com/News/Listing?symbol=NBS&exchange=2)) announced Thursday that it will receive one of the largest research grants of its kind to support its pioneering treatment for patients with stage III recurrent or stage IV metastatic melanoma, a potentially breakthrough approach that teaches the immune system which cells to attack and kill.

The $17.7 million grant from the California Institute for Regenerative Medicine (CIRM), a distinguished and independent scientific body, is a significant endorsement of the potential for NeoStem's novel approach for treating metastatic melanoma, the most deadly form of skin cancer. The sheer scope of the award has important implications as it is expected to fund a significant portion of the pivotal Phase 3 clinical trial investigating a personalized cancer treatment and currently enrolling patients at centers across the United States.

NeoStem's therapeutic candidate, NBS20, uses a patient's own cancer cells – in essence, turning the cancer against itself – in a way that is unique. Instead of attacking the bulk of cancer cells, NeoStem's therapy targets a patient's cancer-initiating cells, often called "cancer stem cells," which are those that proliferate cancer cells, spreading the disease and forming new tumors throughout the body.

Trial results to date support the expectation that this novel approach will improve overall survival. In a Phase 2 randomized, controlled trial of the therapy, results showed a 72 percent two-year survival rate, compared with 31 percent in the control group.

NeoStem's belief is that if the Phase 3 trial is successful, and the therapy is then approved by the FDA, it could be a breakthrough in the treatment of metastatic melanoma patients and available as early as 2018.

The product candidate has been granted both Fast Track and Orphan Drug designations from the FDA, and the protocol is the subject of a Special Protocol Assessment with FDA. Additionally, the European Medicines Agency has classified NBS20 as an Advanced Therapeutic Medicinal Product.

"We are grateful to CIRM and the cancer experts who reviewed and endorsed our application. We firmly believe that this therapy has the potential to help people survive cancer longer and this grant helps us advance towards bringing this treatment to market," said Dr. David J. Mazzo, NeoStem's Chief Executive Officer. "The grant substantiates our approach to identifying and securing non-dilutive funding for our development programs and helps position NeoStem as a leader among immuno-oncology therapy developers."

The grant comes during National Melanoma Skin Cancer Awareness Month and just ahead of the summer outdoor season when preventing skin cancer – the most common type of cancer in the United States – is a priority.

Norm Beegun, a patient in one of the Phase 2 trials for NBS20 who lives in Los Angeles, California, commented, "After several years of receiving other treatments with limited long-term success, I have been disease-free for the past 11 years since the NeoStem treatment – and I remain so today. The medical need for those with late-stage melanoma is profound, but so is the potential of this therapy. It deserves attention and support. That's why I am so glad that CIRM is funding the kind of research that can help so many people."

NeoStem's research has potentially widespread applications for people with cancers of various solid tumor types, including ovarian, lung, colon, liver, renal and brain cancers.

Other therapies may treat existing cancer, but they may not be as likely as NeoStem's approach might be to prevent a recurrence of tumors. NeoStem is one of a small group of companies with an immunotherapy product actually in Phase 3 development.

NeoStem is a biopharmaceutical company developing novel personalized immuno-oncology medical treatments. CIRM, whose mission it is to accelerate the availability of stem cell treatments for patients with unmet medical needs, funds promising research programs involving all types of stem cells and across a wide range of diseases.

The grant is the largest CIRM has made since its Dec 31, 2014 official launch of CIRM 2.0 and one of the largest the organization has ever made to an industry sponsor. Use of any of the CIRM grant funds is subject to dollar-for-dollar match funding by NeoStem.

"CIRM 2.0 is designed to accelerate the development of treatments for people with unmet medical needs, and this project clearly fits that description," says Dr. C. Randal Mills, President and CEO of CIRM. "With the Board's approval today we will now get this work up and running within the next 45 days. But that's just the start. We are not just providing financial support; we are also partnering with these groups to provide expertise, guidance and other kinds of support that these teams need to help them be successful. That's the promise of CIRM 2.0. Faster funding, better programs and a more comprehensive approach to supporting their progress."

Unlike many other immuno-oncology products, in order to work as hypothesized NBS20 does not depend on a low-level intrinsic immune response from the patient. The therapy is intended to induce an immune response by "teaching" the immune system what cells to attack and kill. Furthermore, because of its unique mechanism of action, the therapy will likely be complementary and potentially even synergistic with other available therapies. It is expected to be very well tolerated by patients because it uses their own immune system to create a specific therapy.

The Intus trial is supported by results from two Phase 2 trials conducted with dendritic cells loaded with antigens from autologous tumor stem cells, now known as NBS20. The more recent of the two trials was a randomized trial comparing subcutaneous injections of NBS20 versus injections of autologous irradiated (inactivated) tumor stem cells in patients with advanced melanoma. The two-year survival rate of 72 percent corroborated one of the findings of the previous Phase 2 trial, in which a 73 percent two-year survival rate was demonstrated along with a five-year survival rate of 50 percent.

The U.S. melanoma market is approximately $1 billion per year, which is predominantly spent to treat metastatic melanoma.

For more information on the Intus Study, which will be enrolling at approximately 50 sites across the U.S, Canada, Australia and New Zealand, please visit [www.theintusstudy.com](http://globenewswire.com/Tracker?data=PDy9cunhB_7pQa1k5vMzDioqw6xphDtjF6mpZT1jJrmHZBAQkGvnyTbjzT_W4mmDbp-xks3cxxL9bWmoyqK3Kw%3D%3D) or [www.clinicaltrials.gov/ct2/show/NCT01875653](http://globenewswire.com/Tracker?data=JN6CGODKwfhOmh-04A_VV4UcSNQhLSMCWbsp8vvUFxyuhJ5l1H8oBeLAzUgwK9voK1wzs1vLcH8ji0xPd6Z1kLqxS5d1hwviSBYQ2QKWj8DsshDB3kNmvH5wx9ZHfe_uFvZddZLYw2-d1t6LLsjCDA%3D%3D).

**About Metastatic Melanoma**

Melanoma, which originates in pigment-producing cells known as melanocytes, is the most lethal form of skin cancer. Melanoma is often caused by unrepaired DNA damage to skin cells from UV radiation. Patients who have progressed to stage IV melanoma have a cancer that has metastasized—or spread—to distant sites in the body such as the lymph nodes, lungs, liver or brain. As a result, advanced melanoma is exceedingly difficult to treat, with a 5-year survival rate of approximately 15%. There are 20,000 estimated new cases of metastatic melanoma, and an estimated 10,000 deaths from metastatic melanoma, each year in the United States.

Treatments for stage IV melanoma are typically directed at slowing the growth of the cancer and prolonging survival. Current treatment options include radiation, chemotherapy, surgical resection, immunotherapy, or a combination approach. Unfortunately, most current approaches provide only temporary relief, either halting the growth of tumors for an average of 6-10 months or eliminating all tumors in only 10-20% of patients. Administering certain anti-melanoma drugs at higher doses appears to be more effective, but often results in more severe side effects.

**About CIRM**

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and to act with a sense of urgency commensurate with that mission. To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies. With $3 billion in funding and over 280 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of medicine closer to reality.  
[https://www.cirm.ca.gov/](http://globenewswire.com/Tracker?data=N8uAxGsj9nQjLhj3VTq70ZZtJa_XVCzucfIiXaup0MwS-lnWF-E1EOpRsdlPN2e6uu9hpXKvtx0O8sDlDAH2zA%3D%3D)

**About NeoStem, Inc.**

NeoStem is a biopharmaceutical company pursuing the preservation and enhancement of human health globally through the development of novel cell based individualized medicine therapeutics that prevent, treat or cure disease. The Company is developing therapies based on three platform technologies (immuno-oncology, ischemic repair and immunomodulation) with a focus on its lead, Phase 3 clinical program for NBS20 in immuno-oncology. The combination of a rich therapeutics pipeline and an externally recognized in-house center for cell therapy process development and manufacturing has created an organization with unique capabilities for accelerated and efficient product development. [www.neostem.com](http://globenewswire.com/Tracker?data=s2J8sc0wtG8NW_w4c9_YYrKGUkjh33mnVM9IYSl58TwfOUXX0Fw_lrhfZm4aD-Ov6C6rygmYV2AE01K4QgnC-g%3D%3D)

**Forward-Looking Statements**

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements reflect management's current expectations, as of the date of this press release, and involve certain risks and uncertainties. Forward-looking statements include statements herein with respect to the adequacy of the grant for funding a significant portion of the Company's pivotal Phase 3 trial, the potential impact of NBS20 in the treatment of metastatic melanoma patients and the date NBS20 will be available, the efficacy of other cancer treatments as compared to NeoStem's approach, the applications of NeoStem's research to cancers of various solid tumor types, the degree to which NBS20 will be complementary to and synergistic with other therapies, successful execution of the Company's business strategy, the Company's ability to develop and grow its business, the successful development of cellular therapies with respect to the Company's research and development and clinical evaluation efforts in connection with the Company's Immuno-Oncology Program, Ischemic Repair Program, Immune Modulation Program and other cell therapies, the future of the regenerative medicine industry and the role of stem cells and cellular therapy in that industry. The Company's further development is highly dependent on future medical and research developments and market acceptance, which is outside of its control. The Company's actual results could differ materially from those anticipated in these forward-looking statements as a result of various factors. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of the Company in general, see the factors described under the heading, "Item 1A. Risk Factors" in the Company's Annual Report on Form 10-K filed with the SEC on March 2, 2015 and those described in the Company's other periodic filings with the SEC. The Company undertakes no obligation to update or revise any forward-looking statements.

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