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Scientists Use Chemical Biology Approach to Assist Cardiac Regeneration

San Diego, Calif. (July 7, 2011) – Scientists at ChemRegen, Inc., a San Diego for-profit company focused on developing small molecule regenerative medicines for human diseases, the Human BioMolecular Research Institute (HBRI) in San Diego, CA, and Sanford-Burnham Medical Research Institute (Sanford-Burnham) in La Jolla, CA, have reported a new method of cardiomyocyte formation from human embryonic stem cells (hESC) using pharmacological inhibition of Wnt signaling. The Wnt signaling pathway is a key mediator of development and stem cell differentiation. The paper, published online today in *Circulation Research*, reports that pharmacological Wnt inhibition can be used to induce cardiomyocytes from hESC and describes the technology.

In the United States, curing heart disease is a major unmet medical need. Of the 300,000 individuals suffering from heart disease who would benefit from a heart transplant, less than one percent of those people will actually receive a heart transplant. During a heart attack, heart tissue is irreparably damaged and scar tissue can form around the heart that may eventually lead to heart failure. If regenerative medicine could provide a way to regenerate damaged tissue, it would be a significant benefit to millions of people who are currently living with heart disease. Another application of the technology is to generate large numbers of cardiomyocyte cells for transplantation purposes.

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Scientists at ChemRegen, in collaboration with HBRI and Sanford-Burnham, developed and tested a high-content screening assay to discover small molecules that drive cardiogenic differentiation from human embryonic stem cells in order to identify small molecules to make large numbers of human cardiomyocytes. The potent Wnt inhibitors were discovered from about the 550 pathway modulators from EMD/Millipore/Merck that were screened. These inhibitors demonstrated that Wnt inhibition alone is sufficient for producing cardiomyocytes from hESC-derived mesodermal cells. This may have practical applications for making large numbers of cells from hESC for utility in a biotechnology setting. Currently, there are few ways to readily make large numbers of human heart cells.

The chemical biology research approach used in this study represents a new method for discovering potential heart disease therapies that might result in drug leads for human cardiac regeneration or practical approaches for producing large numbers of human cardiomyocytes.

About ChemRegen Inc.: ChemRegen is a for-profit company doing research directed at identifying small molecules of use for addressing human diseases. The approach is to develop regenerative medicines to work in conjunction with human embryonic stem cells to cure major human diseases including heart disease, cancer and other diseases. For more information, visit www.ChemRegen.com .

About HBRI: The Human BioMolecular Research Institute is a non-profit research institute conducting basic research focused on unlocking biological and chemical principles related to diseases of the human brain, cardiovascular disease and cancer. The Institute conducts fundamental studies of central nervous system disorders, heart disease and cancer including stem cell approaches and translates findings into new drug development to address human illness. In addition, the institute promotes scientific learning through community service and public access by disseminating information and sharing research with collaborators, colleagues and the public. For more information, visit www.HBRI.org.

About Sanford-Burnham: Sanford-Burnham Medical Research Institute is dedicated to discovering the fundamental molecular causes of disease and devising the innovative therapies of

tomorrow. Sanford-Burnham, with operations in California and Florida, is one of the fastest-growing research institutes in the country. The Institute ranks among the top independent research institutions nationally for NIH grant funding and among the top organizations worldwide for its research impact. From 1999 – 2009, Sanford-Burnham ranked #1 worldwide among all types of organizations in the fields of biology and biochemistry for the impact of its research publications, defined by citations per publication, according to the Institute for Scientific Information. According to government statistics, Sanford-Burnham ranks #2 nationally among all organizations in capital efficiency of generating patents, defined by the number of patents issued per grant dollars awarded.

Sanford-Burnham utilizes a unique, collaborative approach to medical research and has established major research programs in cancer, neurodegeneration, diabetes, and infectious, inflammatory, and childhood diseases. The Institute is especially known for its world-class capabilities in stem cell research and drug discovery technologies. Sanford-Burnham is a nonprofit public benefit corporation. For more information, please visit www.sanfordburnham.org.

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* Willems E, Spiering S, Davidovics H, Lanier M, Xia Z, Dawson M, Cashman J, and Mercola M (2011) Small molecule inhibitors of the Wnt pathway potently promote cardiomyocytes from human embryonic stem cell derived mesoderm. *Circ Res* July 7 (Epub ahead of print).