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With the CADUCEUS Study Showing their Cardiac-Derived Stem Cells are not only Safe but Reduce Scar Size and Generate New Heart Tissue, Capricor, Inc. is now Preparing for their Fully-Funded, 274 FDA-Approved Phase I/II ALLSTAR Trial

Biotech
Stem Cell
(Private)

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Dr. Linda Marbán, Ph.D.
Chief Executive Officer

BIO:
Co-founder of Capricor, Dr. Linda Marbán is Chief Executive Officer of the company. Dr. Marbán combines her research background and business experience to lead Capricor and create a path to commercialization of its novel stem-cell cardiac therapies. She was the lead negotiator in the licensing agreements that have created Capricor's intellectual properties and oversees Capricor's operations, fundraising, manufacturing, business development, and marketing efforts.

Dr. Marbán was previously with the biotechnology start-up company Excigen, Inc., where she was responsible for business development and then operations. At Excigen she raised capital from Medtronic Corporation through a Series A-2 preferred stock financing round.

Dr. Marbán also served on the faculty in the Department of Pediatrics at Johns Hopkins University Medical School and worked for two years as a postdoctoral fellow there in cardiology, studying novel treatments for myocardial infarction (MI) in animal models.

She earned a Ph.D. from Case Western Reserve University in cardiac physiology.

Company Profile:
Capricor's cardiac-derived stem cells (CDCs) represent a novel treatment to repair the heart after muscle loss following large heart attacks. This is accomplished through the regeneration of heart muscle and the shrinking of scar tissue. The CADUCEUS trial, which utilized Capricor's CDCs, showed a 30-47% reduction in scar size and roughly 50% more viable heart muscle in the infarction zones 1-year post heart attack.

This fall, Capricor plans to begin enrollment of the ALLSTAR trial, a Phase I/II FDA approved study using allogeneic CDCs to treat patients 30-days to 1-year after a large heart attack. The objective of ALLSTAR is to demonstrate that the infusion of CDCs to damaged myocardium will show a relative reduction in scar tissue. The worldwide market opportunity is over $5bn. Capricor also plans on beginning the CADRE-MI study in the next 12 months, in which heart attack patients will receive CDCs immediately after reperfusion. The goal is to limit infarct size and reduce acute myocardial damage. The worldwide opportunity for this indication is $1.5bn.

Interview conducted by:
Lynn Fosse, Senior Editor
CEOCFO Magazine

CEOCFO: Dr. Marbán, what is the idea behind Capricor?
Dr. Marbán: Capricor was founded in 2006 in Baltimore, Maryland as a spin-off from intellectual property from John Hopkins University and the University of Rome at La Sapienza. The purpose of the company was, and continues to be, to bring stem cell therapy to patients with heart disease.

CEOCFO: Why is that the particular focus?
Dr. Marbán: Our background, specifically the founders and our intellectual base, is in cardiology and the treatment of the patho-physiology of cardiac disease. We discovered a cell type that is unique, but at the time of the founding of the company we just had a hypothesis in some small animal data that the cells we had would repair the heart. Now we have data both in animals and in people that proves our thesis on the regenerative qualities of our cells.

CEOCFO: How does the science work?
Dr. Marbán: We infuse the damaged area of the heart with our cells, which then harness the endogenous repair

capacity of the heart itself. They have all kinds of positive effects on existing cells, in that they prevent cells that might die from dying and allow those that are still alive to potentially regenerate themselves.

CEOCFO: At what point would cells be used and how would the treatment eventually work?

Dr. Marbán: What we call ischemic injury to the heart would be a situation in which heart muscle dies. This usually occurs in a heart attack, and the continuum of treatment for our product would be from the time the person has a heart attack all the way through the development of heart failure, which is the result of multiple or long-term ischemic injury to the heart.

CEOCFO: Where are you in the process?

Dr. Marbán: We will initiate a Phase II efficacy trial in the US called ALLSTAR, which has already received approval from the FDA, this fall. As we recruit patients, we have to do some safety work because we are switching from an autologous cell, which we already had proof of concept in humans with, to an allogeneic cell. Autologous means the cells are taken from you as an individual, grown up, and then delivered back to you. An allogeneic cell is one that is prepared from a donor heart, so we get donor tissue and make many thousands of doses. This product will be seen in humans for the first time in our ALLSTAR trial, and as such we are going to do a 14-patient lead-in safety study as the first part of ALLSTAR. This will be followed by the second part of ALLSTAR, which will be a larger efficacy study. This will hopefully start next spring and will substantiate the fact that our cells both reduce the scar tissue that develops after a heart attack and increase viable heart muscle. I should note that our allogeneic cells have been proven as safe and efficacious as autologous cells in animal studies.

CEOCFO: Why do you make the change?

Dr. Marbán: There are several benefits of allogeneic vs. autologous cells. A patient has to undergo a great deal of personal time and an invasive procedure to harvest tissue to grow autologous cells. Allogeneic cells require none of that preparatory work, as they are grown from donor heart tissue. A patient can just show up at a hospital and get treated with our cells, as opposed to growing autologous ones, which can take several weeks. This is critical, as it also allows us to treat patients very early on in their disease process. If you have a heart attack and are brought into the emergency room, our cells can be infused immediately, hopefully preventing the damage that can occur in your heart. Finally, allogeneic cells are more cost-effective to manufacture, as we can get thousands of doses from one donor heart as opposed to one dose per person.

CEOCFO: Are there any side-effects?

The time to get involved in stem cells for heart therapy is now. In Capricor, investors have a good opportunity to work with a cell product that is backed by strong science, $24 million of grant funding and that may prevent the progression of heart disease into heart failure. Capricor can transform the treatment of heart attack patients, creating a market measured in the billions. - Dr. Linda Marbán, Ph.D.

Dr. Marbán: The allogeneic cells have not been used in a person yet, but in a large number of animal studies neither we nor the FDA have seen any side-effects that we are concerned about. We are using as our benchmark study the CADUCEUS trial, which was run by our group at Cedars Sinai Medical Center using the autologous product. The study, whose results were recently published in The Lancet, showed there were no side-effects. Notably, 17 of 17 patients treated with cells benefited from the therapy.

CEOCFO: Is there much research in the area of stem cells as far as heart regeneration, and what is different about your approach?

Dr. Marbán: It is one of the hottest research topics in all cardiovascular medicine at this time. When we entered the field in 2003-2004, there was not one session at the meeting of The American Heart Association on stem cell therapy for heart disease. Today, there are probably at least two hundred presentations on this very topic at the meeting. Our approach is similar to our competitors and our colleagues, but our results are very different. What we have found, which is fascinating and very exciting, is that our cells seem to work better than any of our competitor's cells. This includes hematopoetic stem cells, bone marrow mononuclear cells or any of the other cell types that are being studied for treatment of heart disease. Our cells work better as they conclusively reduce scar size by an amount that has not been reported by any other group. We also see for the first time ever, in both animal studies and the CADUCEUS trial, that our cells generate new viable heart tissue.

CEOCFO: Why do they work better?

Dr. Marbán: We have a strong collaboration with The Heart Institute at Cedars Sinai Medical Center where they do much of the mechanistic studies and discovery work. We know factors that the cells express. We know that they seem to generate some myocytes and we know that our cells actually disappear completely. We cannot find a single one after three weeks, but we know a year later patients are doing better.

CEOCFO: Has the medical community been paying attention or is it still wait and see?

Dr. Marbán: The first two years it was not wait and see, but instead full-on skepticism. The next few years were continued skepticism, but that then turned into wait, see and curiosity. Now, in the most recent phase, particularly since the publication of the CADUCEUS data, there is resounding enthusiasm for what we are doing. In fact, we seem to stand out as a real potential leader in the field for the treatment of heart disease. To give a sense of the potential for our cells, in CADUCEUS, scar tissue shrank 30-47% percent and patients showed a roughly 50% increase in viable heart

muscle in the infarction zones 1-year post heart attack.

CEOCFO: Do you still find the public has misgivings on stem cells and is not understanding of the differences?
Dr. Marbán: I think it is a very controversial topic for many people. Those that I talk to sometimes do not understand the difference between an adult stem cell, which is what we have, and an embryonic stem cell, which obviously comes from embryos. In general, the public is excited about the promise of stem cell therapy, they just are a little confused as to the how and the when.

CEOCFO: Development and testing are expensive, is Capricor funded to get through the next steps?

Dr. Marbán: No company our size can confidently say that they are funded all the way through to product launch. That said, we are fully funded through completion of the ALLSTAR trial.

CEOCFO: Why should investors pay attention to Capricor now?
Dr. Marbán: I think Capricor is a unique opportunity for investors because we have a product that is proven to work, de-risking our technology. The CADUCEUS data shows our cells are not only safe but reduce scar size and generate new heart tissue, both of which can prevent the progression to heart failure. This opens up a huge market. We have a fully-funded, 260-patient FDA-approved Phase II trial and in a few short years we hope to begin our pivotal Phase III study. We have a manufacturing model that has been well designed and thought out. Finally, we have a very skilled management team to bring our product to market.

CEOCFO: What should people remember most about Capricor?
Dr. Marbán: The time to get involved in stem cells for heart therapy is now. In Capricor, investors have a good opportunity to work with a cell product that is backed by strong science, $24 million of grant funding and that may prevent the progression of heart disease into heart failure. Capricor can transform the treatment of heart attack patients, creating a market measured in the billions.

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