Translating Broad-Based Regenerative Medicine Research from Bench to Bedside and Beyond

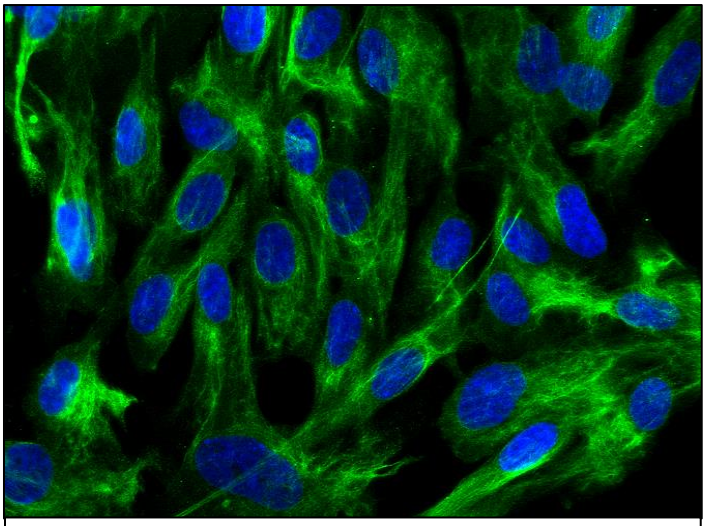
UF CENTER FOR REGENERATIVE MEDICINE

BOUNDLESS POTENTIAL

At the University of Florida Center for Regenerative Medicine (CRM), clinical trials and collaboration are catalyzing the future of health care. The center's expert team of affiliated faculty and clinicians, led by Dr. Keith March, is a national resource for research in regenerative medicine and cell-based therapy. By leveraging the power of the most comprehensive academic health center in the Southeast, the center is driving cross-disciplinary, boundary-breaking clinical trials and translating findings to the clinic to solve today's most challenging unmet medical needs.

The center boasts established partnerships with researchers in UF's six health-related colleges, the UF Clinical and Translational Science Institute, UF's Institute of Food and Agricultural Sciences and other powerhouse research universities across the country. These connections reinforce a robust portfolio of clinical trials utilizing cell-based therapeutics in which the CRM is pioneering new national research that captures the innate healing power of umbilical cord lining-derived mesenchymal stem cells to help or even cure autoimmune and inflammatory diseases. At the same time, the CRM is building a pipeline of future clinicians, researchers and medical experts with one of the only undergraduate regenerative medicine programs in the country.

CLINICAL TRIAL OVERVIEW



"Umbilical Cord Lining Cells in Culture"

Philanthropic support has been critical to the center's advancement of clinical trials. By partnering with the JAR of Hope Foundation and RESTEM LLC, the CRM will be able to initiate an important trial for Duchenne Muscular Dystrophy (DMD). UF has already been selected as the clinical trial site and RESTEM, LLC has received authorization from its Investigational New Drug Application to the FDA entitled **"A phase 1, randomized, double-blind, placebo-controlled Cross Over study to assess the safety, tolerability, and preliminary efficacy of Umbilical Cord Lining stem cells (ULSC) in Pediatric patients with duchenne's muscular dystrophy (DMD)"** to proceed with a Phase 1 trial in 12 patients. Additional support from the JAR of Hope Foundation will help translate Phase 1 and later Phase 2 trial with more answers as to if this will help these children with this debilitating disease. Applying the CRM's research broadly, center leadership is seeing positive early results indicating that regenerative medicine might be a better course of treatment for some patients than immunosuppressive medicines with powerful side effects.

TOGETHER, WE WILL

The CRM is the ideal partner—a leading force in clinical trials allied with numerous departments across UF Health's expansive academic health science center network. Results from the center's research point the way to clear improvements in patient outcomes and have stimulated potentially game-changing developments in clinical care. From the smallest cells to entire communities, the UF Center for Regenerative Medicine is laser-focused on leveraging research that can address the symptoms of disease, trauma or degenerative illness for individual patients and broader populations. Few other regenerative medicine centers in the country can boast a research and clinical enterprise of comparable proportion—with deep expertise that stretches across the breadth of our efforts and leverages disciplines across our comprehensive research university. We appreciate your interest in supporting our shared vision to translate broad-based research into life-saving treatments for a multitude of debilitating diseases and ailments, especially DMD.

About JAR of Hope:

JAR of Hope was born September 13th, 2013, when the founder, Jim Raffone's precious son, James ("Jamesy") Anthony Raffone, was diagnosed with Duchenne Muscular Dystrophy (DMD). JAR of Hope's mission is to find a cure for Duchenne MD.

Jamesy was 4 years old when he was diagnosed. Today he is 13. Each day without a cure his muscles continue to waste away as will all children who suffer from this horrific disease. Statistically, by the age of 12 they will be in a wheelchair, by 15 they will be a quadriplegic, by 18 they will not be able to breathe on their own and eventually will live on a ventilator until their bodies are no longer strong enough to fight this disease. Many don't survive past their mid-20s. The only way this disease will continue to cheat these children of a future is if we stand by and do nothing to find a cure.

DMD is a genetic disorder characterized by progressive muscle degeneration. It primarily affects boys, although there are a few rare cases of girls who have DMD. Duchenne affects 300,000 boys worldwide; 20,000 children in the United States alone. These children are robbed of their childhood as they can't run and play.

In over 200 years, a cure for Duchenne has not yet been found. Not one child has survived this disease, and as you can probably imagine, it takes millions of dollars, massive amounts of time and people committed to finding a cure. JAR of Hope has and continues to dedicate each and every day to finding a cure and living in a world where Duchenne no longer exists. With the help of donors and sponsors, JAR of Hope is funding a team of research scientists and doctors determined to eliminate Duchenne. We also provide financial and emotional support for children and their families who are and have been affected by DMD. We have purchased wheelchair accessible vans, installed chair lifts on stairs in homes for these children to be able to move from one floor to another, funded studies, assisted with medical bills and, unfortunately, have paid for funeral expenses for children that have lost their fight to Duchenne MD.

JAR of Hope's founder, Jim Raffone, has been relentless in his pursuit to defeat Duchenne. Some of his efforts in educating others, raising awareness and funds include breathtaking world-record events such as climbing Mt. Everest, creating race teams to participate in marathons and ultra marathons around the world, gathering thousands of people to simultaneously do pushups together, and creating the longest Lego chain at a school in New Jersey, to name a few. You can see the many events that have taken place on JAR of Hope's website. Jim has received Keys to Cities from various Governors and Mayors as he has traversed around the globe determined to make Duchenne a household name and push science forward. Jamesy is the inspiration behind JAR of Hope and Jim is committed to all of the children with DMD around the world. Jamesy's courage, strength, positive attitude and willingness to do whatever it takes to get healthier and stronger every day motivates Jim to carry on until the first child is cured. Jim and the JAR of Hope team believes that this disease has gone on for too long. Jim's motto is: "I can, I will & I must" find a cure; however, he knows that he cannot do it alone.

Together, we can make history and save lives. Please check out jarofhope.org to learn more about us, for prior and upcoming events, and continuous updates from our researchers.

About RESTEM

RESTEM is an innovation-driven, clinical-stage, biotechnology firm, based in Corona, California, dedicated to the discovery, development and commercialization of novel, next-generation, cell-based therapeutics and tools that aid in the treatment of a broad range of degenerative disorders. The company's mission is focused on improving the quality of life for those who suffer from disabling diseases of the immune system, age-associated disorders, and other degenerative diseases . With over 14 years of research and development focused on the company's novel and proprietary, umbilical cord lining stem cell, or ULSCs, which have novel regenerative properties believed to besuperior to mesenchymal stem cells, RESTEM has emerged as a major contributor to cell-based treatments with ongoing clinical trials that address significant unmet medical need across a range of diseases and disorders.

For more information, please contact:

Keith L. March, MD, PhD
Professor of Medicine
Division of Cardiovascular Medicine
Director, Center for Regenerative Medicine
University of Florida
kmarch@ufl.edu

Margaret Friend
Senior Director of Development
Corporate and Foundation Relations
UF Health Office of Development
frienm@shands.ufl.edu