Stem for Life
a CURA Foundation

CURING DISEASES WITH CELL THERAPY

Spring 2016

LifeLines

www.stemforlife.org

MISSION EXPANDED

Increasing Our Influence Worldwide

The Stem for Life Foundation is pleased to announce that it has expanded its mission to include all types of cell therapy, not just those using stem cells. This change represents our shared commitment to find the best treatments and cures for the many people worldwide in need of a health care solution.

Through joint initiatives, and with the help of supporters, our mission is to give rise to a global movement that will develop vital cellular cures in key areas of medical need. We invite you to join us as we raise public awareness about the therapeutic promise of cell therapy and support the advancement of research and development.

Clinical trials are key to our commitment to find cures for cancer and diabetes, restore tissue and organs and treat neuro-degenerative diseases. We believe a strong collaborative community can unlock the body's natural healing and repair mechanisms. Our hope is to accelerate this vital work by uniting with global leaders in the field of cell therapy.

EDUCATE

We educate the world about the rapid advancements in cell therapy and the need to raise funds to complete testing and clinical development.

INSPIRE

We inspire the next generation of thought leaders, researchers, doctors and business leaders about the importance of cell therapies and the impact these discoveries can have on people's lives.

UNITE

We unite the world's leading scientists, doctors, educators, regulators, business leaders and philanthropists to work together with individual patients to accelerate the development of cell therapy cures.

TO ACCELERATE DEVELOPMENT OF CELL THERAPY TREATMENTS AND CURES...

@STEMFORLIFE
#UNITETOCURE

UNITE TO CURE

Welome Message

Now is a time of great opportunity and infinite possibilities in the field of cell therapy and personalized medicine. During this exciting time, The Stem for Life Foundation is asking you to join us and be part of this medical revolution. We are creating a grass-roots movement to *unite to cure.*

Cell therapies harness the human body's natural repair mechanisms and address the root cause of diseases, offering the possibility for more effective and less costly treatments.

As we take a significant leap forward, I invite those who share a passion for this vision to join us on this epic journey. I hope you will consider becoming an active participant in shaping the future of cell therapy research and development as we work to find cures for the world's most debilitating diseases.

DR. ROBIN L. SMITH
President and Trustee, Stem for Life Foundation

CLINICAL DATA NEWS

RECENT FINDINGS

Clinical Trials In The News

Clinical trials are research studies that explore whether a medical strategy, treatment, or device is safe and effective for humans. The following is a selection of the findings published from some recent studies focused on the use of cell therapy treatments:

- Cytori announced that a single administration of ECCS-50 (*SCLERADEC I trial*) achieved a sustained improvement in hand dysfunction at two years in patients with scleroderma.

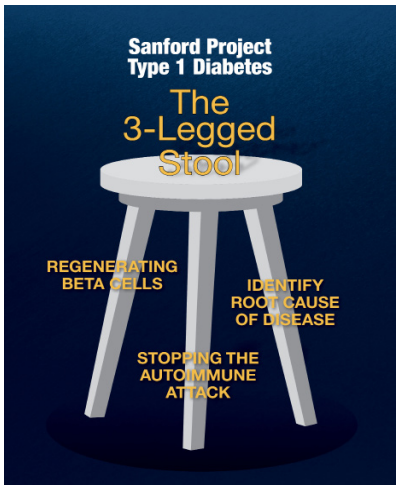
- Mesoblast announced results for *MSC-100-IV* that showed a clinically meaningful responses and significantly increased survival in children with steroid-refractory acute Graft Versus Host Disease (aGVHD). The data from 241 children with steroid-refractory GVHD treated in the Expanded Access Program conducted across more than 50 sites in North America and globally, showed an overall response rate of 65% was seen at day 28 after treatment with MSC-100-IV. A response rate of 81% was seen when MSC-100-IV was used as front-line therapy following steroid failure. In patients with gastrointestinal and liver disease, who have the highest mortality risk, overall response rates were 65% and 62% respectively.

- Athersys announced that the one-year follow-up data from the phase II ischemic stroke study showed that patients receiving *MultiStem* (N=65) continued to improve and had a statistically significant higher rate of "Excellent Outcome" (P=0.02) versus placebo (N=61). These data represented patients treated in the 24-48 hour window. A rating of "Excellent Outcome" was even more pronounced in the patients treated in the 24-36 hour window (P=0.01).

- Vericel announced i*xmyelocel-T* Phase 2b placebo-controlled trial involving 114 patients reduced the number of deaths and hospitalizations of some of the most advanced heart failure patients. To learn more visit: www.clinicaltrials.gov.

A NOVEL APPROACH

A Multipronged Approach to Curing Diabetes



At Sanford Health, the team of scientists and doctors are working to cure Type 1 diabetes. Their approach is focused on three distinct areas:

1 Replacing or regenerating beta cells to restore insulin production by delivering adult-derived stem cells to the pancreas that have the ability to differentiate into beta cells.

2 Stopping the autoimmune attack on beta cells to preserve insulin production by restoring a balanced immune system through expansion of T-Regulatory cells.

3 Identifying the basis for the autoimmune attack. Each clinical trial that has been completed has a few responders indicating a phenotypic response to the therapy, which supports the notion that while the disease is characterized by the loss of beta cells, there could be a variety of mechanisms that precipitate this loss.

Dr. David Pearce, president of Sanford Research at Sanford Health, and Dr. Eckhard U. Alt, the Todd and Linda Broin Distinguished Professor and Chair at Sanford Research, believe success in just one of these areas may not be enough to cure type 1 diabetes. Regenerating beta cells still leaves them prone to autoimmune attack. In order to determine a successful treatment for an individual patient, the root cause of their diabetes must be determined, which is why Pearce and Alt are pursing all three avenues.

GIVING BACK THROUGH CORPORATE SOCIAL RESPONSIBILITY

Making an Impact

Italian sisters, Gabriella and Margot Micallef, founded Gabriella's Kitchen to create nutritious and delicious foods that everyone could enjoy, regardless of their dietary preferences or restrictions.

Naturally, they started with pasta! It was initially about finding an alternative to the delicious but unhealthy traditional pasta. But soon after their initial launch, Gabriella was diagnosed with stage 4 cancer. Eating healthy meals was no longer just desirable, it was necessary to prolong Gabriella's life.

The sisters found, however, that eating healthy often meant eating alone as others shunned the 'bad-tasting-good-for-you' alternatives that were available. They realized that food nourished one's body but sharing food nourished one's soul. So, in 2009, they created a line of high-protein fresh pasta that is enriched with vegetarian protein and is low in carbohydrates and calories.

In 2015, the company was renamed Gabriella's Kitchen in honor of Margot's late sister, who remains the inspiration behind the company. In further recognition of Gabriella and as part of their corporate social responsibility program, the company has chosen to support the Stem for Life Foundation's efforts to create a future in which others don't lose loved ones to lung cancer or any cancer.

All skinnypasta™ products are GMO-free and have no additives or preservatives. Dairy-free and gluten free options are also available. To learn more, visit gkskinnypasta.com.

MARGOT MICALLEF
CEO, Gabriella's Kitchen

THE CONFERENCE

THIRD INTERNATIONAL CONFERENCE

Regenerative Medicine–Progress and Its Cultural Impact

Held at The Vatican April 28-30, 2016
Cellular Horizons™

Join the conversation on Twitter with **#UniteToCure** and Follow us **@StemForLife**

DAY 1	DAY 2	DAY 3
Thursday, April 28 "Hope for our Children"	Friday, April 29 "Cellular and Technological Breakthroughs in Cancer"	Saturday, April 29 "Cellular Frontiers: Research, Regulation and Funding"

Mission and Goals

DISCUSS and understand the importance of scientific advancements, technology and data in the paradigm shift toward regenerative medicine, with a particular focus on cellular therapies.

UNITE people, without prejudice, creating an open dialogue about the interconnections between cellular research, technology, faith and culture.

FOSTER an international conversation among researchers, physicians, philanthropists, faith leaders and policymakers to bring cellular cures to those in medical need and identify a pathway to help reduce human suffering throughout the world.

CREATE and inspire the next generation about the vital linkages between cellular sciences, technological innovation and data to optimize health care at the patient level.

CATALYZE the necessary funding to support the development of cell therapies, which will cure and treat a broad range of debilitating diseases and medical conditions.

ESTABLISH a global movement of cellular researchers, medical practitioners, technological innovators and cultural and religious thought leaders devoted to advancing the paradigm shift toward using cell therapy to treat and cure diseases.

Pontifical Honors and Awards to be presented:

KEY INNOVATION

The Key Innovation Award recognizes game-changing medical innovation exemplified by transformative thinking, creativity and ingenuity used to deliver the highest quality of medical care.

KEY PHILANTHROPY

The Key Philanthropy Award honors an individual who exemplifies care for all of humankind and demonstrates exceptional commitment to tackling difficult issues in order to improve the human condition.

KEY VISIONARY

The Key Visionary Award recognizes medical innovators who change the course of history and reduce suffering on a global scale by blending visionary thinking with real action.

THE POWER OF CELL THERAPY

A HOPEFUL JOURNEY

Cellular Solutions

When she was 17, Grace Meihaus noticed that patches of her skin had suddenly become tight and her fingers swelled up and turned blue whenever she was cold. Previously an active teenager, Grace suddenly fatigued easily and started losing focus in school. After first being misdiagnosed with lupus, it was soon determined that Grace had scleroderma, or systemic sclerosis (SSc).

SSc is a rare autoimmune rheumatic disease that affects the skin and other organs. Typically a disease of young women, SSc affects upwards of 100,000 people in the United States.

By 2015, Grace's symptoms worsened and she left college. Through a scleroderma support group, she learned about Dr. Richard Burt at the Feinberg School of Medicine at Northwestern University and an experimental scleroderma treatment. Grace was enrolled and received an autologous hematopoietic stem cell transplant. Within months, her skin loosened, her internal inflammation stopped and her immune system improved significantly. Her shortness of breath also vanished completely and she was again able to enjoy sports.

GRACE MEIHAUS

NEW TREATMENT

Cutting Edge Technology

Nicholas Wilkins was diagnosed with acute lymphoblastic leukemia when he was just four years old. After three relapses, he had exhausted traditional therapies and enrolled in a clinical trial at the Children's Hospital of Philadelphia using chimeric antigen receptor T-cells, known as CAR-T cell therapy.

Millions of Nicholas' T-cells were taken from his blood, reengineered in a lab then reintroduced into his system through injection to find and destroy cancerous cells. Now 17-years-old, Nicholas is cancer free and enjoys an active lifestyle.

NICHOLAS WILKINS

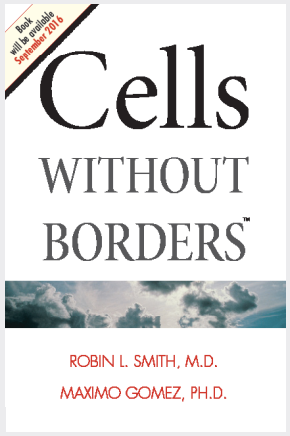
SPREADING THE WORD

RAISING AWARENESS

Expanding the Conversation

In their latest book, *Cells Without Borders, Biological Solutions To Help Rebuild Healthier, Better Bodies*, Dr. Robin Smith and Dr. Max Gomez describe how virtually any disease that results from malfunctioning, damaged or failing tissues may potentially be cured through a variety of regenerative medicine therapies that draw upon the intrinsic healing system of the body.

Readers of *Cells Without Borders* will get an up-close-and-personal look at amazing research innovations that are impacting the body's internal healing system and come to understand why they have the potential to trigger a profound change in medical practice. *Cells Without Borders* can be pre-ordered at www.amazon.com with a release date of September 1, 2016.



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ELANA SIMON

PRECISION MEDICINE

Inspiring the Next Generation

At the age of 12, Elana Simon was diagnosed with fibrolamellar hepatocellular carcinoma, or FHCC, a rare liver cancer. Thankfully, Elana's doctors were able to remove her 15 cm tumor, which had enveloped two thirds of her liver, and she has been in remission ever since. This early battle with cancer sparked her interest in science and forever changed the course of her life.

While in high school, she learned genetic sequencing, and at the age of 18, together with her team, discovered one genetic mutation common to all patients with FHCC. Her findings, published in *Science* magazine, have been re-affirmed by researchers worldwide.

Elana is a wonderful example of a next generation scientist focusing on cancer research. In 2014, she received the Association for Cancer Research's first Young Champion Award, and in 2015 was asked to write President Obama's introductory speech to launch his Precision Medicine Initiative. Currently enrolled at Harvard University, she is studying computer

ROBIN ROBERTS (L) AND SALLY-ANN ROBERTS.

A LOVING GIFT

Giving Can Save A Life

When her younger sister needed help, Sally-Ann Roberts stepped up. Robin Roberts, of Good Morning America, was diagnosed with myelodysplastic syndrome in 2012. Fortunately, Sally-Ann was a perfect donor match. That summer she underwent a small procedure withdrawing some of her stem cells that were then transplanted to Robin. Four years later, Robin is healthy and Sally-Ann has become a prominent advocate for organ and bone marrow donation.

ALI GUTHY

PERSONALIZED MEDICINE

One Person Can Affect Many Lives

At the age of 14, Ali Guthy lost vision in her left eye and was diagnosed with Neuromyelitis Optica (NMO), a rare autoimmune neurological condition affecting the optic nerve and the spinal cord.

After Ali's diagnosis, her parents, Victoria Jackson and Bill Guthy, created the Guthy-Jackson Charitable Foundation, which has become a global community dedicated to better understanding the disease, discovering therapies (including stem cell therapies) and ultimately finding a cure. Ali and her mother co-authored *Saving Each Other — A Mother-Daughter Love Story*. Ali refused to allow her condition to control her life and resolved to not only manage her symptoms and survive, but also to find a way to thrive in every aspect of her life. Today, Ali is 22 years old. She graduated from the University of California, Santa Barbara, in 2015 and now works for Tradesy, the popular California-based Internet website and app.

REGULATORY ENVIRONMENT FOR CELLULAR THERAPY

CELL THERAPY REGULATIONS

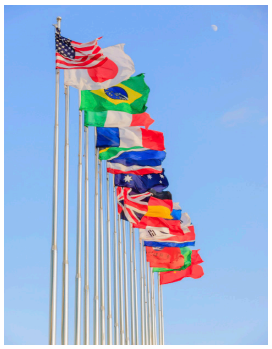
Current Challenges

There are many barriers preventing cellular therapeutics from reaching patients, including scientific, technical, legal and regulatory. A brief overview of cellular therapeutics regulations puts this into perspective. In the USA, surgical transplantation of a piece of tissue is recognized as "practice of medicine" and performed at the discretion of licensed physicians, exempting it from the Food and Drug Administration's (FDA) 21CFR 1271 regulations.

However, once individual cells are isolated from that same piece of tissue, their use becomes regulated. Even if cells are transplanted back into the same patient, human cells are regulated as drugs, unless they are shown to be essentially unchanged, or "minimally manipulated", from the state in which they were originally isolated, and also used in the same anatomical environment for "homologous" purposes.

FROM THE BENCH TO THE PATIENT

Country by Country



Despite profound differences between living human cells and inanimate chemicals, the regulatory framework for cellular therapeutics applied by the FDA remains largely based on that which was developed for chemical drugs.

Other regulatory jurisdictions have pioneered attempts to resolve these issues; the European Union has created a separate class of Advanced Therapy Medicinal Products that are regulated differently and introduced Adaptive Licensing, while Japan also introduced new legislation in 2014 to accelerate adoption of cell-based therapy.

Many believe the USA needs a new regulatory paradigm so that cellular therapies can reach patients in need while there is still time to have an impact.

PROACTIVE ADVOCACY AND OUTREACH

ADVANCING MEDICAL INNOVATION FOR A HEALTHIER AMERICA

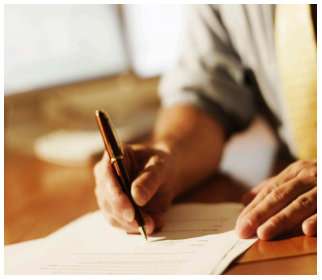
Legislative Updates

Founded in 2007 in Washington D.C., the Bipartisan Policy Center (BPC) is a non-profit think tank that combines politically balanced policymaking with proactive advocacy and outreach.

In July 2015, in response to a request for technical assistance from the Chairman of the Senate Committee on Health, Education, Labor and Pension, BPC released a report, *Advancing Medical Innovation for a Healthier America,* that stressed the need for clarity regarding regenerative medicine regulations.

The BPC convened a panel of prominent American domain experts and in December 2015 published *Advancing Regenerative Cellular Therapy,* a report promoting recommendations to accelerate availability of potentially beneficial therapies.

Independently, Senator Mark Kirk (R-IL) had also been drafting a Bill to accelerate the delivery of cellular therapeutics and the Senator's office worked with BPC, its expert panel, and other academic, patient and



Legislative Updates Cont'd

industry organizations to develop legislation. On March 16, 2016, Senators Kirk, Manchin (D-WV) and Collins (R-ME) introduced bipartisan bill, S. 2689, the REGROW Act, followed by corresponding House of Representatives bill, HR 4762, introduced by U.S. Representatives Coffman (R-CO), Takai (D-HI) and Griffith (R-VA).

This Act proposes to create a new conditional approval pathway for cellular therapeutics where, once safety and preliminary efficacy have been demonstrated by Phase I/II trials, the FDA may grant a 5-year conditional approval, prior to Phase III trials. During this conditional approval period further evidence of safety and efficacy must be gathered and submitted for full approval of a "BLA" - a Biologics License Application.

RAISE YOUR VOICE

GET INVOLVED

Show Your Support

If you are a patient, academician, or scientist and want to learn more and even support the bill, visit http://www.celltherapynow.org.

JOIN THE MOVEMENT

BECOME AN ADVOCATE

Make a Contribution

LEARN

Discover the Stem for Life Foundation's cell therapy awareness toolbox. Sign up today to receive subsequent issues of the LifeLines newsletter and to learn about future conferences, events and activities.

DONATE

Donate now to play a part in creating better medicine. Your support will help educate and advance initiatives to bring cell therapy cure to patients in need. Gifts may be made in honor of a friend or loved one.

SHARE

Help spread the word about cell therapy. Invite others to join you as you raise awareness by engaging in social media channels and participating in community outreach.

JOIN US

To learn more: Call 212.584.4176 or visit *www.stemforlife.org*

To make a contribution: Visit *www.stemforlife.org/donate* or send a check to:

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