FINDINGS

Nebraska Coalition *for Lifesaving Cures*

The Newsletter of the Nebraska Coalition for Lifesaving Cures

December 2014

Wally and Barbara Weitz to be Honored at Spring Luncheon

The Nebraska Coalition for Lifesaving Cures will honor Wally and Barbara Weitz at its annual tribute luncheon, to be held April 20, 2014 at the Happy Hollow Club.

Wally and Barbara are active in the local non-profit community, focusing their efforts on education, social justice and the arts.

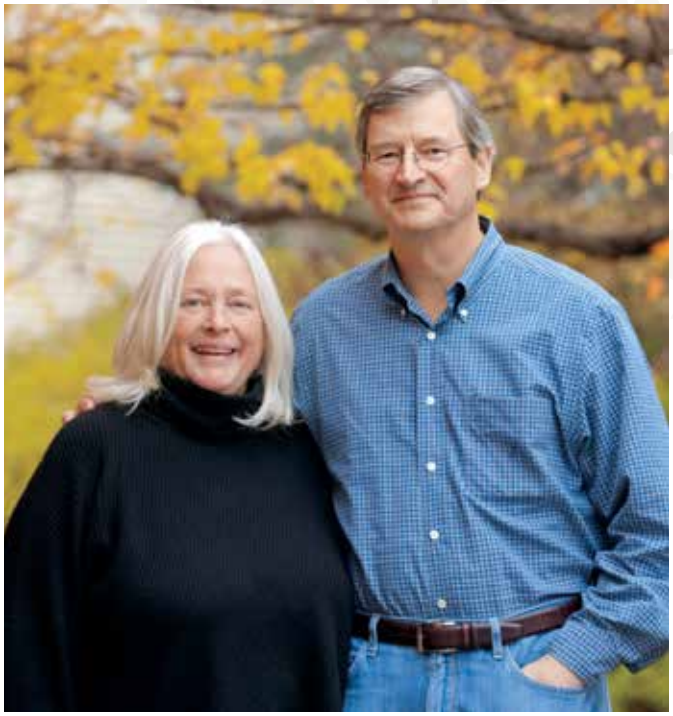
Wally was born in Pittsburgh to a social worker and a college professor. He grew up in New Orleans in the 50s and 60s, where the roots of both entrepreneurial instinct and social conscience were evident early.

He earned a B.A. in economics at Carleton College, where today he serves as a trustee, and went to New York to work for a small Wall Street firm. In 1973, Wally and Barbara moved to Omaha, in pursuit of Midwestern surroundings. Wally joined Chiles, Heider, a regional investment firm where he spent 10 years managing accounts for individuals and doing equity research.

In 1983, he founded Weitz Investment Management which now manages about $6 billion in assets through its family of mutual funds and separately managed accounts.

Barbara was born in Lincoln where she lived with her family until her father completed law school and they moved to Charleston, SC. Barbara says she learned to speak with a delightful southern accent, until her family relocated to Chicago and then at age seven to Omaha, where she graduated from Westside High School. It was at Carleton College where she met Wally. With her degree she entered the job market and landed in the Minnesota Governor's office for a brief career as a speech writer.

Wally and Barbara married and Barbara moved to New York City where she went back to school at New York University for her Master's in Public Administration.



Wally and Barbara Weitz

When they returned to Omaha, she began a career as a mother, volunteer, church educator and finally and "most happily a social worker." She received her MSW from University of Nebraska Omaha and taught in the School of Social Work for 15 years.

Wally and Barbara are strong supporters of the Nebraska Coalition for Lifesaving Cures. Wally serves as a member of the board and Barbara's support is personal, after watching her sister, Vicki, die at age 55 after 52 years of fighting brittle juvenile diabetes and her father, Robert, who died of Parkinson's disease.

Wally and Barbara have three children and six grandchildren all living in Omaha.

FROM THE PRESIDENT

Stem Cells in 2014

by Dr. David Crouse
President, Nebraska Coalition for Lifesaving Cures

The past year has been a remarkable one in the arena of stem cell science and clinical applications. Those advances have come in nearly every domain of work with these interesting cells. The advances are too many to detail in a short newsletter but include: demonstration that research cloning of human cells (SCNT) to produce cells with the characteristics of embryonic stem cells was finally reached by Mitalipov's group in Oregon and Lee & Chung's group in Korea (Science 344:462, 2014); numerous clinical trials with adult, umbilical cord/placental, or neural stem cells have been initiated with patients suffering acute spinal cord injury, several neurological diseases (ALS, MS, CP), heart failure, inflammatory bowel disease, and diabetes, as well as other critical diseases; investigators have increasingly used embryonic stem cells (ESC) and induced pluripotent stem cells (iPSC) to produce cell lines derived from individuals with known diseases to allow study of "diseases in a dish". Along with all of this activity has come markedly escalated attention from the FDA to regulate clinical use of "stem cells" much like a drug. This oversight has undoubtedly provided protection to consumers/patients but has also slowed the delivery of treatments to patients and increased the financial burden on investors. It may also contribute to the development and expansion of stem cell-related medical tourism where patients leave the US for treatment in countries with less regulatory oversight in spite of increased health risk and little evidence to support the treatment.

Some of the most dramatic changes in 2014 have been with respect to the opening of several new clinical trials with ESC derived cells and one with iPSC. First, the biotech company ACT has supported ongoing trials with ESC derived retinal cells and they continue to be very successful for age-related atrophic macular degeneration and Stargardt's macular dystrophy. Some patients have shown a remarkable return of visual acuity (Lancet, Oct 2014). Using the same type of cells, ACT is sponsoring a new trial treating severe myopia and it has been approved by the FDA. Additionally, a similar study, the first to use iPSC, is now underway in Japan using iPSC derived retinal cells to treat macular degeneration. Second, studies supported by ViaCyte have been FDA approved and launched to treat type I diabetes. This study uses a very new approach in which the ESC derived pancreatic islet cells are implanted subcutaneously in a small permeable container (i.e., encapsulated) that allows the cells to sense sugar levels and respond by producing insulin. Third, ESC derived cardiac progenitors will be studied in a clinical trial in France. They will be implanted at the same time patients are undergoing bypass surgery and are intended to facilitate recovery and improve ventricular function. It is not expected that they will actually engraft and replace muscle but rather help in the "healing process". Finally, the study that was the first to use ESC derived cells in a human was resumed. The trial treating acute spinal cord injury, sponsored by Geron, began in 2011 and enrolled a number of patients who all progressed with no adverse outcomes. In late 2011, the company reorganized and dropped stem cells as a "product line" to focus on oncology treatments, however they did provide funding and a process to continue to follow and study the previously enrolled patients for 15 years. The now-resumed study on repair of spinal cord injury will be sponsored by Asterias Biopharmaceutics and supported with a $14.3 million grant from the California Institute of Regenerative Medicine (CIRM). This represents quite a turn-around for the study and the treatment story will take years to come to a conclusion.

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Related to that clinical trial, a new book that chronicles the story of the first ESC treated patient, T.J. Atchison, has been written by Tory Williams, a close family friend. In that writing process, she has become a vocal advocate for the research and has become close to many other patients, the scientists and clinicians who were behind the research and treatment, as well as the lead figures in other advocacy organizations. It is a real "behind the scenes" look at the family, the decisions to participate in the study and the controversy that surrounded the clinical use of these remarkable cells. The book, "Inevitable Collision" should be available soon and is published by Mary Ann Liebert, Inc.

NEWS & NOTES

From Stem Cells to Billions of Human Insulin Producing Cells

by B.D. Colen
Harvard staff writer, Harvard University
News Release, October 9, 2014

Harvard stem cell researchers announced that they have made a giant leap forward in the quest to find a truly effective treatment for type 1 diabetes, a condition that affects an estimated three million Americans at a cost of about $15 billion annually.

With human embryonic stem cells as a starting point, the scientists are for the first time able to produce in the kind of massive quantities needed for cell transplantation and pharmaceutical purposes, human insulin-producing beta cells equivalent in most every way to normally functioning beta cells.

Doug Melton, who led the work and who twenty-three years ago, when his then infant son Sam was diagnosed with type 1 diabetes, dedicated his career to find a cure for the disease, said he hopes to have human transplantation trials using the cells to be underway within a few years.

"We are now just one pre-clinical step away from the finish line," said Melton, whose daughter Emma also has type 1 diabetes.

World-First Embryonic Stem Cell Trial for the Heart

by Cynthia Fox
Science Writer, Bioscience Technology
Originally published on October 30, 2014

The first embryonic stem (ES) cell trial for severe heart failure is launching now in Paris.

The long-awaited trial comes after much preclinical cell work on more than 350 rats, 50 immunodeficient mice, and 32 non-human primates.

"After 20 years in the stem cell area and a daily practice of cardiac surgery, I am very cautiously optimistic," Principle Investigator Philippe Menasche told Bioscience Technology. Menasche is chief of the Heart Failure Surgery Unit of the Hôpital Européen Georges Pompidou, and director of an INSERM (France's National Institute of Health and Medical Research) lab devoted to cell therapy of cardiovascular diseases.

"These patients are, by definition, very sick. I hope the trial will be just a step from which we can learn how to move the field forward," he said.

The world-first trial will give cardiac progenitor cells made in a lab from human ES cells to six patients. The patients will receive the cells when they undergo scheduled coronary artery bypasses, or mitral valve procedures.

ViaCyte's VC-01™ Investigational Stem Cell-Derived Islet Replacement Therapy Successfully Implanted into First Patient

PR Newswire
Release date October 29, 2014

VIACYTE
Regenerating Health™

ViaCyte, Inc., a privately-held regenerative medicine company, announced in a news release on October 29, that the first patient in its Phase 1/2 study was successfully implanted with VC-01™, its embryonic stem cell-derived islet replacement product candidate being developed as a treatment for type 1 diabetes. This Phase 1/2 clinical trial, designed to evaluate the VC-01 product candidate directly in patients with type 1 diabetes, is initially being conducted at UC San Diego Health System, with the support of the UC San Diego Sanford Stem Cell Clinical Center, under the direction of Principal Investigator Robert Henry, MD.

Dr. Paul Laikind, President and CEO of ViaCyte, said, "We are very excited to begin the clinical stage of development in our quest to transform the way patients with type 1 diabetes are impacted by the disease. To our knowledge, this is the first time that an embryonic stem cell-derived cell replacement therapy for diabetes has been studied in human subjects, and it represents the culmination of a decade of effort by the ViaCyte team, our collaborators, and our supporters at the California Institute for Regenerative Medicine and at JDRF."

NEWS & NOTES

UCLA Team Cures Infants Of Often-Fatal "Bubble Baby" Disease By Inserting Gene In Their Stem Cells; Sickle Cell Disease Is Next Target

by Todd Dubnicoff
California Institute of Regenerative Medicine
November 18, 2014

Poopy diapers, ear-splitting cries, and sleepless nights: sure, the first few weeks of parenthood are grueling but those other moments of cuddling and kissing your little baby are pure bliss.

That wasn't the case for Alysia and Christian Padilla-Vacarro of Corona, California. Close contact with their infant daughter Evangelina, born in 2012, was off limits. She was diagnosed with a genetic disease that left her with no immune system and no ability to fight off infections so even a minor cold could kill her.

Evangelina was born with Severe Combined Immunodeficiency (SCID) also called "bubble baby" disease, a term coined in the 1970s when the only way to manage the disease was isolating the child in a super clean environment to avoid exposure to germs. Bone marrow transplants from a matched sibling offer a cure but many kids don't have a match, which makes a transplant very risky. Sadly, many SCID infants die within the first year of life.

Until now, that is.

A UCLA research team led by Donald Kohn, M.D., announced a stunning breakthrough cure that saved Evangelina's life and all 18 children who have so far participated in the clinical trial.

Inserting the missing gene, called ADA, into the blood stem cells restores the cells' ability to produce a healthy immune system. And since the cells originally came from the infant, there's no worry about the possible life-threatening complications from receiving non-matched donor cells.

This breakthrough didn't occur overnight. Kohn and colleagues have been plugging away for over twenty years.

"All of the children with SCID that I have treated in these stem cell clinical trials would have died in a year or less without this gene therapy, instead they are all thriving with fully functioning immune systems," according to Kohn.

For the Padilla-Vacarro family, the dark days after Evangelina's grave diagnosis have given way to a bright future. "It was only around six weeks after the procedure when Dr. Kohn told us Evangelina can finally be taken outside. To finally kiss your child on the lips, to hold her, it's impossible to describe what a gift that is," said Alysia, Evangelina's mother.

The team's next step is to get approval by the Food and Drug Administration (FDA) to provide this treatment to all SCID infants missing the ADA gene.

Parkinson's Disease Breakthrough: Stem Cells May Replace Damaged Nerves, Reverse Symptoms

by Samantha Olson
Medical Daily
Originally published November 9, 2014

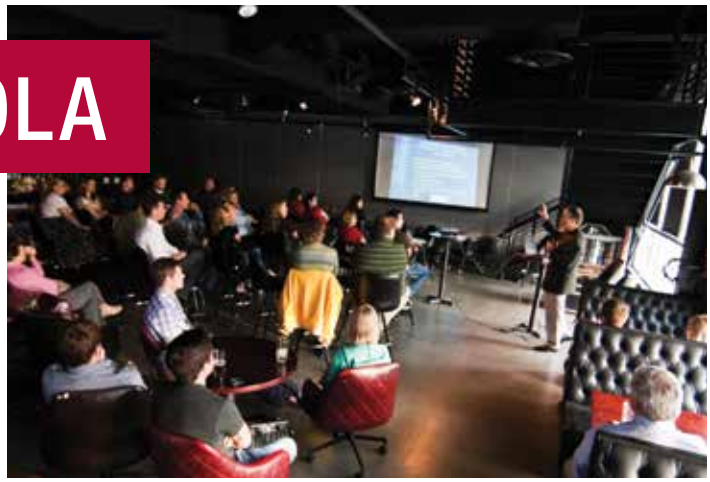
Parkinson's disease patients can find hope in a new treatment, thanks to breakthrough stem cell research that successfully replaces damaged nerves. Swedish researchers have figured out how to create motor neurons that become lost in the brains of Parkinson's disease patients. They published their findings in the journal Cell Stem Cell.

Researchers from Lund University took human embryonic stem cells (hESC) from in vitro fertilization embryos and grew them into motor neurons. The neurons were transplanted into the brains of rats with Parkinson's disease, and over the course of five months, their dopamine levels rose back to normal. There are currently one million individuals living with Parkinson's disease in the United States, and 96 percent of them were diagnosed after the age of 50.

Parkinson's is an incurable progressive disease that takes over your body, rendering you without control, according to the Parkinson's disease Foundation. It affects the nervous system and movement, causing tremors, stiffness, slow movements, impaired posture and balance, speech changes, and other life-changing symptoms. This tumbling loss of motor skills is partially caused by the death of nerve cells that control dopamine in the brain. Researchers don't know exactly why the chemical messenger begins to die, but once dopamine levels decrease, the brain loses the ability to regulate critical muscle movements.

"Our study represents an important milestone in the preclinical assessment of hESC-derived dopamine neurons and provides essential support for their usefulness in treating Parkinson's disease," said the study's lead author Malin Parmar of Lund University in a press release.

SCIENCE CAFÉ & EBOLA



Ebola Questions Answered at Science Café

Dr. Ali Khan
Dean, University of Nebraska Medical Center, College of Public Health

Ebola became personal to Nebraska residents when the first patient with the disease touched down in the state and was treated – successfully – at the University of Nebraska Medical Center's bio-containment unit.

At the November Science Café, of which the Nebraska Coalition for Lifesaving Cures is a sponsor, Dr. Ali Khan, dean of UNMC's College of Public Health, provided the facts about the disease.

He told the audience that fruit bats in Africa were most likely to blame for the spread of the disease by biting animals such as chimps or antelopes, which were then handled by people in the area who became infected.

Dr. Khan dealt with Ebola and other deadly viruses in his previous job with the U.S. Centers for Disease Control and Prevention.

The current Ebola outbreak, which has occurred primarily in three west African countries – Sierra Leone, Guinea and Liberia – caught health officials in those countries off-guard, Khan said.

"It's easy to understand how you could completely miss this upfront," he said. Ebola symptoms are typically fever, headaches and muscle aches, which are similar to those experienced by people battling malaria, dysentery or typhoid fever.

Khan told the audience that burial practices played a major role in the recent spread of the virus. People who prepare the bodies of Ebola victims for burial were getting the infected bodily fluids in their eyes, noses and/or mouths, he said, and they spread the virus to others.

As of November 14, the U.S. Centers for Disease Control and Prevention says, the number of Ebola cases across the globe has exceeded 14,000; the total worldwide death toll from Ebola has topped 5,100. There have been three cases in Mali and three deaths.

Two Americans who contracted the Ebola virus in Liberia have been successfully treated in the biocontainment unit at the Nebraska Medical Center. Dr. Martin Salia, who was in extremely critical condition when he arrived at UNMC, died barely two days into his treatment.

What this outbreak has taught us, Khan said, is that "an infectious disease threat anywhere is an infectious disease threat everywhere." The secret, he said, is to stop the disease where it lives.

Khan answered questions about how long the virus can live on surfaces (a couple days to, possibly, a week), why exposed people would resist being quarantined (if they have no fever, they're not infectious) and whether the virus could become airborne (unlikely, but anything can happen).

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