FINDINGS

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FINDINGS

Nebraska Coalition *for Lifesaving Cures*

The Newsletter of the Nebraska Coalition for Lifesaving Cures

Spring/Summer 2015

Nebraska Coalition honors Wally and Barbara Weitz

David Crouse, Ph.D., President, Nebraska Coalition for Lifesaving Cures, Barbara Weitz, Wally Weitz

The Nebraska Coalition for Lifesaving Cures presented its Lifesaver Award to Wally and Barbara Weitz at its thirteenth annual luncheon, held Monday, April 20, 2015 at the Happy Hollow Club.

At the lunch, the Weitzs voiced their strong support of the stem cell research being done at UNMC and other research centers around the country.

"I grew up believing that everyone believes in science," said Barbara Weitz, a former social worker. "We need to vote for people in the legislature who support research. We need to be the loudest voices in the room."

"We need to start telling people that we believe in science and not assume that everybody does. We need to tell the person we're standing next to in line at Starbucks."

Wally Weitz, a coalition board member, had high praise for Dick Holland, founder of the Coalition and a staunch supporter of research and UNMC.

"Dick didn't have to twist our arms (to provide financial support to the coalition)," Wally said. "Dick is the real hero. He started the whole thing."

Wally Weitz is the founder of Weitz Investment Funds and Barbara Weitz recently retired from The Grace Abbott School of Social Work at the University of Nebraska at Omaha where she taught for more than 10 years.

The Weitzs met at Carleton College in Northfield, MN. Following graduation they lived in New York City while Wally worked for a small Wall Street firm. They returned to Omaha, where Barbara had lived and graduated from Westside High School. Weitz joined Chiles, Heider, a regional investment firm where he spent 10 years managing accounts for individuals and doing equity research before founding his own firm.

Wally Weitz serves on the board of directors of the Nebraska Coalition, and Barbara has a personal stake in protecting research after watching her sister die at age 55 after 52 years of fighting brittle juvenile diabetes and her father die of Parkinson's disease.

> "We need to start telling people that we believe in science and not assume that everybody does. We need to tell the person we're standing next to in line at Starbucks."

UNMC Surgeon receives inaugural Scientific Achievement Award

In 2014, the Nebraska Coalition for Lifesaving Cures established the Chancellor Emeritus Harold M. Maurer, M.D. and Beverly Maurer Scientific Achievement award. The award recognizes the accomplishments of University of Nebraska scientists or clinicians that demonstrate promise in research topics that improve lives of Nebraskans and people around the world.

David F. Mercer, M.D., Ph.D., was selected for his extraordinary work as a surgical innovator and the Director of the Intestinal Rehabilitation program. Through Dr. Mercer's efforts, his nomination read, "He now has arguably the world's largest and most successful experience in a variety of innovative surgical techniques such as the Serial Transverse Enteroplasty (STEP)."

The work of Dr. Mercer and his multidisciplinary team recently culminated in the publication of groundbreaking work demonstrating successful treatment of children with ultrashort length of bowel. Again, quoting his letter of recommendation, "This work is transformative in the field."

Partial funding for this award comes from a grant from Ellie Batt in memory of her brother, Larry, a long-time supporter of the efforts of medical research.

Ellie Batt, Leann Mercer, David F. Mercer, M.D., Ph.D., Beverly Maurer, Harold M. Maurer, M.D.

Nebraska Coalition sponsors Lincoln Science Café and Kearney Lunch and Learn featuring CIRM President

Dr. C. Randal Mills, President & CEO, California Institute for Regenerative Medicine

California Institute for Regenerative Medicine president and chief executive officer Dr. C. Randal Mills was the featured speaker at a Science Café in Lincoln and a Lunch and Learn in Kearney during this year's Science Festival the week of April 13. His talk was titled, "The Impact of Investing in Stem Cell Research: California's Story."

Mills previously served as CEO of Osiris Therapeutics. Under Mills' leadership, Osiris developed the world's first approved stem cell drug to treat children suffering from graft versus host disease, an often fatal side effect of a bone marrow transplant.

Mills is a founding member of the University of Florida Tissue Bank and currently serves as Chairman of Tissue Banks International, the largest provider of ocular tissue for vision restoration. He also serves on the Board of the Alliance for Regenerative Medicine and the Wake Forest Institute of Regenerative Medicine Advisory Board.

The Nebraska Science Festival is a collaboration of organizations and individuals interested in the advancement of science literacy. The Science Festival is designed to make science accessible, interactive, relevant and fun for kids and adults alike. In addition to UNMC, other sponsors to date, include: Nebraska Medicine, the Nebraska Coalition for Lifesaving Cures, Metro Credit Union, HDR, West Corporation and media sponsors: KETV and the Omaha World-Herald.

FROM THE PRESIDENT

Nebraska Coalition for Lifesaving Cures - luncheon comments

HISTORY OF NCLC

April 20, 2015

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

As a background comment, the University of Nebraska Medical Center has a long and distinguished national and international reputation in stem cell transplantation. Following some 20 years of basic science research around the world and gradual refinement of clinically applied transplantation, the first patients at UNMC were transplanted with bone marrow cells in 1983 and sometime later, using techniques pioneered at UNMC, with peripheral blood derived stem cells. During most of this time and continuing today, basic science research and clinical applications related to stem cells have been an important part of UNMC's portfolio. I would like to thank those individuals and groups for their work.

With that background, I would like to outline the history of our organization. In November of 1999 the Omaha World Herald, ran a front page story entitled, "NU Uses Fetal Cells in Studies" which set off a cascade of follow-up interviews and stories. Legislative bills over the next two to three years would propose to ban and even criminalize such research in Nebraska, or at least at UNMC. In the fall of 2000, Nebraskans for Research was organized to voice its support of the important research at UNMC. It was an effective move, and after much work by many parties, the restrictive research bills failed to move forward – at least for a time. But science and legislators do not stand still.

In the same time period, we can pick up the second track. The first description of the isolation and characterization of human embryonic stem cells (hESC) occurred in 1998. Soon thereafter, NU President Dennis Smith established a Bioethics Advisory Committee, chaired by now Chancellor Harvey Perlman. They were to develop guidelines for such hECS research conducted by the University of Nebraska. The resulting, very forward-looking, recommendations were discussed intensely and adopted by the Board of Regents in the spring of 2001. They prohibited research cloning and required the University to follow federal guidelines, among other things. Therefore, when President Bush restricted the use of federal funds for hESC research to stem cell lines created prior to August 9, 2001, the University was duly limited to using only the "Bush" approved hESC lines (22 at the time). It was not long before the need for additional, more diverse and technically more useful hESC lines became a major issue.

The Nebraska Legislature grappled with hESC and cloning research legislation from 2001 to 2007. In 2007, the Nebraska Coalition for Lifesaving Cures, which had merged with Nebraskans for Research, continued to stand in support of UNMC. Many restrictive bills were deflected and in 2008 a compromise, the Stem Cell Research Act (LB 606), was finally reached. The compromise was widely supported by both proponents and opponents of the research inside and outside of the Legislature, as well as by the Governor. It allowed research on hESC lines as long as the cell lines were developed elsewhere and is still effective today.

On March 9, 2009, President Obama signed an Executive Order changing the Bush limitations on federally funded hESC research. On March 6, in anticipation of that change, opponents of stem cell research testified in a hearing before the University of Nebraska Board of Regents that such research in Nebraska should not be expanded beyond the Bush-approved hESC lines and urged the Board to revisit its policy. This was clearly in contrast to their very recent support of the compromise Stem Cell Research Act (LB606). The Coalition stood in support of the current law and continuation of the research. The hearing did not lead to any action at that time, but a resolution to restrict hESC research at the University was soon proposed.

In October and November of 2009, the Board of Regents considered the University policy regarding Embryonic Stem Cells in some detail and had a vigorous and lengthy discussion. Finally, in a four-to-four split vote, the NU Stem Cell Guidelines were retained.

The Nebraska Coalition for Lifesaving Cures remains steadfast in our support of an open research environment where all federally permitted research into the causes, treatments and cures of devastating diseases can be openly conducted by caring and talented clinicians and scientists.

NEWS & NOTES

Stem cell therapies on mice reduce Parkinson symptoms

by Rick Nauert Ph.D.
D'OR Institute for Research and Education/EurekAlert!

Brazilian researchers announced progress toward the use of implanted stem cells as a treatment for Parkinson's disease.

Investigations at the D'OR Institute for Research and Education (IDOR) and Federal University of Rio de Janeiro (UFRJ) report that their newly developed therapy reduced symptoms in mice.

Using an FDA approved substance for treating stomach cancer, S.K. Rehen and colleagues were able to grow dopamine-producing neurons derived from embryonic stem cells. The cells remained healthy and functional for as long as 15 months after implantation into mice — restoring motor function without forming tumors.

Parkinson's, which affects as many 10 million people in the world, is caused by a depletion of dopamine-producing neurons in the brain.

Current treatments include medications and electrical implants in the brain which cause severe adverse effects over time and fail to prevent disease progression.

In the current study, researchers build upon past investigations that have indicated the transplantation of embryonic stem cells improves motor functions in animal models. However, until now, the procedure has shown to be unsafe, because of the risk of tumors upon transplantation.

To address this issue, the researchers pre-treated undifferentiated mouse embryonic stem cells with mitomycin C — a drug already prescribed to treat cancer.

The researchers used mice modeled for Parkinson's. The animals were separated in to three groups. The first one, the control group, did not receive the stem cell implant. The second one received the implant of stem cells which were not treated with mitomycin C, and the third one received the mitomycin C treated cells.

After the injection of 50,000 untreated stem cells, the animals of the second group showed improvement in motor functions, but all of them died between three and seven weeks later. These animals also developed intracerebral tumors.

In contrast, animals receiving the treated stem cells showed improvement of Parkinson's symptoms and survived until the end of the observation period of 12 weeks post-transplant with no tumors detected. Four of these mice were monitored for as long as 15 months with no signs of pathology.

Furthermore, the scientists have also shown that treating the stem cells with mitomycin C induced a four-fold increase in the release of dopamine after in vitro differentiation.

"This simple strategy of shortly exposing pluripotent stem cells to an anti-cancer drug turned the transplant safer by eliminating the risk of tumor formation," said the leader of the study, Stevens Rehen, Professor at UFRJ and researcher at IDOR.

The research is scheduled to be published in the journal *Frontiers in Cellular Neuroscience*.

NeoStem announces extension of study under grant from California Institute of Regenerative Medicine (CIRM) to fund research of retinal disease

GLOBE NEWSWIRE
April 9, 2015

NeoStem, Inc. (Nasdaq:NBS), a biopharmaceutical company developing novel cell based personalized medicine therapies, announced today the extension of its study under a 2014 Early Translational grant from the California Institute of Regenerative Medicine for research leading to the development of a treatment for retinal diseases, including macular degeneration and retinitis pigmentosa.

Under the $4 million grant made to the University of California, Irvine, NeoStem is entitled, through a sub-award, to $1 million of new funds adding to the original half awarded. The goals of the research are to generate three-dimensional retinal tissue, to investigate the ability of adult induced pluripotent stem cells to restore sight in rodent models of retinal degeneration and to make eventual preparations for clinical use of the tissue.

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NEWS & NOTES

(CONT. FROM PAGE 4)

The grant supports a three-year study led by Dr. Hans S. Keirstead, President of NeoStem Oncology, and Dr. Magdalene J. Seiler, Project Scientist V at the University of California, Irvine, and its Sue & Bill Gross Stem Cell Research Center.

"This study exemplifies our commitment to utilizing non-dilutive funding sources for discovery programs exploring application of our technologies in other indications. A self-sustaining development pipeline depends on the generation of new development programs that are reasonable in terms of size of opportunity and clinical investment," said Dr. David J. Mazzo, Chief Executive Officer of NeoStem.

In the first year of the study, NeoStem fulfilled its primary goal of reproducibly generating retinal pigment epithelium (RPE) and layered retinal progenitor tissue, which include progenitors of the major retinal cell types including photoreceptors. This success enables the rest of the study, which will focus on transplantation and testing of these tissues in rodent models of retinal degeneration.

Approximately 11 million people in the United States have some form of age-related macular degeneration. This number is expected to double to nearly 22 million by 2050. Estimates of the global cost of visual impairment due to age-related macular degeneration is $343 billion, including $255 billion in direct health care costs.

Nine things to know about stem cell treatments

Many clinics offering stem cell treatments make claims that are not supported by a current understanding of science

International Society for Stem Cell Research

Stem cells have tremendous promise to help us understand and treat a range of diseases, injuries and other health-related conditions.

There is still a lot to learn about stem cells, however, and their current applications as treatments are sometimes exaggerated by the media and other parties who do not fully understand the science and current limitations, and also by "clinics" looking to capitalize on the hype by selling treatments to chronically ill or seriously injured patients.

It is important to discuss these Nine Things to Know and any research or information you gather with your primary care physician and other trusted members of your healthcare team in deciding what is right for you.

1. Currently, very few stem cell treatments have been proven safe and effective.
   - Beware of stem cell treatments offered without regulatory approval or outside the confines of a legitimate and registered clinical trial.
2. There is something to lose when you try an unproven treatment.
   - Unproven treatments present serious health, personal and financial considerations. Consider what might be lost and discuss these risks with your family and healthcare providers.
3. Different types of stem cells serve different purposes in the body.
   - Be wary of clinics offering treatments with stem cells originating from a part of your body unrelated to your disease or condition.
4. The same stem cell treatment is unlikely to work for different diseases or conditions.
   - View clinics that offer the same cell treatment for a wide variety of conditions or diseases with extreme caution. Be wary of claims that stem cells will somehow just know where to go and what to do to treat a specific condition.
5. The science behind a disease should match the science behind the treatment.
   - Your best protection against clinics selling unproven stem cell treatments is an understanding of the science behind your disease, injury or condition.
6. Cells from your own body are not automatically safe when used in treatments.
   - Every medical procedure carries risk; be wary of clinics that gloss over or minimize the risks associated with their treatments.
7. Patient testimonials and other marketing provided by clinics may be misleading.
   - Beware of clinics that use persuasive language, including patient testimonials on the Internet, Facebook and newspapers, to market their treatments, instead of science-based evidence.
8. An experimental treatment offered for sale is not the same as a clinical trial.
   - Beware of expensive treatments that have not passed successfully through clinical trials.
9. The process by which science becomes medicine is designed to minimize harm and maximize effectiveness.
   - Beware of clinics that circumvent the accepted process by which science becomes medicine.