FINDINGS

Nebraska Coalition for Lifesaving Cures
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Cures Save Lives

SAVE THE DATE

Monday, April 18, 2016

14TH ANNUAL TRIBUTE LUNCHEON
A Salute to Jim & Diny Landen

Board of Directors

FINDINGS

Nebraska Coalition *for Lifesaving Cures*

The Newsletter of the Nebraska Coalition for Lifesaving Cures

December 2015

NCLC loses long-time supporter

Long-time Nebraska Coalition for Lifesaving Cures board member, Fred Simon, passed away on Sept. 28. Simon was the executive vice president of Omaha Steaks, a company founded in 1917 by Simon's grandfather and great-grandfather.

Fred was instrumental in building his family's business into the nation's largest direct response marketer of premium beef and gourmet foods, but it was his love of the arts, or more specifically opera, that filled his time in later years. He served as chairman of the Nebraska Arts Council, Opera Omaha Foundation and board and was a member of the Executive Committee of Opera Omaha. In addition, he served on the board of the Santa Fe Opera, the Santa Fe Chamber Music Festival and the Nebraska Cultural Endowment.

In 2012, the Nebraska Coalition honored Fred and his wife Eve, with the Lifesaver Award, presented annually to individuals and couples who have made a difference in the world, and specifically Nebraska, through their passionate support of medical research.

Steve Bloch, longtime friend of Fred, was quoted in *The Jewish Press* saying,

> "Fred was a special man. His spirit was charitable; his love for his family, the arts, his synagogue and our community were passionate and his willingness and ability to show the way to others was exemplary."

Fred leaves behind his wife Eve, son Todd and daughter-in-law Betiana Simon; daughter Venus Simon French; step-son Richard and wife Sheila Ortega; step-daughter Marisa and husband Michael Mayhan; brother Alan and sister-in-law Anne Simon; and eight grandchildren.

Holland honored with lifetime achievement award

The Association of Fundraising Professionals Nebraska Chapter honored Richard "Dick" Holland, board chairman, with its Lifetime Achievement Award in November. Holland was nominated for the award by the Nebraska Coalition for Lifesaving Cures.

"Dick Holland's impact on the quality of life in Omaha and in the state of Nebraska can't be understated," said Victoria Kohout, executive director of the Nebraska Coalition for Lifesaving Cures. "Through his philanthropy, his lifetime of giving will impact generations to come."

Holland was also recognized for his life-long support of several causes including the arts. The Holland Performing Arts Center, which opened its doors in Omaha 10 years ago, was made possible in large part by Mr. Holland's philanthropy.

Dick Holland receiving the Lifetime Achievement Award for philanthropy, November 3, 2015

NEWS & NOTES

NCLC Supports State Science Meets

University of Nebraska Medical Center

The Nebraska Coalition for Lifesaving Cures donated $15,000 this year to the Nebraska Area Health Education Center (AHEC) to help support the nine regional science meets for approximately 500 eighth graders across Nebraska in 2016. Regional meets take place in Ainsworth, Crete, Fremont, Grand Island, Omaha, Norfolk, North Platte and Sidney.

The top students from the regional meets are invited to attend the State Science Meet at UNMC in Omaha on June 2-4, 2016. The state meet is designed to introduce students to careers in health care or science. During the state meet, students stay at the UNO Scott Residence Hall. Hands-on activities take place at the Peter Kiewit Institute of Information Science, Technology and Engineering, and at the University of Nebraska Medical Center. The students also visit Mahoney State Park, and on Saturday morning the event concludes with teams competing in the "Quiz Bowl".

Parkinson's IPS Cell Trial in Japan Switching to Allogeneic

The Niche
Knoepfler lab stem cell blog

In a major shift earlier this year, the induced pluripotent stem (IPS) cell trial in Japan for treatment of macular degeneration (MD) switched gears from using the patients' own cells (called "autologous") to using banked cells from other people, termed "allogeneic".

Dr. Masayo Takahashi, the leader of this MD trial, indicated the main reason was due to regulatory changes related to stem cells in Japan.

Now a second clinical study in Japan is also using IPS cells but as the basis for treatment of Parkinson's Disease (PD). The PD trial, run by Dr. Jun Takahashi, reportedly will also switch to focus on allogeneic cells.

The advantages of allogeneic cells include the fact that they can be validated and batch-prepared in advance. In theory, in this allogeneic system there might be no waiting period for patients while their own cells are turned into IPS cells. However, finding matches from a bank of IPS cells may prove somewhat difficult for allogeneic use for some patients.

Together these changes in IPS cell clinical plans suggest a significant, broader shift in the field potentially toward allogeneic use of IPS cells.

Stem Cell Stories That Caught Our Eye: Three Teams Refine Cell Reprogramming, Also Stem Cell Tourism

CIRM: The Stem Cellar
Don Gibbons

Getting the cells needed faster. Pretty much everyone's cell therapy wish list contains cells that genetically match the patient, and often with the added feature of genetic modification to correct an in-born error. We have the technology to do this. But the combined processes can take three months or more; time patients often don't have.

Researchers at the University of Wisconsin's Morgridge Institute and the Murdoch Children's Research Institute in Australia have sped up that process to just two weeks. They found a way to do the stem cell conversion and the genetic correction at the same time and used the trendy new gene-editing tool, CRISPR, which is faster and simpler than other methods.

When iPS-based therapies become a reality, the faster method will be critical for certain patients such as children with severe immune deficiency or people with rapidly deteriorating vision.

Duchenne Muscular Dystrophy is a Stem Cell Disease

Nature Medicine via Medical Press
Ottawa Hospital Research Institute

A new study from The Ottawa Hospital and the University of Ottawa is poised to completely change our understanding of Duchenne muscular dystrophy and pave the way for far more effective treatments.

"For nearly 20 years, we've thought that the muscle weakness observed in patients with Duchenne muscular dystrophy is primarily due to problems in their muscle fibres, but our research shows that it is also due to intrinsic defects in the function of their muscle stem cells," said Dr. Michael Rudnicki, senior author of the study. "This completely changes our understanding of Duchenne muscular dystrophy and could eventually lead to far more effective treatments."

Dr. Rudnicki is the Director of the Regenerative Medicine Program at The Ottawa Hospital and a professor at the University of Ottawa. He also holds the Canada Research Chair in Molecular Genetics.

This research was conducted in mouse cells, but it is expected that the findings will hold in humans, as the dystrophin protein is almost identical in all animals.

FROM THE PRESIDENT

Notes from the World Stem Cell Summit, Atlanta GA

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

The 12th annual meeting of the World Stem Cell Summit (WSCS) in Atlanta was held December 10-12. Presentations covered all aspects of stem cells from basic science discoveries to progress in clinical applications as well as the important connections to the supporting corporate biosciences and major advocacy organizations. It was very apparent that stem cell science and the clinical applications using these remarkable cells continue to move forward at an almost frantic pace and include a wide range of stem cell types.

Many sessions of the meeting focused on the FDA and the regulatory framework that oversees proposed clinical applications of this variety of stem cell sources: adult, embryonic, fetal, cord blood, mesenchymal, placental, adipose, induced, etc. It was clear that all cellular sources remain important in the research and potential application and that the regulatory guidelines that help control such activities have struggled to keep up with the rapid pace of biological and medical discoveries. Central to these discussions is when and how does the FDA consider a "stem cell product" to be a drug. The new Deputy Commissioner of the FDA, Dr. Robert Califf, emphasized their desire to accelerate translation from the laboratory preclinical studies into safe and effective clinical therapies. The adjectives of "safe and effective" became a continuing theme, particularly in the face of many "stem cell clinics" in the U.S. and abroad that really step outside the boundaries of accepted medical practices. The FDA has issued a number of "Guidance" documents in the past two years as a stop-gap measure prior to formal regulations being implemented. Indeed, because of the many concerns expressed by the attendees, Dr. Califf invited members of the public, scientists and clinicians to contact the FDA directly if they have concerns and specific information about clinics or practitioners advertising "stem cell treatments" that seem too good to be true. Many of them are exactly that.

Nonetheless, the number of valid, FDA approved and registered clinical trials continue to rise. Trials with embryonic stem cell (ESC) derived cells are ongoing for Type I diabetes, spinal cord injury and macular degeneration. They continue to move forward without significant adverse events for the patients (for example, no tumors, no rejection issue and no other bad reactions). Very surprisingly, trials with induced pluripotent stem cell (iPSC) derived cells are already underway in Japan and about to start in the U.S. These will be focused on the same diseases as the studies with ESC but have been expanded to include Parkinson's disease.

Some of the most interesting findings presented at the WSCS were related to clinical applications employing cells derived from umbilical cord blood stem cells (UBSCs), adipose derived stem cells (ASC) and mesenchymal stem cells (MSC). Detailed work with these cells all over the world is revealing their potential power in a variety of diseases. These include in repair of: sports related musculoskeletal injuries (cartilage tears, bone healing, etc); post-surgery or soft-tissue injury (breast cancer reconstruction, traumatic bone/tissue injuries, etc); immune modulation in tissue or bone marrow transplants, and many other areas. As the biology of these uses is better understood, it now appears that the majority of the positive effects seen are not due to any cell or tissue replacement activity from any "stem cells" in the product, but rather to other pro-regenerative effects induced by the cells. These include suppression of inflammation, production of anti-fibrotic factors, stimulation of growth of new vasculature and general augmentation of immune or resident stem cell responsiveness.

Probably the most memorable report came from Dr. John Wagner at the University of Minnesota (Cell Stem Cell, December 2015). His group has developed a procedure that augmented the effectiveness of UBSCs to engraft in clinical transplants and revealed a potential to use the approach in grafts normally restricted to adult bone marrow. This growth factor and culture-based system led to the near elimination of graft versus host disease and more rapid engraftment with greatly enhanced recovery of platelets and neutrophils. He also speculated that it may be possible to "bank" the culture and growth factor modified cells as a product useful in many therapies now restricted to adult bone marrow.

All in all, the continued progress in stem cell based therapies gives great hope that more and more diseases will be successfully addressed using these approaches. The continued availability of stem cells from all sources will clearly enhance the progress of these important therapies.