findings
The Newsletter of the Nebraska Coalition for Lifesaving Cures Winter 2013
Nebraska Coalition for Lifesaving Cures

Nebraska Coalition to honor Dr. Harold M. and Beverly Maurer

The Nebraska Coalition for Lifesaving Cures will honor Dr. Harold M. and Beverly Maurer at its annual tribute luncheon, to be held April 28, 2014 at the Happy Hollow Club.

Dr. Maurer came to the University of Nebraska Medical Center as medical dean in 1993 and quickly rose to chancellor of UNMC in 1998.

During his tenure, Dr. Mauer tripled its external research funding, with research dollars now at almost $100 million annually. He secured $14 million in tobacco settlement funds from the state to support biomedical research; created a vice chancellor for research position at the medical center; developed programs in regenerative medicine, nanomedicine, drug delivery and bioterrorism preparedness; raised more than $150 million in private support to construct the Durham Research Center and Durham Research Center II, which combined have more than 200 research laboratories.

In total, Dr. Maurer has raised close to $1 billion in philanthropy for the medical center.

"I would like for my legacy to be, 'he built research at UNMC,'" Dr. Maurer said. "I'm also very proud of our many accomplishments in education, clinical care and community outreach."

One very visible honor for the Maurers came in 2011 when, at the insistence of donors the new public health building was named the Harold M. and Beverly Maurer Center for Public Health.

In September of 2012, Dr. Maurer announced that he would make the transition to a fundraising role for the new UNMC cancer center at the Foundation.

Dr. Harold M. and Beverly Maurer

Beverly Maurer is a former school principal, serves on numerous civic boards, including the Joslyn Museum and the Omaha Community Playhouse and is a full-time volunteer at UNMC. She assists with recruiting, orchestrating functions, developing community support and fundraising.

It was Beverly, along with a handful of others in 2001 who saw a need for an organization supporting open and free research in the state of Nebraska and who founded Nebraskans for Research, the organization which joined with and became Nebraska Coalition for Lifesaving Cures.

Hal and Beverly have been married for more than 50 years. They have two daughters and four grandchildren.

FROM THE DESK OF

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

The 2013 year is coming to a close and the work of stem cell research continues to advance at a pace that challenges even the dedicated scientist. Over the next few months, either through our printed newsletters or our e-Newsletters, I will attempt to summarize the two or three findings that have stood out so far and look forward to what we can expect in the year to come.

In the realm of basic research studies it is hard to ignore how prominent the rapid advances related to induced pluripotent stem cells (iPSC) have become. Indeed, Dr. Shinya Yamanaka was awarded the Nobel Prize for his discovery of this process just a year ago. As a reminder, iPSC are derived from mature normal cells (skin, blood cells or almost anything else), not an embryo. They have been biochemically directed by the investigator to take on the characteristics of pluripotent embryonic stem cells (ESC) by exposing them to appropriate factors, typically in a culture system.

The factors used to coax these cells from their comfortable mature state into their highly flexible, experimentally useful, immature state like ESC, were usually factors discovered by studying actual ESC lines. Recall that these ESC lines were derived after extensive informed consent from left-over, frozen in vitro fertilization embryos slated for discarding.

In iPSC research, over the past year investigators have made great progress in deriving many such iPSC lines from patients with existing medical conditions that are not well understood (Alzheimer's, Parkinson's, fragile X syndrome, Huntington's syndrome, muscular dystrophy, autism, diabetes, and many other congenital diseases). Generally, the intent is not to produce cells that could be used for treating the same patient, but to develop model systems that may recapitulate the developmental defect and allow better understanding of the nature of the disease and even the development of disease-specific drugs. In an article which appeared in *Science*, the advent of reprogramming mature cells by "cellular engineering" will facilitate the non-transplant therapy of these difficult diseases (Doulatov & Daley, Nov. 2013) but does not preclude the potential use of iPSC in transplantation (Rao, Stem Cell Reports, Oct. 2013). In related studies, a better understanding of the same biochemical signaling factors may also lead to an ability to "regrow" or otherwise regenerate/repair damaged tissues (Science Daily, Nov. 2013).

In coming months, I will look at the domain of stem cell clinical trials and the progress with therapies based on ESC, which has been slow but ongoing.

I will also examine the enthusiasm for "stem cell cures" which has led to an explosion of clinics in the US and abroad, that are willing to offer untested and potentially dangerous therapies using a bewildering variety of "stem cell" transplants, cell extracts or even topical creams and ointments.

Finally, I want to thank all of our members for your continuing support of the mission of the Coalition – which is to promote, support and advocate research to advance our quality of life and economy.

Nebraska Coalition to host stem cell advocate as part of The Nebraska Science Festival

Dr. Paul S. Knoepfler

The Nebraska Coalition for Lifesaving Cures will host Paul S. Knoepfler, Ph.D., an associate professor of Cell Biology and Human Anatomy at University of California Davis School of Medicine during the Nebraska Science Festival.

Dr. Knoepfler studies stem cell and cancer cell epigenetics at his lab. The big picture goals of his lab are to make stem cell-based regenerative medicine therapies safer and to develop innovative cancer therapies.

He teaches histology to medical students and developmental biology to graduate students. He is also a cancer survivor as well as patient advocate and is the recipient of the Genetic Policy Institute 2013 National Advocacy Award, previously won by Michael J. Fox.

Knoepfler is the author of the new book, Stem Cells: An Insider's Guide and was named in 2013 as one of the top 50 most influential people in the world in the stem cell field.

When he should be enjoying REM sleep, he writes the popular "Knoepfler Lab Stem Cell Blog", located online at **www.ipscell.com**.

Schedule of Events

- **Friday, April 25, 2014** | Lincoln
- **Saturday, April 26, 2014** | Omaha

Both events open to the public

UNMC announces Richard Holland Future Scientist Award winners

Six undergraduate students from four Nebraska colleges and universities recently received the 2013 Richard Holland Future Scientist Award from the Nebraska Coalition for Lifesaving Cures

Pictured left to right are:
Taylor Mighell, Gloria Larson, Lana Zholudeva, Shanice Harris, Lisa Poppe.

(Not pictured: Karl Krieser)

Six undergraduate students from four Nebraska colleges and universities recently received the 2013 Richard Holland Future Scientist Award from the Nebraska Coalition for Lifesaving Cures.

The students received cash prizes totaling $2,700 on Aug. 6 at the annual INBRE (Institutional Development Award (IDeA) Networks of Biomedical Research Excellence Program) conference in Grand Island.

The awards are named in honor of Richard Holland, an Omaha philanthropist and longtime supporter of research.

The students were judged in two categories, representing oral and poster presentations of the research work they conducted this summer as part of the INBRE program.

The INBRE program is overseen by James Turpen, Ph.D., professor of genetics, cell biology and anatomy at UNMC. Dr. Turpen is the principal investigator of the $17.2 million National Institutes of Health grant that funds the program.

Established in 2001, the INBRE Scholars program was created to expose students to serious biomedical research and build a statewide biomedical research infrastructure between undergraduate and graduate institutions.

The students, called INBRE scholars, enter the program after completing their sophomore year of college upon recommendation of their college professors. Each undergraduate school nominates approximately three students a year for the program.

The award winners and their hometowns are listed below.

Oral

1st place: Karl Krieser, Omaha

2nd place: Shanice Harris, Dickens

3rd place: Lisa Poppe, Fremont

Poster

1st place: Lana Zholudeva, Omaha

2nd place: Gloria Larson, Omaha

3rd place: Taylor Mighell, Geneseo, Ill.

Regenerating Heart Tissue Through Stem Cell Therapy

November 6, 2013 | *Mayo Clinic's Discovery's Edge*

Two years after a heart attack left him too exhausted to even tend his garden, Miroslav Dlacic is now able to walk again without becoming worn out. An international team of Mayo Clinic doctors and researchers, led by André Terzic, M.D., Ph.D., director of Mayo Clinic's Center for Regenerative Medicine, helped Dlacic reclaim his life by discovering a way to regenerate heart tissue through stem cell therapy.

For decades, treating cardiac patients has typically involved managing heart damage with medication. It's a bit like driving a car without fixing a sluggish engine; you manage the consequences as best you can and learn to live with them. But in collaboration with European colleagues, Mayo Clinic researchers have discovered a revolutionary means of repairing a damaged heart—of actually fixing the machine.

"It's a paradigm shift," says Dr. Terzic. "We are moving from traditional medicine, which addresses the symptoms of disease, to being legitimately able to cure disease."

In this breakthrough process, stem cells are harvested from a cardiac patient's bone marrow. They undergo a laboratory treatment that guides them to become cardiac cells. The treated cells are then injected into the patient's heart in an effort to grow healthy heart tissue. The study is the first successful demonstration in human beings of the feasibility and safety of transforming adult stem cells into cardiac cells.

"We guide the stem cells to become something useful, in this case, cardioprogenitors," Dr. Terzic explains.

This discovery has implications for millions of people. Cardiovascular disease is the leading cause of death worldwide. In the U.S. alone, about 5.8 million people have heart failure, and the number is growing.

Researchers model familial amyloidosis in vitro using iPSC technology

November 25, 2013 | *National Institutes of Health*

Researchers from Boston University School of Medicine (BUSM) and Boston Medical Center (BMC) have generated the first known disease-specific induced pluripotent stem cell (iPSC) lines from a patient with familial transthyretin amyloidosis (ATTR). The findings, which are reported in *Stem Cell Reports*, may lead to new treatments for genetic diseases such as familial amyloidosis.

Familial transthyretin amyloidosis is a slowly progressive condition characterized by the buildup of abnormal deposits of a protein in the body's organs and tissues. In ATTR, protein secreted from the liver aggregates and forms fibrils in target organs, chiefly the heart and peripheral nervous system, highlighting the need for a model capable of duplicating the multisystem complexity of this clinically variable disease.

According to researchers using iPSC technology, cell lines can be established that are genetically identical to the individual from whom they are derived, allowing for disease modeling and development of novel therapeutics in the personalized genetic context of the patient from which they are made.

In this study, the researchers used the iPSC to generate liver cells that secrete the disease-specific mutant protein as well as cardiac and neuronal cells, the downstream target tissues of the disease. Upon exposure to the mutant protein, the heart and neuronal cells displayed signs of stress and an increased level of cell death as compared to those exposed to normal protein, thereby recreating essential aspects of the disease in vitro. Furthermore, small molecule stabilizers of the mutant protein that are being tested in clinical trials show efficacy in this model, validating this iPSC-based, patient-specific in vitro system as a platform for testing therapeutic strategies.

Nebraska Science Festival To Return

Second Annual Event, Co-Sponsored by Nebraska Coalition for Lifesaving Cures, will run from April 24-27, 2014

The Nebraska Science Festival returns in 2014 with expanded programming that offers greater opportunities for students of all ages.

In its second year, the Nebraska Science Festival – scheduled for April 24-27, 2014 – features an array of science- and technology-related activities, as well as extends programming into Lincoln as it builds toward being a statewide celebration.

Headline presenter Adam Savage, co-host of Discovery Channel's "Mythbusters"

Adam Savage, co-host and executive producer of Discovery Channel's "Mythbusters," will headline the 2014 Nebraska Science Festival.

On April 25, Savage will present at Joslyn Museum's Witherspoon Concert Hall. His 7:30 p.m. presentation is free to the public and is suitable for ages 12 and older. Tickets are available first-come, first-served at Joslyn Museum between 10 a.m. and 4 p.m. on Jan. 25 or 26.

New in 2014, the free Science Expo will be open to school groups over the course of two days and at two different locations: The Durham Museum in downtown Omaha and the Strategic Air and Space Museum near Ashland, Neb.

Teachers may register online at www.nescifest.com to bring their elementary-age students (ideally third- through fifth-graders) to the Science Expo at either:

- The Durham Museum: Field trip choices are from 8:30 to 11:15 a.m. or 11:30 a.m. to 2:15 p.m. on both April 24 and 25
- The Strategic Air and Space Museum: Field trip choices are from 8:30 a.m. to 11:15 a.m. or 11:30 a.m. to 2:15 p.m. on both April 24 and 25

The Science Expo at The Durham Museum will be open to the public on April 26 from 9 a.m. to 1 p.m.

Also, proposals are being taken for hands-on, kid-friendly science booths and presenters at the Science Expos, as well as community sites that want to host an event for the general public. Visit **www.nescifest.com** for more info or to register as a booth and/or community site.

Science Festival announcements will be made as sites – and programming – are confirmed.

Presented by the University of Nebraska Medical Center, the Nebraska Science Festival is a growing collaboration 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 The Nebraska Coalition for Lifesaving Cures and media sponsors KETV and the Omaha World-Herald.

Progress Toward Therapies:
Projects in the Pipeline

Source: California Institute for Regenerative Medicine

Alzheimer's Disease

$20,000,000 University of California, Irvine *(StemCells, Inc.)*

This project proposes to evaluate the use of human neural stem cells as a potential innovative therapy for AD. AD results in neuronal death and loss of connections between surviving neurons. The hippocampus, the part of the brain responsible for learning and memory, is particularly affected in AD, and is thought to underlie the memory problems AD patients encounter. Evidence from animal studies shows that transplanting human neural stem cells into the hippocampus improves memory, possibly by providing growth factors that protect neurons from degeneration. Translating this approach to humans could markedly restore memory and thus, quality of life for patients.

Heart Disease

$19,999,899 Stanford University *(The J. David Gladstone Institutes)*

Stem cell therapy may be a promising strategy for improving heart failure patient outcomes by transplanting cells rather than a whole heart. Several studies have convincingly shown that human embryonic stem cells can be differentiated into heart muscle cells (cardiomyocytes) and that these cells can be used to improve cardiac function following a heart attack.

ALS/Lou Gehrig's Disease

$17,842,617 Cedars-Sinai Medical Center, Los Angeles

This project aims to use a powerful combined neural progenitor cell and growth factor approach to treat patients with amyotrophic lateral sclerosis (ALS or Lou Gehrig's Disease). Human neural progenitor cells found early in brain development can be isolated and expanded in culture to large banks of billions of cell. When transplanted into animal models of ALS, they have been shown to mature into support cells for dying motor neurons called astrocytes.

findings

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