Nebraska Coalition
FOR LIFESAVING CURES

FINDINGS

FALL 2017 NEWSLETTER

Ten Undergraduate Students Awarded Holland Future Scientist Prizes

(Reprinted with the permission of the UNMC public relations department)

Ten undergraduate students from five Nebraska colleges and universities recently received the 2017 Richard Holland Future Scientist Award from the Nebraska Coalition for Lifesaving Cures.

The students received cash prizes totaling $5,000 at the annual INBRE (Institutional Development Award (IDeA) Networks of Biomedical Research Excellence Program) conference on August 8 in Nebraska City.

The awards are named in honor of the late Richard Holland, an Omaha philanthropist and longtime supporter of research. This is the 10th year the Holland Future Scientist Awards have been awarded.

The students were judged for their oral and poster presentations of the research work they conducted this summer as part of the INBRE program.

The 2017 INBRE Award Winners

Pictured from left to right are the five oral presentation winners: Christina Ternent, Jessica Hotovy, Marlene Djidjoho, Robyn Scott and Alison Guyer.

Oral Presentation Winners:
First: Alison Guyer, Creighton University;
Second: Christina Ternent, College of Saint Mary;
Third: Jessica Hotovy, University of Nebraska-Lincoln;
Honorable Mention: Robyn Scott, Creighton University; and
Honorable Mention: Marlene Djidjoho, College of Saint Mary

Pictured from left to right are the five poster presentation winners: Truc Doan, Harim Won, Tiffany Truong, Rebecca Zawistowski and Samantha Stoupa.

Poster Presentation Winners:
First: Harim Won, University of Nebraska at Omaha;
Second: Rebecca Zawistowski, Creighton University;
Third: Samantha Stoupa, Creighton University;
Honorable Mention: Tiffany Truong, University of Nebraska-Lincoln; and
Honorable Mention: Truc Doan, Doane University

The INBRE program is overseen by James Turpen, Ph.D., associate vice chancellor for academic affairs at the University of Nebraska Medical Center. Dr. Turpen is a professor in the UNMC Department of Genetics, Cell Biology and Anatomy and is the principal investigator of the $16.2 million grant funded by the National Institute of General Medical Sciences of the National Institutes of Health 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, referred to as INBRE scholars, enter the program after completing their sophomore year of college upon recommendation of their college professors. It is a two-year comprehensive training program to prepare the students for graduate school.

FROM THE PRESIDENT

Dr. David Crouse
President, Nebraska Coalition for Lifesaving Cures

In June of 2016 in this Newsletter, we pointed out that the International Society for Stem Cell Research (ISSCR) had produced a set of Guidelines for Stem Cell Research and Clinical Translation that included comments addressing Stem Cell-Based Marketing Innovation. More specifically, they had a sidebar "WARNING ON THE MARKETING OF UNPROVEN STEM CELL-BASED INTERVENTIONS." To quote this section directly: "… as a rule, stem cell-based products should rarely, if ever, be developed outside of a formal clinical trials process." The established medical and research communities have fully supported an increased attention regarding clinics offering unproven treatments.

In August 2016, Leigh Turner and Paul Knoepfler published a widely discussed Open Access article entitled "Selling Stem Cells in the USA: Assessing the Direct-to-Consumer Industry" (Cell Stem Cell 19:154-157, 2016). In their survey they identified "… 351 U.S. businesses engaged in direct-to-consumer marketing of stem cell interventions offered in 570 clinics." These businesses have a nationwide geographic distribution but much higher density in California, Texas and Florida. The various clinics pose a bewildering variety of putative stem cell and regenerative medicine products to treat nearly every disease or condition that ails mankind! In a previous publication this has been described as exhibiting a wild west or cowboy culture. In addition to the obvious ethical issues that underlie making unfounded claims about these proposed therapies, they also make it clear that there are real safety and efficacy issues in such treatments.

Fortunately, in the past few weeks the Food and Drug Administration (FDA) has targeted some larger clinics touting the wide range of treatments using their unproven stem cell or regenerative medicine therapies. On August 24, 2017, one of these clinics, US Stem Cell Clinic, LLC, received a very pointed Warning Letter that identified in great detail the specific violations that required prompt action to avoid possible "…seizure and/or injunction." This clinic claimed to treat ALS, Parkinson's, rheumatoid arthritis, COPD, heart disease, pulmonary fibrosis, Crohn's, colitis, lupus, diabetes and a number of other diseases or conditions. They proposed to use autologous (your own) adipose tissue as a source of cells and other materials to provide these treatments. In essence, the FDA reiterated its position that cells or cell products that are "…more than minimally manipulated" or that are not "…intended for homologous use" require FDA review and approval prior to clinical application. The US Stem Cell Clinic therapeutic products were both more than minimally manipulated and used in a site that was not homologous. No FDA review or approval had been sought. On inspection of the actual clinic, the inspectors also pointed out many other violations related to lack of sterility, laboratory controls, clinic environment, adequate written procedures, record keeping, quality control and several other deficiencies. The publically available letter is very informative and we encourage reviewing it on **nebraskacures.com**

> *"The FDA will not allow deceitful actors to take advantage of vulnerable patients by purporting to have treatments or cures for serious diseases without any proof that they actually work."*
>
> —**Dr. Scott Gottlieb**
> FDA Commissioner

A few days after this letter, the FDA acted even more aggressively toward StemImmune Inc. operating a set of clinics called California Stem Cell Treatment Centers

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Former Quadriplegic Patient Finds Hope from Clinical Trial

Lucas at the Milwaukee Brewers game. (Photo courtesy of Caitlin Moyer.)

Lucas Lindner suffered a severe spinal cord injury when his car swerved off the road into a tree to avoid hitting a deer in May 2016. He was flown to the hospital and received immediate surgery to stabilize his spine. He was left without the ability to move his limbs below the neck and upper arms.

In June 2016, Lucas participated in an investigational stem cell therapy for complete cervical spinal cord injury, receiving 10 million cells of AST-OPC1 in Asterias Biotherapeutics' ongoing clinical study at the Medical College of Wisconsin.

Lucas has since regained the use of his fingers, hands and lower arms and in August threw out the ceremonial first pitch at a Milwaukee Brewers' baseball game.

"Throwing out the first pitch at a Major League game is not something I could have imagined a year ago," said Lucas. "I want to show everyone that there is hope that spinal cord injury patients can regain function. I am looking forward to going back to school, pursuing my dream of working in the IT field and living independently someday."

Asterias has now completed enrollment and dosing in four of the five planned SCiStar study cohorts and enrolled 22 patients in the SCiStar study. Twenty-seven patients have been administered AST-OPC1 after including patients from a previous Phase 1 safety trial, and results-to-date continue to support the safety of AST-OPC1. In June 2017, Asterias reported nine month data from the AIS-A 10 million cell cohort that showed improvements in arm, hand and finger function observed at three and six months following administration of AST-OPC1 were confirmed and in some patients further increased at nine months. The company intends to complete enrollment of the entire SCiStar study later this year, with multiple safety and efficacy readouts anticipated during the remainder of 2017 and 2018.

> *"I want to show everyone that there is hope that spinal cord injury patients can regain function."*
>
> —Lucas Lindner

PRESIDENT (continued)

treating patients in Rancho Mirage and Beverly Hills, CA. US Marshalls Service seized vials of vaccinia virus that were used to create a dangerous, unapproved stem cell product for treating cancer patients. The FDA Commissioner, Dr. Scott Gottlieb, stated that "The FDA will not allow deceitful actors to take advantage of vulnerable patients by purporting to have treatments or cures for serious diseases without any proof that they actually work."

Unfortunately, at least one of these rogue clinics is now located at three sites in Nebraska and proposes to treat a similarly enormous range of conditions. They have recently placed a detailed ad in a local business publication. In that ad, in addition to more common orthopedic conditions and those in the previous list cited by the FDA, they propose to treat aging, MS, stroke, asthma, erectile dysfunction, fibromyalgia, osteoporosis, osteoarthritis and scleroderma among even more. How is that even believable? It is our hope that the FDA, along with local clinical practitioners, will help to end this kind of misleading advertising and better control such practices.

Glaucoma Breakthrough by UNMC Research Team Published in Journal **Stem Cells**

Professor Iqbal Ahmad, Ph.D., on the right.

(Reprinted with permission from the UNMC public relations department)

A University of Nebraska Medical Center researcher has discovered that a common form of glaucoma that strikes adults may have early origin. The discovery, which is detailed in the August 9 issue of the journal *Stem Cells*, could result in earlier diagnosis and treatment of the disease that is the second leading cause of irreversible blindness and affects more than three million people in the United States and 60 million people worldwide.

Iqbal Ahmad, Ph.D., a professor in the department of ophthalmology and visual sciences at UNMC, led the team of investigators. He has spent more than a decade studying the stem cell approach to understand and treat glaucoma, which is called a silent robber of vision because it strikes without warning or any noticeable symptoms.

"There are several forms of glaucoma but all have two things in common—the progressive degeneration of retinal ganglion cells (RGCs) and the irreversible loss of vision," Dr. Ahmad said.

The primary function of RGCs is to tell the brain through a series of synapses and connections what the eye sees, he said. Without RGCs, there is no perception of vision.

Since glaucoma is generally a late onset disease and RGCs are formed during gestation, Dr. Ahmad's team had to find a way to study the degeneration process, which they hypothesized was because of a developmental abnormality.

Using blood from patients carrying a specific gene variation and also suffering from primary open angle glaucoma (POAG), one of the more common forms of the disease, Dr. Ahmad and his team created a pluripotent stem cell-based model of POAG to understand why and how RGCs degenerate.

Dr. Ahmad's team was able to show that RGCs from POAG patients were different from those generated from healthy donors.

"They were developmentally abnormal in form, function and gene expression," he said, adding that knowing the molecular basis of the defect and its biomarkers will allow early diagnosis and treatment.

"We are excited, as it is an important first step toward early diagnosis and treatment of this debilitating disease," Dr. Ahmad said.

Shane Haven, M.D., a glaucoma specialist at UNMC's Truhlsen Eye Institute, said, "Dr. Ahmad's work could help us better understand the pathophysiology of degenerative conditions and in turn, reveal new treatment targets and cell replacement therapies."

NCLC Begins Strategic Planning for the Future

The Nebraska Coalition for Lifesaving Cures board of directors is working through a strategic planning process. Last spring the board held two sessions to discuss and develop an innovative plan. At that time three strategic directions were established: education initiative, increasing capacity and strengthening governance.

The board also approved new vision and mission statements:

Vision statement: Leading society to understand and embrace the importance of scientific research.

Mission statement: The Nebraska Coalition for Lifesaving Cures promotes, supports and advocates research to advance our quality of life and our economy.

Four new committees have been formed as a result of the new strategic plan: Governance, Education, Development and Nominating.

16TH ANNUAL TRIBUTE SPRING LUNCHEON

SAVE — THE — DATE

MONDAY, APRIL 30TH, 2018

Honoring

Ken Cowan, M.D., Ph.D.
Director,
Fred & Pamela Buffett Cancer Center and Eppley Institute

and

Alison Freifeld, M.D.
Professor,
Internal Medicine
Division of Infectious Disease

Monday, April 30, 2018
Happy Hollow Club
1701 South 105th Street
Omaha, Nebraska

An Omaha "Light" Has Gone Out

Eunie Denenberg

Everyone thought they were Eunice Denenberg's best friend, because whenever she was talking to you, you were the only person in the room.

Eunie died Thursday, September 14, of an aneurism at University of Nebraska Medical Center. She had been diagnosed with colon cancer two years ago and was given about a year to live, but never slowed down.

A founding member of Nebraskans for Research, which later became Nebraska Coalition for Lifesaving Cures, Eunie gave 100 percent to whatever she set her mind to. At her funeral, her daughter Debbie said of her mother that she was "light" — a force of energy and positivity that was unstoppable.

Eunie graduated from Central High and Omaha University (now the University of Nebraska at Omaha). She and Norman Denenberg married in 1950, and they had three children. Besides Debbie, a theater producer, they are Larry Denenberg of Newton, Massachusetts, senior technical manager for Trip Advisor; and Dr. Steve Denenberg of Omaha, a facial plastic surgeon. She also is survived by daughters-in-law Rachael Rosner and Tippi Denenberg and nine grandchildren.

FINDINGS

FALL 2017 NEWSLETTER

Nebraska Coalition for Lifesaving Cures
900 S. 74th Plaza, Ste. 301
Omaha, NE 68114

402.390.2461
nebraskacures.com

SAVE THE DATE:

16th Annual Tribute Spring Luncheon on April 30th

Honoring
Ken Cowan, M.D., Ph.D. and
Alison Freifeld, M.D.