COMMUNITY HEALTH CHARITIES℠ NEBRASKA

CHARITY NEWS & UPDATES

The ALS Association Mid-America Chapter

- The discovery of the NEK-1 gene, now known to be among the most common genes that contribute to the development of ALS, made headlines around the globe. More than 80 researchers in 11 countries conducted this largest-ever study of inherited ALS.
- The U.S. Food and Drug Administration (FDA) accepted, and is currently reviewing, a New Drug Application (NDA) for Edaravone for the treatment of ALS.
- One of the nation's largest precision medicine programs began enrolling in October, with nine centers at universities and hospitals across the United States. The ALS Association committed $3.5 million in Ice Bucket Challenge-raised money to this exciting collaborative effort.

Alzheimer's Association Nebraska Chapter

- Aducanumab, an experimental Alzheimer's disease treatment, seemed to slow cognitive decline in a small, preliminary study. The findings from this early phase clinical trial are encouraging, but additional testing is needed in a larger number of people for longer periods of time.
- An unprecedented $414 million FY2017 budget increase for Alzheimer's research, education, outreach and caregiver support stemming from the efforts of Alzheimer's Association advocates was passed swiftly by Congress. The Association commends the bipartisan work of our elected officials to make Alzheimer's disease a national priority.
- With the evolution of Alzheimer's research, the progress being made in the area of biomarkers has the potential to more accurately identify people with disease-related brain changes, at an earlier point in the biological process. Research to validate biomarkers for Alzheimer's disease is one of the most active areas in Alzheimer's science.

American Diabetes Association of Nebraska

- **Accelerator/New to Diabetes Research Diabetes Research Grant:** Project focuses on insulin action and signal transduction in persons with Type 2 Diabetes.
- **Nitrate supplementation and exercise tolerance in patients with type 2 diabetes:** The proposed study will examine whether dietary nitrate supplementation increases bioavailable NO and subsequently improves 1) blood flow and oxygen delivery to skeletal muscle, 2) utilization of oxygen within skeletal muscle and 3) ultimately exercise capacity and tolerance in patients with T2D.
- **The role of prolactin receptor signaling in maternal glucose homeostasis and islet function:** The project focuses on Gestational Diabetes –Pregnancy/Beta Cell Mass in Diabetic Pregnancy.

American Lung Association in Nebraska

- The American Lung Association is a leader in asthma education, in addition to presenting health education programs about tobacco use and chronic obstructive pulmonary disease. Their goal is to prevent lung disease through smoking cessation, promoting clean air environments and more.
- The American Lung Association also supports basic and clinical research through training and "seed" grants for researchers who are at the early stage of their careers. As part of its commitment to research, the American Lung Association created the Asthma Clinical Research Centers Network (ACRC), which seeks to conduct large clinical trials that will provide useful information about asthma and benefit patients directly.

Brain Injury Alliance of Nebraska

- There is a growing body of evidence revealing the positive effects exercise has on brain structure and cognitive function following a traumatic brain injury. It is proposed that exercise assessment and aerobic exercise training may reduce concussion-related physiological dysfunction by restoring autonomic balance and improving autoregulation of cerebral blood flow. A speaker at the 2017 Brain Injury Conference will discuss the details of these studies and use of exercises in rehabilitation.

Crohn's & Colitis Foundation of America, Nebraska/Iowa Chapter

- **Microbiome Initiative**
    - People carrying different IBD genes, such as NOD 2, have different compositions of gut bacteria. A person's genes can therefore affect which gut bacteria they host. Different subsets of IBD can be characterized by signature bacterial compositions, defined by risk signature of ~20 microbial families. A novel way to study intestinal bacterial metabolism in humans have been identified.
- **Pediatric Risk Study**
    - 17 genes are expressed differently in intestines of patients who developed a stricture. The group is developing a model for the expression of these genes to be used as predictors of risk for stricture development. Children diagnosed under 10 years of age have distinct blood profile antibodies in their blood. These results suggest that there are distinct mechanisms of CD in patients < 10 years.
- **Genetics Initiative**
    - 200+ genes associated with IBD have been discovered, as well as 5 high-priority genetic pathways.

Cystic Fibrosis Foundation – Nebraska Chapter

- During the past two decades, several new drugs/therapies have come to market for cystic fibrosis patients. The Cystic Fibrosis Foundation funded many of these drugs, which treat the symptoms of the disease; i.e., excess mucous, inflammatory response, antibiotics and digestive issues. As a result, more than 50% of our patients are over the age of 18 and our patient population has more than doubled since 1996. Recently, the U.S. Food and Drug Administration (FDA) approved the use of lumacaftor/ivacaftor (Orkambi®) today for children with cystic fibrosis ages 6 to 11, who have two copies of the most common CF gene mutation, F508del. The decision means that about 2,400 additional children in the U.S. are eligible to receive the drug, bringing the total number of those eligible for the treatment in the U.S. to nearly 11,000.

JDRF International – Heartland Chapter

- More than 80 percent of JDRF's expenditures directly support this goal through research and research-related education.

The Leukemia & Lymphoma Society

- The goal of former VP Joe Biden's moonshot to cure cancer is to achieve a decade's worth of progress against cancer in 5 years. LLS announced a significant commitment to this goal, by going on the offensive against one of the most deadly cancers, AML. In October, we launched our groundbreaking Beat AML Master Trial, an unprecedented collaborative clinical study aimed at advancing precisely targeted treatments for patients who desperately need new & better outcomes.

March of Dimes, Nebraska & Western Iowa Market

- In 2015, the March of Dimes issued a national Prematurity Report Card to raise awareness of the frequency and perils of premature birth. The nation as a whole got a "C." Nebraska as a state received a "B," with a prematurity rate of 9.1%. Thanks in great part to our 39 Weeks Project, 363 babies were spared preterm birth in Nebraska, representing savings of more than $11 million in health care costs. Prematurity has grown inexplicably by 25% over the past 25 years and continues to be one of our nation's most serious health problems.

Muscular Dystrophy Association of Nebraska

- Eteplirsen granted accelerated approval to treat DMD – This is the first disease modifying drug available to treat DMD.
- FDA approved the first ever treatment Spinraza for all types of SMA.
- A Pharma company has announced encouraging 12-month efficacy and safety data for a drug for the treatment of ALS.

National MS Society, Mid America Chapter

- In 2016, Ocrelizumab, which was shown to slow primary progressive MS and to reduce MS relapses in clinical trials, was granted "Breakthrough" status by the FDA and a decision about its possible approval is expected by the end of March 2017.

Nebraska Chapter of the National Hemophilia Foundation

- uniQure recently announced that their investigational hemophilia B gene therapy, AMT-060, has received "breakthrough" therapy designation from the U.S. Food and Drug Administration (FDA). UniQure is an Amsterdam-based company specializing in developing gene therapies to treat rare conditions, such as central nervous system disorders, liver/metabolic and cardiovascular diseases. Breakthrough drugs are put on a fast-track approval program and given intensive guidance from the FDA.
- The technology behind AMT-060 employs adeno-associated viruses (AAV). These small viruses, which do not cause disease and typically produce mild immune responses, are used as vectors (delivery vehicles) to introduce a functioning factor IX (FIX) gene into the liver cells of patient's with hemophilia B. The goal of the trial is to trigger long-term FIX protein production through a single administration of the therapy. This could dramatically reduce the frequency of bleeding episodes in people with hemophilia B.
- Medscape Activity Focuses on Optimizing Hemophilia Treatment Guidelines: Assessing Your Skills in Integrating Treatment Guidelines for Hemophilia" is the latest educational opportunity to become available from Medscape. This activity is intended for hematologists, pediatricians, nurses and other healthcare professionals who treat patients with hemophilia. It is part of the online series, "Clinical Advances in Hemophilia: Management for Life."

Nebraska Hospice and Palliative Care Association

Advocacy Update: On July 19, 2016, NHPCA representatives attended National Hospice and Palliative Care Organization's Capitol Hill Day in Washington, D.C., to call on Congress to support the Rural Access to Hospice Act, the Personalize Your Care Act of 2016, and the Care Planning Act. The Nebraska advocates met with all five of Nebraska's congressional delegation. Representative Smith agreed to co-sponsor the Rural Access to Hospice Act prior to the visit and Representative Ashford and Representative Fortenberry agreed to co-sponsor after the visit.

Nebraska Kidney Association

- First U.S. trial of wearable artificial kidney successful. The present prototype of WAK is a 10-pound device, powered by nine-volt batteries and worn around the waist.
- Home Hemodialysis has a better mortality rate with more frequency than in center treatments. It also frees up some of the dietary and fluid restrictions

Susan G. Komen® Great Plains

- Research supported by Susan G. Komen® sheds new light on estrogen receptor-positive breast cancer that has become resistant to therapy and metastasized.
- Susan G. Komen®, Milburn Foundation and the Inflammatory Breast Cancer Research Foundation push for breakthroughs in IBC research
- Susan G. Komen® announces nearly $33 million in new research funding to support bold goal of cutting breast cancer mortality by 50 percent
- Susan G. Komen announces $27 million health equity initiative to reduce breast cancer deaths in African-American community

Team Jack Foundation

- The Team Jack Foundation has invested in multiple research projects across the United States. One of the Foundation's earliest grants was to Dana Farber Cancer Center in Boston, MA. The following is a report of research made possible by this grant: Precision medicine—in which diagnosis and treatments are keyed to the genetic susceptibilities of individual cancers—has advanced to the point where it can now impact the care of a majority of children with brain tumors, a new study by investigators at Dana-Farber/Boston Children's Cancer and Blood Disorders Center suggests. In the largest clinical study to date of genetic abnormalities in pediatric brain tumors, researchers performed clinical testing on more than 200 tumor samples and found that a majority had genetic irregularities that could influence how the disease was diagnosed and/or treated with approved drugs or agents being evaluated in clinical trials. The findings demonstrate that testing pediatric brain tumor tissue for genetic abnormalities is clinically feasible and that in many cases, the results can guide patients' treatment.

United Cerebral Palsy of Nebraska

- Although researchers are actively seeking a cure for Cerebral Palsy, much focus is placed on acquiring an understanding of the condition, identifying risk, determining cause, advancing treatment and implementing preventative measures. Recent studies have focused on eye-tracking technology, stem cell treatments, repairing damaged brain cells, pain management, bio-medical advancements, assistive technology and surveillance.
- Australia's first clinical trial of stem cell infusion from cord blood as a possible treatment for cerebral palsy (CP) is commencing in Melbourne. The safety trial, led by the Murdoch Children's Research Institute (MCRI), will recruit patients nationally and take place at The Royal Children's Hospital. Expected to take two years, the study is being funded by the Cerebral Palsy Alliance Research Foundation and Cell Care, Australia's largest private cord blood bank. It is the first step in a promising process that eventually aims to find out whether cord blood infusion is both safe and efficacious for children with the condition.