Seattle Children's Hospital Guild Association

Funding Focus Update

Fiscal Years 2004 - 2011

Learn how Guild Association Funding Focus investments are making a difference for children and their families.

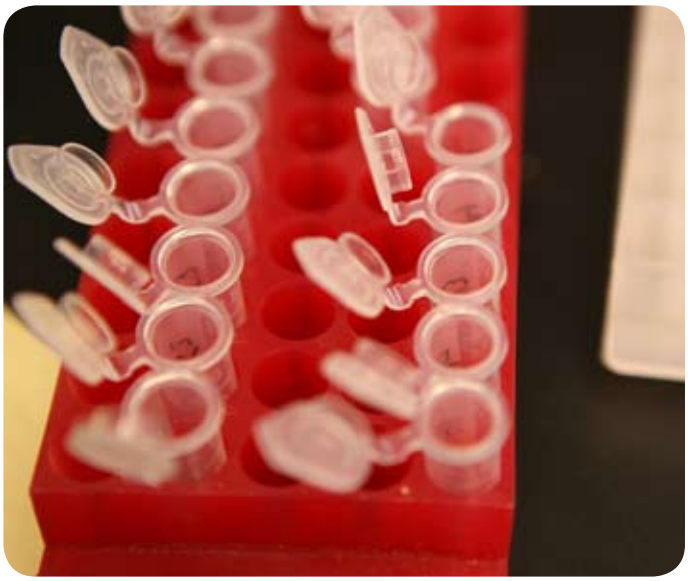
Seattle Children's
HOSPITAL • RESEARCH • FOUNDATION

Seattle Children's Hospital Guild Association has raised millions of dollars for needs such as clinical services, facilities and research through the Funding Focus program. A small portion of the money we raise annually is directed to priority funding needs within the hospital.

Thank you for helping us improve care for sick and injured children — and work to prevent childhood illness and injury — through Funding Focus initiatives. Examples of programs and facilities that have benefited from your generous support include:

- Adolescent and Young Adult Oncology Program
- Global Alliance to Prevent Prematurity and Stillbirth
- Program in Cardiac Innovation
- Center for Integrative Brain Research
- Center for Tissue and Cell Sciences
- Immunology research
- Health outcomes research
- Infectious disease research
- Urology
- Janet Sinegal Patient Care Building
- Childhood Communication Center
- Brain tumor research
- Neurosurgery
- Intensive care

We are pleased to share a summary of our current Funding Focus — which will conclude in 2011 — and report on the results of Funding Focus investments from fiscal years 2004 through 2008. If you have questions about Funding Focus programs or this report, please contact Aileen Kelly, Guild Association executive director, at 206-987-4816 or aileen.kelly@seattlechildrens.org.



Adolescent and Young Adult Oncology Program
Prematurity and Stillbirth Prevention Program in Cardiac Innovation

FY 2009 through FY 2011

Our current Funding Focus will raise $1.5 million for each of three innovative programs through fiscal year 2011.

Adolescent and Young Adult Oncology Program

Research shows that if teens with cancers such as leukemias and sarcomas are treated at pediatric institutions with protocols developed specifically for them, long-term survival is 25% higher than if they are treated at adult medical centers. Funding Focus gifts are helping Children's Adolescent and Young Adult Oncology Program improve facilities, expand support services, hire staff, initiate community education and advance clinical research to better serve teens and young adults with cancer.

Leah Kroon, clinical nurse specialist (left) and Dr. Becky Johnson (right) created the Adolescent and Young Adult Oncology Program.

Global Alliance to Prevent Prematurity and Stillbirth

Throughout the world, prematurity is the leading cause of newborn deaths. In addition, millions of stillbirths occur late in pregnancy or just minutes before birth. The Global Alliance to Prevent Prematurity and Stillbirth (GAPPS), an initiative of Seattle Children's, is leading an international effort to address these challenges. Funding Focus gifts are helping GAPPS speed the adoption of effective prevention and treatment options through research. *Please see page 9 for more information about GAPPS.*

GAPPS seeks to prevent prematurity and stillbirth.

Program in Cardiac Innovation

Given that nine of every 1,000 babies are born with a heart defect, access to outstanding cardiac care is vitally important for children in the Northwest. Funding Focus gifts are supporting the Program in Cardiac Innovation, through which Children's Heart Center investigators are researching, designing, developing and testing devices that improve diagnostic techniques, treatment options and quality of life for children with heart conditions.

Dr. Gordon Cohen oversees the Heart Center's Program in Cardiac Innovation.

Center for Integrative Brain Research
Center for Tissue and Cell Sciences

FY 2008

During fiscal year 2008, the Guild Association Funding Focus helped advance pioneering research at Seattle Children's Research Institute by raising $1.5 million split between the Center for Integrative Brain Research (CIBR) and the Center for Tissue and Cell Sciences (CTCS).

Center for Integrative Brain Research

The Funding Focus gift to CIBR supported the recruitment of Dr. Jan-Marino "Nino" Ramirez, CIBR director and *Guild Association Investigator in Integrative Brain Research.*

Ramirez joined Children's from the University of Chicago, where he chaired the Department of Organismal Biology and Anatomy. He continues his innovative research at Children's, where a major focus is the cellular basis of disorders that affect the autonomic nervous system. For example, one project aims to unravel the basis of erratic breathing, one of the most debilitating symptoms of Rett syndrome.

"It's heartbreaking to see children struggling to breathe who are afflicted with Rett syndrome," says Ramirez. "Equally devastating are breathing disorders in which children 'forget' to breathe or don't respond properly to challenges that require complex autonomic responses. There is currently neither a treatment nor a cure for these neurological disorders. Hence, as scientists with a mission to prevent, treat and someday eliminate pediatric disease, we have the enormous responsibility of developing new therapies and concepts to combat these neurological conditions."

Dr. Jan-Marino "Nino" Ramirez, director of the Center for Integrative Brain Research, is the *Guild Association Investigator in Integrative Brain Research*.

In his role as director of CIBR, Ramirez is establishing integrated research programs that will lead to a better understanding of neurological, neurodevelopmental and neuropsychiatric disorders in children. CIBR's approach integrates knowledge at the genetic, molecular, cellular, network and behavioral levels to develop new ways of preventing, treating and potentially curing neurological conditions in children.

CIBR research currently includes studies of the following conditions: congenital malformations and craniofacial disorders; eating and endocrine disorders; epilepsy and seizure disorders; hydrocephalus, cerebrospinal fluid disorders and headache; metabolic disorders; movement disorders; psychiatric and autism spectrum disorders; respiratory, autonomic and sleep disorders; stroke, injury and infection; and tumors of the central and peripheral nervous system.

Funding focus update for FY08

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Center for Tissue and Cell Sciences (CTCS)

The Guild Association's gift of $750,000 to CTCS will support the recruitment of a talented scientist to join the Myocardial Regeneration Initiative led by Dr. Mark Majesky, associate director of CTCS.

The Myocardial Regeneration Initiative aims to develop new ways of repairing the heart and regenerating heart tissues using stem cells. When fully assembled, the Myocardial Regeneration Initiative team will include experts in developmental biology, genetics, bioengineering, pathology and clinical pediatrics.

An expert in stem cell biology and regenerative medicine, Majesky also leads the stem cell research efforts for Children's Heart Center, where his work will benefit the heart failure program. Majesky and his team are conducting detailed studies of how heart and coronary vessels develop. Ultimately, it may be possible to create healthy heart cells in the laboratory and then transplant those cells into patients with chronic heart disease to repair the heart and coronary vessels without invasive surgery or a heart transplant.

This work exemplifies the spirit of the CTCS. By understanding the body's response at the molecular, cellular, tissue and whole-organ levels, CTCS researchers are developing innovative therapeutic interventions that will ultimately help the body repair itself.

The knowledge gained from the center's research will help investigators find ways to heal damage caused by developmental abnormalities and illnesses such as chronic kidney disease, heart disease, asthma and craniofacial malformations.

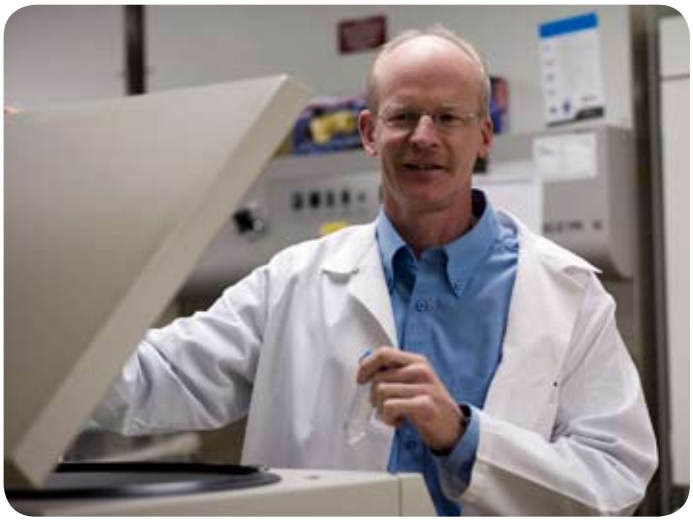
The *Guild Association Investigator in Tissue and Cell Sciences* will join the Myocardial Regeneration Initiative led by Dr. Mark Majesky, associate director of the Center for Tissue and Cell Sciences (pictured above). Recruitment for the *Guild Association Investigator* is underway.

The Myocardial Regeneration Initiative recruitment effort — made possible by the Guild Association Funding Focus gift — is currently underway. CTCS leaders are negotiating with a leading investigator in the field and hope to complete the hiring process by fall 2010. The new recruit will hold the title of *Guild Association Investigator in Tissue and Cell Sciences*.

The Myocardial Regeneration Initiative aims to develop new ways of repairing the heart and regenerating heart tissues.

Pediatric Immunology Research | FY 2007

The Children's Hospital Guild Association Endowed Chair in Pediatric Immunology Research supports the leadership of Dr. David Rawlings, director of the Center for Immunity and Immunotherapies (CIIT) at Seattle Children's Research Institute.



Dr. David Rawlings, director of the Center for Immunity and Immunotherapies, holds the *Guild Association Endowed Chair in Pediatric Immunology Research*.

Under Rawlings' leadership, CIIT brings together distinguished experts across multiple disciplines to solve some of the immune system's most complex problems. The CIIT team is researching new ways to treat diseases such as immune deficiency, autoimmunity, allergies, infections and cancer.

An internationally renowned immunologist, Rawlings is working to understand how dysregulated B cell development and signaling leads to immunodeficiency, autoimmunity or lymphoid malignancies. He is also a leader in developing genetic therapies for immune diseases. As co-director of the Northwest Genome Engineering Consortium along with Dr. Andrew Scharenberg, Rawlings is part of a multidisciplinary effort to potentially cure single-gene diseases of the immune system and blood through a form of gene therapy known as gene repair.

Rawlings' most recent gene therapy research breakthrough could dramatically improve care for children with X-linked agammaglobulinemia (XLA), a primary immune deficiency disease in which patients lack mature B cells and immunoglobulin — and thus the ability to produce antibodies. As a result, individuals with XLA are vulnerable to life-threatening bacterial infections. The current treatment for XLA, which includes antibody replacement therapy and antibiotics, is only partially effective — and patients can experience long-term complications. Many individuals with XLA die before midlife.

Through laboratory studies in mice, Rawlings and his team have demonstrated the potential of gene therapy to address the underlying problem. Ongoing work to fine-tune the process is underway, and Rawlings is hopeful that this work will lead to a clinical trial for children with XLA in the coming years.

"Our current research in this area represents a major step toward developing an alternative therapy for children affected by XLA," says Rawlings. "Ultimately, we hope to improve B cell function in children with XLA by isolating their existing blood stem cells, introducing a copy of the normal gene and returning them to the patient. The idea is that the corrected cells will reproduce more effectively than the defective cells, improving the patient's immune system function, health and quality of life."

Pediatric Health Outcomes Research | FY 2006

The Children's Hospital Guild Association Endowed Chair in Pediatric Health Outcomes Research advances Dr. Frederick Rivara's efforts to improve children's health.

Dr. Frederick Rivara holds the *Guild Association Endowed Chair in Pediatric Health Outcomes Research.*

Rivara, who leads the Division of General Pediatrics at the University of Washington, is an international leader in outcomes research. His interests include the efficacy and promotion of bicycle helmets, prevention of pedestrian injuries, youth violence, the epidemiology of firearm injuries, intimate partner violence, interventions for alcohol abuse in trauma patients and the effectiveness of care for trauma patients.

Currently, one of Rivara's primary areas of study is outcomes among children with traumatic brain injuries — including concussions. "Historically, concussions have been brushed off as minor events," says Rivara. "However, we are increasingly concerned that these are significant injuries."

To understand how traumatic brain injuries affect children and their families, Rivara and his team have been studying 700 children for the past four years. "We are looking at the entire spectrum of injury, from mild through moderate to severe, across all ages, from infancy through adolescence," notes Rivara. "Our work has important implications for the care that should be delivered to children and their families in the hospital, the clinic, schools and the community."

Through this study, the team discovered there are no standard guidelines for the rehabilitation of children with traumatic brain injuries. "We believe rehabilitation is key to improving outcomes," says Rivara. "However, the lack of standard guidelines has hindered progress." To address this challenge, Rivara and his team are in the process of developing national guidelines for the care of traumatic brain injuries. This work will improve the health of patients with traumatic brain injuries by standardizing care at rehabilitation centers across the country, including Seattle Children's.

Another factor that affects a child's recovery from injury is the support and resources that families can devote to the child. If a parent has been injured in the same accident, the level of support available to the child is often diminished. Rivara is leading an effort to improve outcomes for these children by learning how to better coordinate care when more than one family member is injured.

Rivara is also working to expand the number of health professionals interested in improving outcomes for injured children. He recently secured funding from the National Institutes of Health for a multidisciplinary training program in outcomes research and interventions.

Pediatric Urology

FY 2005

The Dr. Michael Mitchell Endowed Chair in Pediatric Urology has played a pivotal role in the growth of Seattle Children's Urology program.

Established in honor of Children's long-time chief of Urology who retired in 2006, the *Mitchell Chair* helped the hospital recruit Dr. Martin Koyle as chief of Urology in 2008. The chair supports Koyle's leadership in patient care, research and medical education.

Prior to joining Children's, Koyle led the Pediatric Urology program at The Children's Hospital in Denver. He is renowned for his pioneering work in urological surgery. For example, during his 19-year tenure in Colorado, Koyle was the first to use laparoscopic surgery, a minimally invasive technique, to remove a kidney in an infant.

Koyle's leadership has already made a difference for thousands of children with urologic conditions. Since arriving in Seattle, he has partnered with his team to improve access to Children's urology services. As a result of this work, wait times for clinic appointments have been reduced from over a month to just a couple of days, and wait times for non-emergent surgical procedures dropped from three months to approximately two to four weeks. Koyle also cares for patients at clinics in Everett and Tri-Cities in Washington and in Missoula, Montana, which saves families the expense and stress of traveling to Seattle.

Koyle is part of a team that's exploring the use of technology to further expand access to Children's urology experts. After reviewing patient records from the past several years, the team determined that many urgent visits to Children's urology clinic and emergency room for urologic conditions were unnecessary. They hope to launch a pilot project that allows families in these situations to securely share information about the child's condition with clinicians at Children's via the Internet.

Dr. Martin Koyle, who leads Children's Urology Department, holds the *Dr. Michael Mitchell Endowed Chair in Pediatric Urology.*

"This is one way that we can improve accessibility to our expertise while minimizing travel for families," says Koyle. "By talking with parents via phone and looking at digital photos that show their child's condition, we can determine if a visit to Children's or the nearest emergency room is necessary. We think this approach will give families peace of mind while saving time and money. If this pilot project works, it could serve as a model for medical centers nationwide."

Pediatric Infectious Disease Research | FY 2004

In the United States, prematurity affects one in eight babies and accounts for $26 billion in healthcare costs annually. Though half of premature births have no known cause, research has shown that Group B streptococcus (GBS) plays a role.

Dr. Craig Rubens, executive director of the Global Alliance to Prevent Prematurity and Stillbirth (GAPPS), holds the *Guild Association Endowed Chair in Pediatric Infectious Disease Research.*

GBS is also the leading cause of bacterial infection in newborns, causing pneumonia, meningitis and blood infections. Under the leadership of Dr. Craig Rubens, the first holder of the *Children's Hospital Guild Association Endowed Chair in Pediatric Infectious Disease Research*, investigators at Seattle Children's are working to better treat and someday prevent GBS infection.

This effort is part of the Global Alliance to Prevent Prematurity and Stillbirth (GAPPS), an initiative of Seattle Children's directed by Rubens. The GAPPS team is working to raise awareness of prematurity and stillbirth, understand the causes, develop solutions and advocate for policy changes that save lives.

Since the *Guild Association Endowed Chair in Pediatric Infectious Disease Research* was established, Rubens and his team have worked tirelessly to determine the genetic and biochemical basis of GBS interaction with pregnant women and newborns. For example, they are exploring how specific genetic mutations affect the bacterium's ability to interact with tissue, to grow in amniotic fluid, to cause infection and meningitis, and to evade the immune response.

"The incidence of prematurity is on the rise," says Rubens. "Infection of the placental membrane and subsequent inflammation have been implicated as major causes. Yet very little is understood about the mechanisms and interactions that initiate this disease process."

To address this knowledge gap and begin developing effective therapies, Rubens and his team are building a model system that will help them determine how bacteria like GBS cause premature labor and delivery.

Rubens is also working to train more specialists. Children's is fortunate to have funding from the National Institutes of Health for fellowship training in infectious diseases, but the program is at risk due to budget cuts. The *Guild Association Endowed Chair in Pediatric Infectious Disease Research* will provide vital support during the next four years.

"This endowment will help us expand research opportunities in pediatric infectious diseases and develop the next generation of talented investigators," notes Rubens.

Our Mission

To serve as advocates for Seattle Children's through our volunteer, philanthropic and educational endeavors to ensure excellent care for all children in our region.

Hope. Care. Cure.™

Seattle Children's
HOSPITAL • RESEARCH • FOUNDATION

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