In 2012, CCF awarded $250,000 to the National Heart, Lung and Blood Institute’s Pediatric Cardiomyopathy Registry (PCMR), a national clinical database of children diagnosed with cardiomyopathy. This summer, findings from two CCF-supported studies were published in Circulation and American Heart Journal. The two ancillary studies analyzed extensive patient data in the PCMR and provide significant new guidance on heart transplantation for children with cardiomyopathy.

“These studies are game changers for physicians, surgeons and parents as they point the way to improve transplant outcomes,” said Steve Lipshultz, M.D., professor of pediatrics and the George Batchelor Endowed Chair in Pediatric Cardiology at the University of Miami Miller School of Medicine. Lipshultz also is the director of the Batchelor Children’s Research Institute and the principal investigator of the PCMR.

Published in the July issue of Circulation, the study “Outcomes of Restrictive Cardiomyopathy in Childhood and the Influence of Phenotype: A Report from the Pediatric Cardiomyopathy Registry” examined 3,375 cardiomyopathy patients, comparing transplant outcomes based on several risk factors such as prior heart failure and thicker heart walls. The multi-center study represents the largest cohort of children with restrictive cardiomyopathy reported to date. In the past it was difficult to accurately assess outcomes and risk factors on a national basis for this rare disease.

Restrictive cardiomyopathy (RCM) has generally been associated with poor prognosis in childhood. “This study found that transplant-free survival is poor for RCM in childhood and overall outcomes are worse than for all other forms of cardiomyopathy,” said James Wilkinson, M.D., M.P.H., professor of pediatrics and epidemiology and the director of the PCMR administrative coordinating center. “The study further found that children with RCM and heart failure are twice as likely to fail medical management and need a transplant compared with patients who...”

continued, page 3
From Lisa Yue,
CCF Founder & President

2012 marks the 10th anniversary of CCF! A decade ago, outcomes for children with cardiomyopathy were dismal with little research being conducted and no existing support services for those living with this chronic heart disease. In 2002, my husband Eddie and I set out to change this and dedicated ourselves to accelerating research and establishing resources to help affected families.

Many have since joined our cause, and the CCF community has expanded to 1,900 members from 52 countries. While we still have a way to go in our search for cures, we are proud of the strides we have made.

In the past 10 years, CCF has supported numerous studies on pediatric cardiomyopathy resulting in 145 presentations and publications that help improve diagnosis, treatment and patient care. CCF was the first to organize a scientific conference and establish a DNA and tissue repository for the disease. In addition, our patient education materials are distributed to more than 80 hospitals, and we introduced the first cardiomyopathy bill to the U.S. Senate and the House this year.

These achievements would not have been possible without your support. The positive testimonies I receive from families and physicians always warms my heart, and being named a top-rated nonprofit inspires me to do more. Just imagine what we can accomplish in the next ten years with your continued involvement!

We hope you enjoy our 10th anniversary issue as families and physicians share their thoughts on CCF (pgs. 6-7). You also can read about the latest happenings from CCF’s Annual Golf Classic (pg. 5) and family fundraisers (pg. 8) to updates on our funded research (pg.1) and advocacy initiatives (pgs. 9-10).

Thank you again for being with CCF every step of the way these past ten years!

Lisa Yue
CCF-FUNDED STUDY to Expand the DONOR HEART POOL RECEIVES NIH GRANT

A CCF-supported study, focused on expanding the donor heart pool for children with advanced heart failure, has received additional funding from the National Heart, Lung and Blood Institute (NHLBI), a division of the National Institutes of Health. The study, "Expanding the Donor Pool for Pediatric Heart Transplant," led by Carmelo Milano, M.D., associate professor of surgery at Duke University, has been awarded $500,000 by the NHLBI.

Only 2,200 heart transplants are performed annually in the U.S., a number mainly limited by the severe shortage of donor hearts. The shortage is greatest for children with advanced heart failure who are three times more likely to die waiting for a heart transplant compared to adults. In an effort to change this, Dr. Milano's research goal is to transplant alternative "donation after cardiac death" (DCD) hearts from individuals who have suffered severe brain injury and have had life support withdrawn causing the heart to stop beating. Currently, pediatric heart transplants are only using hearts from deceased donors that are brain dead but continue to have cardiac function. DCD hearts are not being used because physicians are concerned about possible cardiac injury, which may affect post-transplant survival.

In 2011, CCF provided seed funding to Dr. Milano to develop a clinical protocol for heart recovery and screening of pediatric DCD hearts for transplant consideration. Part of the grant enabled the research team to purchase a critical piece of equipment to evaluate the cardiac function of DCD hearts.

“The MPVS-Ultra Foundation System enables us to obtain the most sensitive measurements of cardiac function on our current system, which stimulates heart transplantation to see how well a donor heart works,” said Dr. Milano. CCF's support of the initial feasibility study helped Dr. Milano to secure an outstanding score on his NIH R-21 grant and multi-year funding.

The NHLBI grant will allow Dr. Milano’s team to continue with their human heart experiments, further demonstrating their ability to recover DCD hearts using the LifeCradle cardiac perfusion device. It also will enable them to identify plasma biomarkers that can predict which hearts can be successfully transplanted.

According to Dr. Milano, 15 people die each day waiting for an organ transplant, and half of the heart transplants required in the near future will be for cardiomyopathy patients. The long-term goal of the study is to expand the donor heart pool by improving the preservation of DCD hearts and developing a clinically relevant and reliable procedure to evaluate the function of DCD hearts after restoring blood flow to the heart. It is estimated that within three years his research may facilitate an increase in the national heart donor pool by more than 20 percent — an accomplishment further benefiting pediatrics where the donor pool is severely limited and alternative heart failure therapies are lacking.

Findings from Two Nationwide Studies Provide New Transplant Guidance  continued from page 1

didn’t have heart failure. That strongly suggests that these pediatric patients should be listed for an early heart transplant.”

The findings are important because RCM patients typically receive a transplant only after their heart fails and they are put on life support systems. This study shows that patients could have a better outcome if they list earlier for a transplant before their heart fails.

The second study, “Outcomes in Children with Noonan Syndrome and Hypertrophic Cardiomyopathy: A Study from the Pediatric Cardiomyopathy Registry,” was published online in August in American Heart Journal.

Outcomes for children with hypertrophic cardiomyopathy (HCM) who present in the first year of life are usually worse than when HCM is diagnosed after the first year of life. The leading cause of HCM in infants is Noonan syndrome, a systemic genetic disease. The PCMR study was the first multi-center study that compared the survival experience of 74 children with Noonan syndrome and HCM with 792 children with idiopathic or familial HCM to identify clinical and echocardiographic predictors of clinical outcomes.

It was found that pediatric patients diagnosed with Noonan syndrome and HCM before age 6 months with heart failure had a 69 percent mortality rate at one year — a much worse outcome than the HCM patients without Noonan syndrome. Predictors of poor outcomes in patients with Noonan syndrome and HCM were found to be decreased height-for-age and lower left ventricular fractional shortening measurements.

“Our findings strongly suggest that infants with Noonan syndrome, HCM and heart failure should have an aggressive therapeutic approach including potential early listing for cardiac transplantation, even if their symptoms have transiently improved,” Wilkinson said. “These pediatric patients also may require a defibrillator to stay alive until a transplant can be performed if they have life-threatening abnormal heart rhythms and a risk for sudden death.”

The PCMR was established in 1994 to describe the epidemiologic features and clinical course of selected cardiomyopathies in patients aged 18 years or younger and to promote the development of cause-specific treatments. The registry has collected clinical data on more than 3,500 children diagnosed with dilated, hypertrophic or restrictive cardiomyopathy from 100 pediatric cardiac centers in North America. CCF continues to collaborate with the PCMR in sponsoring working group sessions and providing support for data analysis and publication of manuscripts.
Echocardiograms are the primary method for evaluating the size of the heart and how it functions. However, there is a wide range of factors that cause variability in echocardiographic measurements, including how echocardiograms are performed and interpreted by different sonographers or cardiologists. These variables in measurement can be problematic in a study where researchers need to compare changes in heart function values over time. Currently, researchers and healthcare providers do not know how reproducible some of the measurements are in children. Reproducibility is the degree of agreement between measurements or observations conducted in a study by different people at different locations.

The PHN-conducted VVV study results were published in the Journal of the American Society of Echocardiography (JASE) in August 2012. The study followed 131 children with dilated cardiomyopathy (DCM) aged 0-22 years from eight pediatric clinical centers. Clinical data was collected along with 150 common echocardiographic indices of ventricular size and function. Data was collected multiple times and variables were studied to understand what influences reproducibility and measurement error in these pediatric patients.

The results conclude that certain measurements are highly reproducible, while others, despite beat averaging, are poorly reproducible. Beat averaging, specifically measuring three consecutive heartbeats and calculating the average, as well as the use of a core laboratory with a single person observing the measurements can reduce variability in echocardiographic measurements. These findings could influence echocardiography methods and help in the development of future clinical trials on cardiomyopathy.

“This study provides fundamental knowledge about quantitative methods in echo for children with dilated cardiomyopathy, and the lessons learned from this study will be essential to designing the trials of tomorrow,” said Jonathan Kaltman, M.D., chief of the heart development and structural diseases branch, NHLBI Division of Cardiovascular Sciences.

Dr. Colan, who has spent a large part of his career focused on developing standards in echocardiograms, believes that the impact of this study is far reaching. “The feedback I have gotten so far leads me to believe the ripple effects of this on the field may be huge,” said Dr. Colan. Besides the JASE paper, three additional papers are in submission; three are in final preparation; and five are in analysis at the current time. Dr. Colan, a member of the advisory panel of the American Society of Echocardiography, is working on a set of recommendations based on the PHN study findings to standardize echocardiography testing for both children and adults.

The results of this PHN study will not only provide standards on how echocardiograms are performed in clinical studies, but also improve how children with cardiomyopathy are diagnosed and cared for: “This study promises to provide significant improvements in how we manage our patients,” said Dr. Colan. “We are already utilizing three beat averaging in our clinical practice at Children’s Hospital Boston.”

CCF Participates in New York REGIONAL HCM CONFERENCE

The 3rd Regional Winthrop Hypertrophic Cardiomyopathy (HCM) Conference took place at the Cartlun on the Park in Long Island, N.Y., on October 20. Lisa Yue, CCF founder and president, was invited to speak about HCM pediatric concerns and participated in a panel discussion with several HCM specialists from Winthrop University Hospitals’ HCM Treatment Center.

Organized by Srihari Naidu, M.D., director of the HCM Center at Winthrop, the half-day event gathered HCM specialists and 60 HCM families for a half day of education, awareness and social networking. The meeting covered topics such as: HCM treatment guidelines; understanding echocardiograms; the role of cardiac MRI, arrhythmia, pacemakers and ICDs; and pediatric issues. The Winthrop HCM Treatment Center manages roughly 300 HCM individuals and is one of only two in the New York metropolitan area offering specialized services to HCM families.

CCF has compiled a list of HCM treatment centers in the U.S. to assist those with a family history of HCM and have a diagnosed adult family member or child who needs to transition to adult care. For more information on these centers, please contact Chris Colán at ccolon@childrenscardiomyopathy.org.

The 2011 American College of Cardiology and American Heart Association published “Guidelines for the Diagnosis and Treatment of Hypertrophic Cardiomyopathy” recommends that HCM patients be seen at specialized centers committed to the ongoing, comprehensive and multidisciplinary diagnosis and management of patients with HCM. This includes being experienced in high-volume clinical care, pediatrics, advanced imaging, electrophysiology, medical and surgical interventions, and genetic testing and counseling.
On July 16, CCF marked 10 years of progress in pediatric cardiomyopathy at CCF’s 10th Annual Golf Classic at the Montclair Golf Club. More than 225 attendees celebrated CCF’s Anniversary with Founders Eddie Yu and Lisa Yue who spoke movingly about how CCF started, where it is today, and the need to continue CCF’s mission. The event raised $380,000 for CCF’s research and education programs. “It’s amazing to think of all that CCF has accomplished in 10 years,” said CCF Board Member Brian Nold. “Through the help and support of our generous donors, we continue to raise awareness of pediatric cardiomyopathy, and we are getting closer to hopefully one day finding cures.”

This year’s winning foursomes were: John McCormick, Mike Dowdell, Peter Wilson, Pat Lanigan (first prize); John Campbell, Mark Colm, Riaz Haidri, Brian Nold (second prize); Dan Ryan, Brian Hewitt, Steve Sander, Tom Schneider (third prize); and Brad Roberts, Kevin Uarian, John Petrozzi, Rushabh Doshi (fourth prize). The prize for closest to the pin went to Carney Hawks of Brigade Capital Management and Dan Ornstein of Morgan Stanley. Tim Milton and Mike Walker won the prize for longest drive, and Sean Smith of Societe Generale took home the raffle prize.

**2012 Event Sponsors:**
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More event photos are online under “News & Events/Golf Classic.”

**Save the Date!**

5th Annual

All-In For A Cure

No-Limit Texas Hold ’Em

Wednesday, February 6, 2013

Crimson, New York City

Join CCF for an exciting evening of charity poker!

Visit our event web page under “News & Events” for more information.
CELEBRATING 10 YEARS TOGETHER

As the Children’s Cardiomyopathy Foundation (CCF) celebrates its 10th anniversary, we have asked members of our CCF community—both families and physicians—to share what CCF has meant to them.

We cannot thank you all enough for sharing your stories and your lives not only with CCF, but also with each other every day.

CCF is my safe harbor, my port in a storm. I know when things are going well I have people to celebrate with. When things are not going so well I have people who commiserate. I have met friends here who will last a lifetime. We laugh together and cry together. We share information and news and ideas. No matter how lost and confused I am there is always someone there to lend a hand and point out a new direction.

Sharon Belleville, mom to Luke, 15, DCM/LVNC

Nearly 14 years ago, my youngest son, Nathan, was diagnosed with myocarditis as a newborn. This led to his dilated cardiomyopathy. I discovered CCF soon after it started and it has changed my life. Not only has CCF been an invaluable source of information and resources, it has connected me with other families. I now have a worldwide network of friends who understand my fears and my hopes.

Carol Davis, mom to Nathan, 13, DCM

As a healthcare provider, CCF has helped me have a better understanding of the cardiomyopathy journey. Stories vary so much, yet the core emotions of fear, anguish and despair parents experience are the common thread that bonds them. Each story I read makes me more passionate about what I do for these special children and their families.

Jenny Nova, RN, Transplant Coordinator, Children’s Hospital at Montefiore, Bronx, N.Y.

CCF was there for me less than one day after I found out that my son had hypertrophic obstructive cardiomyopathy. From that point on, CCF has been my lifeline. I have inherited a community of friends that feels more like family every day. The CCF forum has given me the help and hope that I need to go on with my life knowing that I’m not alone on this tough, unpredictable journey.

Janell Gregerson, mom to Chase, 2, HCM

Over nearly a decade, CCF’s support of the Pediatric Cardiomyopathy Registry has resulted in dozens of publications in medical research journals, whose findings positively affect the lives of children with cardiomyopathy and their families.

James Wilkinson, M.D., M.P.H., professor of pediatrics and epidemiology, University of Miami Miller School of Medicine, Fla.

CCF is the only place I can talk to someone who truly knows the ups and downs, the struggles, and the times of perseverance when dealing with this disease our children live with. It’s a group of people I’ve never met yet connect with in a very unique and special way.

Aimee White, mom to Tyler, 4, DCM

When I was first diagnosed with cardio-myopathy, my mom found CCF and they have been there for us ever since. We never felt alone because there was always a link to someone in CCF’s supportive community who could help. When I needed a heart transplant, instead of being scared, we had a level of understanding and comfort that we never would have had without CCF.

Joe DiSanto, 17, HCM diagnosed at age 11

After Jaxson was diagnosed with hypertrophic obstruction cardiomyopathy, I found CCF online and it gave me a reason to hope. Without CCF and the many families I have met through the forum I might not have known to look for a specialist and better facility.

Andi Coffman, mom to Jaxson, 3, HCM

When my daughter was 3 1/2 months old, she was in heart failure, and we were given little hope for her future. I have received priceless advice, support and love from the caring people who work at the organization, as well as from the parents who I connect with on the Listserv.

Heather Tucker, mom to Evangeline, 5, DCM
CCF has built strong bridges between patients, physicians and scientists so that they can work together to improve the lives of children with cardiomyopathy.
Seema Mital, M.D., Hospital for Sick Children, Toronto, Canada

Everyone has two kinds of family - family by blood and family by choice. CCF is a type of family by blood - the blood that circulates (not always too well!) in our children’s hearts as well as a family by choice in that we choose to be together. When one celebrates, we all celebrate. And when one grieves, we all grieve. We all understand one another no matter our backgrounds, our religious views, even our languages.
Dorie Neuhaus, mom to Matthew, 13, DCM

Nobody else knows exactly what it is like to live with cardiomyopathy, but for the families that live it CCF connects those families and gives us the strength we need to move forward.
Jennifer Ayers, mom to Eleanor, DCM, deceased

Since CCF was formed, there has been much progress in the field of pediatric cardiomyopathy. As a CCF grant recipient, I know the impact CCF’s support can have. To finally have answers for parents is incredibly rewarding, and this would not have been possible without funding from CCF.
Stephanie Ware, M.D., Ph.D., co-director of the Diagnostic Cardiomyopathy Clinic, Cincinnati Children’s Hospital, Ohio

CCF has brought me close to life-long friends who I know are always there for me when we receive good and not-so-good news about Annabelle’s diagnosis and disease. We are a close group who offer unconditional love and support to each other.
Jessica & Rick Marschall, parents to Annabelle, 4, DCM

CCF has been a Godsend to me personally and it has helped me to keep my sanity when I thought I was losing it. I would like to say thank you to CCF, Lisa Yue, all the staff and all the families that are on the Listserv. You have all blessed my life in more ways than I can count and in ways that I could never have imagined. I am a stronger person and a better parent.
Faith Patton Settles, mom to Ryan 13, HCM

I am immensely grateful for CCF’s support, which gave me the resources to collect critical data and jump-start my research.
J. Carter Ralphe, M.D., assistant professor of pediatrics, University of Wisconsin School of Medicine, Wis.

I joined CCF in 2008 while my son was being worked up for a heart transplant at 3 weeks old. There was not much hope, and he was admitted into hospice. The CCF community was there for us the entire time. I found a place I could lean on others for support and knowledge. I even found information for treatments that probably contributed to his unexpected and dramatic improvement in 2010 leading to hospice discharge. After more than 4 years, I am still a part of this community. I will never forget how much CCF helped our family both emotionally and physically!
Kristi Pena, mom to Christopher, 4, DCM/LVNC

I want to personally thank Lisa and Eddie Yue and the staff at CCF who dedicate every day of their lives to helping children and their families with cardiomyopathy. They are doing great work for all of us.
Matt Protas, 21, HCM diagnosed at age 15

More testimonials about CCF can be found at www.greatnonprofits.org
Garland Bell, age 8, sold the most expensive cup of lemonade in North Carolina history to benefit CCF. With help from mom, Allison, Garland and her brother Ford, age 5, set up a CCF lemonade stand on their street and sold lemonade and cookies throughout the summer.

Garland was diagnosed with dilated cardiomyopathy two years ago, and the family has been eager to increase awareness of the disease with an activity that Garland and her brothers could participate in. “The lemonade stands became a fun way for the kids to be involved,” says Allison. The Bells were excited to raise more than $1,700, but it was a contribution from a neighbor they didn’t know well that moved them the most. The neighbor purchased lemonade and cookies for her grandchildren and took a CCF pamphlet. That evening she returned with a check for $1,000. She had been so moved by Garland’s story and what she had read about pediatric cardiomyopathy that she felt compelled to make a larger donation. “The generosity that she showed was really amazing,” said Allison. The heart-warming story became front-page news in the Greensboro News & Record.

MORE FAMILY FUNDRAISERS...

- **Gary Stone Memorial Run**
  Kristi Stone held the G. Stone Memorial Run on April 29, in memory of her late husband Gary, who passed away unexpectedly from arrhythmogenic right ventricular cardiomyopathy. Approximately 60 people attended the 5K Run, held at Lake Quannapowitt in Wakefield, Mass. “We had a nice day honoring Gary, enjoying each other’s company and supporting CCF,” says Kristi. The event raised more than $1,000.

- **Every Penny Counts at Watchung Elementary School**
  Students in Marissa Donovan’s first grade class at Watchung Elementary School in Montclair, N.J., showed their big hearts by selecting CCF as the beneficiary of their “Penny Wars” competition. Class student, Seamus McNamara, son of CCF staff member Sheila Gibbons, persuaded his classmates to donate the pennies raised to CCF.

- **From the Heart for Heart Month**
  Mark and Kim Hobbs of Merritt Island, Fla. organized a community fundraiser for CCF in honor of Cristina Cinca, age 11, who has hypertrophic cardiomyopathy. The event was held in February to coincide with Go Red for Women’s Heart Health Month. For a $20 donation, attendees enjoyed a fun evening of pottery making and refreshments.

- **A Birthday Tribute to CCF**
  For the fourth year in a row, 8th grader Macie Bridge of Groton, Mass, asked for donations to CCF in lieu of birthday presents. Macie is a former student of CCF family member Scott Middlemoss, whose son Joey has left ventricular non-compaction cardiomyopathy.

Lisa Yue, nominated CCF and did a presentation on cardiomyopathy. Afterwards, students unanimously voted for CCF as their charity of choice. “I know how hard it is to have cardiomyopathy so I wanted to do something to raise money for research” says Audrey.

- **3rd Annual Casen Riley 3-on-3 Basketball Tournament**
  Casey and Heather Riley held the 3rd Annual Casen’s Crew for CCF on August 25 in memory of their son Casen, who died at 6 months due to hypertrophic cardiomyopathy. The Texas event included a 5K run/walk, a 3-on-3 basketball tournament and a chicken spaghetti lunch sponsored by Haskell National Bank. “It warms my heart to think that a child that was alive for only 6 months can make a difference in the lives of so many,” says Heather. The event raised more than $5,000.

- **Dwight Englewood Book Sales**
  Third graders at Dwight Englewood School in Englewood, N.J. selected CCF as the recipient of their book sale proceeds totaling $1,400. Audrey Yu, a student and daughter of CCF Founder Audrey Yü, a student and daughter of CCF Founder Lisa Yue, nominated CCF and did a presentation on cardiomyopathy. Afterwards, students unanimously voted for CCF as their charity of choice. “I know how hard it is to have cardiomyopathy so I wanted to do something to raise money for research” says Audrey.

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Q: Why is helping children with cardiomyopathy important to you?

A: As a parent, I remember worrying over my children when they had a simple cold, so I cannot imagine the pain the parents of a child with cardiomyopathy must go through. Cardiomyopathy levies an incredible toll on the families it affects, so it is important that they have access to the resources they need and to be as informed and prepared as possible.

Q: How would the Cardiomyopathy HEARTS Bill help children with cardiomyopathy?

A: This bill will provide parents, school personnel and health professionals with information to increase awareness of cardiomyopathy and sudden cardiac arrest and to be prepared in the event of an adverse cardiac event. This information will encourage families to evaluate their cardiac history, check for cardiomyopathy symptoms and know when to seek medical screening. With these efforts, more individuals with cardiomyopathy will be diagnosed and appropriately treated before they fall victim to sudden cardiac death.

Q: How important is it to hear from constituents, and what can they do to help support this bill?

A: Grassroots advocacy is a vital part of our democratic system and a key component in every legislative success. Members of Congress rely on their constituents to raise awareness on issues and to bring legislation to their attention. Calling and writing your representatives in Congress to let them know you support the Cardiomyopathy HEARTS Act, and that they should show their support by becoming a cosponsor, is a crucial step in raising awareness about cardiomyopathy and building support for the legislation.

Q: What are the next steps needed to get this bill passed?

A: Contacting your representatives and asking them to cosponsor the bill is a key step in building support and getting it through the legislative process. Once there is a significant amount of support for the bill, the committees of jurisdiction – the Health, Education, Labor and Pensions Committee in the Senate and the Energy and Commerce Committee in the House of Representatives – can take action to report the bill to the full House and Senate for a vote. The legislative process is long and often difficult. However, with your advocacy and continued support, it is not impossible. Together we can work to pass this bill and provide children with cardiomyopathy, their families and their schools with the information they need to stay informed about cardiomyopathy and to be properly prepared for sudden cardiac arrest.

CCF Named TOP-RATED HEALTH ORGANIZATION for SECOND YEAR

Thanks to the glowing reviews of CCF family members, CCF was again named a top-rated health non-profit by America’s leading charity evaluators GreatNonprofits, CharityNavigator, GuideStar and GlobalGiving.

Read what families have to say about CCF at www.greatnonprofits.org.
CCF Presents Testimony on NEW JERSEY CHILDREN’S SUDDEN CARDIAC EVENTS REPORTING Act

Lisa Yue, CCF’s founder and president, presented testimony to the New Jersey Senate Health and Human Services and Senior Citizens Committee in June to support the “Children’s Sudden Cardiac Events Reporting Act.” The Bill (S.1911) would mandate the Department of Health and Senior Services (DHSS) to establish a Children’s Sudden Cardiac Events Registry and require healthcare professionals in New Jersey to report to the DHSS all sudden cardiac events in children under 19 years old.

The Bill introduced by Senator Fred Madden is one of a series of bills that came from recommendations made by the New Jersey Student Athlete Cardiac Screening Task Force. The task force was created in 2007 to develop recommendations for preventing sudden cardiac death in student athletes.

On October 1, the bill cleared the New Jersey Senate Budget and Appropriations Committee. “This state legislation is a great first step for recognizing that SCA is a serious problem and needs to be better understood,” said Lisa Yue. “We hope other states consider similar legislation to make sudden cardiac arrest a reportable condition so that we have the information that we need to prevent sudden cardiac death.”

Taking a Stand Against SUDDEN CARDIAC ARREST

As part of the Sudden Cardiac Arrest Coalition, CCF is working alongside more than 46 organizations to get thousands of signatures to raise awareness of sudden cardiac arrest and the importance of automatic external defibrillators (AEDs).

The goal is to obtain 10,000 signatures by February 2013 in honor of National Heart Month. The signatures will then be presented to Arne Duncan, U.S. Secretary of Education and to Kathleen Sebelius, U.S. Secretary of Health and Human Services to ask for increased AEDs in schools and public places.

Sudden cardiac arrest (SCA) claims more than 380,000 lives per year. Most youth who suffer SCA have an undetected heart disease like cardiomyopathy. AEDs can increase the survival rate for SCA up to 90 percent by delivering a life-saving shock within the first few minutes of an attack.

To date, the Coalition has collected more than 6,000 signatures. You can support this initiative by visiting CCF’s Facebook page and clicking on the “causes” tab to add your name to the “Help Stop Sudden Cardiac Arrest” petition.

Q: What is the focus of your pediatric cardiomyopathy research?
A: My research is focused primarily on using genomic information to guide individualized or personalized medicine in children with cardiomyopathies. The first approach relies on next-generation sequencing (a technique that can produce thousands of DNA sequences at once) or genotyping to identify genetic factors that increase the risk of disease progression so that at-risk patients can be treated more aggressively. In addition, genes can help predict who will respond to certain heart failure medicines. This knowledge can help us choose the right drug for a patient. We have successfully generated heart cells from human stem cell platforms to model cardiomyopathy phenotype in a dish. The ability to model disease in a dish is revolutionizing the search for new drugs by allowing rapid screening of hundreds of compounds for effectiveness and safety. We have successfully generated heart cells from...
the skin cells of patients with genetic cardiomyopathies. These cells are being used to screen new and existing drugs for effectiveness. We are applying this approach to patients with RASopathies (Leopard syndrome, Noonan syndrome) and with hypertrophic cardiomyopathy caused by sarcomeric mutations with the eventual goal of screening new compounds for drug development.

Q: Why did you decide to study pediatric cardiomyopathy?
A: While new drugs have significantly improved survival and outcomes in adults with heart failure, children with cardiomyopathy have failed to reap these benefits. My desire to study pediatric cardiomyopathy stems from the emergence of new genomics and stem cell research to find cures for a disease that to date has had poor outcomes. As more disease-causing genes are identified, research is needed to develop new treatments targeted to these gene defects. I have focused my research on applying this knowledge towards this goal with particular emphasis on translating research findings to the bedside.

Q: What kind of impact is your research making?
A: My first research project on the genetic determinants of outcomes in pediatric cardiomyopathy received pilot funding from the CCF in 2004 and helped me move the research in the right direction. Over the years, we have established a team of basic researchers, stem cell biologists, pharmacologists, geneticists and cardiologists, who are working together to understand the molecular basis of the disease and use this knowledge to identify drugs that can be effective in preventing disease or reducing its progression. To facilitate clinical translation, we are developing pre-clinical models for drug testing and have established a population-based research network to conduct early stage clinical trials of new drugs. This collaborative approach will facilitate the application of basic science discovery to patient care.

Q: What motivates you to study pediatric cardiomyopathy?
A: As a transplant cardiologist, I am often faced with hard choices when caring for children with end-stage heart failure. Given the chronic shortage of donor organs, as many as a third of children with end-stage cardiomyopathy die before they make it to a heart transplant. Survival after transplant is limited by occurrence of rejection, infection and other complications highlighting the need to develop therapies that can extend survival in cardiomyopathy without the need for transplant. This critical need is what motivated me to pursue this field of research and apply my training in translational research to help meet this goal.

Q: What is the future of genetic research on cardiomyopathy in children?
A: Next-generation sequencing has revolutionized our understanding of the genes that cause human disease in a way that was not possible until a few years ago. We can now study the entire human genome and identify small changes that influence risk of disease. This is allowing us to identify the cause of cardiomyopathy in a growing number of patients. Genomics research in the last five years has led to the identification of genetic mutations involved in over 70 percent of patients presenting with hypertrophic cardiomyopathy, ARVD, RASopathies, and in a growing number of patients with other forms of cardiomyopathies. In the coming years, we will also develop a better understanding of which genes modify disease outcomes and which drugs should be used to target specific gene defects. As we get closer to faster and cheaper ways to sequence the human genome, it will expedite our ability to develop better diagnostic tests and therapies that are individualized to improve survival and health outcomes in children with cardiomyopathy.
MEMBER SUPPORT SERVICES

CCF offers a variety of ways for members to share information on pediatric cardiomyopathy and provide support to one another. CCF offers a member forum, local support group meetings and Facebook chats.

For more information about these services or the below scheduled events, please contact Chris Colon at ccolon@childrenscardiomyopathy.org.

CCF Facebook Chat Schedule

- **Topic: General Discussion**
  - Wednesday, December 19, 2012
  - 8:00 p.m. EST
  - Tuesday, January 22, 2013
  - 9:00 p.m. EST
  - Monday, February 18, 2013
  - 4:00 p.m. EST
  - Thursday, March 21, 2013
  - 8:30 p.m. EST

To join, you must be a member of the CCF Community. You can register at www.facebook.com/groups/iheartccf/

CCF Forum Guest Q&A Sessions

- **Nutrition in Pediatric Cardiomyopathy**
  - Tracie Miller, M.D.
  - University of Miami School of Medicine
  - November 26 – December 3, 2012

CCF: Celebrating 10 years of helping children and families with cardiomyopathy

“We’re indebted to CCF for helping us through all the struggles and celebrating with us our triumphs.”

- Heather, mom to Evangeline, DCM

Read more testimonials on pages 6-7.

Find us on Facebook

Became a CCF fan, group member and/or cause supporter.

Invite your friends and family to help spread awareness of pediatric cardiomyopathy!