President’s Message
From Lisa Yue, CCF Founder & President

It’s been a busy few months with the review of submitted research grants and the planning of our scientific workshop. The scientific workshop, held January 25-26, was an important milestone for CCF, and we are excited about disseminating our learnings to the scientific and medical community in the coming months. I thank our dedicated medical advisors who have put many hours into various CCF initiatives in spite of their busy schedules.

I am happy to report that 2006 was a year of tremendous growth. With the generous support of our donors, we received $514,754 in total revenue. Our annual appeal with the theme of “A Little Heart Goes a Long Way” kicked off in November 2006 and has raised more than $57,500 to date. With this growth, we are looking to expand our hospital and patient outreach and support, which will entail establishing more local support groups and updating our website to include more patient resources. On the research front, we will continue to search for new cardiomyopathy studies to fund and will look into collaborations with relevant research study groups. We will also be finalizing a joint American Heart Association and CCF research grant, which will be announced year-end.

We hope the momentum of what we’re doing now will carry over to the second half of the year. Our annual fundraiser, the 5th Annual Golf Classic, is planned for July 23rd. Due to the success of past outings, we will be upgrading to a 36-hole course at the Montclair Golf Club in New Jersey. This is an important event because it dictates what we will be able to fund in research grants and patient programs in 2008. As such, I hope that all of you will somehow participate as a sponsor, donor, or attendee at this meaningful event.

CCF is pleased to announce that the Board has selected Dr. Anne Dipchand and Dr. Tain Yen Hsia as 2007 research grant recipients. Dr. Dipchand will be awarded $42,642 to analyze heart transplantation as a treatment for children with cardiomyopathy. Dr. Hsia will receive $50,000 to study the importance of the extracellular matrix of the heart in pediatric cardiomyopathies.

Dr. Dipchand is a pediatric cardiologist and the head of the Pediatric Heart Transplant Program at the Hospital for Sick Children in Toronto, Canada. She is one of four cardiologists in the Heart Failure and Cardiomyopathy Program, as well as an echocardiographer. Her study proposes to describe what happens to children with cardiomyopathy once they get listed for heart transplantation. Although the treatment for heart failure from various forms of cardiomyopathies continues to improve, the number of children who require heart transplants has not declined.

“We do not have enough information on Highlights

- Updates on CCF’s 2006 Funded Research .......... pg. 3
- Q&A: Medical Insurance Issues ......................... pg. 5
- Points to Consider: Thinking about Family Planning .. pg. 6
- Children’s Cardiac Camps ............................... pg. 10

First International Pediatric Cardiomyopathy Scientific Workshop TAKES PLACE

On January 25-26 more than 30 of the leading experts in the field of pediatric cardiomyopathy gathered together in Bethesda, Maryland for the first international scientific workshop on pediatric cardiomyopathy. The conference was jointly organized by CCF and the University of Miami Miller School of Medicine. The conference was also supported in part by grants from the National Heart, Lung, and Blood Institute, Genzyme Therapeutics, and CIBC World Markets.

The attendees included clinicians and researchers specializing in a range of disciplines: pediatric cardiology, pediatric gastroenterology, surgery, genetics, adult internal medicine, adult cardiology, biostatistics, and epidemiology. Attendees came from all over the United States, Canada, and Australia to participate in this important meeting.

continued, page 8

continued, page 4
CCF ATTENDS NHLBI MEETING

CCF was recently invited to attend the eighth annual Public Interest Organization (PIO) Meeting organized by the National Heart, Lung, and Blood Institute (NHLBI). Held February 12-13 in Bethesda, Maryland, the meeting was attended by eighty public interest organizations. This meeting marks the fourth year that CCF has been invited to participate.

During the meeting, Dr. Mark Gladwin (NHLBI) shared his findings on nitrite therapy, Mr. Rino Aldrighetti (Pulmonary Hypertension Association) provided viewpoints on pulmonary hypertension, and Dr. Carl Roth (NHLBI) outlined the NHLBI grants process. Dr. Lawrence Friedman (NHLBI) spoke about understanding clinical trials, and Dr. Alice Mascette (NHLBI) presented clinical perspectives on stem cell technology. There was also a PIO panel discussion on “Engaging the Scientific Community in Research on Rare Disease”. Among the key concerns of the NHLBI and attending organizations was more funding for research and greater attention to health on the part of the general public.

CCF CENTER SURVEYS: A Reminder to Physicians and Nurses

For those hospitals and clinics that received our center survey in January, please take a few minutes to complete the survey. We are in the process of compiling a physician/center referral guide for at risk or diagnosed families and would like to update your center’s information.

To request a survey, please contact Stormy Hill, CCF Patient Outreach and Support Coordinator, at thill@childrenscardiomyopathy.org.
• Exercise Intervention in a Pediatric Population with Cardiomyopathy

– Tracie Miller, MD

Dr. Tracie Miller of the University of Miami Miller School of Medicine is currently doing research to determine whether the benefits of physical activity can be applied to children with cardiomyopathy. More specifically, she is evaluating the effects of exercise on heart function and size, functional capacity to do work, and quality of life in children with cardiomyopathy. This study will also determine if exercise is safe in children with cardiomyopathy such that it does not adversely affect their cardiac health. This topic is critically important as current clinical care downplays the importance of exercise in children with cardiomyopathy, and most clinicians do not advocate for structured physical activity that pushes the child to his/her cardiac threshold.

The exercise rehabilitation program currently has seven subjects enrolled, and recruitment for the three-month program is on-going. Dr. Miller recently presented preliminary results at CCF’s scientific workshop on two subjects who completed the study. The initial results show improvements in flexibility, strength, aerobic capacity (a measure of cardiac health), bone mineral density, and muscle mass. Additionally, improvements were seen in quality of life and physical activity as measured by questionnaire surveys. The measures of cardiac function (through echocardiography) showed that the children’s hearts remained stable and did not deteriorate with three months of training. The blood for mitochondrial mutations is being banked until Dr. Miller reaches final recruitment. It will then be analyzed to determine whether there is a genetic association to the child’s response to exercise.

The results, although promising, are considered preliminary as the original study proposed to recruit 20 children. Recruitment remains one of the greatest challenges as this study requires a full three-month commitment. Dr. Miller hopes to augment enrollment by presenting preliminary results to local cardiologists to stimulate more referrals. Furthermore, with school ending during the summer months, it is expected that more children will be able to participate since there will be less conflict in their schedules.

• Identification of Mutations in Genes Associated with Hypertrophic Cardiomyopathy and Dilated Cardiomyopathy

- Jeff Towbin, MD

Dr. Jeff Towbin has started his analysis of cardiomyopathy related candidate genes as proposed in his study. Several novel genes for dilated cardiomyopathy and hypertrophic cardiomyopathy have been identified by Dr. Towbin’s laboratory during the past year. The focus of the studies has been on the Z-disk and the genes encoding proteins involved in the structure and function of the sarcomere. These studies were designed to evaluate the mechanosensors of the sarcomere and Z-disk in particular. In addition to the genes identified, animal models and mechanosensory-stretch-induced mechanistic evaluations have been performed. Although significant progress has been made, the specific details of the underlying mechanisms resulting in the clinical features of the disease have not yet been completely worked out. Studies designed to answer these questions are ongoing.

• Update on CCF’s Funded Pediatric Cardiomyopathy Repository

The concept for the Pediatric Cardiomyopathy Repository was initiated by CCF in 2004 and launched in late 2005 with the participation of 11 top pediatric cardiomyopathy centers. The goal of the repository is to collaborate with investigators interested in defining the causes of pediatric heart muscle disease and heart failure and in understanding the genetic and clinical correlations. The repository is linked to the National Institutes of Health (NIH) funded Pediatric Cardiomyopathy Registry and is supervised by a steering committee consisting of Lisa Yue (CCF Executive Director), Steve Lipshultz, MD (PCMR Principal Investigator, University of Miami), Lynn Sleeper, ScD (New England Research Institute), Jeff Towbin, MD (Laboratory Director, Baylor College of Medicine), Steve Webber, MD (University of Pittsburgh), Bruce Gelb, MD (Mount Sinai), Elizabeth McNally, MD, PhD (University of Chicago), and Wendy Chung, MD, PhD (Columbia University).

The repository, housed at the Baylor College of Medicine, has enrolled a significant number of multi-center-multi-site subjects and has successfully obtained specimens (blood, tissue) from these subjects. In addition, clinical data from the enrolled patients has been obtained and stored at the Data Coordinating Center at the New England Research Institute. Over 100 blood and 15 tissue samples have been collected to date and active recruitment continues.

The Director of the Biological Specimen Laboratory, Jeff Towbin, MD, has recently applied for supplementary funding from the NIH (National Heart, Lung, and Blood Institute). The grant received an excellent score and the likelihood of funding will be confirmed in the next 2-3 months. If funded, this would enable a dramatic increase in collaborative samples to be available for scientific and clinical evaluation. This, in turn, will advance the study and understanding of pediatric cardiomyopathy in a more rapid fashion than was previously possible.
The purpose of the conference was to determine the focus of research on pediatric cardiomyopathy and to discuss research possibilities. The conference presentations centered on three general themes: Molecular and Genetic Issues, Epidemiology, Etiology and Outcomes, and Clinical Issues. Each session featured presentations from top cardiomyopathy specialists followed by a round table discussion and a question and answer period.

Opening remarks were given by Dr. Steve Lipshultz, Chair of CCF’s Medical Advisory Board and Chairman of Pediatrics, University of Miami Miller School of Medicine and Lisa Yue, CCF’s Executive Director. They each expressed their excitement at being able to have this pivotal and innovative conference.

The first workshop, “Molecular and Genetic Issues” was moderated by Dr. Jeff Towbin (Texas Children’s Hospital). This session consisted of eight presentations centered on genetics, targeted therapies, genetic causes of hypertrophy, mitochondrial cardiomyopathies, mouse models for the disease, cell based therapies, and stem cell based cardiac repair.

The second workshop was moderated by Dr. Steve Lipshultz and Dr. James Wilkinson (University of Miami Miller School of Medicine). The topic of this session was “Epidemiology, Etiology and Outcomes.”

The afternoon consisted of eight more presentations on the Australian experience with cardiomyopathy, the North American Pediatric Cardiomyopathy Registry data, diagnosis of metabolic and genetic etiologies, predictive genetic testing, myocarditis, arrhythmogenic right ventricular dysplasia/cardiomyopathy registry, and restrictive cardiomyopathy.

The third and final workshop was moderated by Dr. Steven Colan (Children’s Hospital Boston) and revolved around “Clinical Issues in Pediatric Cardiomyopathy.” These eight presentations focused on such topics as functional status, peripartum cardiomyopathy, beta-blockers and dilated cardiomyopathy, and novel medical therapies for heart failure. Enzyme replacement, surgical management, heart transplantation, age-related differences in heart transplantation, and physical activity as it relates to cardiomyopathy were also covered.

During the evening session there was a preview of CCF’s patient video on pediatric cardiomyopathy followed by several presentations by young investigators. Topics reviewed include LAMP2 animal models of hypertrophic cardiomyopathy, Noonan syndrome mutations, serum creatinine as a pre-transplant predictor of outcome, neurohumoral and inflammatory association in heart failure, adequacy of echocardiographic screening in relation to genetic testing, and outcome predictors for dilated cardiomyopathy.

The conference was the first of its kind and brought together leading physicians and researchers to brainstorm and share ideas with one another. It was an exciting “first step” towards better understanding the disease and finding cures. An important outcome of this meeting will be the ability to share this vital information with the rest of the pediatric cardiology community and to draw attention to the state of the disease. Proceedings from the meeting and a consensus statement from the participants will appear in a three part series in Progress in Pediatric Cardiology, a leading journal on pediatric cardiology. The first issue is expected this summer. Presentations from the meeting will be available for viewing via webcam on CCF’s website in the near future.

HELP CCF FUND MORE RESEARCH
Provide Contacts for Corporate Donations

CCF is always searching for new contacts for corporate donations, matching gifts, and foundation grants to help us further our research efforts and expand our programs. Last year, we raised close to $104,000 from corporate and foundation sources. Many of these donations and grants materialized from CCF donor referrals and nominations.

Does your company have a corporate giving/charity program or provide foundation grants in which CCF can be considered? Perhaps you know someone who has contacts to pharmaceutical companies, medical device companies, or other charitable giving programs that CCF can apply to for assistance. Having an inside contact or referral can make a tremendous difference in securing project funding.

For more information, please contact: Pauline Pierrot, Assistant Executive Director at ppierrot@childrenscardiomyopathy.org or 866-808-2873 ext. 902.
1) What are the toughest issues regarding insurance and medical costs that you see in families affected by cardiomyopathy?

Some of the major concerns are referrals to the cardiologist and co-payments for both visits and medications. Families need to check in with the hospital social worker as well as their employers to find out what benefits they may or may not have with regards to the Family Medical Leave Act and how this may affect insurance.

When a parent receives any concerning diagnosis for their child they should also contact their insurance company and make an appointment to speak with a case manager to understand what their policy covers.

2) How should families go about finding out what assistance programs are available in their state?

Consult both the hospital social worker and, if available, internet sites. If a family is seen in a hospital clinic, the pediatric cardiologist should be able to put them in contact with the social worker. If they receive care at a private practice, the primary care physician should be able to get them in touch with a social worker as well. Or, families can always contact case management through their insurance company.

One of the most important things to remember with a diagnosis such as cardiomyopathy is that the parents become the child’s biggest advocate the moment they hear the diagnosis. As soon as possible, parents should begin speaking with the cardiology team at the hospital and find out what resources are immediately available to the patient and the family as a whole.

3) If a family does not have private or group insurance, what options are available to them?

Medicaid is one option, depending on the family’s income. In Michigan, there is a program called MI Child that provides insurance for uninsured working families. In addition, families should inquire into the possibility of applying for Children’s Special Healthcare Services. Services vary widely across states. Prior to moving, I would encourage all families to verify coverage of their child’s diagnosis in the state to which they are moving. In addition, all pharmaceutical companies have funding for indigent patients. Individual companies would need to be contacted.

4) What should a family do if they are about to max out on their child’s health insurance?

A family should consider applying for Supplemental Security Income through the Social Security Department as soon as their child has been diagnosed with cardiomyopathy. Families should consult a hospital’s financial coordinator if one is available, as well as the hospital social worker. All families should inquire about coverage through supplemental insurance such as the Children’s Special Health Care Services, as these vary state to state.

5) What alternatives are there once they have a pre-existing condition?

Families should discuss alternatives with the case management services through their insurance. Also, always check in with the hospital social worker.

6) Some parents are worried that their child will not be able to get life insurance once they are diagnosed or if their other unaffected children are screened and found to have cardiomyopathy. Some have elected to get life insurance before screening their other children. What assistance programs or piece of legislation should families be aware of related to this issue?

This is a very individual family decision. The health of the children should always be paramount; so, if a child needs medical intervention, life insurance should be a secondary concern. However, there is the Genetic Information Nondiscrimination Act that prohibits discrimination in health insurance and employment prospects based on genetic information. For federal and group employer plans, health insurers are prevented from denying coverage or adjusting premiums based on an individual’s predisposition to genetic conditions. Families should call their state representatives and consult the state websites to find additional information.

7) Many families overseas need medical care in the U.S. but do not have the financial means to pay for it. Are there any organizations that assist in covering medical costs and travel expenses?

Depending on the country, there may be government programs that assist in funding for travel and procedures. Also, families may want to seek assistance from their local religious organizations. I recently heard of an organization, First Hand Foundation, located in Kansas City. The contact person for this is Mary Nelson, RN, BSN (816) 201-1569. The Foundation assists with medical needs for kids age 18 and under; including transplant expenses, assistance for international patients and procedures, office visits, prescriptions costs, etc.
After a child is diagnosed with a genetic condition such as an inherited cardiomyopathy, parents who are considering having another child, or other family members who are considering starting a family, often want to know if there are options available to them to have a child that is not at risk for developing cardiomyopathy. It is recommended that couples meet with an informed outsider, a genetic counselor, to understand their risk factors and to discuss family planning options that may best fit with the couples’ personal beliefs and value systems.

The best time to meet with a genetic counselor is prior to pregnancy. This allows couples time to consider all feasible options and to complete any genetic testing that may be needed for the child diagnosed with cardiomyopathy. Some couples will use information gained through prenatal genetic counseling and testing to prepare themselves for the possibility that a future child may develop cardiomyopathy. Some will use the information to help them determine whether or not to continue with the pregnancy; whereas others will decide that they feel most comfortable entering a pregnancy without information prior to the birth of their child. Any decision that a couple may make related to family planning is valid, and the genetic counselor’s role is to guide them through this difficult and emotional decision-making process.

Although genetic testing options for inherited forms of cardiomyopathy have increased over the past several years, there are still many families who will go through the genetic testing process, and a genetic cause for the condition in their family will not be identified. In this scenario, a meeting with a genetic professional is still beneficial. It can help to determine the manner in which the condition appears to be running in the family based on medical history and echocardiogram and electrocardiogram results from all first degree relatives (brothers, sisters, mother, father or children) of the affected individual. Determining the mode of inheritance or whether the cardiomyopathy appears to be originating from one or both sides of the family allows for a better risk assessment of children that may inherit the disease.

For families who have performed genetic testing and a specific genetic mutation has been identified to cause cardiomyopathy in the family, preimplantation and prenatal genetic testing are options. If the condition appears to be X-linked in the family, micro sort can also be considered.

• **Preimplantation Genetic Diagnosis (PGD)**

PGD is testing done prior to pregnancy and involves harvesting eggs from the mother and then proceeding with in vitro fertilization in a petri dish using sperm from the father. Once the embryos have grown to the 8-cell stage, one to two cells are removed from each embryo and sent for genetic testing and chromosomal analysis. Once results come in, couples would be able to implant only those embryos that do not carry the genetic mutation known to cause cardiomyopathy in the family. The risks of PGD are similar to that of a traditional in vitro fertilization procedure.

• **Prenatal Diagnostic Testing**

Amniocentesis testing involves sampling the fluid surrounding the developing fetus and then performing chromosomal and genetic testing for cardiomyopathy on these cells. This testing can be performed at the 16-18th week of pregnancy. There is a small risk of miscarriage associated with amniocentesis.

• **MicroSort**

If the disease is determined to be a X-linked form of cardiomyopathy where the disease is passed on from the mother to a male child (i.e. Barth Syndrome, Danon Disease or Duchenne Muscular Dystrophy). MicroSort may be used to screen for gender. This fairly new technology involves sorting the father’s sperm by weight, as X chromosome sperm have a different weight than Y chromosome sperm. Then the couple either proceeds with intrauterine injection or in vitro fertilization of the “X” sorted sperm to increase the likelihood of conceiving a female.

For couples that do not have a specific genetic diagnosis, there are different options for decreasing the risk of future children developing cardiomyopathy. These options include egg or sperm donation if the cardiomyopathy is transmitted from one side of the family or embryo and traditional adoption for cardiomyopathies that are recessive in nature (both parents contribute a defective gene).

• **Sperm or Egg Donation**

If the parent identified to have cardiomyopathy would like to remove the possibility of passing on the disease to their future children, this option would allow a couple to proceed with a pregnancy with either donor sperm or donor egg. Some centers do perform limited genetic testing on their egg or sperm donors for carrier status of common inherited conditions.
Planning

• Embryo Adoption
Many couples that have gone through the in vitro fertilization process have excess frozen embryos that they may make available to other couples for adoption. This form of “adoption” allows couples to experience the pregnancy and birth of their adopted child while removing the chance that their future child may have cardiomyopathy.

• Adoption
Traditional adoption is always an option for couples that would like to parent another child but are concerned with the risk of passing on an inherited condition to their child.

Some of the options mentioned above are not available at all medical institutions, and some are only available at specialized reproductive centers. Additionally, several of these options, such as PGD and Microsort if individuals are utilizing the procedure for family planning based on having a previous child with an X-linked condition.

Nicole Johnson is a genetic counselor for the Cardiomyopathy and Heart Failure Group at John Hopkins University (JH). She is also a study coordinator for the JH Familial Cardiomyopathy Registry. Nicole can be reached at 410-502-2578 or njohnso5@jhmi.edu

Schools’ Obligation to have PORTABLE PUBLIC DEFIBRILLATORS

By Rachel Moyer

The federal government has established under the Rehabilitation Act of 1973 Section 504 that a child cannot be denied access to “reasonable accommodations” in his or her school regarding health issues that may impair his or her ability to function in major life activities. An example of impairment would include difficulty in walking, seeing, hearing, speaking, breathing or taking care of oneself.

If your child has been diagnosed by a physician or cardiologist to have a heart condition or health impairment, you are entitled to the benefits of this legislation. Examples of “health impairment” include but are not limited to: asthma, allergies, seizures, epilepsy, hypertrophic cardiomyopathy, Marfan syndrome, Barth syndrome, Brugada syndrome, Wolff-Parkinson-White syndrome, Long and Short QT, ARVD, Myocarditis or heart murmur.

Your medical doctor or cardiologist may write a prescription for an automated external defibrillator (AED) to be available on school grounds and also on field trips for your child.

If your school district refuses to provide an AED for your child at the 504 hearing, and you have provided a prescription for an AED from your doctor, you can then file a complaint with your regional Office of Civil Rights of the U.S. Department of Education or sue in federal court.

For information on how to file a 504 complaint, go to www.ed.gov/about/offices/list/ocr/complaintintro.html or call (800) 421-3481 (TDD) (877) 521-2172.

For additional information about Section 504, visit the Department of Education website: www.ed.gov/about/offices/list/ocr/504faq.html

Rachel Moyer, a CCF member, lost her 15 year old son to sudden cardiac arrest from undiagnosed dilated cardiomyopathy. She was recently featured on NBC Nightly News for her lobbying efforts to get portable defibrillators (AED’s) into schools. Rachel can be reached at Rachel@parentheartwatch.com

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how to predict when these children should be listed for a heart transplant and what things might make them more or less likely to survive a heart transplant,” notes Dr. Dipchand. “Different doctors and hospitals use different criteria to make decisions about the need for and timing of listing for a heart transplant with very little clinical information to guide the decision-making process.” Dr. Dipchand feels that timing is crucial when it comes to putting a patient on the heart transplant list — too soon and the patient could be deprived of less invasive treatments like good medications; too late and the patient could end up very sick on the bridge to transplant machines or not survive in time for the transplant.

CCF is pleased to announce that the Board has selected Dr. Anne Dipchand and Dr. Tain Yen Hsia as 2007 research grant recipients.

Dr. Dipchand’s interest in cardiomyopathy evolved from her job as a pediatric cardiologist involved in heart transplantation. “About 50% of kids who come to need a heart transplant have some type of cardiomyopathy. It is hard to take care of this challenging group of patients so regularly and still not know for some of them what is the best thing from a survival and quality of life perspective. I want to have a better way to predict when to list them for a transplant so that they have the best chance at long term survival and a great quality of life.”

Using the information gathered from more than 1,100 cardiomyopathy patients in the Pediatric Heart Transplant Study Group, Dr. Dipchand will identify factors that make these types of patients more likely to need a heart transplant, to survive a heart transplant, and the timing they should be listed. The study can help determine how such pre-transplant factors such as sex, age, type of cardiomyopathy, and treatment can impact post-transplant survival and quality of life. According to Dr. Dipchand, “the information learned from such a large group of patients will almost certainly be important for the clinical management of and future study of children with cardiomyopathy.”

CCF’s second grant recipient is Dr. Tain Yen Hsia. Dr. Hsia is a Pediatric Cardiothoracic Surgeon at the Medical University of South Carolina Children’s Hospital. He is one of only two surgeons in South Carolina specializing in congenital heart diseases. He is also an Assistant Professor of Mechanical Engineering and Bioengineering at Clemson University.

Dr. Hsia’s interest in pediatric cardiomyopathy was sparked during his medical training while caring for children afflicted with the disease. In one case, a 13 year-old girl came down with flu-like symptoms and was subsequently diagnosed with viral dilated cardiomyopathy. Unfortunately, her heart did not respond to any medical therapy and she quickly was in need of a heart transplant. While waiting for a donor heart to become available, she was temporarily given a mechanical device called the Berlin Heart. She eventually underwent a successful transplant, but the experience made Dr. Hsia realize that cardiomyopathy is a disease that is not well understood. “As a surgeon, what I have to offer is imperfect in treating the disease. Heart transplantation is just another disease we trade cardiomyopathy for because it has its own set of complications and problems.” He saw this as a challenge to better understand the disease, and to come up with more reliable and less complicated ways to monitor and treat it.

Dr. Hsia chose to focus on the heart’s extracellular matrix as a potential area of exploration. The extracellular matrix consists of proteins, collagen and elastins that form the integral structure of the heart. Dr. Hsia likens it to the wooden frame of a house. “It provides the scaffolding upon which the cells of the heart can build healthy architecture which is essential for normal heart function.” In the heart, the regulation of the extracellular matrix belongs to a set of enzymes called matrix metallo-proteinases (MMPs) and their inhibitors (TIMPs). MMPs’ function is to chew away excess structure that the heart does not need whereas TIMPs prevent this. In a healthy heart, MMP and TIMP work in harmony to ensure a balance between matrix degradation and buildup. Dilated cardiomyopathy (DCM) is an example of what happens when the MMPs are too active, causing too much of the matrix to be degraded and the heart to become thinned. Hypertrophic cardiomyopathy (HCM), on the other hand, is an example of what happens when the TIMPs are too active, preventing the MMPs from doing their job and causing too much extracellular matrix to be deposited. This contributes to the heart becoming thicker in HCM.

The objective of Dr. Hsia’s research is to uncover the specific role MMPs and TIMPs play in pediatric cardiomyopathies, to examine differences from adult cardiomyopathies, and to develop a way to measure these enzymes from small blood samples. The study will examine serum and tissue MMPs and TIMPs in children afflicted with idiopathic DCM collected through biopsies, explanted hearts, and stored samples from CCF’s Pediatric Cardiomyopathy Repository.

Dr. Hsia hopes that the findings will explain why cardiomyopathy issues in children are so much more severe than in adults. “We also aim to show a direct relationship between heart failure severity and these enzyme levels. This will allow us to use MMPs and TIMPs as new biomarkers to predict the advent of heart failure and the progression of the disease before symptoms appear,” he says. Depending on the research results, improvements in cardiomyopathy screening, diagnostics, prognostics, and treatment can be made.
TWELVE-STEP SCREENING
May Help Reduce Sudden Death In Young Athletes

A 12-point screening process could help reduce sudden cardiac death in high school and college competitive athletes according to The Recommendations for Preparticipation Cardiovascular Screening of Competitive Athletes, published in the March issue of Circulation, a journal of the American Heart Association. The recommendations include 12 questions about personal and family medical history and a physical examination to uncover aspects of a potential athlete’s health that could signal a cardiovascular problem. If any of the 12 screening questions has a “yes” answer, the participant would be referred for further cardiovascular examination.

The incidence of deaths is roughly one in 200,000 high school-age athletes per year, based on a twelve year Minnesota study of 1.4 million student-athlete participations in 27 sports. “Although the frequency of these deaths in young athletes appears to be relatively low, it is more common than previously thought and does represent a substantive public health problem,” said Dr. Barry J. Maron, director of the Hypertrophic Cardiomyopathy Center at the Minneapolis Heart Institute Foundation and the chair of the Circulation writing group.

In the United States, these deaths occur most commonly in basketball and football — high intensity sports with high levels of participation. Although the European Society of Cardiology and International Olympic Committee include routine electrocardiograms (ECG) for all potential athletes, there is some debate on whether mass prescreening of competitive athletes should include an ECG before they are allowed to participate in team sports. Dr. Maron says current U.S. recommendations don’t include widespread, routine ECGs, largely due to a lack of policy mandate and infrastructure to support this. In addition, there is a significantly higher number of competitive athletes in the U.S. Currently, “the relevant athlete population available for mass screening may be as large as 10 million people per year,” the panel wrote. The total estimated cost of mass screening for that many athletes, along with the follow-up required for abnormal findings, is more than $2 billion a year. Given the lack of physicians and other medical resources for performing and reading ECGs, and no laws to mandate the standards for pre-participation screening, Dr. Maron believes the cost effectiveness and feasibility of such a program in the United States cannot justify such a recommendation at this time.

The panel does, however, recommend the development of a national standard for cardiovascular screening of high school and college athletes and notes there has been significant improvement in the support and adherence to life-saving screening processes for youth participating in sports. As of 2005, 81% of states support adequate screening processes.

This story has been adapted from a news release issued by the American Heart Association.

Focusing on PRE-PARTICIPATION ATHLETIC SCREENINGS

According to the Center for Disease Control (CDC), 300,000 people die annually from sudden cardiac arrest, with 10% of those deaths occurring in young people between the ages of 15 and 34. It is also estimated that 14,000 children and infants die annually from sudden cardiac death (SCD). Often the primary cause of SCD in young people is cardiomyopathy because many victims are not diagnosed in time for treatment. The CDC claims that such deaths are preventable through the proper and timely use of echocardiogram screenings.

An echocardiogram, an ultrasound of the heart, is the gold standard for detecting fatal heart conditions. However, nationwide screenings have not been possible because echocardiograms are quite expensive and not usually covered by insurance companies.

One organization trying to increase screening availability is The Chad Foundation for Athletes and Artists (www.chadfoundation.org). The Foundation was established by Arista in memory of her son, Chad Butrum, who died of SCD. Chad was 26 when he collapsed during a game of football and later found to have dilated cardiomyopathy. The Chad Foundation exists to raise awareness of SCD through educational lectures and free cardiovascular screenings in high schools and colleges. From previous echocardiogram screenings, The Chad Foundation have detected, on average, 10 structural anomalies in every 100 children screened. They also point out that in Italy where a nationwide screening system was implemented, the incidence of sudden cardiac arrest among competitive athletes declined significantly.

In August, the foundation will be partnering with Dr. Theodore Abraham from the Johns Hopkins’ Hypertrophic Cardiomyopathy Clinic in a community screening drive. Heart screenings are also being planned in Minneapolis and Harlem in the Fall of 2007. CCF is working with the Chad Foundation to support their New York City heart screenings.
Cardiac camps have been established throughout the country to allow children with heart disease to enjoy a variety of fun physical activities in a medically safe environment and in the company of other children with similar health issues. The programs and facilities are tailored to the needs of young heart patients with medical personnel on staff to supervise and attend to medical emergencies. These programs, specific to children with congenital or acquired heart disease, have been praised for helping children accept their heart condition, build self confidence, develop life skills, and meet other children with similar health, emotional and social concerns.

Camp Bon Coeur
www.heartcamp.com
- Two 2 week sessions in July; one session for ages 8-12, one session for ages 12-16
- Located in Richardson, LA and includes a junior Olympic pool, miniature golf, nature trail, craft room, tennis courts, and canoe lake
- Cardiac nurses available 24 hours/day; camp infirmary is equipped to deal with cardiac emergencies and includes a defibrillator; Lafayette General Medical Center is nearby
- $1200 to attend but financial assistance is available
Tel: 337-233-8437
E-mail: info@heartcamp.com

Camp Braveheart
- Organized by the Children’s Healthcare of Atlanta for children ages 7-18
- Takes place one weekend in October at Camp Twin Lakes in Rutledge, GA
- Facility designed for children with medical concerns; volunteer doctors and nurses on site
- No fee to attend
Tel: 404-785-6735
E-mail: campbraveheart@choa.org

Camp Del Corazon
www.campdelcorazon.org
- Three 5 day sessions each year; Memorial Day weekend for ages 7-17, end of August for ages 7-12, and Labor Day weekend for ages 13-17
- Located in Malibu or Catalina Island, CA
- Run by volunteer doctors, nurses, and counselors; fully equipped infirmary and emergency airlift plan
- No fee to attend
Tel: 888-621-4800
E-mail: information@campdelcorazon.org

Camp Odayin
www.campodayin.org
- 3-day day camp for ages 6-7, 5-day residential camp for ages 8-17, and weekend family camp for families with at least one child with heart disease
- Located in Minnesota from end-July to mid-August or Wisconsin in mid-October
- Activities include archery, hiking, arts and crafts, golf, and talent shows
- Medical professionals including cardiologists and cardiac nurses available either on-site or on-call; counselors have been trained for medical emergencies
- $25 registration fee
Tel: 651-351-9185
E-mail: info@campodayin.org

Edward J. Madden Memorial Open Hearts Camp
www.openheartscamp.org
- Four 12-day sessions for ages 8-12, 13-14, 15-year-olds, and 16-year-olds held from end June-end August
- Located in Berkshires, MA
- Program designed in consultation with pediatric cardiologists specifically for children who have recovered from open-heart surgery or heart transplant surgery
- $250 to attend.
Tel: 413-528-2229
E-mail: info@openheartscamp.org

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CCF Local Support Group Activities

Georgia Group
CCF families, Brian & Elizabeth Tasker, Doug & Audrey Callahan, and Todd & Amy Urowsky participated in the American Heart Association Annual Heartwalk held Nov 5th to raise funds for pediatric heart research.

Michigan Group
First meeting held Nov 19th at Children's Hospital of Michigan and moderated by Dr. Richard Humes, Jill Matson, RN, and Rebecca Morocco, MSW. Attended by CCF families, Scott & Penni Newport, Steve & Tina Paulson, Daren & Stormy Hill, Brian Hill, and Taunya Gaston

Representatives of the Michigan Group

NATURE’S FOOD PATCH Fundraiser

Sean and Nicole Balsley turned the 20th Anniversary of their health food store Nature’s Food Patch Health into a fun-filled fundraiser to benefit CCF. The outdoor event, held on February 18th in Clearwater, Florida, attracted approximately 2,000 people. The Balsley’s thought it would be a great opportunity to raise awareness and funds for CCF in honor of their son, Rain, their 7 year old son, who was recently diagnosed with restrictive cardiomyopathy and is currently waiting for a heart transplant.

The all day event included free food and drinks, live music, a variety show, a balloon clown, and face-painting for the kids. There was also a raffle for numerous gift baskets and donated items. A special booth was set up to distribute information about cardiomyopathy, which included CCF fact sheets and brochures.

50% of the raffle proceeds and 15% of the day’s sales will go to CCF. “All in all, a terrific turn-out. We are so grateful to all that made this event possible,” say Sean and Nicole.

Living Courageously with Cardiomyopathy

This new section of the newsletter is for parents to share advice, ideas, and experiences about living daily with CM. Each issue will feature a different question posed to members of CCF’s listserve.

What has helped you and your child the most in managing the anxiety of living with the unknowns of the disease?

“I have a friend who has lived through stage 4 brain cancer who told us to put up a big piece of paper on our wall and carry around a little notebook, and any time something made us feel a little better, to write it down. Sometimes these were little things, like, “his laminar blood flow looks fine,” or, “take a breath and just get through the next hour.” Sometimes we wrote things down to practice saying and thinking them, like, “we will get through this.””

Jonah (HCM)

Karen Jefferson, mother of Spencer (HCM)

“Sometimes we wrote things down for poster meant that though we were hearing all sorts of scary, destabilizing things we also had to listen for the good. And still now when we feel hopeless, we stand in front of our four posters and read the encouraging, hopeful, and simple things written there and almost always feel a little better. We remember that even when things seem at their worst there are good things out there to cast out to and hold onto. The unknown brings so much anxiety not exactly because it is unknown, but because we fill so much of our worst fears. We have to remind ourselves that we can fill the unknown up with all sorts of things, some of them even good.”

- Tara Geer, mother of Jonah (HCM)

My oldest son was diagnosed two years ago with HCM because his father was diagnosed with severe HOCM. While it’s not what I wanted to hear from the doctor; I consider the diagnosis to be a gift. It is a gift because at least now I have a fighting chance to protect him. By having this knowledge I can avoid activities that are usually innocuous. It is a gift I am thankful for every time he advocates for himself. It is a gift I will be thankful for every day of my life. Without this gift of an HCM diagnosis, it could have ended in tragedy.

- Karen Jefferson, mother of Spencer (HCM)

“I get great comfort from the CCF listserve. It is so nice to be able to talk to people who understand what we are going through. I get great comfort hearing about children who are older than my son who are living a fairly normal life with cardiomyopathy. It gives me hope for the future.”

- Carol Davis, mother of Nathan (DCM)

“We have two children with restrictive cardiomyopathy and one child that has already passed away a little over a year ago, four healthy children, and one on the way (expected in June). Probably the best thing that has helped our entire family with the anxiety of living daily life, knowing some of our children have this disease, was to ‘Let it Go!’ We live life day to day as normal as possible, and do not dwell in that unknown… That does not mean we are not educated or realistic about what we are dealing with by any means… Having already lost one child, I know what we are up against, so we have our minds made up that we will love them for however long we are blessed to have them and so we just choose to LIVE and let it all go.”

- Leslie Rossi, mother to Yelayna (RCM), Brody (RCM)
CCF family members, Francisco and Debbie Pereda of Virginia were planning a ten year memorial service for their son when Debbie contacted CCF about increasing awareness of cardiomyopathy and raising money for research. Her son, Timothy, was 17 years old when he died suddenly on November 26, 1996 while playing basketball after school. He was determined to have Arrhythmogenic Right Ventricular Cardiomyopathy (ARVC) after his death.

Debbie approached CCF about developing a “tribute card” to be used as table favors to honor her guests. CCF worked with Debbie to create personalized tribute cards to inform her guests that a gift was made to CCF in honor of them. Debbie also set up a table with CCF patient education materials to raise awareness. “The cards and all the educational materials were a great source of identity for Tim’s Memorial. Everyone who attended was deeply touched by our donation in their honor to Tim’s memory,” said Debbie.

The memorial service was followed by a “Reflections Brunch” where family and friends paid tribute to Tim Pereda. A balloon release at the Arlington National Cemetery completed the day. Debbie added, “It came as some closure in knowing that there is a foundation that exists for awareness of his condition.”

TOP DONORS Recognized in NYC

On February 26th, CCF held an dinner for 30 of CCF’s top donors in the tri-state area. Held at the New York Athletic Club in New York City, Lisa Yue, CCF Executive Director, updated donors on CCF’s collaborations, new projects, and current funding needs.

The Children’s Cardiomyopathy Foundation (CCF) is a national non-profit organization focused on pediatric cardiomyopathy - a chronic and potentially life-threatening heart disease that affects children. CCF is dedicated to improving treatments and finding cures through the support of research, education, and increased awareness and advocacy.

www.childrenscardiomyopathy.org
Tel: 866.808.CURE
Fax: 201.227.7016