From the PRESIDENT

A common question on our family listserv is, “How much exercise should I allow my child to engage in?” This topic always stirs up a lot of discussion, probably because there are no clear-cut answers. This is why we are excited about supporting Dr. Tracie Miller’s study on Exercise Intervention on Children with Cardiomyopathy. Her research will allow us to answer some fundamental questions on exercise with reliable data.

In this issue, we present the framework of Dr. Miller’s study along with a summary of the last published guidelines on sports and exercise by Dr. Barry Maron for the American Heart Association Committee on Exercise.

Another update in the research area is CCF’s grant recipient, Dr. Ju Chen, has secured a multi-year, million-dollar grant from the National Heart, Lung and Blood Institute for the research that we supported in 2005. We are thrilled with this accomplishment given the competitive NIH review process (only 10% of submitted applications are funded by the federal government).

In order to fund more pilot projects of this caliber, we continue with our fundraising efforts. Fortunately, our 2005 Holiday Joy campaign was well received, and we have raised $44,000 to date. My sincere thanks goes to those CCF families who helped to send appeal cards and who contributed their inspirational stories to our campaign.

Our work does not stop with one campaign. If we are to grow and achieve greater things, public support is needed year round. This simple concept forms the basis for our Spring Appeal theme. Please take a moment to read our “Plant a Seed of Hope” letter enclosed with this newsletter and participate in any way that you can. As the saying goes “Hope springs eternal”, and with these parting words, we hope that you will continue to make room in your heart for CCF.

– Lisa Yue, President & Founder

CCF Awards 2006 RESEARCH GRANT

CCF is proud to announce the recipient of its 2006 Research Grant Award.

A national request for applications focused on pediatric cardiomyopathy was issued early last year, and submitted research proposals were ranked and selected based on peer review by CCF’s Medical Advisory Board. This year’s grant of $45,000 went to Dr. Tracie L. Miller to investigate the effect of exercise in children with cardiomyopathy.

Miller, a pediatric gastroenterologist by training, heads the Division of Pediatric Clinical Research at the University of Miami School of Medicine. Along with Dr. Paolo Rusconi, a pediatric cardiologist and Medical Director of the Pediatric Heart Failure and Cardiac Transplant Program, Miller proposes to test what kinds of exercise, if any, can be recommended for children with cardiomyopathy.

Currently, it is accepted medical practice for parents and physicians to limit and even ban exercise for children with cardiomyopathy, even though most recommendations are based on anecdotal experience and not on scientific clinical trials. As a result, children with the disease become inactive, inactivity leads to deconditioning, and deconditioning leads to further inactivity. Interestingly, many studies support the prescription of exercise as a component of cardiac rehabilitation. For example, exercise

CCF Funded RESEARCHER RECEIVES MULTI-YEAR NIH FUNDING

The National Heart, Lung, and Blood Institute (NHLBI), part of the national medical funding arm of the U.S. government, will award a five year $1.25 million research grant to Ju Chen, PhD (University of California, San Diego) for his study “Role of Cypher in Cardiac Muscle.” His study will investigate the role of the Cypher gene (in humans known as ZASP) and its mutations in cardiac function.

Findings from this study will help us to understand the biological function of this newly identified gene at the molecular, cellular, and physiological levels. Furthermore it will provide insight into the mechanisms by which mutations in Cypher/ZASP cause human dilated cardiomyopathy and left ventricular non-compaction.

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This NHLBI funded study is a continuation of a study that CCF supported in 2005 entitled “Involvement of Cypher Specific Isoforms in Cardiomyopathy.” Cypher is a gene encoding 6 cytoskeletal proteins (isoforms) that expresses in both skeletal and cardiac muscles. Its malfunction is believed to contribute to the development of dominantly inherited dilated cardiomyopathy. The initial seed funding that CCF awarded to Dr. Chen provided the essential preliminary data to obtain funding from the NHLBI.

The original intent of Chen’s CCF funded project was to understand the role of the disease-causing Cypher gene by generating mouse lines to be models for these human mutations and to perform preliminary characterization of them. After one year of funding, Dr. Chen successfully generated two mouse lines in which long and short Cypher isoforms were deleted, and then observations were made on which mouse models developed cardiomyopathy, at what age and life stage, it’s severity, and final outcomes.

Preliminary data shows that both mouse lines developed cardiomyopathy with different phenotypes. Mice that expressed only the short isoform of Cypher developed a more severe form of dilated cardiomyopathy early in life at 2-3 months. These mice also did not respond to dobutamine, a strong drug used in cardiac therapy. Mice that expressed only the long isoforms of Cypher showed compromised cardiac function at 12 months, although normal heart function was observed at 3 months. In the next six months, Chen plans to alter the amino acid in the Cypher gene to create a mouse model that more closely replicates DCM found in humans. Additional characterizations of the new mouse model will be performed to gain a better understanding of the biochemical pathways leading to cardiomyopathy and to suggest therapeutic options to eliminate or correct the genetic abnormality causing the disease.

In Chen’s earlier studies, it was demonstrated that mice lacking the Cypher/ZASP gene did not survive after birth, whereas mice with short or long isoforms were able to survive to adulthood. In a 2003 Journal of the American College of Cardiology publication, Vatta, Towbin et al. suggest that Cypher/ZASP appears to be among the most commonly mutated gene for familial or sporadic DCM in both very young and older children to date.

**CCF Funded Researcher Receives Multi Year NIH Funding**

This NHLBI funded study is continued from the previous page.

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The Children’s Cardiomyopathy Foundation, including all parties to or associated with Heart to Heart will not be held responsible for any actions readers take based on their interpretation of articles in this newsletter. As always, readers are encouraged to discuss medical evaluations and treatments with their own physicians.

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CCF has slowly been growing in size since its creation four years ago. In addition to the Executive Director (Lisa Yue), CCF has a part-time staff that includes an Assistant Executive Director; two Project Coordinators and a Bookkeeper and Data Entry Assistant. As a team, they provide strategic and administrative support to keep the Foundation running smoothly.

Pauline Pierrot is the Assistant Executive Director. She is a development professional with over 15 years of fundraising experience with leading non-profit organizations. Prior to joining CCF, Pauline was the Director of Individual Giving for A Better Chance, a national educational support organization. Previously, she served as the Director of Major Gifts with Volunteers of America, the Director of Development with the Girl Scout Council of Greater New York, and as a Senior Manager at the Asia Society, the Children’s Health Fund and the Association on American Indian Affairs.

As Assistant Executive Director, Pauline is responsible for developing and managing CCF’s development and fundraising efforts. She also works closely with Lisa, the Executive Director, on strategic planning and assisting with the day-to-day operations of the Foundation. Pauline says, “I enjoy working on behalf of CCF’s children and families to provide education, support and research to a very high standard, while having the benefit of being involved in a wide range of activities, including fundraising.”

Project Coordinator Renee Thekkekara has a Masters in Business Administration from George Washington University and formerly worked as an Associate Publicist for Oxford University Press in New York City. Renee started out as a CCF volunteer and became a staff member in February 2005. “I found out about CCF through a website offering volunteer opportunities in Bergen County. Lisa’s story and mission moved me; I wanted to help out, even in a small way.”

Since Renee joined CCF, she has worked on a variety of communications projects including community and physician outreach, developing CCF’s press kit, writing press releases and letters of introductions, creating patient support materials, and putting together the Heart to Heart newsletter. Renee spent most of last summer organizing a silent auction and getting press coverage for CCF’s Annual Golf Classic fundraiser.

Project Coordinator Harriet Salk, with us since November 2005, is CCF’s newest staff member. Harriet worked for over ten years in sales, marketing and public relations in the broadcast media industry. At CCF, Harriet manages the family database and direct mailings, arranges conference calls and meetings, handles physician outreach and provides necessary administrative support to Lisa and Pauline. She also works on special projects such as organizing fundraisers and conferences as they come up. Harriet is glad to be at CCF “to help out and to learn.”

Finally, Christine Chun is a “retired” attorney, having worked for 7 years in New York City, and is now a stay-at-home Mom to her two young children. At CCF, she takes on administrative duties, doing data-entry, donation processing, and bookkeeping work. She learned about CCF when a friend invited her to a CCF fundraiser in 2004. Christine initially started as a volunteer helping with the annual golf fundraiser but became more involved in the summer of 2005. “I wanted to help Lisa in whatever way I could, because Lisa does so much!” said Christine.

Eligibility Requirements: Principal investigator must hold an MD, PhD or equivalent degree and reside in the United States. The investigator must have a faculty appointment at an accredited U.S. institution and have the proven ability to pursue independent research as evidenced by original research in peer-reviewed journals.

Available Funding & Award Duration: Funding is available in the range of $25,000 to $50,000 for one year of total direct costs. For grant renewals, CCF funding is limited to two years (consecutive or otherwise) of support.

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(Dilated, Hypertrophic, Restrictive, or Arrhythmogenic Right Ventricular Cardiomyopathy)
CCF Awards 2006
Research Grant  continued from page 1

is known to benefit adults with heart failure. Studies have shown that exercise programs help cardiac patients become more active with less fatigue and therefore feel better. Exercise also has proven physiological benefits, from improved blood chemistries to increased vital capacity. This CCF grant supports research designed to ask a basic question: can exercise benefit children with cardiomyopathy?

Dr. Miller has devised a clinical trial to test whether or not children with dilated cardiomyopathy (DCM) can benefit from a structured exercise program. The study will recruit twenty children with DCM from Miami-Dade County, home to Holtz Children’s Hospital. Prior to starting the exercise program, the children will undergo baseline studies of cardiac function, including echocardiography, metabolic stress testing, Holter monitoring, evaluation of body composition (how much lean and fat tissue a child has), strength and flexibility, blood vessel (arterial) function, and mitochondrial DNA assessment of both mutation and function. The participants will also complete quality of life surveys.

After completing the baseline assessment, the children will be instructed and carefully monitored in twice-weekly programs in the hospital’s exercise laboratory, performing both aerobic and resistance training. At the end of twelve-weeks, all baseline tests will be repeated. The participants will then be instructed in a three-month home-based exercise regimen, with telephone support and monthly evaluations at the exercise laboratory.

Twenty healthy controls, either siblings or friends of similar age and gender, will also be recruited to participate in this study. The use of controls will allow the researchers to compare improvements in cardiac function, body composition, quality of life, and vascular function between each study subject as well as between study subjects and controls.

The aim of the study is to prove that supervised exercise regimens will improve cardiac function, body composition, and quality of life for children with cardiomyopathy. Furthermore, some basic scientific research will be performed to explain how exercise helps the heart improve: are there greater effects to the heart tissue itself, are the beneficial effects targeted more to the blood vessels, or are there effects to both the heart and blood vessels? Additionally, this study will evaluate the effect of exercise on abnormalities in the mitochondrial (parts of the cell that produce energy) structure and function frequently found in children with DCM. It has been shown that endurance exercise training in healthy patients leads to significant increased mitochondrial volume and mass. This causal relationship could mean that the total productivity of mitochondria could potentially increase for children with cardiomyopathy, even if their mitochondria are defective. Higher mitochondrial productivity would allow these children to work harder without tiring as quickly.

Should this study prove that children with DCM can benefit from rehabilitative exercise, there are at least three positive outcomes. Firstly, physicians and parents will at last have proven, scientific guidelines to follow. Secondly, children themselves could experience a two-fold benefit: their health will improve, and they will be able to participate more fully in their own childhood by playing actively with their peers. Finally, with substantive scientific evidence, insurance companies will be compelled to pay for the services of this health benefit.

Dr. Miller’s interest in exercise research has grown directly from her work with children and nutrition. Since the late 1990s, Dr. Miller has been studying the nutritional needs of children with HIV infection. She has determined that exercise as a nutritional intervention can improve muscle mass, help control lipid levels, and prevent the progression of diabetes in a group of children who are at very high risk. Additionally, she has found that this exercise intervention improves overall activity, quality of life, and self-esteem. Dr. Miller has applied these same principals to a study of children who have survived cancer treatments.

Dr. Miller started her career both as a researcher and practitioner at the Children’s Hospital of Boston. She was then the Division Chief of Pediatric Gastroenterology and Nutrition at the University of Rochester before coming to the University of Miami to start the Division of Clinical Research in Pediatrics two years ago. Since her appointment, about half of the one hundred and fifty faculty members are now engaged in clinical research trials.
To gain insight into the kinds of information that would be most beneficial to newly diagnosed families, CCF conducted a national needs assessment study in 2005, comprised of online surveys with parents of children with cardiomyopathy and telephone interviews with medical professionals. Based on the research findings, CCF has developed new patient education materials to meet the demand for comprehensive and easy-to-understand pieces. Supported by a grant from the Medtronic and eBay Foundations, the series includes a 14-page color booklet, Understanding Pediatric Cardiomyopathy, and three shorter pamphlets focused on the most common forms of the disease: dilated, hypertrophic, and restrictive cardiomyopathy. In this way, families can take home a general explanation of the disease as well as more specific information on the type of cardiomyopathy their child has.

Working in concert with some of the leading physicians who specialize in pediatric cardiomyopathy, the materials were designed to answer many of the questions that parents have after diagnosis or while managing their child’s medical care. The main booklet provides an overview of pediatric cardiomyopathy, covering topics such as:

- What is pediatric cardiomyopathy?
- What causes it?
- How is it diagnosed?
- What are the symptoms?
- What are available treatment options?
- What is the likely prognosis?
- Should my family undergo screening or genetic testing?
- How will the disease impact my child and family?
- How do I cope with a chronic disease?

The booklet also includes a section on what research is underway and other resources. The accompanying inserts highlight the differences in causes, symptoms, diagnosis, treatment and prognosis among the different forms of pediatric cardiomyopathy.

To make these materials accessible to diagnosed families, CCF will approach the top forty pediatric cardiomyopathy, heart failure, and heart transplant centers for distribution. 210 additional centers will be mailed introductory packets, and they will be given the opportunity to order the materials at no cost. CCF will offer the materials directly to interested families as well. The full series will also be downloadable from CCF’s website under the Support Services/Publications submenu: www.childrenscardiomyopathy.org/site/pamphlets.php

For more information about the materials or to request copies, please contact Stormy Hill at 866-808-CURE, ext. 905 or thill@childrenscardiomyopathy.org with your order quantities and mailing address.

Foundation awarded $40,000 towards the development of a patient DVD. This audio-visual piece will complement the existing printed materials for families. The DVD will present, in a more personal and comforting manner, quality of life issues and the psychosocial aspects of being diagnosed with a chronic heart disease. To date, the American Legion Child Welfare Foundation has awarded over $8 million to educational projects that contribute to the physical, mental, emotional and spiritual welfare of children in the U.S.

CCF received $10,000 from CIBC World Markets through their signature giving initiative, Miracle Day, held on December 7, 2005. This was the fourth year that CCF was nominated to participate. More than $6.6 million was generated this year from trade generated fees and commissions to support charities that assist children in need or at risk. CCF will use the awarded funds to offset PEP administrative costs related to patient outreach and support.

The Medtronic Foundation awarded $30,000 for CCF’s Patient Outreach Initiative. The goal of this initiative is to reach more affected families through the top specialty treatment centers and to provide more targeted patient resources. This marks the second year that CCF has received program funding from Medtronic’s Patient Link Program. Through their Patient Link grants, the Medtronic Foundation supports programs that improve the lives of people with chronic diseases.

The DVD and patient outreach initiatives both evolved from CCF’s latest research findings. Results highlighted the need for a visual communication device and a dedicated patient support resource. CCF will continue to seek grants in 2006 in order to fully fund these two important projects.
SPORTS AND EXERCISE GUIDELINES
for Patients with Genetic Heart Disease

While most children diagnosed with cardiomyopathy are restricted from playing competitive sports, many still wish to participate in recreational and leisure-time activities to take advantage of the health benefits of exercise.

At this point, there is very little medical literature on what are acceptable levels of physical activity for children with cardiomyopathy. The last article published on this subject was in the June 8, 2004 issue of Circulation entitled “Recommendations for Physical Activity and Recreational Sports Participation for Young Patients with Genetic Cardiovascular Diseases.”

In this consensus paper, Dr. Barry J. Maron and a panel of clinical cardiovascular specialists and molecular biologists from the Working Group of the American Heart Association Committee on Exercise, Cardiac Rehabilitation, and Prevention provide recommendations on generally acceptable sports activities and recreational (non-competitive) exercise for young patients with genetic cardiovascular disease (GCVD). This group of congenital and/or inherited heart diseases include hypertrophic cardiomyopathy, long-QT syndrome, Marfan syndrome, Brugada syndrome, and arrhythmogenic right ventricular cardiomyopathy, which are all associated with an increased risk of sudden cardiac death during exercise. These guidelines extend to those diagnosed patients who are not trained athletes, but who wish to participate in recreational sports activities.

Exercise that are acceptable include:
- Moderately intense activities such as biking on flat terrain, modest hiking, informal jogging without a training regimen, lap swimming, sailing, doubles tennis, lifting free-weights, and walking on a treadmill.
- Low-impact activities such as bowling, golf, horseback riding, and recreational skating.

Activities that are highly not recommended include:
- “Burst” exertion activities, characterized by rapid acceleration and deceleration over short distances. These include basketball, soccer, tennis, ice hockey, racquetball, squash, and touch football.
- Physical exertion in adverse environmental conditions, such as particularly cold or hot temperatures, high humidity, or substantial altitude.
- Exercise programs that require systematic and progressive levels of exertion and are focused on athletic conditioning such as cycling, rowing, and road running. HCM patients, in particular, are recommended against such activities.
- Extreme sports such as bungee jumping and hang gliding.

Amusement park rides that are associated with sudden acceleration in heart rate and abrupt changes in centrifugal or centripetal forces such as roller coasters.
- Intense, static (isometric) exertion associated with activities such as bodybuilding and weight lifting.
- Excessive participation in sporting activities such as downhill skiing for long periods.

Sports in which the potential for traumatic injury or drowning is high if an individual is at risk for impaired consciousness (fainting). In this case, activities include horseback riding, weightlifting, rock/mountain climbing, ice hockey, downhill skiing, motorcycling, scuba diving, snorkeling, surfing, windsurfing, and diving.
- Contact sports or any activities that could lead to bodily trauma for individuals with an implantable cardioverter-defibrillator.

In regard to physical education classes in elementary and junior high school, physicians recognize the social and psychological significance of participation. Children forced to sit on the sidelines or take “alternative health” classes subject themselves to further scrutiny and ridicule from their peers. Therefore, it is recommended that participation in “normal” gym activities with some modification be determined in consultation with the pediatric cardiologist and school officials. This should be done after a careful review of the level of exertion involved and the assurance that the physical education instructor can monitor children with GCVD.

The American Heart Association panel stress that these recommendations do not apply to individuals with the following clinical features: symptoms of fainting, dizziness or impaired consciousness, prior cardiac surgery (myectomy), presence of an implanted cardioverter-defibrillator or pacemaker, and evidence of life-threatening arrhythmias. Other factors to take into consideration are the psychological and physical stress associated with sports participation, use of cardio-active drugs, environmental conditions, and the precise clinical profile of each patient.

“consultation with a physician is necessary to weigh the perceived risk with the benefit of exercise”

It is also important to note that recommendations for the article were formulated based on collective experience rather than quantitative data or published evidence. Therefore, consultation with a physician is necessary to weigh the perceived risk with the benefit of exercise. Dr. Maron writes, “...these recommendations are, to an extent, necessarily subjective and represent only a starting point for clinical judgments on individual asymptomatic patients with clinically evident GCVD.” Thus, as in most situations, these guidelines must be adapted to the needs of the patient in question and can vary from person to person.
SOY DIET Worsens HCM in Male Mice

In a recent study published in the January 4, 2006 edition of Journal of Clinical Investigation, University of Colorado researchers found strong links between environmental factors, namely diet, and cardiac disease. Specifically, the investigators observed a detrimental effect of a diet rich in soy-protein on male mice with a genetic predisposition to hypertrophic cardiomyopathy (HCM). However, the soy-protein diet did not have the same effect on female mice with HCM, nor did it have any effect on healthy mice of both genders.

For the study, Dr. Leslie A. Leinwand, Chair of the Department of Molecular, Cellular, and Developmental Biology at the university, and her colleagues compared the heart functions of male and female mice with HCM when given either soy-protein diets or milk-protein diets. They also compared the effects of a soy-protein diet and a milk-protein diet on healthy male and female mice without HCM.

They found that the hearts of the male mice with HCM that were on the soy-protein diet exhibited signs of heart failure such as depressed contractile function, thinning walls, and dilated left ventricular chambers. In contrast, when put on a milk-protein diet, the heart function of HCM male mice improved, and disease progression stopped. The female HCM mice were unaffected by diet.

The biological consequences of a soy-diet have been attributed to the presence of phytoestrogens (plant-derived compounds that resemble estrogen). Dr. Leinwand said that the difference in responses between the female mice and the male mice might be related to the fact that the female mice already have high levels of estrogen, so their bodies are less sensitive than males to the effect of the phytoestrogens and to changes in estrogen level from their soy diet.

The researchers are still uncertain as to why soy might adversely affect the HCM mice, and the results warrant further study. “We are currently extending these studies to include the effects of a soy diet on other disease models and are conducting more extensive studies on normal healthy mice,” Dr. Leinwand added.

She also stated that it is premature to extend the findings to human beings. Firstly, “...these mice eat a lot of soy. It is their only source of protein,” says Leinwand. Such an extreme situation does not exist in the more varied diet of humans. Secondly, “...these are mice, not humans,” she adds. Therefore, Dr. Leinwand would rather not make any recommendations about what physicians should say to patients regarding a diet rich in soy-based foods. “It would be a huge extrapolation to say people with hypertrophic cardiomyopathy shouldn’t eat soy,” says Leinwand.

Dr. Steve Colan, a pediatric cardiologist at Boston Children’s Hospital, agrees with Dr. Leinwand’s reticence. “In general, the applicability of animal data to humans is challenging. Rodents have a different molecular pattern of expression in their myocardium than other mammals, including humans. Also, given the differences in disease behavior between species, it is really hard to know how applicable findings like this are in humans.”

The study results in a sex-specific disease with male mice developing dilated cardiomyopathy and female mice developing hypertrophic cardiomyopathy. This is an unusual pattern not found in humans. In addition, the progression to dilated cardiomyopathy in humans who carry this gene defect is rare, whereas it is a universal occurrence in male mice.

Right now, Dr. Leinwand and others in the scientific community prefer to focus on the fact that diet can have an influence on heart disease, a finding that they did not anticipate, even on such a narrow population. For years, soy-rich diets have been thought to protect against heart disease but most of the studies on soy on heart disease, a finding that they did not anticipate, even on such a narrow population. For years, soy-rich diets have been thought to protect against heart disease, a finding that they did not anticipate, even on such a narrow population. For years, soy-rich diets have been thought to protect against heart disease but most of the studies on soy have been inconclusive and often contradictory. Dr. Craig T. Basson, Director of Cardiovascular Research at Weill Medical College of Cornell University, is optimistic about the recent findings, “We always speculate about how environmental factors influence cardiovascular disease, and this is the first time we’ve had clear data about the environment...It opens doors to some very exciting clinical interventions down the line.” What this means for another highly specific population – humans living with HCM – remains to be seen.
Implantable Cardioverter-Defibrillators in Children with CARDIOMYOPATHY

Implantable cardioverter defibrillators are an important component of the treatment options available to adult and pediatric cardiomyopathy patients. The selection criteria, indications for implantation, techniques for pediatric ICD implants, and impact of having an ICD are reviewed in this brief article.

Indications for ICD in Childhood Cardiomyopathy

The use of an implantable cardioverter defibrillator (ICD) in patients with hypertrophic or dilated cardiomyopathy is well established for high-risk adult patients. In adult patients, ICDs are being used both as secondary prevention (after someone has already had a cardiac arrest or resuscitated sudden death episode) and as primary prevention (implantation prior to a serious arrhythmic event). Risk-stratification is used to help decide who should receive an ICD, taking into account family history or genetic testing, thickness of the ventricular wall, history of fainting and other symptoms, presence of ventricular arrhythmias, and response to exercise testing.

The indications and criteria for selection of pediatric cardiomyopathy patients are less well defined. Pediatric cardiologists and electrophysiologists usually recommend ICD implantation following a cardiac arrest (i.e. secondary prevention), but there is less of a general consensus for primary prevention, especially in smaller children. One problematic issue is that the failure rate of ICD leads is significantly higher in children than in adults, related, at least in part, to continued growth and more vigorous activity. In addition, the life expectancy of a child with cardiomyopathy is decades longer than adults receiving an ICD. Therefore, multiple procedures will be required for battery and lead replacements, carrying additional risks and costs.

The risk: benefit ratio of receiving an ICD may differ in a young child with cardiomyopathy compared with an older adolescent. Prevention of sudden cardiac death is one of the main goals in the care of children with cardiomyopathy. However, it is important to recognize that all patients with cardiomyopathy will not develop a fatal rhythm, and not all children with cardiomyopathy require an ICD. Further refinement of risk-stratification to determine who is the optimal candidate for an ICD, and deciding when is the best timing for implantation, is still necessary.

New Techniques for ICDs in Infants and Small Children

The downsizing of the ICD over the past 15 years along with novel implant approaches now allow it to be used in just about any size child. Originally, the generators were too large to be implanted in a child and required major surgery for implanting in adults. Nowadays, most ICDs are implanted in the cardiac catheterization lab, similar to pacemaker procedures, with the device placed under the skin on the chest wall. The leads are placed either by threading through veins into the heart or surgically placing them directly onto the heart surface. Miniaturization of the ICD generator (can) and reduction in
the diameter of the leads has made it feasible to implant these devices in adolescent patients and school-age children. However, in infants and the smallest of children, the ICD generator is still too large to be accommodated in the pectoral region, and the device is placed in the abdomen.

Recently, a minimally-invasive technique was developed at the Children’s Hospital Boston to place an ICD lead just under the skin of the chest wall, avoiding the need for leads in small veins or open-chest surgery. This new technique is now being used at several pediatric specialty institutions to allow ICD placement in infants and small children with structural heart disease.

**Impact of Having an ICD as a Child**

While an ICD may be life-saving, there are some psychosocial concerns related to having a device. Adult studies have shown a higher degree of anxiety and depression among ICD patients, somewhat related to the underlying cardiac disease. We recently administered a psychological survey to our adolescent ICD patients to assess the quality of life and psychosocial impact of having a defibrillator. Whereas parents of teenage ICD patients did not report any adverse effects on family dynamics, some adolescent ICD patients reported mild anxiety and depression, which impacted family functioning and quality of life. Children were equally concerned between how it will feel when the ICD fires, and what happens if the ICD doesn’t work.

Other important issues in young ICD recipients are restrictions on sports and driving. Having an ICD should not impose additional restrictions above and beyond the recommended athletic restrictions for the underlying disease. In other words, patients with cardiomyopathy that is significant enough to warrant an ICD would have the same restrictions whether or not they have an ICD. It is prudent to advise restriction from high-level and/or hard-contact sports to minimize the risk of damage to an ICD. Participants in contact sports should wear adequate protection to safeguard the device from impact damage. The rules and restrictions regarding driving with an ICD vary by state, and are often extrapolated from other diseases such as epilepsy. Many states either recommend or require abstention from driving for an arbitrary period of time (usually around 6 months) after a syncopal (fainting) event, ICD implantation, or receipt of an appropriate shock.

**Safety and Efficacy of ICDs in Children**

The ICD is a safe and effective therapy for prevention of sudden arrhythmic death in cardiomyopathy patients. Despite the inordinate media attention given to ICD recalls over the past year; they are very reliable – with failure rates of less than 1 in 1000 generators (even those devices on manufacturers recall). Of the nearly 200 children with ICDs that are followed at the Children’s Hospital Boston, we have seen appropriate shocks in 30% of cases during the first 2 years after implant, and only 2 ICD patients died from ventricular arrhythmia. A multi-center study looking at children with hypertrophic cardiomyopathy who received an ICD revealed that 70% of children implanted for secondary prevention received an appropriate ICD shock, and 40% of primary prevention patients also received an appropriate ICD shock for ventricular tachyarrhythmia. This large pediatric series demonstrates the value of the ICD as part of the medical management of selected pediatric hypertrophic cardiomyopathy patients. However, individuals should seek their own physician’s advice and expertise regarding the indications for an ICD and various treatment options for their child’s medical care. Additional information about arrhythmia and treatment programs at Children’s Hospital Boston can be found at www.childrenshospital.org/az/Site473/mainpage5473P0.html

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In 2005, The Genetic Information Nondiscrimination Act, which recognizes that “all individuals, whether they are healthy or sick, and all medical information, genetic or otherwise, should be afforded the same protections under the law,” was introduced to Congress in order to prohibit genetic discrimination by employers and health insurers. Although it was approved unanimously in the Senate and President Bush supports the bill, it remains in deliberation within the House of Representatives.

At least 20 bills have been introduced in Congress to prohibit genetic discrimination in the past 10 years. However, only one has passed, offering limited protection against genetic discrimination for the group health insurance market. Many groups that represent employers have expressed concerns that this bill addresses a type of discrimination that does not exist and will leave companies open to new kinds of lawsuits.

The random nature of these laws brings to the fore the need for an all-encompassing piece of legislation ensuring all people, regardless of the company they work for or the state in which they live in, the same rights against abuse of their personal genetic information.

In a recent turn of events, one such company, IBM, pledged to not use genetic information in its hiring practices or in deciding eligibility for health insurance for its 300,000 employees. The only other employer known to offer such protection is the U.S. government. In 2000, President Clinton signed an executive order protecting 2.7 million federal employees from such misuse of personal information.

Currently, individual states have genetic non-discrimination laws in place, with each state guaranteeing varying degrees of protection. The random nature of these laws brings to the fore the need for an all-encompassing piece of legislation ensuring all people, regardless of the company they work for or the state in which they live in, the same rights against abuse of their personal genetic information. This includes protecting against employers deciding not to hire someone based on their genetic predisposition to a disease and insurers deciding to raise premiums or decline coverage for individuals at a higher risk for a disease.

The recent case involving former Chicago Bulls basketball player Eddy Curry and the team’s request for DNA testing to diagnose a heart arrhythmia highlights an employer’s interest in genetic information and the need to protect from possible job related discrimination.

As more genetic tests are developed to enable early detection, protections against the potential misuse of genetic information becomes ever more important. Once such protection is in place, people can safely participate in genetic research studies and diagnostic testing without fear of professional repercussion.

The National Heart, Lung, and Blood Institute (NHLBI) hosted its Seventh Annual Public Interest Organization Meeting in Bethesda, Maryland on January 30-31. The meeting was organized to encourage collaboration and sharing of best practices among public interest groups. Sixty-four public interest organizations (PIO) attended, and invited groups had open access to NHLBI directors. This was the third year that CCF was invited to this important meeting, and it provided another opportunity to strengthen CCF’s relationship with the NHLBI and discuss new ways to work together.

Dr. Elizabeth Nabel, the new NHLBI Director, opened the meeting with a summary of her first year as director and presented NHLBI’s vision for the future. Dr. Carl Roth highlighted NHLBI’s success and accomplishments. This was followed by Mr. Carl Weixler of the Hemophilia Federation of America who spoke about his personal perspective on the disease and Dr. Harvey Alter of the National Institute of Health Clinical Center who presented his research findings on “Eliminating Post-Transfusion Hepatitis C Virus Infection.” Other NHLBI staff presentations included “The NIH Grants Process, Priority Setting and Strategic Planning;” “The State of Stem Cell Technology Today;” “Stem Cells, Tissue Engineering, and Bioengineering for Therapy;” and “NHLBI Cohort Studies and Genetic Research.” Overall, it was a productive meeting that increased communication between PIO’s and key members of the NHLBI staff.
A Parent’s Perspective

The Warrior Mentality

By Scott Newport
Father to Evan (age 4 - HCM)

“Mr. and Mrs. Newport, we think you should consider signing a DNR order.”

“What does DNR stand for?” I asked.

“What does DNR stand for?” I asked.

“Do Not Resuscitate.”

What the doctors were saying about my seven-month old son, Evan, sank in slowly.

Let me back up to the day that Evan was born. That was the day my wife, Penni, and I first heard the words “Noonan’s syndrome.” It was February 5, 2002 and we didn’t know for sure if Evan was going to live through his birthday.

But Evan did live.

When he reached six months of age, the doctors were still giving him a poor prognosis for a full life. Thinking that he might not make it to his first birthday, the hospital staff threw a huge six-month birthday party. In the pictures, Evan is surrounded by balloons and well-wishers, his frail body hooked up to various tubes, monitors, and machines.

A month later, the doctors told Penni and me that we weren’t just dealing with Noonan’s syndrome. Evan also had an incurable heart disease called hypertrophic cardiomyopathy. That’s when we faced the decision to sign a DNR order.

The morning after the DNR discussion, I went to Evan’s bedside and told him what the doctors had said. Then I moved some of the tubing that entangled Evan, laid my head next to his, and whispered into his ear what our family interpretation of DNR was: “Do Not Retreat.”

You see, even though Evan was a very sick little boy, he had gifts. I’d say this to everyone who entered his hospital room, and they always looked at me a little funny.

I’d explain, “Evan has the gift of teaching,” and I’d point above all the hospital monitors to our family plaque, entitled simply “The Warrior Mentality.”

THE WARRIOR MENTALITY

Warriors know how to take on affliction. Warriors may get knocked down, but they always get back up.

Warriors know the greater the battle, the greater the reward. Warriors never go into battle alone.

Finally, after 252 days in the hospital, Evan was discharged. Accompanied by a full staff of nurses and enough medical equipment to fill his whole bedroom, Evan was sent home to die in peace.

But Evan didn’t die.

That year held many hospitalizations, close calls, and complications; through it all, I kept the warrior mentality in my heart. And so did Evan.

We recently celebrated Evan’s fourth birthday. Every day, he’s living out the warrior mentality and loving every minute of it. He is a warrior, a soldier, and one tough little kid. He’s also my hero.

Forever,

Evan’s Dad

Scott Newport lives in Michigan with his three children and wife, Penni. His son, Evan, now four years old, is diagnosed with Noonan’s syndrome and hypertrophic cardiomyopathy. If Scott had a bumper sticker on his truck, it would read: “Proud Parent of a PICU Soldier.”
Heart to Heart
Volume 3, Number 1 • Spring • Summer 2006

The Newsletter of the Children’s Cardiomyopathy Foundation

P.O. Box 547, Tenafly, NJ 07670

Address Service Requested

Spring Appeal Campaign Underway
Donate to CCF’s Research Fund.

Plant a seed in our garden of hope, embrace it with sunshine and shower it with love!

We need your help to thrive! The amount of research that we are able to fund depends primarily on public contributions. Please help us to nurture what we have started and support us in our ongoing efforts to find causes and cures for pediatric cardiomyopathy. Together we can watch our “garden” grow, bringing positive change to the lives of thousands of children with cardiomyopathy.

A donation and company matching gift can be made using the remittance envelope inside.

We Want to Hear from You!
Please help us to make Heart to Heart a better newsletter. Drop us a line to let us know what topics you would like covered in future issues. If you are a parent/relative of a child with cardiomyopathy or a physician/nurse that focuses on cardiomyopathy, we encourage you to submit an article as well. Articles can be up to two pages single spaced in length and sent as a MS Word file to newsletter@childrenscardiomyopathy.org. Photographs and artwork can be submitted as standard image files (.tif, .gif, .jpeg). The submission deadline for our Fall/Winter issue is September 1, 2006.

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

www.childrenscardiomyopathy.org

Tel: 866.808.CURE
Fax: 201.227.7016

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