



## Review

## From partnership to progress in the field of pediatric cardiomyopathy



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A decade ago, cardiomyopathy was a rare heart disease that was poorly understood and insufficiently studied in children. Medical progress has been relatively slow, and survival rates for children with cardiomyopathy continue to lag behind advances in congenital heart disease. Even so, interest in pediatric cardiomyopathy has steadily increased among clinicians and researchers over the years. The Third International Conference on Cardiomyopathy in Children, held May 2014 and hosted by the Children's Cardiomyopathy Foundation, exemplifies how far the disease has come in terms of research and education. The Children's Cardiomyopathy Foundation (CCF) is a national non-profit foundation established in 2002 to address the lack of medical progress and non-existent family support for children with cardiomyopathy. Through CCF's partnerships with research groups, federal agencies, industry, and other organizations focused on cardiac disease, the foundation has been able to capitalize on several unique opportunities to move the field forward.

Before CCF was founded, pediatric cardiomyopathy was disadvantaged in several areas: low public awareness, challenging research obstacles, and variable standards of care. In 13 years, CCF has raised more than \$9 million to address unmet needs in research, education, patient support, awareness, and advocacy for pediatric cardiomyopathy. This has led to integrative programs that (1) stimulate and accelerate research to advance understanding of pediatric cardiomyopathy, (2) educate and support physicians and caregivers to improve patient outcomes, and (3) increase public awareness and legislative action to identify more at-risk individuals with cardiomyopathy.

As the founder and executive director of CCF and a parent personally affected by the disease, it has been a privilege to work with various groups to elevate a neglected heart disease and expand its presence in the field of pediatric cardiology. This article will give an overview of how pediatric cardiomyopathy has progressed in the past decade and

how CCF's involvement has advanced the field, leading to improvements in medical care, quality of life, and disease representation in the public health and legislative arena.

## 1. Research Challenges

Cardiomyopathy in children is a serious disease that can lead to heart rhythm disorders, disability, heart transplantation, and death. Cardiomyopathies result in some of the worst pediatric cardiology outcomes and are responsible for nearly one-half of all pediatric heart transplants [1]. Nearly 40% of children die or require a heart transplant within 2 years of diagnosis [2]. I experienced these statistics firsthand when my first son died of a sudden cardiac arrest in 1999 from hypertrophic cardiomyopathy that was unfortunately undetected. Two years later, I lost another son to the disease as he waited for a heart transplant. At the time, pediatric cardiology textbooks stated that hypertrophic cardiomyopathy is rarely seen during infancy and childhood, and clinical manifestations usually do not develop before adolescence [3,4]. However, this was not the case with my family and countless other families who I would encounter over the years.

Misinformation about the disease in the 1990s mainly stemmed from the absence of population-based studies on pediatric cardiomyopathy. It was not until the year 2000 that Steven E. Lipshultz, M.D., would highlight in *Progress in Pediatric Cardiology* the challenges associated with research and medical management of children with cardiomyopathy. In the article, "Ventricular Dysfunction Clinical Research in Infants, Children and Adolescents," Lipshultz outlined key issues with the disease, including reliance on data from adult studies, lack of evidence-based medicine, over-reliance on anecdotal experience, lack of longitudinal and natural history studies, and scarce patient data [5].

Before CCF's establishment in 2002, there were very few multi-center studies on pediatric cardiomyopathy listed on PubMed. Studies were generally observational or retrospective, single-center, and based on limited or biased patient data. Additionally, most genetic studies were performed on adults with a strong family history of the disease. Many pediatric specialists state that children should not be considered "small adults," and findings about cardiomyopathy in adults cannot routinely be applied to infants and young children because of differences in the disease process [5]. Earlier research on cardiomyopathy in children did not adequately explain the large number of idiopathic cases in children.

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Although pediatric cardiomyopathy is a serious and complex disease in children, it has not attracted funding from the pharmaceutical industry and government agencies, which has had a direct impact on the state of research. Pediatric cardiomyopathy is a woefully underfunded disease when compared to other major illnesses. According to the National Institutes of Health Reporter, only \$28 million was awarded to studies on “pediatric cardiomyopathy” between 1991 and 2001. Funding has increased since CCF’s involvement, with almost \$126 million awarded from 2002 to 2014. However, this amount of research spending is still low when compared to other diseases, such as cystic fibrosis (\$231 million), pediatric AIDS (more than \$4 billion), and childhood leukemia (more than \$1 billion). As a frame of reference, cystic fibrosis affects 30,000 adults and children in the U.S., which is similar to the estimated number of children affected by primary and secondary forms of cardiomyopathy [6].

Lack of research funding has been an obstacle, but the inherent variability of the disease also has contributed to slower progress in the field. Cardiomyopathy is not one disease but a set of heart muscle-related diseases with different phenotypes (dilated, hypertrophic, restrictive, left ventricular non-compaction, arrhythmogenic right ventricular dysplasia), multiple causes, and individualized treatment plans. In many cases, it is a primary disease with genetic origins affecting only the heart; other times, it is a secondary disease caused by another systemic disorder. Cardiomyopathy can also be acquired as a result of a viral infection or chemotherapy. Among the primary forms of cardiomyopathy, the disease is genetically heterogeneous. Members from the same family can be affected quite differently ranging from no symptoms to severe symptoms of heart failure. These factors complicate the process of developing a research plan, standardizing treatment and finding a cure because there is no single mutation to identify and no single therapy that can treat all forms of cardiomyopathy.

## 2. Addressing Research Needs Through Partnerships

Low awareness and few medical advances characterized the state of the disease in the late 1990s and early 2000s. This created an opportunity for a disease-specific organization to emerge and focus on multiple forms of the disease in infants, children, adolescents and their families. The stage was set for a national organization that could address the numerous research challenges and advocate for change. Medical literature highlighted many obstacles in research: the underfunding of pediatric research, the lack of trained pediatric clinical investigators, difficulties in translating research into clinical practice, small and underpowered studies, paucity of data, heterogeneity of studies, and lack of awareness of existing research efforts [7]. It became apparent that funding and dedicated effort from a patient-centered group would be required before a potentially fatal disease such as pediatric cardiomyopathy could be prevented and cured.

The Children’s Cardiomyopathy Foundation (CCF) embraced this challenge when it was formed in 2002 with five board members and five medical advisors. As the first and only organization devoted specifically to cardiomyopathies affecting children, CCF is actively involved in all aspects of the disease from research and education to patient support, awareness, and advocacy. The Foundation works with basic scientists, cardiologists, epidemiologists, geneticists, cardiac surgeons, and pediatric professionals to improve outcomes, prevent premature deaths, and maintain a good quality of life for affected children. To stimulate and accelerate research on pediatric cardiomyopathy, the foundation partnered with established research groups like the North American Pediatric Cardiomyopathy Registry (PCMR) and Pediatric Heart Transplant Group (PHTS) to increase the number of multi-center studies. The PCMR is a National Heart, Lung, and Blood Institute (NHLBI)-funded patient registry established in 1995 to provide a population base to describe the epidemiologic features and clinical course of selected cardiomyopathies in patients age 18 years or younger.

The Foundation’s outreach to the PCMR in 2002 was well timed. The PCMR was in need of additional funding to analyze clinical, genetic, and

functional status data from 3,500 children with cardiomyopathy collected from 98 medical centers across the United States and Canada. With CCF’s support, the PCMR was able to evaluate regional, ethnic, gender, and age differences in cardiomyopathy and investigate whether certain baseline factors at diagnosis or trends over time can predict congestive heart failure, mortality, need for heart transplantation, and success post-transplant. Study findings from PCMR have been presented at more than 10 national scientific conferences, and 18 manuscripts have been published in top peer-reviewed medical journals. The Foundation’s collaboration with the PCMR has steadily grown over the years to include sponsoring working group sessions; funding data analysis, manuscript development, and journal submissions; assisting with patient recruitment costs; and covering the remuneration of a dedicated research associate.

The partnership between CCF and PCMR has been pivotal in providing physicians with evidence-based treatment guidelines to improve patient outcomes. Determining the optimal clinical management for children with cardiomyopathy has been and continues to be a challenge because of its rarity and variability. The population-based risk factors published by the PCMR have been critical in aiding physicians in patient treatment decisions.

The success of the PCMR partnership encouraged CCF to collaborate with the Pediatric Heart Transplant Study Group (PHTS) in 2006 on a study titled, “Outcomes of Pediatric Patients with Cardiomyopathy: A Multi-Center Review of Pediatric Patients Listed for Transplant in the Pediatric Heart Transplant Study.” This study attempted to predict which children with cardiomyopathy were more likely to need a heart transplant, the optimal time of transplant listing, and what factors might make them more or less likely to survive a heart transplant. This study, which analyzed clinical data from more than 1100 children with cardiomyopathy listed for a heart transplant in North America, was the first multi-institutional review of post-transplant outcomes that considered factors at the time of diagnosis.

The Foundation’s collaboration with the PCMR and PHTS tackled some of the most pressing research obstacles—dispersed and limited patient data. The resulting publications, funded by CCF and based on a well-documented sample of children with cardiomyopathy, was significant because physicians could utilize published guidelines to assist them in evaluating and treating children with different forms of cardiomyopathy.

## 3. Expanding the Field of Research

To further advance the science behind the disease, CCF’s medical advisors and board of directors felt it was important to develop research programs that would attract scientists and physicians from related medical disciplines, to encourage them to develop new innovative studies, and to support new researchers with an interest in pediatric cardiomyopathy. In 2003, the Foundation formalized its research grant program to offer funding to investigators conducting basic science, clinical, population-based, and translational research on cardiomyopathies affecting children up to 18 years old.

The primary focus of CCF’s research grant program has been to expand basic knowledge of the disease and translate findings to cause-specific treatments for children with cardiomyopathy. The program provides investigators with seed funding to test their initial hypotheses and to collect preliminary data with the goal of securing long-term funding from the National Institutes of Health (NIH) or other major grant-making organizations.

Over the years, CCF’s research grant program has steadily grown, and grant submissions have increased from six proposals in 2004 to 39 letters of intent in 2015. To date, CCF has awarded 36 research grants to various medical centers across the United States and Canada, resulting in more than 72 manuscripts and presentations at national scientific conferences. Additionally, many CCF-funded studies have gone on to receive multi-year funding from the NIH.

In an attempt to elevate CCF's profile and credibility in the cardiac research community, the Foundation partnered with the American Heart Association in 2007 to offer a joint research grant program on pediatric cardiomyopathy. The joint award was developed to draw attention to the study of pediatric cardiomyopathy and encourage researchers to develop an interest in the disease. The program was successful in attracting new investigators who did not know about CCF's research grant program.

CCF also foresaw the need to train the next generation of researchers, especially as the field continues to expand but research funding for young investigators declines. This year, CCF established a Pediatric Cardiomyopathy Research Scholar position with the Kyle John Rymiszewski Foundation to provide research training to a promising pediatric cardiology fellow.

The synergy of CCF's research grant program, the American Heart Association joint research award, and the Pediatric Cardiomyopathy Research Scholar position has had a positive effect on the level and quality of research on pediatric cardiomyopathy. In comparing 12 years of U.S. and Canadian publications and abstracts listed on PubMed, there has been a three-fold increase on pediatric cardiomyopathy publications. During CCF's existence, there was an increase in the number of articles from 255 citations (years 1989–2001) to 680 citations (years 2002 to 2014).

Looking forward, CCF plans to continue funding basic, translational, and clinical research to accelerate the search for cures. This includes more emphasis on translating basic science into therapeutic applications and initiating drug discovery and biomarker programs in partnership with academic institutions, pharmaceutical and biotech companies, and government agencies.

#### 4. Removing Research Barriers with A Repository

In 2006, CCF took the lead in setting up the Pediatric Cardiomyopathy Specimen Repository (PCSR) in response to insufficient tissue and blood samples for larger scale genetic studies. Genetic research is critical for medical progress because the cause of cardiomyopathy is unknown in up to 75% of diagnosed children [8,9]. The biospecimen repository was set up with the goal of encouraging and stimulating basic, clinical, and translational research; understanding the molecular mechanisms of the disease; and promoting new therapeutic strategies. The foundation worked closely with the PCMR network to collect biologic specimens from children with cardiomyopathy from 11 cardiomyopathy specialty centers. By 2010, more than 1,000 well-characterized and categorized specimens were collected and stored in a central repository maintained under the joint stewardship of the PCMR and CCF. Corresponding clinical data from the same PCMR patient base were matched with the collected specimens.

For a rare disease such as pediatric cardiomyopathy, the collection of genetic samples paired with clinical information was a valuable scientific resource to be utilized. The establishment of the repository would be essential for genetic and viral genome screening to identify new genetic causes specific to children. Correlating screening results with clinical data from the PCMR would allow for a wide range of studies to predict a child's clinical course and outcomes.

The repository eventually garnered the support of the National Heart, Lung, and Blood Institute (NHLBI), and in 2012, the NHLBI awarded multi-year funding for a national study titled, "Genotype-Phenotype Associations in Pediatric Cardiomyopathy." This study, which is ongoing, will identify novel genes that cause cardiomyopathy in children and influence the progression of the disease. The findings will have an impact on disease prevention, surveillance, early management, and prognosis. The foundation is helping to accelerate patient enrollment by covering each center's patient recruitment fees. Over the course of 2 years, 900 patients, parents, and affected relatives have been enrolled in this national study.

The PCMR and the PCSR initiatives both demonstrate how three separate entities – a patient advocacy group (CCF), academic institutions (PCMR investigators) and a federal agency (NHLBI) – can successfully work together to advance research on a rare disease. Based on the established groundwork, CCF will continue to take a leadership role in working with the PCMR and PCSR to develop new studies utilizing existing resources.

#### 5. Providing A Platform for Sharing

With an increase in research, a venue was needed to bring together the leading scientists and clinicians in the field and provide them with an opportunity to exchange knowledge and explore new approaches to disease prevention, diagnosis, and treatment. In January 2007, CCF organized the first international conference on pediatric cardiomyopathy in partnership with the PCMR administrative coordinating center. The scientific conference, Idiopathic and Primary Cardiomyopathy in Children: Research Directions and Strategies Conference, was a natural extension of CCF's research and education initiatives. Prior to the first conference, the topic of pediatric cardiomyopathy had not been comprehensively covered at national conferences, such as the American Heart Association and the American College of Cardiology Scientific Sessions.

The highly-focused meeting assembled a multidisciplinary group of scientists, epidemiologists, and clinicians, to review the latest developments in prevention, etiology, diagnosis, mechanisms, and treatment and to identify the most critical areas for research. Two subsequent conferences on pediatric cardiomyopathy were held May 2010 and May 2014. All three conferences were co-sponsored by the National Heart, Lung, and Blood Institute with NHLBI representatives in attendance.

These international scientific gatherings, hosted by CCF, provided a platform for sharing current information about the disease across disciplines, facilitated the identification of research needs and data gaps, and initiated the evaluation of new treatment approaches and technologies. By involving medical and research professionals from outside the cardiology field, it has encouraged different specialists to collaborate on the evaluation and management of patients. As more cardiomyopathies in children are determined to have a genetic basis, it has become increasingly important for cardiologists and geneticists to work together to provide a comprehensive evaluation of the child and family [10]. It is expected that genetic testing will take on a greater role in the initial evaluation and ongoing care of the child and family [10].

Each conference has included a session where pre- or post-doctoral fellows were invited to give presentations on their current research. This has given young researchers the opportunity to interact with international leaders in pediatric cardiomyopathy and receive constructive feedback on their work. The experience has proven to be beneficial for young researchers interested in pediatric cardiomyopathy. From the five fellows who presented at the Second International Conference on Cardiomyopathy in Children in 2010, 36 papers were published in peer-reviewed journals over 3 years.

From 2007 to 2014, conference attendance increased from 40 to 65 participants from the U.S., Canada, Europe, and Australia. The attendance increase highlights the growing interest in cardiomyopathy and the commitment of the academic research and clinical care communities to better understand cardiomyopathy's effect on children. The most recent conference in 2014 received extremely positive feedback with 96% of attendees stating they were "very satisfied" with the conference topics, opportunities for networking, and usefulness of the material presented. Many attendees felt that the meeting provided relevant medical information to aid them in managing their patients. Research priorities also were identified at the conference to help shape the direction of future research studies. Following the meeting, additional working group sessions were planned to finalize a pediatric cardiomyopathy research agenda and develop action plans for collaborative studies.

The dissemination of conference proceedings to the broader medical community has been an important feature of CCF's scientific conferences. Conference proceedings were published in nine issues of *Progress in Pediatric Cardiology* in 2007, 2008, 2011, 2014, and 2015. As of December 2014, more than 74 articles on pediatric cardiomyopathy have been published, and 20 additional articles will be published before the end of 2015. Due to the general lack of information on the disease, articles from the first two conferences were downloaded between 3,000 and 4,000 times in their first year of online availability. According to the scientific journal, many of these articles were among the most downloaded, accessed and cited to date.

In addition to CCF-hosted scientific conferences, the Foundation has co-sponsored and presented at other national medical meetings, including the Pediatric Heart Failure Group Meeting, Understanding Genetic Cardiomyopathy Conference, Youth Sports Safety Alliance Meeting, and the American Heart Association's Conference on the Scientific Basis of Heart Failure in the Young. The Foundation's presence at a wide range of medical conferences targeted to geneticists, genetic counselors, pediatric nurses, social workers, child life specialists, and pediatric cardiologists has ensured continued focus on the disease from all medical disciplines and subspecialties.

## 6. Educating Families To Advocate For Better Treatment

While the focus has been on advancing research and educating physicians, CCF has also placed equal emphasis on supporting and educating affected individuals and their families. Research has shown that educating patients can enhance their participation in healthcare decision making, improve their ability to cope with illness, and positively affect their outcome [11]. The Foundation has always encouraged parents to be well informed about the medical and psychosocial aspects of the disease so that they can proactively manage their child's health and well-being.

For many families, CCF serves as a hub for information about pediatric cardiomyopathy, offering medical details and daily tips on living with a chronic disease. Since its establishment 13 years ago, the Foundation has responded to more than 5,000 calls, letters, and e-mails on the disease. Four key messages have emerged that are specific to a child with cardiomyopathy: (1) get evaluated and treated at a pediatric cardiomyopathy specialty center with a multidisciplinary, comprehensive approach to care; (2) consider screening and genetic testing of other family members; (3) be knowledgeable about the disease; and (4) find a reliable support network. These messages have been communicated through CCF's website, quarterly newsletters, patient education materials, webinars, and cyberguest Q&A sessions.

In the 1990s before CCF's existence, a parent's need for basic information about the disease and the practical guidelines for coping with a chronic disease were not always met [12]. Most web-based articles only addressed the disease from an adult perspective, and articles generally did not acknowledge the psychosocial aspects of the disease or cover the transition from pediatric to adult care. The Foundation filled this void by developing educational materials that considered the concerns of parents trying to raise a child with cardiomyopathy and the challenges they might encounter as their child got older. To develop a variety of educational materials to meet different patient needs, CCF worked with several national organizations, such as the National Organization for Rare Diseases, the American Heart Association, the Pediatric Heart Transplant Foundation, the National Society of Genetic Counselors, and the Centers for Disease Control and Prevention.

Materials created included a 14-page overview booklet, *Understanding Pediatric Cardiomyopathy*, in English and Spanish with accompanying inserts on dilated, hypertrophic, restrictive, and left ventricular non-compaction cardiomyopathy; a 29-page children's booklet *Cardiowhat*; a 30-minute DVD, *Secrets of the Heart: Living with Pediatric Cardiomyopathy*; a school resource kit, *Ensuring a Good Learning Environment*; and a variety of fact sheets covering a wide range of topics such as transition to care,

genetic testing, nutrition, cardiac screening, risk factors for sudden cardiac arrest, and travel and exercise guidelines. Online resources that were developed include, *Pediatric Heart Transplants: A Guide for Patients and Families*, *Introduction to Pediatric Cardiomyopathies* and *A Pediatric Cardiomyopathy Disease Report*. To date, more than 32,000 pieces of literature on pediatric cardiomyopathy have been distributed to major medical centers, school nurses, and relevant medical meetings in the U.S. and Canada.

To complement CCF's educational resources, CCF offers a full range of family support. Children diagnosed with cardiomyopathy are now living longer, and many survive into adulthood, at which point they may require more varied resources and support services [12]. The Foundation currently offers local support groups, a family matching service, an ambassador program, a private discussion group, and a Facebook group page. In addition, more than 2,500 CCF members in 70 countries have access to CCF's webinar and cyber guest Q&A sessions to connect with experts in the field. In the past year, CCF added a teen Facebook group, a youth ambassador program, and a buddy-matching program to meet the needs of a growing teen and young adults segment. These support services were not available for families 13 years ago. Now family members no longer feel isolated; they have a virtual venue to share information and provide emotional support to each other.

The Foundation has also developed relationships with other organizations that address cardiomyopathy-specific areas and regularly refers families to their services. These resources have included charity travel providers, automated external defibrillator (AED) providers, CPR and AED training programs, and international support groups. For children with cardiomyopathy that is determined to be secondary to another systemic disease, CCF will refer the family to the appropriate disease organization, such as the Noonan Syndrome Foundation, the Barth Syndrome Foundation, the Fatty Acid Oxidation Disorder Family Support Group, the Parent Project Muscular Dystrophy, or the National Ataxia Foundation.

By serving as an information clearinghouse and support resource center, CCF has addressed a critical need that was not being met by the medical community. Studies have shown that patients remember only about 50% of what they are told during a medical visit [13]. Many families involved with CCF have expressed feeling lost, anxious, and overwhelmed after their child's initial diagnosis. Through the Foundation's hospital network, physicians have been distributing CCF's materials as a take-home resource to reinforce information parents may have received during their child's doctor visit. This enables parents to become more knowledgeable about the disease and have more productive conversations with their child's physician.

## 7. Raising Awareness of The Disease

Early on, CCF realized that medical progress would depend on increased interest from the medical community and general public. Not only did new research opportunities need to be created, but pediatric cardiomyopathy needed to be more widely recognized as a neglected children's heart disease. The Foundation followed the model of other rare disease organizations, such as spinal muscular atrophy, Duchenne muscular dystrophy, Progeria, Barth syndrome, and Tourette syndrome. These organizations have used their strong leadership to draw attention to their disease through medical and industry collaborations and active involvement from their family members.

Cardiomyopathy is not always a visible disease in children, which has made generating awareness of the disease challenging. It is difficult to convey the seriousness of the disease when affected children do not look sick and symptoms are absent or mild. In reality, when cardiomyopathy is undiagnosed, the consequences can be devastating for a family. According to the Centers for Disease Control and Prevention, more than 2,000 people younger than 25 die of sudden cardiac arrest every year in the U.S., and many times the underlying cause is a heart condition like cardiomyopathy [14].



Over the years, CCF has focused on increasing public awareness of the disease and on identifying children at risk of cardiomyopathy and sudden cardiac arrest. Public awareness initiatives have included featuring articles on cardiomyopathy, highlighting the experiences of CCF family members, working with national partners to elevate the profile of the disease, and developing community awareness activities for National Heart Month in February and Children's Cardiomyopathy Awareness Month in September. These activities have urged families to know their cardiac history, learn the signs and symptoms of cardiomyopathy, and understand that if detected and treated early, sudden cardiac death can be prevented.

In September 2014, the Children's Cardiomyopathy Awareness Month was launched nationally. This year, twelve national partners have joined CCF in promoting awareness month, including the American Academy of Pediatrics, the American Academy of Cardiology, the American Heart Association, the Centers for Disease Control and Prevention, the School-Based Health Alliance, and the National Association of School Nurses. Thirty days of educational activities are planned around primary prevention, including circulating information on the warning signs and risk factors for cardiomyopathy, sharing stories of families affected by the disease, organizing community awareness events, and issuing a call to action to support cardiomyopathy-related legislation.

In addition to this initiative, CCF has partnered with the American Camp Association and congressional members to organize an Automatic External Defibrillator (AED) scavenger hunt to highlight that AED usage during a cardiac emergency is paramount to survival. This secondary prevention initiative was well received and helped to elevate the profile of pediatric cardiomyopathy during the summer months before the Awareness Month in September.

## 8. Advocating For Change

Before 2011, pediatric cardiomyopathy was not represented in Washington D.C., and there were few legislative champions for our cause. The Foundation began advocating for the needs of affected families by working with congressional leaders to enact legislation to identify and protect undiagnosed, at-risk children from sudden cardiac arrest. In the past four years, CCF has supported federal legislation focused on secondary prevention related to AED accessibility and helped to introduce two cardiomyopathy bills in the U.S. House of Representatives and Senate on primary prevention related to developing educational materials and risk assessment forms on cardiomyopathy.

In 2012, CCF partnered with Senator Robert Menendez (NJ) and Representative Frank Pallone (NJ-6) to introduce the Cardiomyopathy Health Education Awareness Risk Assessment and Training in the Schools Act (Cardiomyopathy HEARTS Act). It was the first federal bill centered on cardiomyopathy, aimed at increasing awareness of cardiomyopathy and the risk of sudden cardiac arrest among parents, school personnel, and health professionals. Through CCF's outreach efforts, the bill secured the support of 37 organizations and 29 legislators by the 113th Congress. This achievement was followed by the introduction of a broader-based bill, the Supporting Athletes, Families and Educators to Protect the Lives of Athletic Youth (SAFE PLAY) Act in collaboration with the National Athletic Trainers' Association, Senator Robert Menendez, and Representatives Bill Pascrell (NJ-9) and Lois Capps (CA-24). This bill, which currently has 38 co-sponsors, promotes the safety of young athletes and encourages the development of best practices to prevent, to document, and to address both cardiac emergencies and common sports-related injuries associated with school athletics.

From 2010 to 2013, the Foundation served as a steering committee member of the Sudden Cardiac Arrest Coalition, working in cooperation with the Congressional Heart and Stroke Caucus and 46 heart organizations to host briefings on Capitol Hill and petition the U.S. Secretary of Education and U.S. Secretary of Health and Human Services for the

placement of AEDs and the implementation of emergency response plans in all schools and places of public gathering.

For three consecutive years, CCF was successful in securing report language on cardiomyopathy in the Senate Labor, Health, and Human Services subcommittee appropriations bill that was approved by the Senate in the final omnibus appropriations bill for fiscal year 2013 and 2014. More recently, CCF was able to include language in the Every Child Achieves Act of 2015 (S. 1177) that encourages local educational agencies to use Title IV ESEA state funding toward activities and programs that address cardiac conditions such as cardiomyopathy.

These advocacy initiatives have helped to raise awareness of pediatric cardiomyopathy among key legislators and federal agencies. The foundation continues to strengthen relationships with congressional leaders and grow its presence in Washington D.C. to ensure that the legislative sector retains their interest in pediatric cardiomyopathy.

## 9. Conclusion

Cardiomyopathy in children is a serious and chronic heart disease, which had not received much public attention or medical focus before 2000. Thirteen years ago, there was no national organization to encourage collaborative research among centers or to advocate for the needs of this neglected pediatric population. Fortunately, the field of pediatric cardiomyopathy has expanded and there is now increased awareness and interest on this rare heart disease.

The Children's Cardiomyopathy Foundation (CCF) has taken a leadership role in getting pediatric cardiomyopathy recognized in the research and public health arena, and in advocating for the needs of diagnosed and at-risk children and their families. The Foundation has viewed its role as a facilitator in setting priorities, identifying resources and encouraging collaboration across various disciplines to move the field forward. The Foundation's outreach to researchers, clinicians, cardiac disease organizations, and legislators has laid the groundwork for more productive partnerships and new projects to be developed.

While there has been progress in the areas of research, education, family support, awareness, and advocacy, there is much more to be accomplished. The Foundation is committed to keeping the research community engaged, informing the NHLBI on the state of the science and challenging physicians to rethink current approaches to therapy development and patient management. Through specific education and awareness measures, CCF will continue to serve as a conduit for information from the medical and research community to patients and the general public. These actions will have a direct impact on the pace of research, medical progress, and patient outcomes. It is the Foundation's hope that progress in pediatric cardiomyopathy will continue to gain momentum into the next decade.

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