

Review

Advances in pediatric heart failure and treatments

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ABSTRACT

Heart failure remains a significant contributor to morbidity and mortality in pediatric patients with congenital and acquired heart diseases. However, there are several new promising therapies for acute and chronic heart failures including the development of new pediatric specific ventricular assist devices. There remains a need for collaborative research including the creation of a large-scale registry of pediatric heart failure, increased utilization of genetic testing, and an improved understanding of the optimal model of disease management that maximizes quality care.

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1. Introduction

The onset of heart failure leads to a significant increase in morbidity and mortality in patients with congenital and acquired heart disease [1–5]. The risk of hospital mortality in children hospitalized with heart failure is over 20-fold compared to hospitalized children without heart failure and the overall transplant free survival for patients diagnosed with dilated cardiomyopathy is approximately 50% at 5 years [4,6,7]. However, there have been significant improvements in the care of patients with heart failure with recent data demonstrating an improvement in hospital and overall survival (Fig. 1) [3,8]. While survival has improved, morbidity and costs remain high and not clearly improving. Importantly, the hospital length of stay and hospital charges for children hospitalized with heart failure are increasing and at a rate greater than seen among adults [3].

This is an exciting time for the field of pediatric heart failure. There are several promising new medications for the treatment of acute and chronic heart failure [9–11]. Additionally, mechanical circulatory support continues to advance with the increasing utilization of continuous flow devices in pediatric patients with a planned trial of the Pediatric Jarvik ventricular assist device as part of the NIH sponsored Pumps for Kids, Infants, and Neonates (PUMPKIN) trial [12–17]. However, these advances are occurring in the environment of value-based medicine where improvement in outcomes are measured in conjunction with the absolute and incremental costs incurred [18]. In order to improve outcomes and reduce costs of caring for pediatric heart failure patients, a broader understanding of the diseases leading to heart failure [19],

understanding variability in care, and developing optimal care models is essential [18].

2. Creation of an international pediatric heart failure registry

The use of registries has been well described in both pediatric and adult cardiovascular disease and has resulted in greater understanding of many cardiovascular diseases. Registries in adult heart failure such as the Acute Decompensated Heart Failure National Registry (ADHERE) have resulted in multiple publications and facilitated practice change [20]. The Pediatric Cardiomyopathy Registry (PCMR) has also been instrumental in the understanding of pediatric cardiomyopathy and heart failure and has generated much of the existing literature regarding pediatric cardiomyopathy outcomes [4,21–26]. However, the PCMR has collected data on only North American children with cardiomyopathy. Furthermore, at least half of all children with severe heart failure have an underlying congenital heart disease. In the current era of electronic medical reporting and bioinformatics, we have an opportunity to expand such data collection efforts to include children from all over the globe with heart failure. Such an effort is currently underway under the auspices of the International Society of Heart and Lung Transplantation. This is likely the only real opportunity that exists to do large scale analyses in pediatric heart failure given prospective trial design limitations secondary to cohort size.

3. Increased uptake of genetic testing and focus on phenotyping

Genetic and genomic data are increasingly being generated as part of research studies and clinical care and may be used for prevention and treatment of cardiovascular disease [27]. Large-scale genetic

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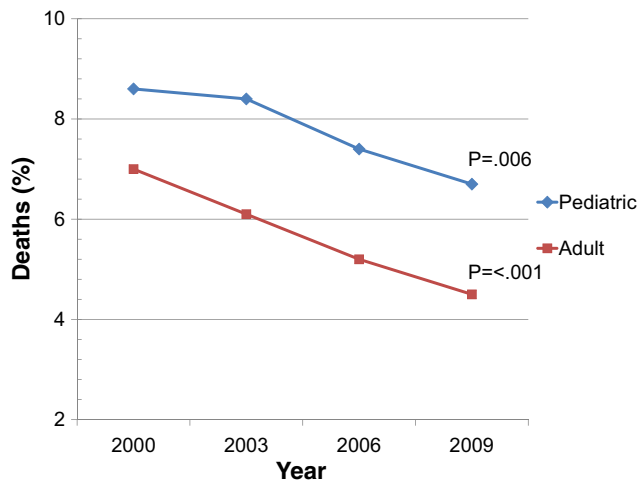


Fig. 1. Mortality of pediatric and adult cardiomyopathy and heart failure related hospitalizations over time. Reproduced with permission from Wittlieb-Weber CA, Lin KY, Zoutis TE, et al. Pediatric versus adult cardiomyopathy and heart failure related hospitalizations: A value-based analysis. *J Card Failure*. 2015;21:76–82.

investigation continues to be part of many investigations and has resulted in a broader understanding of many common diseases including ischemic heart disease, systemic hypertension, arrhythmias, valvular disease, dyslipidemia, and thoracic aortic aneurysmal disease [28–33]. Pharmacogenetic data can also be used to guide therapy as well as predict adverse drug reactions [34–36]. Genetic data can be used for disease diagnosis and assist in clinical management. The genetics of cardiomyopathy are being better delineated and the use of genetic testing has been included in guideline statements [37,38]. However, awareness of the benefits to patients and families remains a vast opportunity for intervention in our field. The use of cascade testing approaches can lead to the identification of family members at risk or with subclinical disease resulting in dedicated longitudinal evaluation and earlier intervention.

More attention to phenotypic description will also be required to better care for patients as well as to leverage the opportunity for genotype-phenotype correlations. A more robust approach to phenotyping is needed and has prompted a call to describe the phenotype as a whole in the form of phenomics [39]. Current phenotypic descriptions are often imprecise, confound our understanding of clinical disease and result in heterogeneity in research [40]. The concept of deep phenotyping has been introduced to provide a precise and comprehensive description of phenotypic abnormalities [40]. Given the complex genetic milieu seen in pediatric heart failure populations, especially in subjects with accompanying congenital heart disease, a more careful approach to phenotypic description will be necessary for clinical investigation and subsequent changes in care.

4. Broader representation of patients

The increased public awareness of life-threatening diseases and the availability and growth of social media have resulted in an ongoing globalization of healthcare over the past few decades [41]. This has resulted in greater opportunities to enhance health care as well as research investigation. This integration of ideas and opinions from around the world places the pediatric heart failure community in a position to substantially advance the existing body of literature. We have a unique opportunity to learn in a rapid manner from our colleagues and enhance our understanding of the natural history of disease and differing management strategies. Much of the existing reports regarding pediatric heart failure typically only describes those patients from developed countries. Current reports underrepresent patient diversity, approaches to management, and outcome data in

pediatric heart failure. The trends being seen in other areas of pediatric medicine may facilitate broader representation of different ethnicities and management strategies that will enrich our existing pediatric heart failure literature. Current trends suggest that the majority of studies for pediatric trials conducted pharmaceutical companies for 6 months of market exclusivity include sites outside the United States with ~11% being conducted exclusively outside the US and a third enrolling patients in developing or transitioning countries [42]. Given the dramatic growth in device and drug investigation in pediatric heart failure, a more global approach affords us an opportunity to conduct studies in a more timely and cost-effective way facilitating progress in pediatric heart failure management across the globe.

5. Developing a focus in quality improvement

Variability in any system can greatly influence the ability to deliver predictable outcomes in a reliable fashion. The need for minimizing care variation and development of a quality improvement focus in medicine is increasingly at the forefront of practitioners across the United States. Performance on quality measures has become increasingly linked to reimbursement and motivated health-care professionals to develop programs and infrastructure around quality improvement. With the implementation of the Affordable Care Act (ACA), efforts in the area of quality are likely to accelerate. Quality measures are designed to enhance the quality of care delivered to a patient and their family and result in improved outcomes such as decreased hospital length of stay, morbidity, and mortality as well as improving patient satisfaction and experience. The development of quality metrics or measurements is the foundation of improvement science. Cardiovascular medicine was one of the first to implement minimum standards into practice measuring adherence to evidence-based practice [43]. Adherence to evidence-based therapies in adult heart failure is being closely monitored in an effort to reduce hospital admissions [44]. Although the penalties for pediatric heart failure readmissions have not been apparent, it is likely that more stringent oversight from payors will be implemented across all services provided in pediatric cardiology. The pediatric heart failure community has an opportunity to be in front of these changes and develop active dialogues with payors to help define benchmarks and metrics of quality. However, to accomplish this, more robust data sets will be needed to understand practice patterns and identify variations that may allow for study and minimizing variation in diagnosis, treatment, and surveillance. Enhanced quality initiatives may provide a more homogeneous group of data that will facilitate an understanding between quality improvement and outcomes. Quality measurement has documented favorable impacts on processes of care but to have maximal impact they must also be linked to clinically relevant outcomes such as mortality or quality of life [45].

6. Development of an optimal care model

Children with heart failure have a complex chronic disease that creates challenges in their care. A multidisciplinary approach to heart failure management with input from medical, surgical, and allied health professionals is advocated by many organizations [46–48]. An example of a multidisciplinary model for the care of heart failure patients is illustrated in Fig. 2; [49] however, the optimal model that maximizes value to the patient and family is unknown and much work is needed to this end. Several studies in adult heart failure patients demonstrate lower hospitalization rates and cost of care and improvement in quality of life, and possibly mortality when heart failure is managed by a specialized clinic [50–55]. Some of these efforts have included a home health component and remote monitoring [53–55]. Such examples of a focused (or team) approach to disease management in improving outcomes also exist in pediatric chronic diseases such as cystic fibrosis, diabetes, and muscular dystrophy [56–58].

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