The dramatic advances in the operative and perioperative care of children with major forms of congenital heart disease have resulted in excellent short- and medium-term outcomes for the most severe forms of heart disease, including those with so-called single-ventricle physiology. This represents one of the great advances in pediatrics over the last 3 decades. A corollary of these observations is that some of the worst outcomes for children with heart disease are now observed in those with cardiomyopathy (whether acquired or congenital). This fact has drawn focus on heart muscle disease as an important cause of morbidity and mortality in children.

Data from the Registry of the International Society for Heart and Lung Transplantation\(^1\) emphasize that cardiomyopathies (notably the dilated form) remain the main indication for heart transplantation in childhood. This international report provides very limited information, however, on outcomes before transplantation. The Pediatric Heart Transplant Study, a multi-institutional research consortium from 3 countries, has focused (since 1993) on identifying risk factors for various outcomes from the time of listing for transplantation in children with end-stage heart disease, the majority of whom have cardiomyopathy.\(^2\)\(^-\)\(^3\) This has provided important information on pretransplantation morbidity and mortality rates in children with advanced heart disease but does not address the epidemiology or outcomes for children at first presentation with heart failure unrelated to structural heart disease.

Recently, 2 groups have provided population-based data on pediatric cardiomyopathies, allowing ascertainment of incidence and spectrum of cardiomyopathy types. The prospective arm of the Pediatric Cardiomyopathy Registry sponsored by the National Heart, Lung, and Blood Institute of the National Institutes of Health provided the first population data on the incidence and types of pediatric cardiomyopathy in the United States.\(^4\) The findings were remarkably similar to the results of a population-based study of primary pediatric cardiomyopathies from Australia.\(^5\) Both the Pediatric Cardiomyopathy Registry\(^6\)\(^,\)\(^7\) and the National Australian Childhood Cardiomyopathy Study\(^8\) have gone on to provide important outcomes data on large numbers of children with pediatric cardiomyopathy.

In the present issue, Andrews and colleagues\(^9\) report a unique population-based study from the United Kingdom and Ireland. This study, performed on behalf of the British Congenital Cardiac Association, differed from the earlier studies discussed above. The authors studied all first episodes of “heart muscle disease–induced heart failure” presenting within a 1-year period at the 17 pediatric cardiac centers. The aims of the study were to prospectively record the national incidence, origins, treatments, and outcomes for children requiring hospital admission for new-onset heart failure that is not due to structural heart disease. As with previous population-based studies performed within the United Kingdom and Ireland,\(^10\) the small number of centers, the close knit pediatric cardiology community, and the central support of the British Congenital Cardiac Association have likely led to near complete case identification.

What did the authors observe? The incidence of new-onset heart failure in children <16 years of age was ascertained and was estimated at 0.87 in 100 000. The median age was young, at approximately 1 year old. Interestingly, 82% were in New York Heart Association (or Ross) class III-IV. This finding is in contrast to what is seen in adults, where a broader distribution of symptom severity is seen at first presentation. As anticipated, the largest group comprised those with idiopathic or familial dilated cardiomyopathy (almost two thirds of cases). Presumed myocarditis was the second most common diagnosis. Of note, occult arrhythmias accounted for almost 7% of cases. This is an important observation, as this group should have an excellent prognosis if the correct diagnosis is made and arrhythmia control is achieved. Anthracycline-induced cardiomyopathy accounted for almost 5% of cases. In stark contrast to new presentation of heart failure in adults, 43 of 104 (41%) children required mechanical ventilation, including 13 who required mechanical circulatory support. Almost a third had sustained arrhythmias that required therapy. Given the critical condition of many of these children, it is perhaps not surprising that the mortality rate was significant. Although 1-year patient survival was 82%, over one third required readmission during the first year after diagnosis. Furthermore, 1-year event-free survival (freedom from death or transplantation) was very poor at 66%. This is far worse than almost all forms of structural congenital heart disease presenting with heart failure.

Although important data on incidence, origin, and outcomes of new-onset heart failure were obtained, information on risk factors for adverse outcomes is sparse. Only a small
The number of variables were available to enter into a multivariate model of risk factors for death or transplantation. The absence of central review of echocardiograms and paucity of hemodynamic data further limit this analysis. Older age and greater systolic dysfunction at presentation were associated with poorer outcome.

What are the important lessons to be learned from this carefully performed study? The incidence and disease severity data help define resource needs for this challenging group of patients. It is clear that deterioration is often rapid, and the majority of these patients required cardiac critical care services. In countries with National Health Services, this will facilitate central planning of supraregional centers of excellence that can provide optimal mechanical circulatory support and transplantation services. Comprehensive heart failure services improve survival and decrease readmission rates in adults with heart failure. Given the complexities of managing infants and small children with advanced heart failure, it seems likely that the same is true (though hard to prove) for the pediatric population. Unfortunately, the relative rarity of this condition poses many logistical challenges for the development of comprehensive heart failure services for children.

The outcomes from the study by Andrews et al provide important new information that will be of value to clinicians when counseling parents of children with new-onset heart failure. Such information has not been available in the past, as it is not possible to extrapolate outcomes from cohorts with specific diagnoses such as dilated cardiomyopathy, where asymptomatic left ventricular dysfunction is common. As the authors note, the study design does not allow any inferences about treatment effects. The high incidence of intracardiac thrombus (almost 10%) in patients with severe systolic dysfunction does suggest that anticoagulation should be provided in such patients. The type of anticoagulation required cannot be defined by this study. Despite the high rates of death and transplantation, outcomes among event-free survivors were encouraging. The median shortening fraction at 1 year was 25%, with very few patients remaining in advanced heart failure. These observations have important implications for design of future clinical trials. In the recent multicenter treatment trial of carvedilol in pediatric heart failure, the rate of improvement in both treatment and placebo arms was higher than anticipated (56% in both groups). This likely contributed to the difficulties in adequately powering the study. Outcome data from carefully studied cohorts of children with heart failure, as in the present important study, should contribute important knowledge for the design of future randomized controlled treatment trials.

Disclosures

None.

References


Key Words: Editorials • cardiomyopathy • heart failure • pediatrics • population
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