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Risk Prediction Model in Children With Hypertrophic Cardiomyopathy:

A Work in Progress

Robert O. Bonow, MD, MS, **Elizabeth M. McNally, MD, PhD**

Northwestern University Feinberg School of Medicine, Chicago, Illinois (Bonow, McNally); Editor, *JAMA Cardiology* (Bonow); Center for Genetic Medicine, Northwestern University Feinberg School of Medicine, Chicago, Illinois (McNally); Associate Editor, *JAMA Cardiology* (McNally).

Managing sudden death risk in young patients with hypertrophic cardiomyopathy (HCM) is challenging for its lifetime implications from both action and inaction, and this decision often relies on shared decision-making by families and clinicians. Thus, the development of risk assessment tools to guide parents and health care professionals are welcome additions to the scientific literature. The study by Norrish and coworkers[1] in this issue of *JAMA Cardiology* is an initial step in creating a risk tool, and as a first step, the editors hope this work will stimulate further discussion and investigation to better refine this tool.

The imperfect prediction model proposed by Norrish et al[1] is based on a retrospective sample of 1024 children younger than 16 years with HCM. The international collaboration, involving centers from Europe, Argentina, Australia, and Japan, created a broad cohort from which to draw conclusions. The pediatric HCM risk tool follows methodology similar to the existing European risk tool in adults, which is embedded in the 2014 European Society of Cardiology guidelines with recent external validation.[2] The European risk tool for adults is more specific, but less sensitive, than the US approach.[3]

The current score for children proposed by Norrish et al[1] is based on 89 events using 5 preselected risk factors: history of syncope, maximal left ventricular wall thickness, left atrial size, left ventricular outflow tract gradient, and nonsustained ventricular tachycardia. This pediatric risk predictor tool did not include age, family history, or gene mutation status in its calculation. In adults with HCM, sarcomere gene mutation status identifies patients with HCM at higher risk.[4] Symptomatic status was evaluated and did not improve the performance of the model.

There are limitations to this childhood risk prediction model worth noting. At best, it had only moderate discrimination (the C statistic is 0.69). There was no external validation. Nearly half of the children from which the model is derived (497 [48.5%]) who experienced nearly two-thirds of the end points (55 [61.8%]) did not have complete data. The median

Corresponding Author: Robert O. Bonow, MD, MS, Northwestern University Feinberg School of Medicine, 600 N St Clair St, Ste 600, Chicago, IL 60611 (robert.bonow@nm.org).

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(interquartile) follow-up period was 5.3 (2.6–8.3) years, and this relatively short follow-up interval may be insufficient for the difficult decisions surrounding whether to proceed with the consequences of lifelong implantable cardioverter-defibrillators in children.

However, as with many first-generation risk tools, a less-than-perfect risk score incites future iterations—this is exactly our hope. The importance of a sudden death risk calculator for children with HCM is clear and is of great public health benefit, but this risk score should be viewed as a work in progress. External validation is necessary, and refinement with additional variables such as gene mutation status and magnetic resonance imaging markers will likely enhance the model, as these have been shown to be powerful predictors of outcome. Incorporating these data can be expected to improve performance of the risk tool so that it can used as a primary driver for device implantation decisions in children.

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