Research Explained

Randomized controlled trials in children's heart surgery in the 21st century: a systematic review

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Medical research, as a general rule, comes in two specific types: retrospective and prospective. Retrospective research is, without a doubt, the most common. It is easier to do and offers no risk to the patient since there is no intervention, yet always entails "looking back" at a group of patients to learn about their condition. Really well-designed retrospective work can be quite powerful and teach us a lot about various cardiac conditions and treatments. Furthermore, there are loads of good retrospective studies that come out every month about congenital heart disease.

Prospective work, however, has long been thought to be the "gold standard" when it comes to research and front-and-center in prospective research is the Randomized Clinical Trial. The way these trials work is to take a group of patients and perform an intervention one way in a random half of the group and another way on the other half. Then, you study the outcomes to learn if the intervention works. The point of randomization is to make two groups that are nearly equal, differing only by the intervention that they received. While randomized trials can be incredibly powerful, they are also incredibly expensive, can last many years and can be really difficult to recruit patients to since it requires parents to allow their child to be randomized to a specific treatment group (possibly not the treatment they would otherwise want).

Nigel Drury took a look at how much prospective research was present in congenital heart disease. He looked at nearly the last 20 years of research and found 333 trials total. The vast majority of these were called "early phase trials" which are usually medication trials that simply look to see, on a small scale, if new drugs are safe. Early phase trials do not tend to look at if those drugs actually work to treat the disease. Why are so many early phase trials? They are easier to recruit patients to, don't cost as much and don't require long-term follow up. Studying those medications in late phase trials becomes challenging to accurately and cost-effectively follow a large group of patients over time – and thus many never progress past early phase

He also found only 11 trials that looked at a surgical intervention. These are really challenging and really expensive to do since many families are frequently not OK randomizing to a certain way to do a surgery. They usually want the surgeon to do it the way they are most comfortable. Still, there have been a few big trials in congenital heart disease with the most recent being for Hypoplastic Left Heart Syndrome looking at shunt type – published almost 7 years ago.

The authors also recognized that adult cardiac disease has far more trials present. There are a few reasons for this. First of all, there are many many more adults with heart disease than children making recruiting far easier. Second, congenital heart disease is incredibly varied, with no two children acting exactly the same way with their disease.

This makes creating equal groups incredibly hard when the patients differ by so much more than the intervention. Finally, government and businesses pour far more money into research on adult heart disease than they do on pediatric heart disease – a necessary resource to successfully complete these trials