Heart-transplant rules meant to save more children haven't worked as hoped, study finds

3 October 2019, by Tom Avril, The Philadelphia Inquirer

A national rule meant to increase the number of heart transplants for the sickest children has not resulted in improved survival, a new study has found.

The March 2016 change in how organs are allocated gave first priority to children born with significant heart defects, such as when most of the left side is missing. Those with a kind of pumping deficiency called cardiomyopathy, on the other hand, were in most cases given lower priority—on the theory that their cases were more manageable with medication.

But since the change took effect, physicians have been more likely to seek special exceptions for certain cardiomyopathy patients, bumping them up to top-priority status and partly undoing the intent of the policy, the authors wrote in the *American Journal of Transplantation*.

Survival rates did not improve among any categories of children waiting for heart transplants, and the rates even got a bit worse for patients with certain kinds of cardiomyopathy who were not assigned top-priority status.

The study authors did not fault the physicians who sought exceptions, as they were trying to save the lives of desperately ill children. But the policy language that allows for the exceptions may be "too loosely worded," said senior author Brian Feingold, medical director of pediatric heart failure and heart transplantation at UPMC Children's Hospital of Pittsburgh.

"There is no ill intent here," Feingold said. "Obviously we have to prioritize, and we have to do it in the best way possible to maximize survival."

It can be hard to predict which children are in greatest need of a transplant, in part because there are few of them overall, encompassing a wide range of complex conditions, he said. Each year, roughly 50 to 70 children die while waiting for a donor heart, according to the U.S. Organ Procurement & Transplantation Network.

"I would love to have a tool, a calculator that I could plug in various characteristics of a patient's case and understand with good certainty whether this was a patient who had two weeks to survive, three months to survive, or three years to survive." he said. "We don't have that."

At Children's Hospital of Philadelphia, pediatric cardiologist Matthew O'Connor said he had obtained exceptions as allowed by the policy, acquiring the highest priority on the wait list for the most gravely ill cardiomyopathy patients.

Many such patients can delay the need for a transplant by getting an implanted pump called a ventricular assist device. But some smaller children might not be good candidates for the device, leaving a transplant as the best option, said O'Connor, who was not involved with the study. He agreed with the study authors that better tools are needed to predict survival.

"I think it's still showing that we are having a difficult time determining who really the sickest patients are in our field," he said.

The head of the national committee that oversees pediatric heart transplant allocation policy, Dallas-based pediatric heart surgeon Ryan Davies, was not available for comment.

To obtain top-priority status for a heart-transplant candidate who would not otherwise qualify, a physician must demonstrate "using acceptable

medical criteria, that a heart candidate has an urgency and potential for benefit comparable to that of other candidates at the requested status," according to the policy.

Physicians were most likely to obtain exceptions for patients with a form of cardiomyopathy called "dilated," in which the ventricle is stretched thin and cannot pump as strongly. Some were younger than one year of age.

In the region of the national organ-transplant system that includes Pennsylvania and New Jersey, patients with dilated cardiomyopathy were significantly more likely to obtain top-priority exceptions after the policy was changed.

From December 2011 to March 2016, two out of 57 candidates in the region obtained that type of exception—a rate of less than 4%. From March 2016 to September 2018, nine of 32 regional candidates obtained that type of exception, a rate of 28%.