Modify Guidance for Pediatric Heart Exception Requests to Address Temporary MCS Equipment Shortage

OPTN Heart Transplantation Committee

Purpose of Approved Emergency Action

- Respond to potential pediatric patient safety risk from shortage of durable pediatric mechanical circulatory support (MCS) devices and supplies
- Support National Heart Review Board for Pediatrics (NHRB) reviewers and transplant programs by describing appropriate circumstances for submitting and approving exception requests
  - Update OPTN *Guidance for Pediatric Heart Exception Requests (Guidance)*
  - Identify circumstances pediatric dilated cardiomyopathy (DCM) candidates may be eligible for status 1A by exception when timely access to MCS devices and equipment is limited by a shortage
  - Describe clinical factors indicating when status 1A by exception is appropriate

Approved Emergency Guidance Action

- Updated *Guidance*
- Clarifications addressed access to transplant issues certain pediatric candidates may experience during MCS and supply shortage

Approved Emergency Guidance Action (Cont.)

- Added description of clinical factors indicating who should be considered for status 1A by exception

New Guidance	"In the event of a recognized national shortage of mechanical circulatory support (MCS) devices and/or a national shortage of the equipment necessary to operate such MCS devices and no acceptable alternative is available, then candidates not meeting the above size criteria, but whose clinical condition is evidenced by poor systemic perfusion while supported by high-dose inotropes as defined in Table 1, may be eligible for status 1A by exception."

Rationale

- Currently, there is reduced availability of durable pediatric ventricular assist devices (VAD), supplies, and support equipment
  - VADs are primary MCS therapy used to bridge pediatric candidates who fail inotropic support
  - Only one VAD has FDA approval for use in small children and infants
  - Device and supply shortage limits access to this therapy
  - FDA placed pediatric VADs on its *Device Shortage List* in July 2025
- Updated *Guidance* addresses patient risk by providing status 1A exceptions for children who would benefit from, but do not have access to, equipment due to shortage
  - Guidance is not policy
  - Guidance helps consistent review of exceptions by NHRB

Rationale (Cont.)

- Due to patient safety risk, Committee pursued emergency approval pathway and OPTN Board of Directors (BOD) approved on 06/09/2025
- To align with emergency pathway criteria, proposal included:
  - Sunset date of 07/11/2026 for when criteria will no longer apply
  - Timeline for retrospective public comment, which is 08/27 – 10/01/2025
- Committee will evaluate device shortage status and report to BOD by 09/10/2025 about continued need for updated guidance

Member Actions

- Transplant program staff should be educated about guidance update
  - Review impact(s) on program's candidates
  - Be aware of type and detail of clinical information to be included with exception requests
- NHRB for Pediatrics reviewers should be familiar with guidance update and circumstances when it applies

What do you think?

- What criteria should the Committee use to evaluate whether equipment shortage no longer exists?
- From the perspective of patients, families, living donors, donor families, how has the shortage impacted pediatric candidates?

Extra slides

OPTN Management and Membership Policies E.7: Emergency Actions

- Policy proposals that meet at least one of the following criteria may be adopted by the Board of Directors prior to public comment:
  - A proposal that is necessitated by a pending statutory or regulatory change
  - A proposal that is required due to an emergent public health issue or patient safety factors
  - A proposal that is necessitated by a new medical device or technology that affects organ allocation
- Emergency policy development process included the following steps:
  - Designates a future date upon which the policy will expire