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AAP policy clarifies acceptable off-label use of medical devices in children

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In many instances, therapy for childhood illness or conditions involves the use of medical or surgical devices. In the adult world, devices are commonly “cleared” by the Food and Drug Administration (FDA) for specific uses, based on information provided by medical device companies about their safety and efficacy from clinical trials.

Unfortunately, studies of device use in children are less common, so use of devices “off-label,” or in circumstances different than FDA-cleared or -labeled use, is widespread in pediatric practice. The Academy has issued a policy statement clarifying that the use of devices off-label is appropriate in circumstances where no FDA-approved device is available and when the physician believes use of the device is in a child’s best interest.

Off-Label Use of Medical Devices in Children, from the AAP Section on Cardiology and Cardiac Surgery and Section on Orthopaedics, is available at http://dx.doi.org/10.1542/peds.2016-3439 and will be published in the January issue of *Pediatrics*.

The policy also addresses use of devices for rare conditions that have been cleared by the FDA under a Humanitarian Device Exemption (HDE) and makes it clear that these devices should not be considered investigational. Importantly, the statement makes it clear that public and private payers should cover appropriate use of off-label or HDE devices.

The Academy has advocated successfully for requirements and incentives for more testing of drugs in children, and in 2014 issued a policy statement (http://bit.ly/2gNG0AT) regarding the appropriateness of off-label use of drugs in children in certain circumstances.

The new AAP statement extends these policies to cover devices and is even more important, as there are no laws requiring companies to study device use in children as a part of device development. The statement acknowledges that devices used to treat pediatric conditions ideally should undergo rigorous testing, similar to adult uses. It advocates for more research and better approaches through scientific and regulatory innovation.

The new policy also goes a long way to make sure children have access to needed devices and endorses the practice of pediatricians who use off-label devices appropriately.

Recommendations

- Pediatricians should consider off-label or physician-directed use of medical and surgical devices in children as necessary and appropriate when there is no device available that has been approved or cleared by the FDA for the specific pediatric indication.

- Policymakers, institutional review boards and payers should consider the use of HDE-approved medical and surgical devices as appropriate and not investigational. This mechanism often is the only pathway to FDA clearance for devices used in fewer than 4,000 patients annually.
- Public and private payers should approve payment for off-label and HDE-cleared devices when the use of such devices is directed by a physician and provides effective therapy, and when equivalent therapy is not available with other specifically labeled devices.
- Appropriate government agencies should take steps necessary to increase FDA-approved pediatric labeling for all medical devices, especially for the highest-risk devices used in children.
- The FDA, National Institutes of Health and other policymakers should vigorously explore opportunities to facilitate and encourage the design and testing of medical and surgical devices specifically for children through scientific and regulatory innovation, including increasing pediatric expertise within relevant agencies.

Dr. Jenkins, a lead author of the policy, is a member of the AAP Section on Cardiology and Cardiac Surgery Executive Committee.