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Children are not little adults

Federal legislation would help ensure medical, surgical devices meet pediatric patients' needs

Robert Campbell Jr., M.D., FAAP, knows firsthand the challenges children face when it comes to accessing life-saving medical and surgical devices made especially for them.

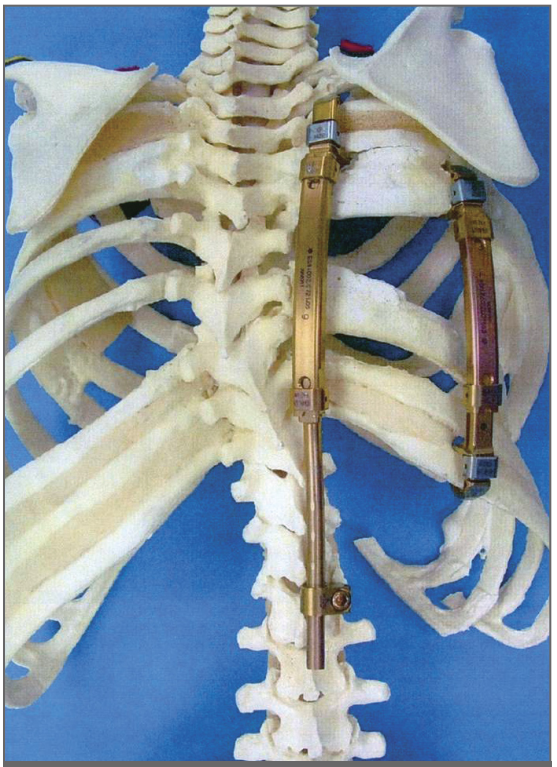
Dr. Campbell

Director of the Center for Thoracic Insufficiency Syndrome at The Children's Hospital of Philadelphia, Dr. Campbell invented the Vertical Expandable Prosthetic Titanium Rib (VEPTR) to treat rare diseases of the spine and chest wall. The device surgically expands a child's chest to correct spinal deformities without stopping growth.

In 1987, Dr. Campbell knew nothing about how to manufacture a medical device; all he knew was that his 6-month-old patient, who was born with severe scoliosis, missing several ribs and relied on a ventilator to stay alive, depended on a surgical solution that didn't exist. Along with Melvin Smith, M.D., a pediatric general surgeon, Dr. Campbell "jury-rigged" an artificial chest wall of orthopedic fracture pins wired vertically to support the lungs and control the scoliosis.

"My shoes filled up with sweat that night," said Dr. Campbell. "One slip and you go into the child's artery. Amazingly though, the surgery worked, and five days later the little guy went off the ventilator for first time in his life and grew and thrived."

Following the life-saving surgery, Drs. Smith and Campbell faced a new challenge: The crude chest wall device would not grow with the patient. Drawing from his engineering background, Dr. Campbell began sketching ideas for expandable artificial ribs, inventing what would become the VEPTR.



This titanium rib, which surgically expands a child's chest wall, was approved by the FDA for pediatric use under its Humanitarian Device Exemption (HDE). The pediatric incentive for HDE devices is set to expire Oct. 1 if it is not renewed by Congress.

"I was learning on the fly," Dr. Campbell said. "There wasn't a phone number in the phone book that said: 'information for inventors for devices for rare diseases.'"

Eventually, an orthopedic device company agreed to manufacture the device in return for positive publicity should the surgery prove successful. With that support, Dr. Campbell and his colleagues replaced the original device with the VEPTR in the young patient in 1989, two years after the emergency surgery.

Fourteen years later in 2004, the Food and Drug Administration (FDA) finally approved the VEPTR under its Humanitarian Device Exemption (HDE) approval pathway. Devices classified as HDEs can be used only to treat or diagnose a disease or condition that affects fewer than 4,000 patients. While HDE manufacturers must demonstrate safety, they are not required to prove efficacy in the same way as larger market devices.

To protect against inappropriate use of the HDE pathway, Congress prevented HDE manufacturers from profiting on the sale of their devices, allowing them only to recoup research and development costs. As a result, the HDE pathway is a less popular avenue for manufacturers to pursue; only 56 devices have been approved since the pathway was created in 1996.

A federal solution

That changed in 2007. After sustained advocacy and with support

from the Academy, then-U.S. Sen. Chris Dodd (D-Conn.) and U.S. Reps. Ed Markey (D-Mass.) and Mike Rogers (R-Mich.) helped pass the Pediatric Medical Device Safety and Improvement Act to incentivize more manufacturers to develop pediatric HDE devices. The law removed the profit restriction for devices marketed and approved as HDEs so long as they were labeled for pediatric populations. Once the device has pediatric labeling, any approved adult uses also can make a profit.

Since 2008, the number of medical devices designated for pediatric patients has increased five-fold, the first step in the HDE approval process.

Every five years, a collection of bills authorizing certain FDA programs are up for renewal. The pediatric incentive for HDE devices will expire if not renewed by Oct. 1, 2012. The Academy is engaged in aggressive advocacy on Capitol Hill to protect the profit incentive for HDE devices labeled for pediatric use in the 2007 law's reauthorization.

The Best Pharmaceuticals for Children Act (BPCA) and the Pediatric Research Equity Act (PREA) are due to be reauthorized at the same time. The longstanding AAP priority programs have resulted in more than 427 drug labeling changes to include pediatric information since 1997.

Bills to reauthorize the HDE law and BPCA and PREA have been introduced in the U.S. House of Representatives, H.R. 3975 and H.R. 4274, both of which the Academy has endorsed. A bipartisan consensus proposal on medical device policy also has been circulated in the U.S. Senate. The proposal preserves the HDE pediatric profit incentive while allowing devices that treat diseases or conditions that do not occur in pediatrics to make a profit, advancing the prospects for new device options for adults with rare diseases. With the support of pediatricians like Dr. Campbell, the Academy was able to negotiate a compromise among the medical device industry, rare disease groups and federal legislators.

Unfortunately, current legislative proposals fail to address inconsistent insurance coverage that has arisen with pediatric HDEs. The Academy will be working with members to resolve issues involving insurance coverage denials of these life-saving devices.

Funding for pediatric device consortia

The Pediatric Device Consortia Grant Program is another initiative in need of reauthorization within the FDA legislative package up for renewal this year. The program funds nonprofit consortia to bridge the gap between developing an idea for a medical device and acquiring funding for its development and understanding on how to conduct clinical trials and navigate the FDA regulatory process.

"With the consortia in place, clinicians and others with a pediatric device idea now have a place to go to receive assistance with many of the issues that go into device development," said Linda Ulrich, M.D., FAAP, director of the Pediatric Device Consortia Grant Program at the FDA. "These include intellectual property protection, prototyping, identifying funding resources, appropriate preclinical and clinical trial design, and regulatory submission."

The five FDA-funded Pediatric Device Consortia have helped advance the development of 135 proposed pediatric medical devices to date. Currently, the consortia are managing 80 projects, including an artificial kidney to treat children with severe kidney failure, a roboimplant to treat severe pediatric spinal deformities and a device that uses two rare earth magnets to slowly reconfigure the chest in children with pectus excavatum.

"In less than three years, the Pediatric Device Consortia have made inroads by advancing pediatric medical devices, leveraging resources to attract additional funding for pediatric device research, and increasing national awareness around pediatric medical device development issues," said Dr. Ulrich.

The program was funded at $3 million in fiscal year 2012 and proposed at $3 million in President Obama's in fiscal year 2013 budget but authorized at $6 million per year. The Academy recently sent a letter to Congress along with 15 other organizations urging funding for the program at $6 million, and will continue to advocate for the passage of H.R. 3975, which would reauthorize the program.

"Children deserve access to devices that are safe, effective and made just for them," said Dr. Campbell. "Nothing is more wonderful than to watch a child that was going to be severely disabled or die early in life grow up and hopefully make a difference for the world in their own way some day.