One-time hydrocephalus operation brings good outcomes for babies

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Hydrocephalus, an abnormal build-up of cerebrospinal fluid within the brain, is typically treated by surgically placing a shunt to continually drain cerebrospinal fluid into the abdomen, protecting the brain from excess pressure. However, shunts nearly always fail over time and must be replaced, requiring emergency neurosurgery—which is not readily available in many areas. A randomized trial in today's *New England Journal of Medicine* shows good results with an alternative, potentially one-time operation called endoscopic third ventriculostomy with choroid plexus cauterization (ETV/CPC).

"There are many advantages to avoiding lifelong dependence on a shunt," says neurosurgeon Benjamin Warf, MD, at Boston Children's Hospital, the study's senior author. "But one important unanswered question has been whether ETV/CPC is as good for infant brain development as placing a shunt."

ETV/CPC was pioneered by Warf 17 years ago while serving as a medical missionary in Uganda, where shunt failures often are fatal. It is a two-part procedure: ETV uses an endoscope to create an opening that allows trapped cerebrospinal fluid (CSF) to escape from the brain cavities known as ventricles, while CPC uses an electrical current to burn off some of the fluid-producing tissue.

Cognitive outcomes: shunting versus ETV/CPC

The NIH-funded study randomized 100 infants at the CURE Children's Hospital of Uganda who had hydrocephalus as a result of a neonatal nervous-system infection. Fifty-one were randomized to ETV/CPC, and 49 to shunt placement. All were under 6 months of age (average age, 3.25 months).

At 12 months, the group randomized to receive a shunt had lower CSF volumes, as expected, but there was no significant difference between treatment arms in regard to brain volume or cognitive scores (measured by the Bayley Scales of Infant Development, administered by trained evaluators who were unaware of treatment assignment).

"Others have suggested that shunts are a better treatment in regard to neurologic development or brain growth, but we found this was not the case, despite the fact that shunts cause more of a decrease in the ventricle size," says Warf.

After one year, treatment had failed in 18 patients randomized to ETV/CPC and 12 patients randomized to shunts, which was not a statistically significant difference. The researchers will continue to follow the infants to learn their two- and five-year outcomes; previous studies suggest that after this early period, failures of ETV/CPC are uncommon.

"We were surprised that there was no significant difference in treatment failure at 12 months, because ETV/CPC is expected to have a higher short term failure rate, whereas shunts tend to fail more over time," says Warf. "We expect that there will be no further ETV/CPC failures, which nearly all occur within six months, but that the shunts will continue to fail as we follow this cohort."

Although this study was limited to infants with post-infectious hydrocephalus, prior research by Warf's group has shown that ETV/CPC

can also prevent the need for a shunt in most infants with other causes of hydrocephalus—including brain hemorrhage from prematurity, spina bifida, aqueduct stenosis, encephalocele and Dandy-Walker malformation.

The current study had some limitations: It was performed at a single center that has developed expertise in ETV/CPC, and it did not have enough statistical power to rule out small differences in outcomes aside from cognitive scores. Also, infants in both groups tended to have lower developmental scores due to the original brain infection.

"We will continue to follow these children, but the results further raise our level of confidence in recommending ETV/CPC as the initial treatment for most infants with hydrocephalus," says Warf. "In the hands of trained surgeons, the operation provides an opportunity to avoid a lifetime of shunt dependence."

Warf, a 2012 MacArthur Foundation fellow, spends much of his time training other surgeons in the ETV/CPC technique. The training will soon be aided by a highly sophisticated, anatomically accurate mannequin developed with the help of special effects artists.

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