CONGRESSIONAL TESTIMONY
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Introduction

Chairman Bass, Congressman Smith, and honored members of the committee, it is a privilege to have been invited to speak to you.

Like many other childhood medical conditions, the one I have been asked to discuss today – hydrocephalus – is common and its consequences are devastating. The good news is that it can be treated with a fairly straightforward and cost-effective operation. The bad news is that children in low income countries so often do not have access to treatment.

In 2000, my family and I moved to Uganda to help begin a neurosurgery hospital for children. From its opening, we were inundated with a steady stream of mothers seeking treatment for the infants with hydrocephalus. Astonished by the staggering volume of these patients, I was confronted with two immediate questions: 1) How common was it and what was causing it? 2) What was the best way to treat it under these circumstances? Over the last 20 years, we have made progress in answering these questions.

Definition of hydrocephalus

The normal brain contains fluid-filled spaces called ventricles that communicate with the fluid space surrounding the brain and spinal cord. This cerebrospinal fluid (CSF) is made inside the brain's ventricles and normally pulsates freely among all these spaces with a balance between production and absorption. Anything that disrupts this normal circulation pattern can cause CSF to accumulate within the ventricles inside the brain, which then gradually enlarge under pressure. This condition is called hydrocephalus.[1]

Causes of hydrocephalus

There are many causes of hydrocephalus.[1] In the US and other developed countries, where circumstances enable prematurely born babies to survive, one of the most common causes of hydrocephalus is bleeding into the ventricles, something to which the premature brain is prone. In low income countries, the premature generally do not survive; but, newborn infections are much more common than in wealthy nations. We now recognize that infection in the brain's fluid spaces is likely the single most common cause of infant hydrocephalus in developing countries. In Uganda, we reported that post-infectious hydrocephalus accounted for 60% of all cases in children.[2] Notably, these two common causes of hydrocephalus are potentially preventable by reducing premature birth and subsequent brain hemorrhages in the US and by reducing the incidence of neonatal sepsis and meningitis in low-income countries.

As well, both post-hemorrhagic hydrocephalus of prematurity and post-infectious hydrocephalus are associated with some degree of primary brain injury from the initial bleeding or the infection, unrelated to the subsequent effects of hydrocephalus. These can result in the persistent cognitive and motor disabilities recognized as cerebral palsy, despite timely and successful treatment of the hydrocephalus. Thus, prevention is important not only to forestall the development of hydrocephalus, which is treatable, but also to prevent the initial brain injury itself, which is not. One study of children successfully treated for post-infectious hydrocephalus in Uganda showed that 1/3 had died by 5 years and 1/3 of the survivors were badly disabled.[3] It is notable that, according to the 2011 World Report on Disability by the WHO and World Bank, children with disabilities have less access to basic healthcare, as well as education and employment opportunities, than their peers.[4]

Common congenital causes of hydrocephalus include aqueduct stenosis (obstruction of an internal CSF pathway called the cerebral aqueduct) and hydrocephalus associated with neural tube defects.[1]

Consequences of hydrocephalus

With hydrocephalus, whatever its cause, CSF builds up inside the ventricles, causing them to expand under pressure. This stretches and compresses the surrounding brain tissue, causing mechanical injury and decreased blood flow. In young infants with compliant skulls the expanding ventricles typically cause an abnormal rate of cranial expansion, resulting in a very large head. There can also be progressive symptoms of irritability, lethargy, poor feeding, vomiting, and abnormal eye movements. Untreated, hydrocephalus leads to progressive brain injury and inhibition of its normal growth. It causes infants to suffer pain because of the pressure, and results in long-term cognitive and motor disabilities, spasticity, visual impairment, and sometimes death.[1] Before there was effective treatment for hydrocephalus, about half of the babies did not survive past the age of two years and survivors were significantly disabled. When hydrocephalus develops in older children, the skull is no longer able to readily expand, and elevated intracranial pressure is a more acute problem resulting in death more quickly than is the case with infants.

Prevalence of hydrocephalus

Hydrocephalus is the most common condition treated by pediatric neurosurgeons and accounts for about 2 billion dollars of health expenditures in the US every year[1]. The prevalence of infant hydrocephalus is roughly one in one thousand[1]; but, the prevalence is estimated to be higher in developing countries[5], where access to treatment is also more limited.[6] We have estimated that in sub-Saharan Africa alone new infant hydrocephalus cases may exceed 200,000 every year, and the majority are likely secondary to neonatal infection[7]. In a recent exhaustive review of the world literature, the incidence was higher in low- and middle-income countries (123 per 100,000 births) than in high income countries (79 per 100,000 births).[5] Worldwide, there may be up to half a million new cases of infant hydrocephalus each year. The greatest burden of disease falls on the African, Latin American, and Southeast Asian regions, accounting for an estimated three-quarters of the total volume of new cases.[5]

Economic impact of hydrocephalus

In the US alone, treatment of pediatric hydrocephalus claims up to 2 billion dollars in healthcare expenditures every year[1] and has been estimated to account for 2% of all pediatric hospital admissions. A published analysis of the consequences of untreated hydrocephalus in sub-Saharan Africa recently estimated annual economic losses of up to $1.6 billion using a human capital approach and up to $56 billion using the value of a statistical life approach.[7] Notably, this economic burden is comparable to published estimates of other, more widely recognized medical conditions in Africa such as malignancies, perinatal conditions, congenital anomalies, cataracts and glaucoma.

Treatment of hydrocephalus

There are currently two types of treatment – both surgical. 1) A ventriculoperitoneal shunt (VPS) can be implanted that will allow CSF to escape from the brain's ventricles through a tube into the abdominal cavity. This commits the child to shunt dependence, typically for the remainder of his or her life. Shunts tend to fail. About half will fail in the first couple of years after placement. About 3 out of 4 will have failed at least once in the first 10 years, typically requiring an urgent operation. On average, shunted children have 3 additional operations for shunt failure over time; but, many children have dozens or even scores of operations over the course of their lives, and shunt failure can be an acute life-threatening emergency. The condition of shunt-dependence generates anxiety for families as well as unnecessary emergency room visits and hospital admissions to rule out possible shunt malfunctions. In addition, shunt operations carry about a 5-10% chance of infection. 2) Another option is an endoscopic operation that does not use an implanted device. Endoscopic third ventriculostomy (ETV) makes a new opening between the ventricles (through the floor of the 3rd ventricle) and the normal CSF space outside brain, where the fluid is supposed to go anyway. This re-establishes the normal circulation of fluid from inside to outside the brain. This is much more successful in young infants if some of the choroid plexus (tissue inside the ventricles that makes some of the CSF) is reduced by cauterization. [1, 8] For young infants we combine ETV with choroid plexus cauterization (CPC) to achieve maximum success. With this method, there is an overall 2 out of 3 chance for long-term success in treating hydrocephalus without the need for a shunt. ETV/CPC failures mostly occur early, within 6 months of the operation, unlike the persistent, lifelong risk for recurring shunt malfunction. The risk for infection is also considerably lower for ETV/CPC, at less than 1%.

The development and study of ETV/CPC as well as research into the causes of the neonatal infections that lead to so much hydrocephalus in Uganda, has been facilitated by generous grant support from USAID and the NIH Fogarty Institute. An NIH-funded randomized trial of ETV/CPC compared to shunt placement in hydrocephalic Ugandan infants has shown no difference in developmental outcome or brain growth.[9] Thus, hydrocephalus can usually be treated just as effectively without creating life-long shunt dependence. Since we developed ETV/CPC in Uganda, first published 15 years ago[8], the technique has been adopted in many centers in the US and around the world. This is an excellent example of how US support for work in a developing country has benefitted the health care of US citizens.

The situation in low resource countries

Of the world's pediatric neurosurgical workforce, 86% operate in high income countries. In low and lower-middle-income countries, around 330 pediatric neurosurgeons care for a total pediatric population of 1.2 billion. In the low-income countries of sub-Saharan Africa there is one pediatric neurosurgeon per 30 million children.[6] On the other hand, three-fourths of the world's new cases of hydrocephalus come from the regions of Africa, Latin America, and Southeast Asia.[5] The regions with the highest volume of hydrocephalus and lowest number of neurosurgeons are precisely where children will benefit the most from treatment that avoids a life-time of shunt dependence whenever possible.

A key component to help address the healthcare gap for hydrocephalus will be training and equipping neurosurgeons in the developing world to treat infant hydrocephalus by using endoscopic surgery instead of placing shunts. This training will be especially important in the national referral hospitals and academic centers of high population centers where new national neurosurgeons are trained, with the aim of training the trainers. A number of organizations are already involved in work to expand access to care, including CURE International, Reach Another Foundation, Bethany Kids, Child Help, and some of the international neurosurgical societies, like the World Federation of Neurosurgery and the International Society of Pediatric Neurosurgery. NeuroKids is a new non-profit organization that hopes to focus on training and mentoring neurosurgeons in urban centers who are responsible for training neurosurgeons in their own country.

Thank you.

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