Hydrocephalus remains an unsolved medical mystery

Hydrocephalus is a chronic medical condition that affects over a million people – from newborns to the elderly – in the U.S. There is no known cure and the only treatment options are surgical procedures developed over 50 years ago.

The first generation of individuals treated in childhood for hydrocephalus are now adults facing a long list of challenges including a lifetime of brain surgeries and uncertainty, and limited access to medical insurance. Imagine a 24-year-old woman who has undergone and withstood over a hundred invasive brain surgeries and faces a future not knowing how many more she will experience over her lifetime.

The challenge is clear. Current surgical treatment options are woefully insufficient. Adequate treatment for hydrocephalus remains a medical mystery. The hydrocephalus medical problem has not been solved.

The public health burden of hydrocephalus is significant

- On average, 6,000 new babies are born with hydrocephalus every year in the United States and thousands of people are diagnosed later in childhood or as adults.
- Over 36,000 shunt surgeries are performed each year in the US (an average of one every 15 minutes).
- About two-thirds are not the first surgery and over half represent life and death emergencies.
- The total cost of shunt surgeries in the U.S. exceeds $2 billion per year (J. Neurosurgery: February 2008) and this does not include the cost of any rehabilitative therapy or educational accommodations.
- Accurately diagnosing and treating the 375,000 adults over 60 with Normal Pressure Hydrocephalus (NPH) could save Medicare $184 Million over 5 years (J. Neurosurgery: July 2007).

Shockingly, less than $5 Million per year in public and private money combined is directed to hydrocephalus research.

Public dollar investment in hydrocephalus research is much lower than that of conditions with similar public health burden

<table>
<thead>
<tr>
<th>Condition</th>
<th>Public Spending</th>
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<tbody>
<tr>
<td>Hydrocephalus</td>
<td>$650K</td>
</tr>
<tr>
<td>Down Syndrome</td>
<td>$19M 29x</td>
</tr>
<tr>
<td>Autism</td>
<td>$103M 158x</td>
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<td>Parkinson’s</td>
<td>$200M 308x</td>
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The current state of treatment

There are two accepted surgical treatments for hydrocephalus and neither is sufficient.

1. The implantation of a device called a shunt to divert CSF into another part of the body has high failure and revision rates.

2. Endoscopic Third Ventriculostomy (ETV), which creates an opening in a membrane between the ventricles, is prone to sudden catastrophic failure that can lead to death if not treated immediately.

Many people with hydrocephalus have had over 10 brain surgeries. There are reported cases of more than 100 brain surgeries for individuals.

Until dramatically improved treatment options and, ultimately, a cure are found, people affected by hydrocephalus will rarely lead lives free of repeated, risky medical intervention.

With the support of the Board of Directors and the Medical Advisory Board, the Hydrocephalus Association has decided to take on a vitally important role – raising and directing money to fund research aimed at improving treatment and finding a cure for hydrocephalus.

Why the Hydrocephalus Association?

The Hydrocephalus Association is uniquely positioned to assume this role and strategically influence the hydrocephalus research agenda. Tapping into its national membership constituency of thousands of concerned individuals the Hydrocephalus Association can leverage private investment and its national advocacy network to increase public investment in hydrocephalus research through increased funding from the National Institutes of Health (NIH) and other federal agencies.

Over its 25-year history the Hydrocephalus Association has served over 40,000 individuals and families as well as clinical and research professionals, earning a solid reputation for effectiveness and integrity. Hydrocephalus Association Partners provide local support, fundraising and advocacy in every region of the country and work in concert to unify and amplify the voice of the hydrocephalus community across the nation. This community is clamoring for more research, and trusts the Hydrocephalus Association to take a lead role in raising and directing money to advance research.

A long history of work and leadership

In 2002 the Hydrocephalus Association developed a self-report registry for patients, enabling over 2,000 patients to provide data on their treatment history and quality-of-life outcomes. In 2004 the Hydrocephalus Association began advocating for more federal support of hydrocephalus research and, in September 2005, the NIH sponsored the first hydrocephalus research workshop “Hydrocephalus: Myths, New Facts, Clear Directions”. Findings from this workshop confirmed that “treatment for hydrocephalus has not advanced appreciably since the advent of cerebrospinal fluid shunts more than 50 years ago” and that “many questions remain that clinical and basic research could address, which in turn could improve therapeutic options.” (J Neurosurgery: Pediatrics; November 2007)
A long history of work and leadership (continued)

In 2006, the Hydrocephalus Association held its first National Advocacy Day in Washington DC. Over 120 individuals personally touched by hydrocephalus met with 48 senators and 51 congressional representatives, most of whom heard the word hydrocephalus for the first time. Just twenty (20) months later both houses of Congress passed a resolution calling for more NIH support of hydrocephalus research. The NIH tripled its investment in hydrocephalus research between 2006 and 2009. However, this level of investment remains woefully inadequate relative to the burden of hydrocephalus on the population.

Mission, vision and goals for the Research Initiative

The mission of the Hydrocephalus Association Research Initiative is to fund research that improves the quality of life for people living with hydrocephalus and find ways to prevent or cure hydrocephalus.

By the end of 2009, the Hydrocephalus Association hopes to have raised $500,000 and completed its first round of grant awards for Mentored Young Investigators. This mechanism fulfills the dual purpose of funding promising research relevant to hydrocephalus while fostering a new generation of researchers developed under the guidance of more experienced mentors. The Hydrocephalus Association has engaged more than 40 respected scientists and clinicians to review applications for its current and future grant cycles.

The Hydrocephalus Association has developed a business plan which provides that by 2013, the Hydrocephalus Association will be directly funding $3 Million in clinical and basic research that will increase the number of researchers focused on hydrocephalus tenfold in five years. Specifically, the Hydrocephalus Association’s Research Initiative includes the following:

- Awarding of career development grants to stimulate the pipeline of new researchers.
- Collection of pilot data necessary for hydrocephalus researchers to gain NIH grant support.
- Creation of critical research infrastructure including registries, tissue banks and animal models.
- Provision of support for sustained scientific collaboration across disciplines.
- Development and widespread use of standardized treatments, through the process of evidence-based medicine and enabled by clinical research networks, which will provide better outcomes for patients. For example, the infection rate for shunt surgeries – currently approximately 10% – will drop by 50% nationwide as neurosurgeons adopt proven techniques.
- Transitional care issues facing young adults will be confronted head on and solutions for continued care will be in place in major metropolitan areas.
- Patients with shunts will benefit from advances in bioengineering and microelectronic manufacturing techniques to eliminate the need for invasive diagnostics for measuring shunt function or intracranial pressure. Shunt failure rates – currently 50% within 2 years and 85% in 10 years – will be decreased.
Mission, vision and goals for the Research Initiative (continued)

By 2020, the landscape of hydrocephalus research and treatment will be dramatically improved. The influx of new researchers and the collaborative environment of the prior decade will produce promising findings in basic science that will have immediate and direct impact for people suffering from hydrocephalus:

- Advances in genomic and proteomic research findings will enable pharmaceutical companies to target drugs to halt or reverse some negative outcomes of chronic hydrocephalus.
- The understanding of the complex brain dynamics of hydrocephalus will have advanced to enable more translational research and better clinical practice.
- Neurosurgical practices will be standardized.
- Outcomes for patients will be greatly improved in terms of quality of life as a result of reduced revision and infection rates, decreased incidence of hydrocephalus through preventive measures and advanced developmental understanding and intervention.

If we don’t act

Children with hydrocephalus will continue to grow into adults who most likely will not have a college degree live independently, hold a meaningful job, have health insurance or be in a committed relationship – basic human needs that most of us take for granted. Medicare will continue to waste close to $40 Million every year and aging adults will continue to struggle needlessly with walking and balance, urinary incontinence and thinking and memory.

For the quality of life of individuals with hydrocephalus, and for the public health burden, the Hydrocephalus Association needs to act now.

Please help the Hydrocephalus Association accomplish these vital goals for people with hydrocephalus.