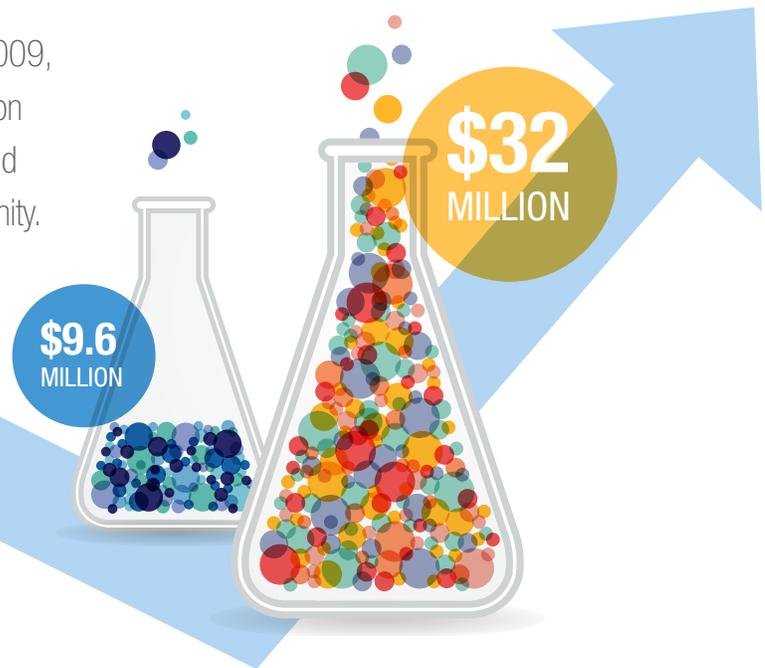


Accelerating Research — 10 Years of Impact

Hydrocephalus Association Research Update Fall 2019

10 Years of Funding High-Quality, High-Impact Research

Since the start of our Research Initiative in 2009, the Hydrocephalus Association has focused on funding high-quality, high-impact research and building the hydrocephalus research community. Over the past 10 years, our impact can be seen throughout the hydrocephalus research landscape.



Since the start of our Research Initiative in 2009, HA has:

- ▶ Spent over \$9.6M on our Research Program
- ▶ Awarded 36 Grants
- ▶ Sponsored 6 Research Workshops
- ▶ Supported 3 Research Networks & 2 Biobanks
- ▶ Developed the 1st Hydrocephalus Patient-Powered Registry

HA-funded Grantees and Clinical Networks have gone on to:

- ▶ Publish 69 Peer-Reviewed Studies
- ▶ Secure over \$32M in Additional Grants
- ▶ Decrease Shunt Infection Rates by 36%
- ▶ Test 7 Preclinical Drug Therapies
- ▶ Obtain 1 New Patent for a Drug Target

Orange County Families Unite to Fund a Cure



Heather Kluter's daughter Eva was born 9 weeks prematurely due to an intestinal twist. After one month in the NICU, doctors discovered that she had developed hydrocephalus.

"We struggled so much those first few years, as doctors painted a bleak picture of her future, filled with developmental challenges and the possibility of being in the hospital more than she was out.

It was scary and depressing," Heather explained. Since then, Eva has had three brain surgeries and five additional surgeries due to related complications. She is now 10 years old, thriving and celebrating life to the fullest.

Throughout Eva's journey, the Kluter family has been actively involved with the Hydrocephalus Association, raising nearly \$140,000 for HA's research, support and education programs. Whether it was through their "Trick or Treat" fundraiser or "Dare to Donate," where people were asked to perform a challenge in exchange for a donation, the Kluters were always raising money for a cure. Some dares included recreating the lift scene in *Dirty Dancing* at the mall or stuffing marshmallows in your mouth one by one, while trying to say hy-dro-ceph-a-lus.

In 2019, Heather was introduced to Erin and Pieter Berger, who also reside in Orange County, CA. The Berger's first child, Seamus (Shea), was diagnosed with hydrocephalus two months after birth and shunted. "We were terrified. We did not know much of anything about hydrocephalus, let alone the treatment options available for our Shea. Fortunately, we were blessed to have a preeminent neurosurgeon at Children's Hospital of Orange County (CHOC), Michael Muhonen, MD, only a

couple blocks from our home. We knew Shea was in the best care, but we had no idea how a condition could exist where the only treatment option requires brain surgery," Erin said.

Now, four years after Shea's diagnosis, the Bergers have joined forces with the Kluters to launch HA's inaugural **Orange County WALK to End Hydrocephalus**. The Bergers are passionate about HA's mission to find a cure and are excited to support HA's efforts.

"Hydrocephalus is personal to us and we are devoted to promoting a cure; if a cure is not in sight at this time, then we are going to work toward better treatment options. This is why research is crucial to our daily lives. We look forward to the day we can troubleshoot a potential shunt malfunction in the comfort of our home, ultimately avoiding another brain surgery. We look forward to removing limitations on our family activities. We look forward to allowing a virus or the flu to simply be that instead of shunt failure. We look forward to the day we do not think about Shea's shunt," Erin and Pieter said.

As co-chairs of the Orange County WALK, Erin Berger and Heather Kluter are making HA WALK history, surpassing the event's initial fundraising goal six weeks before the WALK. It's thanks to the Bergers and the Kluters, and the other amazing volunteers leading 44 HA WALKs across the country, that we know we will find a cure one day!



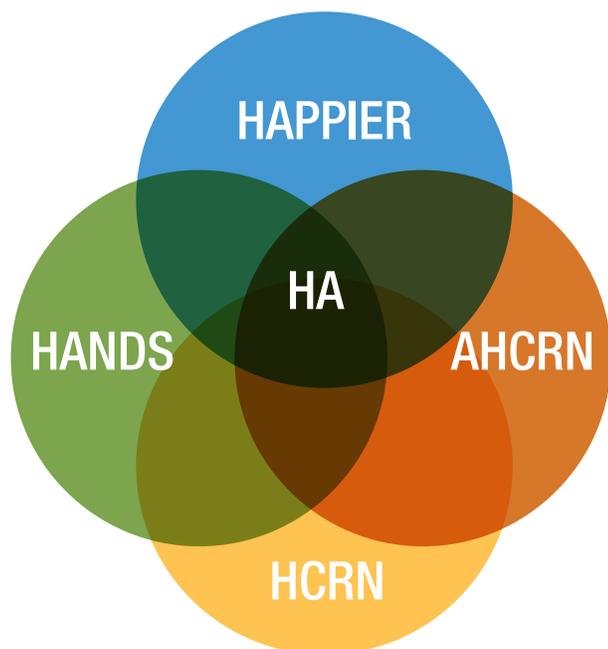
Get HAPPIER!

Be a Part of Research – Get HAPPIER!

The HA Patient-Powered Interactive Engagement Registry (HAPPIER) is an online database created to bring the patient perspective to hydrocephalus research. HAPPIER collects information on the treatment and symptoms, health, and quality of life of people living with hydrocephalus.

The data will be used in three ways:

- 1) To help HA improve our research, advocacy, support, and education efforts.
- 2) To provide researchers with data to conduct research projects.
- 3) To identify patients that are eligible to participate in future clinical trials.



HAPPIER is a part of the larger HA Research Network that is working together to improve outcomes, find new treatments, and develop ways to prevent hydrocephalus all together. With the HA Network for Discovery Science (HANDS), Hydrocephalus Clinical Research Network (HCRN), and the Adult HCRN (AHCRN), HAPPIER will help accelerate hydrocephalus research by providing patient-generated and patient-centered data to the research community.

Join the over
550 HAPPIER participants today
at hydroassoc.org/happier!

Research Updates

On a Mission to Prevent Posthemorrhagic Hydrocephalus



Jennifer Strahle, MD
Assistant Professor of
Neurological Surgery
Director, Pediatric Neuro Spine Program
Washington University in St. Louis

Dr. Jennifer Strahle is focused on understanding how specific blood components (hemoglobin and iron) lead to the development of Posthemorrhagic Hydrocephalus of Prematurity (PHHP). Her recent work has shown that hemoglobin and iron damage cilia, small hair-like structures that move cerebrospinal fluid along the walls of the ventricles. This year, Dr. Strahle received a grant worth \$2.4 million over the next five years from the National Institutes of Health (NIH) to continue this work. The NIH grant is a direct outcome of the funding she received from HA through the 2016 and 2017 Innovator Awards.

New Insights into the Genetics of Congenital Hydrocephalus



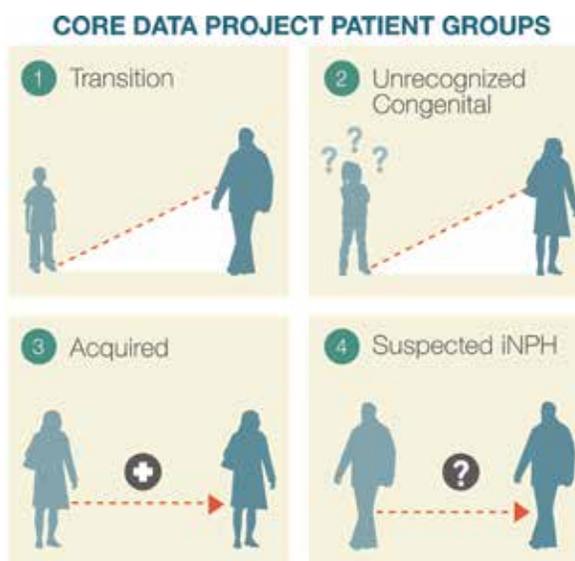
Kristopher Kahle, MD, PhD
Assistant Professor of Neurosurgery and
of Cellular and Molecular Physiology
Director of Neonatal and Congenital
Anomaly Neurosurgery
Yale School of Medicine

With your help, Dr. Kristopher Kahle has discovered three new genes implicated in the development of congenital hydrocephalus. Each of the three genes is involved with brain development and are now being inserted into animal models to help us understand the role they play in hydrocephalus. But, the work is not done! As more families join the study, additional genes will be identified and explored. To learn more about the study go to <https://www.hydroassoc.org/yale-university-study/> or watch the webinar: <https://www.hydroassoc.org/webinars/>.

Understanding our Adult Community: Adult Hydrocephalus Clinical Research Network

Dr. Michael Williams, MD
Professor of Neurology and Neurological Surgery
Director of the Adult and Transitional
Hydrocephalus Program
University of Washington

The Adult Hydrocephalus Clinical Research Network (AHCRN.org) published their first paper, "Insights into the Spectrum of Adult Hydrocephalus from the AHCRN Registry" in the *Journal of Neurosurgery*. The paper analyzes AHCRN data on the first 517 patients enrolled in the registry. Four categories of adult hydrocephalus were defined: transition (treated before age 18 years), unrecognized congenital (congenital pattern, not treated before age 18 years), acquired (secondary to known risk factors, not treated before age 18 or untreated), and suspected idiopathic normal pressure hydrocephalus (iNPH) (\geq age 65 years, not previously treated). The paper highlights similarities and differences in the demographics, etiology, educational attainment, and



neuropsychological assessments between the groups. To access the paper, visit: www.ncbi.nlm.nih.gov/pubmed/31125971.

What's Next: 2019 and Beyond

Pushing Towards a Cure

Hydrocephalus is complex, but scientists are discovering critical features that cut across and link the different forms and causes of hydrocephalus. Later this year, HA will host a Research Workshop that brings these scientists together for an active discussion on which features are key to the development of hydrocephalus and hold the greatest promise for the development of drug and other therapies.

Working with Industry



A prototype of Senseer's "Smart Shunt."

HA has a long history of working with industry partners. In recent years, we've seen an increase in the number of startup companies

focused on intracranial pressure monitoring, automated ventricular volumetrics, detection of shunt failure, alternative shunting methods, and other areas. HA will continue to work with all of these companies to help them understand the needs of our community and assist them as they work through clinical trial design and the Food and Drug Administration (FDA). We will also continue to keep you informed of their progress and setbacks along the way.

Expanding Our Research Reach

Decreasing treatment complications and finding new drug targets for hydrocephalus are critical to HA's mission – but they are not the whole story...



How many people with hydrocephalus have migraines? What pain medications work best for patients with chronic pain? Are there cell replacement therapies that may improve vision? How can patients improve their navigation skills? How can patients improve their memory?

With your help, HAPPIER, HA's patient-powered registry, is generating data that will be used by clinicians and researchers across a broad range of specialties to answer these questions and others.

STAY INFORMED!

Learn about the latest research, support and education initiatives, and events taking place at the Hydrocephalus Association. Sign up for HA's monthly electronic newsletter at www.hydroassoc.org/newsletter.



Answering YOUR Research Questions!

Join HA later this year as we engage our entire community in determining our top 10 Research Priorities for Hydrocephalus. This top 10 list will guide HA research activities and programs in years to come! **Stay tuned for details!**



4340 East West Highway, Suite 905
Bethesda, Maryland 20814-4447

10 Years of High Impact Research Making Your Donations Go Further

In 10 years, HA researchers and research networks have turned our \$9.6M investment into over \$32M in additional grants dedicated to hydrocephalus research.

