



One Mission: End AHC!



YOUR ALTERNATING HEMIPLEGIA OF CHILDHOOD FOUNDATION NEWSLETTER

April 2016

2000 Town Center ■ Suite 1900 ■ Southfield, Michigan 48075

AHCF in ACTION

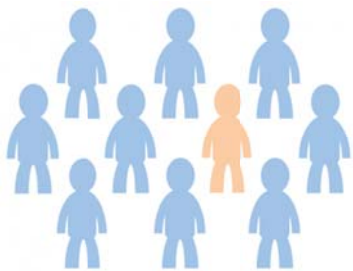
Honoring our Volunteers this Month

This is **National Volunteer Week** and I want to take this opportunity to **thank everyone who volunteers** their time with the goal of ending AHC.

National Volunteer Week first began in Canada in 1943, when some Women's Voluntary Services organized special events to draw attention to the important contributions women made on the home front during World War II. Over the years, the idea grew to include all communities and all volunteers. National Volunteer Week is now celebrated in both nations on the same week every year.

It is only because of the work volunteers take on all year long that we will eventually be successful in ending AHC. Thank you for all that you do for our entire community.

Lynn Egan
AHCF President



AHCF in ACTION

Don't Let AHC Be Left out of the NIH Budget

AHC Families call to action: Advocate for Federal Funding of Medical Research! An Article by Dr. Alfred George, Northwestern University

Your help is needed to **advocate for the funding of the 21st Century Cures Act** and for increasing support for rare disease research through the NIH.

“Rare disease research funding will be the first to get cut if the NIH budget is reduced,” according to Dr. Alfred L. George at Northwestern University. Dr. George, along with his colleague Dr. Kevin Ess at Vanderbilt University, has been studying AHC for the past few years with the support of the AHC Foundation. The doctors are planning to submit a multi-year NIH grant in 2017.

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This affects every AHC family!

How are we going to save Federal funding for AHC and other Rare Diseases?

Please join Dr. Al George on a **webinar on May 1st** to strategize with other AHC families and discuss the planned research that is in jeopardy from cuts to the upcoming federal budget.

Please register for AHC Meeting - Dr. Alfred L. George, Jr. on May 1, 2017 7:30 PM EDT, 6:30 PM CDT, 5:30 PM MDT, 4:30 PM PDT at:

**<https://attendee.gotowebinar.com/register/3470570352286660610>
<<https://attendee.gotowebinar.com/register/3470570352286660610>>**



AHC Families Call to ACTION **Advocate for Federal Funding of Medical Research! Continued...**

A proposal by the White House in March 2017 **to cut the NIH budget by 18% would cripple medical research in the U.S.A.** for years to come and endanger a new generation of innovative scientists at time when powerful new approaches are making it possible to accomplish work that could not be dreamed of just a few years ago.

Last December, the [21st Century Cures Act](#) was signed into law with the purpose of strengthening medical research in the U.S.A., particularly in areas related to brain diseases, cancer and precision medicine.

The **National Institutes of Health (NIH)** is the largest and most successful funder of medical research in the world, and is one of the best investments of tax payer dollars in terms of return on investment, job creation and business development. **Nearly half of all drugs approved in this country between 1998 and 2005 were developed in part because of public-sector support including NIH funding.** In 2015, NIH supported more than 350,000 jobs nationally. When income generated from these jobs along with the purchase of research-related supplies, equipment and materials is considered, the economic impact of NIH is estimated to be more than \$60 billion.

NIH funding is the only mechanism to provide multi-year funding for fundamental science in rare diseases. Medical research into the causes and treatments for rare diseases, such as AHC, is not done by pharmaceutical companies because of high risk and limited market value. **The proposed cuts to the NIH budget are a direct threat to rare disease research, which is expected to be placed at a lower priority than more common disorders.**

AHC Friends and Family Can Make a Huge Difference... Here's How to do it

Please call or email your [congressional representatives and U.S. senators](#) soon and tell them your child's story to emphasize the importance of rare disease research to help fight Alternating Hemiplegia of Childhood and other rare diseases. **Tell them to fully fund the 21st Century Cures Act** and to boost, not slash, NIH funding. The airwaves and inboxes need to be flooded and **your voice needs to be heard!**

Here Are Some Talking Points You May Wish to Share:

- Investing in medical research provides a strong return on investment, promotes job creation and business development
- NIH supports more than 350,000 jobs nationally, and has economic impact in all states
- NIH supports research into the causes and treatments of rare diseases, which is not done by the biopharmaceutical industry because of high risk and small market value
- The 21st Century Cures Act enacted in 2016 will promote discovery of new disease cures that will benefit many Americans; funding this important law should be a high priority
- Cutting the NIH budget by 18% would cripple medical research in the U.S.A. for several years to come and endanger a new generation of innovative scientists
- China could overtake the U.S.A. by 2019 as the world's leader in medical research; this will occur sooner if federal funding for NIH is cut as proposed by the White House

“Our chances of getting an NIH grant have been improved by the generous support from AHCF these past few years” says Dr. Ess.

“But, if the NIH undergoes a drastic budget cut this year, then we may need to wait,” he notes.

AHC ANSWERS

AHCF Medical Advisory Board answers your AHC questions

The AHC Foundation is incredibly fortunate to have **12 internationally recognized AHC experts** serving on our Medical Advisory Board (MAB) **from four different countries**. They represent leading academic facilities, clinical hospitals, and research institutions.

For years, the MAB has been **answering your questions** with the help of Sharon Ciccocola. Under the direction of MAB Chair, Dr. Matt Sweney, and Vice Chair, Dr. Kenneth Silver, we'll be sharing answers to your AHC questions in our forthcoming newsletters.

To have your question answered, contact sharon@ahckids.org



Question posed to our AHC experts: An AHC mom who is 26 weeks pregnant has asked a question about cord blood. "Does anyone know of any research being done currently on cord blood stem cells to potentially help our loved ones with AHC?" The physicians were asked - would you think it a good idea to preserve them for the future in case there is some groundbreaking research using them? Can you see any possible benefit?

Response: From Dr. Hendrik Rosewich:

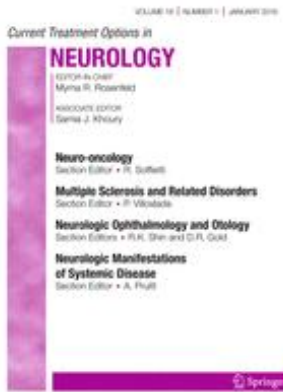
At least to my knowledge, on cord blood stem cells, we are far away from any therapeutic applications. As genetics and medical techniques, in general, develop incredibly fast today; I would not say that it is not worthwhile to collect and store cord blood. My hope is that this technique will help patients with a failure of a protein that is mainly expressed in neurons, at least in my lifetime. Nevertheless, donating cord blood to researchers all over the world with a cord blood biobank might help to speed up techniques and in this way will help indirectly.

Response: From Dr. Kathryn Swoboda:

Agree with Dr. Rosewich. There has been less use than expected of cord blood cells for human disease therapeutics, although recently there has been great interest in collecting cord blood to reinfuse in newborns at risk for hypoxic ischemic encephalopathy when the neonate had history of perinatal distress. However, it is always difficult to say they won't be a jumping off point for future therapeutics development. Nothing in play as of yet or on the horizon that I know of.



SAVE THE DATE
AHC FOUNDATION
2018 FAMILY MEETING
June 22nd – June 24th, 2018
Renaissance Marriott, Woodbridge, NJ



AHC INFO EXCHANGE

Opinion Statement Published in February on AHC Diagnosis and Treatment

In the February 19th issue of *Current Treatment Options in Neurology*, an opinion statement was published titled, “Diagnosis and Treatment of Alternating Hemiplegia of Childhood.” The statement was authored by Melanie Masoud, Lyndsey Prange, Jeffrey Wuchich, Arsen Hunanyan, and Mohamad A. Mikati

The statement opines that the **diagnosis and treatment of patients** with Alternating Hemiplegia of Childhood (AHC) and related disorders should be provided by a multidisciplinary team. The authors are from the **Duke AHC and Related Disorders Multi-Disciplinary Clinic and Program**, as well as CureAHC, and focus on the benefits of their offerings.

Congratulations on getting the word out about AHC and the clinic.

AHC FUNdraising

AHC Doesn't Stop Triathlon Athletes

The first **kid's triathlon** in Kiawah, South Carolina took place on April 15th, at 9 a.m. on the fields of Night Heron Park. **Maddy Munsell participated in the triathlon** as a featured athlete, and dad her dad, Brent, shepherd her through the course. Brent said, “We hope to give Maddy the chance to **participate, raise awareness of AHC, and generate financial support** for Maddy (visit firstgiving.com and search on “mj kids triathlon”).”

Kids aged 3-6 swam 25 yards (swim aids allowed), then rode a .33 mile bike ride, and then completed a .33 mile run. Age group 7-10 did a 75 yard swim, 2 mile bike ride, and .7 mile run. The final category of ages was 11-14 and they swam 125 yards, biked for 3 miles, and finished with a 1.5 mile run.

Congratulations to **the entire Munsell family** for completing an amazing challenge and spreading awareness about AHC at the same time. **Great job!!!**



For all of you folks out east, plan on attending a local screening of the AHC documentary, “Human Timebombs.” The event is hosted by Karen & Matt Shubert and will take place on June 11th.

AHC INFO EXCHANGE

Lessons from the Int'l Task Force on ATP1A3-Related Disorders

On March 2, 2017, an article was published in *Neurology Genetics* titled, “**Research Conference Summary from the 2014 International Task Force on ATP1A3-Related Disorders.**” The recommendations made in the paper were so significant that they will be summarized in several upcoming issues of our newsletter.

Below is a table (#4) of proposed terminology for simplified episode descriptions in AHC.

Primary Episode Name	Episode Description	Episode Subtypes
Hemi-body episode	An event involving one or more limbs on the same side of the body: episodes may or may not include abnormal eye movements or other additional features; if sides switch during an episode but the original affected side recovers, it is considered a continuation of the same episode	1. Hemiplegia/hemiparetic-limbs paralyzed or weak
		2. Dystonic-limbs or body stiff but with involuntary posturing, i.e., stuck into a particular position, head and neck often turned or extended
Full-body episode	Any event in which the 2 sides of the body are affected at the same time; if the right arm and left leg are affected at the same time, it is coded as “full-body”; if transient full-body weakness occurs when hemiparetic episodes switch sides, it would only be coded as full-body if both sides are affected for >5 min	1. Quadriplegia or quadriplegia—all 4 limbs with loss of tone and weakness or paralysis, often associated with loss of speech and altered breathing
		2. Tonic—all 4 limbs in sustained extension
		3. Dystonic—all 4 limbs stiff but twisted into unusual postures, often associated with head turning or head and neck extension. Individual is conscious and may appear to be in significant pain or distress
Seizure-like episode	Any episode in which the patient seems to be unresponsive or unconscious, with or without the following features: repetitive jerking or whole body stiffening or tonic-clonic activity with rhythmic jerking movements, or whole body trembling or twitching movements.	1. Tonic-clonic (repetitive jerking movements involving one or more limbs in focal vs whole body in generalized)
		2. Tonic (stiffening of part of a whole body)
		3. Myoclonic (lightening-like jerking movements)
		4. Complex-partial (confused behavior with inability to follow commands, often in association with more complex behaviors)
		5. Absence-brief episodes of unresponsiveness, often in association with brief cessation of motor activity.
		6. Atonic-sudden loss of whole body tone, resulting in “drop-attacks”

Thank you to everyone who worked on this paper. Your efforts are greatly appreciated

The full article can be found at: <http://ng.neurology.org/content/3/2/e139.short>

AHCF in ACTION

2018 ATP1A3 Symposium in USA

On April 4th, the foundation was notified that our proposal to help organize the 2018 ATP1A3 Symposium was accepted by the ATP1A3 in Disease Organizing Committee. The meeting will primarily be coordinated by the scientific team of Doctors Alison Brashear, Kevin Ess and Alfred George.

Preliminary plans call for the meeting to take place in Chicago, Illinois in coordination with the Child Neurology Society Meeting on October 15 through October 18.

Hosting the meeting in the United States is an incredible honor and the foundation is pleased to collaborate with the international community in preparing another outstanding symposium.



Don't forget to call your **US Representative and Senators** about the 21st Century Cures Act. Here is a link to find your representatives:
www.usa.gov/elected-officials

AHCF in ACTION

Countdown to the 2018 AHCF Family Meeting has begun!

Mark your calendars now and be sure to **join us in New Jersey June 22nd – 24th, 2018** as we come together for the AHCF Family Meeting **at the Renaissance Woodbridge Hotel in Iselin NJ.**



The Marriott Renaissance will be a perfect venue for our meeting with the “Kids” room right next to the conference room, plenty of space to stretch out, free parking and internet. There will be a block of rooms set aside for us at the **discounted rate of \$132 per standard room – \$152 suites** with a discounted breakfast buffet for \$5 for every member of your party.

Take the **free shuttle to local attractions** or to the Metropark Train station to **visit the sites of New York City!**

Look for many more details and announcements each month.

This is your meeting and the Family Meeting Committee would love to hear your suggestions for agenda items, activities, topics, speakers etc...

Please email lynn@ahckids.org or sharon@ahckids.org with your thoughts or comments to make this the best meeting ever!

Start planning now and we'll see you all there!



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