

❖ **INTERNATIONAL FOUNDATION FOR** ❖
ALTERNATING HEMIPLEGIA OF CHILDHOOD

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**Alternating Hemiplegia:
 some new ideas**

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Alternating hemiplegia of childhood (AHC) is a condition whose etiology or cause remains to be defined. Although the condition may not be all that rare, it is diagnosed relatively rarely. There is a real need for collaboration among different centers and polling for patients so that research and treatment approaches can be applied to adequate numbers of patients to allow statistical analysis. Our newly formed group at the Children's Hospital of Michigan is committed to performing in-depth research as well as providing the required clinical services for patients with alternating hemiplegia. We strongly feel that *both* the clinical and research aspects are important and until a cure is found through research, patients with alternating hemiplegia must be carefully monitored and offered the best available treatment. Our major goals are:

- 1) to evaluate new treatment approaches for AHC and improve upon existing treatments
- 2) to investigate the underlying causes of this disorder.

Treatment for AHC

The single most useful agent in the treatment of AHC continues to be flunarazine. Even so, it does not help every patient with AHC and helps some patients only for a finite period of time. Nevertheless, we believe that this drug should be the first to be tried in any

newly diagnosed case of AHC. Since flunarazine is not available in the United States, arrangements should be made by the Foundation to assist parents in obtaining this drug.

For those patients who are only re-

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ceiving partial benefit from flunarazine, additional treatments may be tried. The most common is the use of lorazepam (Ativan) or chloral hydrate at the onset of an attack. Because these drugs result in the child sleeping, they are not ideal. New treatment approaches must therefore be sought. The fact that flunarazine makes a difference in AHC gives us some clues as to what other

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**Making a Difference to
 Those Who Suffer with
 Alternating Hemiplegia
 of Childhood**

Movies like *Lorenzo's Oil* raise our awareness about the pockets of people across the country who have to deal with loved ones affected by rare, incurable diseases.

Research becomes the critical issue.

Loved ones, often parents, enlist the support of others — in an effort to afford the victims of rare diseases a higher quality of life and, ultimately, a cure.

Like the disease in *Lorenzo's Oil*, Alternating Hemiplegia of Childhood ("AHC") is devastating and incurable. It's a progressive catastrophic neurological illness that affects only a very few. This disorder occurs in children and is characterized by repeated, transient attacks of hemiplegia — paralysis of one side of the body, or both. Differential diagnosis includes vascular disorders, such as hemiplegia migraine; cerebral clotting; blood abnormalities; and metabolic disorders, such as muscle disease; encephalopathy; and strokelike episodes syndrome.

Typically, these children have been misdiagnosed with epilepsy and general seizure disorders and have been prescribed drugs, such as phenobarbital, with little or no response from the patient.

Less than 100 children in the U.S. have the disease, including the two-year-old son of one of our Bio Texas members. His son was affected at the age of three months.

The progressive nature of the disease may be delayed by the reduction of the duration and severity of attacks. The

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A Message from the President

Dear Friends,

It is finally happening. The International Foundation for Alternating Hemiplegia of Childhood is a going concern. From our auspicious beginnings in June 1993, we have expanded and moved beyond a "kitchen concern", to an organization that is recognized and registered with the Commonwealth of Massachusetts, and has been given non-profit/tax-free status by the U.S. Internal Revenue Service.

During the early days, the Kiernans along with Kathy and I thought that we were the only ones with AHC kids. With the able assistance of a dear and reliable friend from school days, Joe Stanton, a Certified Public Accountant, we were able to meander through the myriad of forms and papers that we had to file. Joe was always interested and concerned, and when it became time to form our group, he was of indispensable help in taking care of all of the filings, paying for the fees himself, and with no charge for his many hours and days working for the new Foundation.

Not long after this period, we were contacted by two ladies who have become a good part of the backbone of our organization, Laurie Baker and Lynn Egan — from Wisconsin and California respectively. The dedication and efforts that these two people displayed cannot really be justified with words here. But suffice it to say that Laurie and Lynn, together with their husbands, Ray and Mark, played a great roll in keeping the focus and energies from faltering in our burgeoning group.

A brief time went by when I received a call from a couple who live just outside Detroit Michigan, the Georges. Richard and Rhonda were as eager as the rest of us to get things rolling and work towards a cure for AHC. This family, along with a Connecticut family, the Wisyanskis, have generated a genuine interest that has proven itself in donated funds that to date have surpassed the \$50,000 mark. Can we ask for a better

demonstration of dedication and heart than this? Each in their turn can be assured of all of our thanks and support for future endeavors — truly a "Job well done!"

The Foundation was taken a step further at the end of March and early into April, when the Bakers, Egans, and the Georges collaborated to organize a meeting of core participants within the Foundation which was held in the Detroit suburb of Smithfield, Michigan. Other participants included Dennis and Elsie Kiernan, Greg and Donna Cyr, Catherine Mathews, Neal Jackson, Kathy and myself, as well as Dr. Harry Chugani and three of his associates. We had the opportunity to take great advantage of face to face conversation that included comparing notes and asking questions of each others children, as well as sharing concern and support for our families. Dr. Chugani and his colleagues dedicated an entire afternoon to our group's meeting at Children's Hospital in Detroit where he plans to conduct his research into AHC. Near the end of our conference we also had the delight of meeting some of the children by way of video tape, and actually had in our midst little Jimmy Kiernan who accompanied Mom and Dad to Michigan.

I would like to thank all of the families for their attendance at the conference and take great pleasure in welcoming them to their positions within the Foundation. A full degree of gratitude should also be extended to Dr. Chugani who joins us as Medical Advisor to the Foundation. Plans are afoot to include others who have great interest in the AHC area. The promise of these individuals coming on board is sure to have a great impact on the battle that lies ahead, and brings radiant hope to our eventual victory in our cause to eradicate AHC.

Immediate plans have been drawn up and will be executed within a reasonable amount of time, due directly as a result of this recent meeting. These

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Difference

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drug, Flunarazine (trade name Sibelium), manufactured by Janssen Pharmaceutica, has been effective in reducing the duration and severity of the AHC attacks, but has had little effect on the frequency.

Flunarazine was given Orphan Drug status with the FDA in 1986 through the manufacturer of the drug, Janssen Pharmaceutica, and the Janssen Research Foundation in Titusville, New Jersey. In December of 1994, due to budgetary constraints, Janssen terminated its compassionate protocol distribution of Flunarazine to a number of AHC children who were considered a part of the clinical trials.

Currently, the drug is off-patent in the U.S. Due to the lack of marketability and number of AHC patients who would benefit from Flunarazine, Janssen Research Foundation has elected to discontinue its clinical trials on human subjects and, by the end of the year, to terminate the Orphan Drug status currently held with the FDA.

Assistance and Grants

Accordingly, the International Foundation of AHC is seeking the following assistance:

- 1) Sponsor(s) to submit an Investigational New Drug application for clinical trials of Flunarazine leading to FDA approval of the drug for the treatment of AHC.
- 2) Organization(s) capable of funding and conducting research into the causes of AHC, or developing new therapies and/or drugs for the treatment of AHC (with assistance available through the FDA).
- 3) Assistance in raising funds for research in AHC.

The Office of Orphan Products Development ("OPR") of the FDA has a Grants Program to encourage clinical development of products drugs, biologics, medical devices, or medical foods for use in rare diseases or condition.

Currently, only clinical studies qualify for consideration, with a discrete clinical study designed to facilitate FDA approval of the product for a rare disease

or condition. The study may address an unapproved new product (or use for a product) already on the market. Clinical trials in any phase of development are awarded grants of up to \$100,000 of direct costs annually for up to three years. Phase II and III may be awarded grants up to \$200,000 of direct costs per year for up to three years. Additionally, Section 525 of the ODA provides for formal protocol assistance when requested by the sponsor(s) of the drugs for rare diseases or conditions.

For further information about this program, contact the Office of Orphan Products Development (HF-35), FDA, 5600 Fishers Lane, Rockville, MD 20857; or Robert F. Steeves, Assistant to the Director, (301) 443-4903.

Distribution of Flunarazine

In the cases of Flunarazine, it is not necessary for the sponsor to submit certain information with the Investigational New Drug (such as manufacturing and controls information, pharmacology and toxicology data, etc.). Currently, the International Foundation for AHC has been unable to convince the Janssen Research Foundation that it is in its best interest to transfer this data.

Also requested, as an extension of the patent, is assistance in the wholesale importation of Flunarazine for distribution to current patients (barring any problems due to FDA restrictions, regulations and drug import laws). Undue hardship has resulted for the patient and the parents who have been forced to go to England, Germany, Italy and Mexico to obtain the drug — while only being able to import a 90-day supply for their child each time.

(Thanks to Neal Jackson of Houston, TX this article was written by him and printed in the Bio Texas Newsletter, #1 1995. For further information, please contact Mr. Jackson at: 16810 Ashmoor Way, Houston, TX 77058; (713) 280-8028.)

Message

(Continued from page 2)

plans include expanding our medical board, setting up an information resource, a family support and newsletter function, as well as assigning a medical liaison and a compliance liaison.

We are now soliciting membership in the Foundation to strengthen us further. Families with AHC kids are encouraged to join us, and so are medical specialists. The bottom line of our mission, to fund research and treatment — and ultimately cure AHC — has to be met on various levels other than the aforementioned. We must solicit corporate sponsors and donors and do whatever is necessary to get the funding required to fulfill the mission.

For now, let me assure everyone that some sizeable strides are in the works, both for our effort and for that special effort cared on by the devoted physicians who are looking at AHC in earnest. We have a good deal of work cut out for us, but the progress we have made in our young life thus far is very encouraging.

Further progress hinges on alliances and partnerships. These begin with all of us affected by this disorder. And so, on behalf of all of the kids, I ask you to join your time, treasure and talents with ours, so that together we can soon make AHC a cruel memory that we vanquished entirely.

Again, my sincere thanks to all of the hard working members who met in Smithfield, Michigan, and on behalf of all of them, I look forward to working with you all.

With Warm Regards,
Steve Hurley, President

Letters to the Editor

Please send your ideas, suggestions and tips for any topic you would like to discuss i.e. insurance, day care, medicines, alternative methods. I will print them in the next newsletter as space allows. Send to: Lynn Egan, 239 Nevada St., Redwood City 94062; (415) 365-5436

Treasurer's Report

As we close in on the end of our second full year as an official non-profit, tax-except organization, I am pleased to report that the Foundation has begun to receive the donations that can now facilitate our endeavor to provide funding for the research of Alternating Hemiplegia of Childhood.

Our first full year began June 1, 1993 and although we had some discouraging responses to our requests for funding from Corporate and other larger organizations, our second year proved very fruitful indeed. We are most grateful to the efforts of a few families who took the time to raise close to \$60,000 in the past year alone.

Richard and Rhonda George organized a Wine Tasting Fundraiser last year which was attended by many of their friends and family, as well as many business colleagues. Also in attendance were a few AHC families whom by all accounts have described the evening as a lovely fun filled event with food, beverage, and entertainment befitting a grand celebration. The Georges have netted over \$45,000 thus far as a result of their fundraiser and the ongoing commitment of those involved. Because of his proven ability to raise money, Richard has graciously accepted the position of Fundraising Liaison as a means to help others within our membership who would be interested in organizing a fundraiser of their own.

Another AHC family, the Wisyanski's, took another tact which proved very successful as well. Greg did a mailing of a letter he wrote describing his 3 year old son's illness and the Foundation's need for donations. Judging by the response, Greg must have sent it to literally everybody he knows. His plea for help raised over \$8,000. Donations have been coming into the treasury in denominations as small as \$10 all the way up to some very generous contributions of \$1,000. All of this from individuals who know of his son Greg's disease and are sup-

portive of our efforts to find a cure. I must also mention the "Hoop Shoot" fundraiser that the Peckingaugh's of Indiana organized. Being a sports oriented family, they though this would be a fun way to raise some cash for the Foundation. Their efforts produced a total of just over \$1,800 in donations. The Peckinpaugh's have a 12 year old son, R.T. who was diagnosed with AHC about 8 years ago.

In addition to these 3 major fundraising events, we have managed to collect about \$3,000 from other interested individuals. One woman from Wisconsin sends in a check for \$10 every few months. Every little bit helps.

Although Dr. Mikati's appeal to drug companies was not as successful as we had hoped, we did receive a check for \$1,000 from one of them, Ciba-Geigy. Other large companies and non-profit organizations have been contacted i.e.; Newman's Own, the American Legion Child Welfare Foundation, and the Knights of Columbus. We await their reply.

The United Way has been another means of support. Although we are not among their list of member organizations, in some areas we can receive funding as a result of a "write-in" by individuals participating in the United Way's payroll deduction program. An individual simply needs to write in the name of our Foundation (IFAHC) including our address, and we will be contacted for any further information that the regional office may require. Please consider this the next time the United Way Campaign comes around.

It is important to note that most expenses relating to the Fundraising events were not charged back to the Foundation. The Georges donated a substantial amount of their own money to cover some of the costs associated with their Wine Tasting Party. Greg Wisyanski paid for the preparation of his letter as well as the postage involved and the Peckinpaughs absorbed all costs involved in their Hoop Shoot event. On behalf of the Foundation, I would like to thank them all for their significant contributions both in time spent as well as money spent.

Our fiscal year ends May 31, 1995. The Foundation's accountant, Joe Stanton will prepare the financials as well as the necessary filings soon after that date.

Lastly, I am very pleased to announce the newly elected Treasurer, Greg Wisyanski who will take over this office effective with the next fiscal year on June 1, 1995. Greg is an accountant employed by People's Bank. I want to thank him for his enthusiastic participation thus far and am very grateful for the financial expertise that he brings to the Foundation as the new Treasurer.

Katherine Hurley, Treasurer

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Montreal Research

I received the following letter from Dr. Frederick Andermann, M.D.:

Dr. Nicola Di Stefano has recently reviewed the magnetic resonance spectroscopy findings in brain of children with alternating hemiplegia. Previous results published in the *Annals of Neurology* had shown abnormalities in magnetic resonance spectroscopy of muscle suggesting the possibility of mitochondrial disorder. In brain there is a clear difference between the findings in children with benign nocturnal alternating hemiplegia, those with alternating hemiplegia related to specific disorders such as pyruvate dehydrogenase deficiency, and those with classical alternating hemiplegia of childhood.

We would, at the moment, like to carry out magnetic resonance spectroscopy examinations in other children with the classical form of the disorder. This would be done at no cost to the patients and could result in improved clarification for the family and hopefully also improved understanding of the condition which might eventually lead to some avenues of rational treatment.

The procedure is non invasive, but the children would require sedation or in exceptional cases anaesthesia.

If any of the members of your society are interested in pursuing this investigation, please ask them to contact me or Dr. Nicola Di Stefano at this address.

I would like to take this opportunity to wish your association success and remain.

Contact: Dr. Frederick Andermann, MD
or Dr. Nicola Di Stefano
3801, rue University, Montreal,
Quebec, Canada H3A 2B4
(514) 398-6644

MEMBERSHIP CONTRIBUTIONS

AHC is a volunteer organization. Donations are the main source of funding for research and the newsletter. The Foundation would appreciate any donation from parents and \$100 from professional organizations. *Remember, donations are tax-deductible.* Send to: Greg Wisyanski, Treasurer, 409 Buckingham Ave., Milford, CT 06460

Up Coming Conferences

NORD is hosting its Annual Membership Conference at the Sheraton Woodbridge Place, Iselin, N.J. on Sept. 29 - Oct. 1, 1995. The conference welcomes patients, families and support group leaders. For more information contact: Ms. Jean Campbell, 203-746-6518

AND

The Child Neurology Society is hosting its annual conference in Baltimore, Maryland on October 26-28, 1995 at the Hyatt Inn Harbor.

The Foundation is investigating the possibility of renting a booth at this conference. We would need parents to help man the booth. If you are interested in helping out perhaps one or two days, or all three, please contact Karen and Neal Jackson for more information (713) 280-8028

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Address Correction Requested