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Congenital Adrenal hyperplasia Research, Education & Support

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 Summer
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Exercise Study of Patients with Classic CAH

by Deborah P. Merke, M.D.
 National Institute of Child Health and Human Development, NIH

Why Study Exercise in Patients with CAH?

We recently discovered that patients with the most severe form of CAH (classic form) have adrenaline deficiency. Adrenaline is important in exercise and exercise naturally increases adrenaline release. We recently carried out an exercise study in patients with CAH to further explore the effects of adrenaline deficiency in patients with CAH.

Adrenaline and CAH

We recently described that patients with the most severe form of CAH have abnormalities of the adrenal medulla, the central part of the adrenal gland (N Engl J Med 2000; 343:1362-8). Until that time, CAH due to 21-hydroxylase was known to cause abnormalities of the adrenal cortex, the outer part of the adrenal gland and was considered a disease restricted to the adrenal cortex. Cortisol, aldosterone and androgen production all occur in the adrenal cortex. The adrenal medulla secretes epinephrine, also known as adrenaline. In our previous study, patients with CAH had significantly lower adrenaline secretion (measured in 24 hour urine and in blood samples) than healthy controls. We also looked at the adrenal glands of 3 children with CAH who had an adrenalectomy. We found abnormal structure and formation of the adrenal medulla in CAH adrenals. We also found decreased storage of adrenaline hormone in the CAH adrenals compared to normal adrenal glands. Therefore, patients with CAH had decreased production of adrenaline and also had abnormal development and formation of the adrenal medulla. We are currently studying some of the practical implications of this

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**CARES
 CAH Conference**



**Sunday,
 October 24,
 2004**

**Overlook Hospital
 Summit, New Jersey**

See page 8 for details

After 3 years of cramped space and no windows, we've moved into real office space!!

*Come visit us at our new location
 189 Main Street, Millburn, NJ
 (note: our phone numbers remain the same)*



Meryl Stone, Kelly Leight, Laurie Hitzig

A Message from the Executive Director:



Dear Friends,

As spring passes into summer and we enter our third year of existence, I reflect on how wonderful our membership is and how exciting the growth and development of CARES Foundation has been. You, our members, have generously supported us, both financially and with your time. We have now opened our first real offices after being in my basement for 3 years. Our membership numbers have grown and our services have expanded. Our reach and impact is spreading. Newborn screening for CAH has been implemented in 38 states, and we are vigorously working on the balance. I feel a real sense of community amongst our members. I am so grateful for all of the support you have given us and the way the medical community has embraced our efforts. Our future is very bright!

I hope everyone will save the date for our next conference scheduled for Sunday, Oct. 24th, 2004 at Overlook Hospital in Summit, NJ. We have many terrific speakers scheduled and will have many more offerings than in the past. The details of the conference are on page 8.

I hope you have had an opportunity to explore our new web site, www.caresfoundation.org. We have our newsletters archived there (click the NEWS icon), a section on clinical trials and a whole new look. I must give special thanks to Sue Bianchi, volunteer extraordinaire, who has put so many hours into laying out and programming our new site. She deserves our heartfelt thanks and applause for this wonderful, easier to navigate site. I also want to

thank my brother-in-law, Bruce Estes, who designed the new look for the site.

I am delighted to announce that we have 2 new members of the Board of Trustees—Sandra Billings from Houston, TX and Jami Abell Patterson from Los Angeles, CA. Sandra has been an active member of our organization since the beginning and has 2 boys with CAH. Jami, who has a daughter affected by NCAH, wants to focus her efforts on the CAH community in Southern CA and is planning a fundraiser for CARES in spring 2005.

Dr. Mitchell Geffner, a pediatric endocrinologist at Childrens Hospital Los Angeles has agreed to join our Medical and Scientific Advisory Board. He brings great experience to the role in addition to being a really nice guy. We are so honored to have him as an advisor. You can read more about Dr. Geffner's impressive credentials on page 9.

Our State support groups have many fun events planned for this summer, with a picnic in Sacramento, CA and outside Dallas, TX in June and a weekend of fun in coastal CT in July. See page 8 for details.

Please come and visit us in our new offices at 189 Main Street, Millburn, NJ 07041. We would love to give you the tour! Our furniture was all donated by Leisure Life Industries of New York City. They were relocating and gave us their old furniture. We offer our deepest thanks.

Many members called and emailed me about Jennifer Lynn, mother of a child with CAH and one of the leaders of our Indiana support group who suffered a devastating stroke recently which has left her unable to move or speak, and about

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Kelly and Adam Leight

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Executive Director's Message*(Continued from page 2)*

Joseph Hall, a boy with CAH and a brain tumor. Joseph's condition has not changed, but he is doing well and ready for a fun summer. Jenny Lynn's family has set up a website for updates on her condition at www.jennylynn.org. She enjoys cards and pictures if you would like to write to her at 12729 Geist Cove Dr., Indianapolis, IN 46236. Please continue to keep Jenny and Joseph in your thoughts and prayers.

With the busy summer coming, it is a good time to check the expiration date on your bottles of Solu-Cortef. With the heat of summer, please don't keep the Solu-Cortef in the glove compartment of your car or in other high heat areas. Also, remember to keep your children well hydrated with water and electrolyte drinks. Don't forget to bring extra medications (in case of a travel delay or illness) and your Solu-Cortef kit with you when you travel. If you travel by air, you may need a letter from your doctor explaining the need to bring hypodermic needles on the airplane with you. And most of all, have a fun summer!

Warm Regards,

Kelly

Exercise Study*(Continued from page 1)*

very new finding.

Adrenaline is a stress hormone and influences many tissues. Most importantly, adrenaline plays a role in maintaining normal glucose levels, especially in young children. It also has effects on the cardiac system and influences heart rate and blood pressure. Exercise is a natural stimulus of the adrenal medulla. The goal of our exercise study was to evaluate the stress-induced adrenaline response in patients with classic CAH compared to healthy controls, and the effects of adrenaline deficiency on exercise. For this purpose we employed a standardized short-term high-intensity exercise (bicycle) test.

Our Study

Nine otherwise healthy patients with the severe or classic form of CAH (4 females, 5 males, average age 15 years) and nine healthy volunteers matched for gender, age and percent body fat participated in this study. Patients with CAH were receiving hydrocortisone and florninef and were in good clinical control. Physical activity level was derived from information on organized sports and free time activities. For example, subjects were asked how many times per week they engaged in light physical activities, such as golfing or long walks, moderate physical activities, such as hiking or bicycling, and strenuous physical activities, such as running, swimming laps or playing basketball.

Each subject underwent an exercise test to determine fitness level followed by a standardized exercise test one or two days later. The first exercise test, which

measured level of fitness, was approximately 8-10 minutes and was designed to have each subject exercise to exhaustion ("Maximal Exercise Test"). The second or "Standardized Exercise Test" was approximately 20 minutes. This was a standardized high-intensity exercise test and bicycle settings were based on each subject's level of fitness. Therefore, each participant experienced the same relative workout.

All exercise tests were performed using a cycle ergometer (bicycle) and were performed in the morning after an overnight fast (water permitted). About 60 minutes prior to each exercise test, participants drank water. CAH patients received their usual morning dose of hydrocortisone and florninef one hour before each exercise test. The healthy volunteers did not receive any medication. An intravenous catheter (IV) was placed in the arm and was used for drawing blood at predetermined time points before, during and after the exercise tests for measurements various hormones. Subjects wore electrodes for continuous monitoring of their heart. They also wore a fitted nose clip and mouthpiece assembly for measurement of oxygen uptake and carbon dioxide production (see Figure 1). Immediately after the end of each exercise test, subjects were questioned regarding their perceived level of effort and exhaustion.

Our Findings

Overall, the CAH patients were slightly more physically active than the healthy controls. This was most

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Exercise Study

(Continued from page 3)

likely due to the fact that our patients who volunteered for the exercise study tended to be very active in sports. Our patients with CAH who were not physically active, tended not to volunteer for this study. CAH patients had significantly lower adrenaline levels both at baseline (prior to exercise) and at peak exercise (when they were working the hardest) than the healthy controls (see Figure 2). Therefore, when the adrenal medulla is stimulated (by exercise), patients with CAH were not able to mount a normal increase in adrenaline levels.

Our most important finding was in relation to blood glucose levels. The expected normal response to exercise is an increase in blood glucose levels. This occurred in the healthy control subjects (see Figure 3). However, the patients with CAH had a different response. Patients with CAH had blood glucose levels similar to controls before exercise, but they did not experience the normal exercise-induced rise in blood glucose (see Figure 3, page 5). Rather, their blood glucose levels remained about the same during and after exercise. The lack of the normal exercise-induced rise in blood glucose levels was most likely caused by the insufficient adrenaline response. An important role of adrenaline is to increase blood glucose levels during exercise and also during other times of physical stress (such as fevers).

We also measured other hormones which help regulate blood glucose and they did not differ between the 2 groups, with the exception of cortisol. As expected, CAH patients had different cortisol levels than the controls because CAH

patients' cortisol levels are determined by their hydrocortisone dose (their bodies are not making cortisol normally). A normal hormonal response to exercise is a rise in cortisol, but this occurs late and long after the glucose increase. Therefore, cortisol does not appear to play a major role in increasing glucose during short-term exercise. Some people believe that extra hydrocortisone before exercise may help increase blood glucose levels or possibly effect exercise endurance. This is unknown and we are currently studying this. However, our data so far does not support using extra hydrocortisone and suggests that adrenaline has a much more important effect on blood glucose during exercise than cortisol. Unfortunately, there is no known way to replace adrenaline. We do not recommend extra hydrocortisone for exercise. In fact, we discourage this practice because too much hydrocortisone has side effects and can be detrimental to someone's long-term health.

None of our subjects had significantly low blood glucose during exercise or recovery. In general, children are more vulnerable to low glucose levels than adults and their body stores of glucose tend to be lower. CAH patients, particularly children with CAH, might be at increased risk of low glucose during prolonged physical stress. We currently recommend carbohydrate intake prior to prolonged exercise. We also recommend glucose containing fluids (i.e. not just water) during exercise. During illness with fever we also recommend carbohydrates (such as crackers), and glucose containing fluids, in addition to the standard "stress" doses of hydrocortisone.

Patients with CAH often love to exercise and are very active in sports. However, complaints of lack of endurance, tiring more readily than others, or just getting "wiped out" are not uncommon. It is unknown if this is due to a lack of adrenaline and/or lack of an increase in glucose. In our study, the ability to sustain exercise, the level of perceived effort, and exercise abilities were similar in both groups. Despite clearly demonstrated adrenaline deficiency, the CAH patients had normal short-term high-intensity exercise ability and tolerance, and none of our patients experienced low glucose levels. However, it remains to be determined whether adrenaline deficiency decreases endurance and ability to exercise for longer duration. This is unknown and is an area we are currently studying.

We'd like to thank the patients with CAH and the healthy controls who participated in this study!



Figure 1

This is what study subjects wore during their exercise test!

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Exercise Study
(Continued from page 4)

Adrenaline

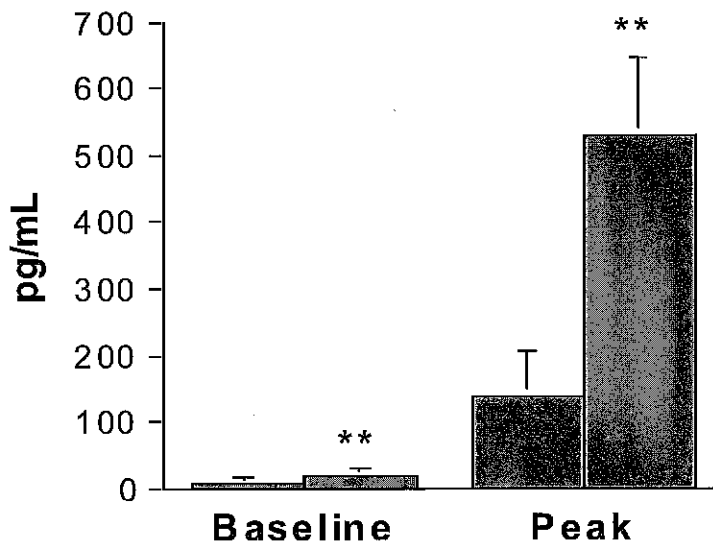


Figure 2

Adrenaline levels before exercise (baseline) and when patients were working the hardest (peak) in patients with CAH (■) and healthy controls (□). Adrenaline levels were significantly lower in patients with CAH at both time points.

** denotes statistical significance (P<0.01) between the 2 groups

from Weise M, et al. *J Clin Endocrinol Metab.* 2004; 89:591-7; with permission

Maximal Exercise Test

Standardized Exercise Test

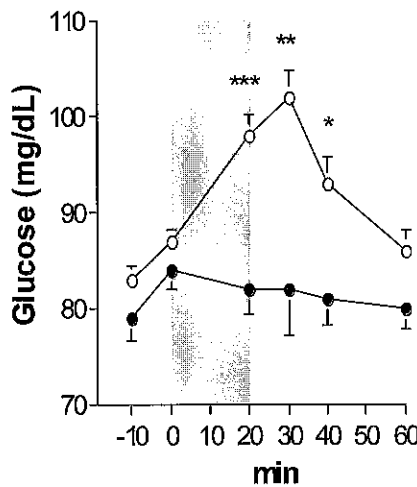
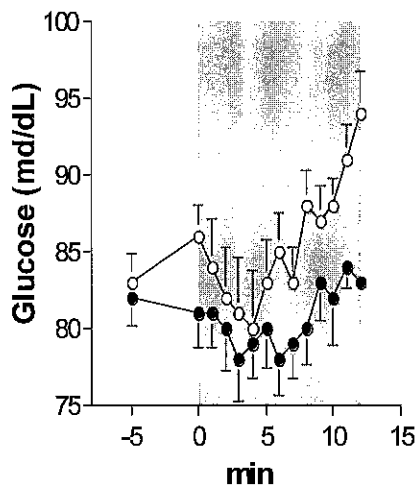


Figure 3

In response to both the Maximal and Standardized Exercise Tests, patients with CAH (●) did not have the normal and expected increase in blood glucose that was observed in the control subjects (○). Grey area indicates time period of exercise.

from Weise M, et al. *J Clin Endocrinol Metab.* 2004; 89:591-7; with permission

CF

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This newsletter is published 3 times a year.

Haitian Adoptees

by Pamela Knight Kretsge

Libby entered the world in 2000 with three strikes against her: She was born in rural Haiti, the poorest country in the Western Hemisphere. She was born with congenital adrenal hyperplasia in a society without the tools to diagnosis or treat the disease. And she was born into a culture that recoiled from the ambiguous genitalia symptomatic of her illness.

Even a technologically advanced society cannot guarantee proper diagnosis and treatment of CAH. In Haiti, this situation is made worse by poverty. And if, by luck and parental care, a child with CAH survives the medical challenges, there are the social issues:

Voodoo, the version of ancestral African worship recently recognized as an official religion in Haiti, regards the ambiguous genitalia that can accompany classical CAH as an evil omen.

Mark Fulton, D.D.S., founder of the Midwestern U.S. chapter of Mission Haiti, a medical relief organization, explains: "In Haiti, when kids have ambiguous genitalia, it's seen as evil. Such 'undesirable' children are treated differently. They may receive less food than other family members, or be left to succumb to adrenal crisis."

This is the story of two little girls who might well have suffered that fate, if not for the determination and bravery of a group of Mission Haiti volunteers from Anderson, Indiana. Members of this faith-based, ecumenical group based at the North

Anderson Church of God have carried out regular medical missions to Saint Ard, Haiti for over a decade. One member, Debbie Alford, has been making six trips a year as part of this outreach program. "Our team stays in the country for a week at a time, traveling to two different villages



Jill & Libby Rogers

each day," explains Alford. "We open up a clinic, and see anybody that comes through--a few hundred people or more a day. It's a pretty intense week."

Alford also travels to and from Haiti to accompany children who, due to extreme medical need, qualify for a medical visa to come to the U.S. for treatment. She has come to know several Haitian families with children that have CAH. According to Alford, Haitian communities may pressure parents of such children to bring the child to a voodoo priest (a male *houngan* or a female *mambo*) or to a *bokor* (a shaman or sorcerer loosely linked to

voodoo practice)...whether for treatment or something more sinister is not clear. While official sources deny that human sacrifice is part of Haitian voodoo rituals, others suspect that it does occur. In any case, the prospect of bringing a child before a *houngan* often fills their families with fear.

To obtain a medical visa to bring a critically ill child out of Haiti to the U.S., all treatment in the U.S. must be donated. Mark Fulton found a willing donor in Martin Kaefer, M.D. of Riley Hospital in Indianapolis. A pediatric urologic surgeon, Dr. Kaefer has performed reconstructive surgery on several Haitian children free of charge. The doctors, nurses, anesthesiologists, and the hospital all donate their services, as well. "It's absolutely amazing," says Alford. "And they're anxious to do more."

Libby was one of the lucky ones, although it hardly seemed that way when her parents brought her to the clinic at Saint Ard. She was nine months old and weighed nine pounds. "The clinic doctors didn't know what was wrong with her. They just knew she had both male and female genitalia and was extremely malnourished," says Alford.

Back in the States, while trying to arrange a medical visa for Libby, Alford received a chilling phone call from Haiti. "Come get her now or she's not going to be alive," she was told by a nurse at the Saint Ard orphanage where Libby was staying. Alford quickly completed the paperwork for Libby and went back

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to Saint Ard to pick her up. "We told her parents she might die in the U.S. because she was so sick, and they were OK with that because at least she would die comfortably," says Alford.

After determining that Libby had classical CAH and getting her medically stabilized, Dr. Kaefer performed surgery on her in March of 2001. Fortunately for Libby, the fact that she would need lifelong medication not available in Haiti meant she was adoptable in the U.S. After being loved and cared for by the Alford family as a foster child, she was adopted by Jill and Jim Rogers, who have attended CARES meetings to learn more about her new daughter's needs. "CARES has helped to bring us to a level of understanding and acceptance of Libby's CAH and given a sense of peace that there are others involved in fighting for the lives of the children here and abroad."

Today, at almost four years of age, Libby has gained a lot of ground. Although her untreated CAH caused adrenal crisis and seizures that left her with permanent brain damage, she is learning to walk and to communicate with word and sign. She is loved dearly by both the Alford and the Rogers. "Libby's birth parents made a great personal sacrifice in giving up their daughter to live in the States," says Jill Rogers. "By doing so, they gave her the gift of life again."

Another Haitian girl with CAH presented a different picture when first seen in the Saint Ard clinic. "Marie was quite a surprise," remembers Alford. "Her mom

brought her to us because she had male and female genitalia, but she was a happy, very healthy, ten-month-old." Marie's parents had fled to a remote mountainous area to get away from some of the voodoo believers who found out she had ambiguous genitalia and felt compelled to involve her in a ritual to drive out "demons".

Dr. Kaefer agreed to perform urogenital surgery, but because the baby was healthy did not suspect CAH. "By the time we got the medical visa," says Alford, "Marie was 11 months old and by then she



The Rogers Family

had started getting sick, vomiting and losing weight. When we got her up here and tested her, it turned out she had classical CAH. She was started on medication and she is doing extremely well." Marie is in the process of being adopted by another member of the Indiana group.

The fate of Mission Haiti and Haitian children with CAH and other disorders is in limbo due to the current political strife. Alford's most recent trip, in February 2004, coincided with the beginning of the riots that ousted Jean-Bertrand Aristide. "We were there to bring another little one up, and got caught locked out of the U.S. Embassy for two hours while they were shooting," she recounts. While travel to Haiti by this and other humanitarian aid groups has become more hazardous than ever, Alford reports she made another trip to Haiti in late April of this year: "I just returned from Haiti with a 2-year old boy for surgery. Praise God--everything went smoothly! Dr. Kaefer will be operating on him May 21. May God bless...all those whose lives have been touched by our CAH children. Libby is the love of our life!"

CF

Physician Listings Available from CARES

CARES Foundation has compiled a large list of pediatric endocrinologists, some adult endocrinologists, urologists and psychologists with experience in treating CAH/NCAH patients. Please contact CARES Foundation for more information.

**CARES
CAH Conference
Sunday, October 24
9:00 am – 5:30 pm
Summit, NJ**

Please join us for our next exciting conference at Overlook Hospital in Summit, New Jersey. Our plans are underway and the speakers we have scheduled to date include:

Susan Baker, Ph.D.
Sheri Berenbaum, Ph.D.
Deborah Merke, M.D.
Maria New, M.D.
Dix Poppas, M.D.
David Sandberg, Ph.D.
Phyllis Speiser, M.D.

Topics include:

- ✓ New Advancements in CAH Treatment and Future Trends,
- ✓ Behavioral and Psychological Aspects of CAH
- ✓ General Treatment of CAH/NCAH
- ✓ Prenatal Therapy
- ✓ Surgical Reconstruction in CAH
- ✓ NCAH/CAH Adult Experience
- ✓ Transition to Adult Care & Adult Healthcare Issues
- ✓ Impact of CAH on Family & Siblings
- ✓ And much more...

Plan ahead and make sure to secure your hotel reservations. We have reserved rooms in two hotels for your convenience. We will provide transportation to and from the hotels to Overlook Hospital.

Holiday Inn (Springfield, NJ) – Single/Double occupancy per night – \$99.00 plus tax. Rate includes a hot and cold breakfast buffet. Call 973-376-9400 and request the CFG-CARES Foundation Group Room Block. This room rate applies from Friday night through Sunday night.

Hilton (Short Hills, NJ) – Single/Double occupancy per night – \$125 plus tax. Call 973-379-0100 and request the CARES Foundation Conference Block. This room rate applies for Saturday night.

For air travelers, the nearest airport is Newark – approximately 15 minutes from the hotels/Overlook Hospital. From NYC take New Jersey Transit from Penn Station to Summit.

We look forward to seeing you there!!

Note: There is NO CHARGE for the conference.

**CARES Family
Support Groups**

ALABAMA

Contact Tonya Judson
(205) 991-8674
tjudson@charter.net

ARIZONA

Contact Michelle May
480-759-0870
michlmay@aol.com

NORTHERN CALIFORNIA

Contact Adria Stoner
(916) 434-8405
stonerfamily@starstream.net

Tammy Hupp

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Tammypoo@pacbell.net

SOUTHERN CALIFORNIA

Contact Jennifer Cribbs
(714) 968-6794
jcribbs@starbucks.com

Jami Abell Patterson

(818) 906-8668
Jami@caresfoundation.org

CONNECTICUT/NEW ENGLAND

Contact Lynn Torony
(203) 264-6898
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**NE/CT Family Gathering
July 24 – 25**

INDIANA

Contact Penny Barrett
(317) 865-9320
onecent721@yahoo.com

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MISSISSIPPI

Contact Susan Aycock
601-833-8373
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TEXAS

Contact Lesly Stevens
817-563-2570
mwlastevens@prodigy.net

Sandra Billings
281-861-6043
billpropl@msn.com

WISCONSIN

Contact Lisa Jaskie
(414) 645-0782
lisa1273@msn.com

Are you interested in starting a support group in your state? Contact the CARES office and we will get you started. **CF**

Change of Work Address for

Dr. Maria I. New

as of

June 1, 2004:

Maria I. New, M.D.
1 Gustave L. Levy Place
Box 1198
New York, NY 10029

Patient inquiries: (212) 241-8210
Fax: (212) 876-4395

Professional website:
www.marianew.com

Maria I. New Children's
Hormone Foundation website:
www.newchf.org

Mitchell E. Geffner, M.D. Joins the CARES Foundation Medical and Scientific Advisory Board

Dr. Geffner is currently Professor of Pediatrics and Director of Fellowship Training in the Division of Endocrinology, Diabetes, and Metabolism at Childrens Hospital Los Angeles, Keck School of Medicine, University of Southern California, and formerly the Chief of the Division of Pediatric Endocrinology at the Mattel Children's Hospital at UCLA.

Dr. Geffner graduated from Queens College of the City University of New York in 1972 and from the Albert Einstein College of Medicine in 1975. He was an intern, resident, and chief resident at the Los Angeles County-University of Southern California Medical Center between 1975-1979. He completed a three-year fellowship in Pediatric Endocrinology at UCLA between 1979-1982 and he remained at UCLA through December 31, 2001, having risen up the academic ladder to the rank of Professor in 1996.

Under the mentorship of Drs. Solomon Kaplan, Barbara Lippe, and David Golde, Dr. Geffner developed a longstanding basic research interest in the general area of hormone resistance. More specifically, he has studied the mechanisms responsible for resistance to ACTH (in the triple A syndrome), thyroid hormones, growth hormone (Laron dwarfism), and combined GH and IGF-I resistance (African Efe Pygmies and HIV-1-infected children with growth failure). To date, Dr. Geffner has authored 93 peer-reviewed publications. On a clinical level, his

research interests have involved studies of new formulations, delivery systems, indications for GH therapy, and optimization of treatment for children with Type 2 diabetes mellitus.

Dr. Geffner has had a career-long interest in medical and patient education. He currently serves as the Chairman of the Program Directors' Committee of the Lawson Wilkins Pediatric Endocrine Society, Medical Advisor to the Turner Syndrome Society of Southern California, Medical Advisor on childhood panhypopituitarism for the MAGIC Foundation and the Pituitary Network, and as a member of the National Steering Committee for the NIH-sponsored TODAY (Treatment Of type 2 Diabetes in Adolescents and Youth) trial. Lastly, Dr. Geffner is co-editor of the next edition of the Wilkins' Textbook of Pediatric Endocrinology.

Dr. Geffner will soon be initiating two clinical research studies related to adrenal function: (1) to address the issue of whether stress glucocorticoid coverage is required in children with non-classical adrenal hyperplasia and (2) to determine the duration of adrenal suppression resulting from intralesional long-acting glucocorticoid injections in the treatment of orbital hemangiomas and, in these children, whether temporary daily glucocorticoid replacement is required. **CF**

CLINICAL TRIALS

VOLUNTEERS FOR RESEARCH STUDY NEEDED! WHIMS

Women's Hormones and Impact on Mood Study

Gail Schoen Lemaire, Ph.D, APRN/PMH, BC, Assistant Professor, University of Maryland School of Nursing, Department of Family and Community Health, Baltimore, Maryland.

If you are between the ages of 18 and 40 and are premenopausal, you may qualify for this study!

Little research has been conducted on the effects of excess androgens (such as testosterone) on women's mood. Dr. Gail Schoen Lemaire is a member of CARES, and a nurse and researcher at the University of Maryland School of Nursing in Baltimore. Dr. Lemaire is currently recruiting volunteers for a research study on androgen excess symptoms and mood. The "Women's Hormones and Impact on Mood Study" or WHIMS, will study how symptoms of androgen excess influence women's mood and well-being and how they affect women's lives.

In order to take part in the study, participants must be premenopausal, between the ages of 18 and 40, and have been diagnosed with NC-CAH. Women who are currently pregnant or lactating, or who have had a hysterectomy (removal of the uterus) or endometrial ablation (destruction of the lining of the uterus), bilateral oophorectomy (removal of both ovaries) or adrenalectomy (removal of the adrenal gland) are not eligible for WHIMS.

Study participation involves completing a one-time, written survey. The survey includes questions about general health, androgen excess symptoms, and emotions. It takes about 30 minutes to complete the survey.

Data for this study is being collected anonymously. Participants will **not** include their name on the survey. Whether or not you decide to take part in the study will not affect your current or future membership in CARES.

For more information or to request a survey, please email Dr. Lemaire at the WHIMS Study Center at lemaire@son.umaryland.edu or call her at 410-706-4914. Once we receive your mailing address, we staff will send you the WHIMS survey, along with a self-addressed, stamped envelope.

The results of this study will provide important information that will help health care providers and scientists better understand and treat androgen excess conditions in women.

CF

PRENATAL DIAGNOSIS RESEARCH PROJECT TO TARGET WHICH WOMEN SHOULD TAKE DEXAMETHASONE DURING PREGNANCY

We are seeking the help of couples at risk for having an affected child with congenital adrenal hyperplasia to participate in a research project in Boston. As you know, some pregnant women are offered the opportunity to take dexamethasone early in pregnancy to reduce the risk of masculinization of a female fetus affected with congenital adrenal hyperplasia (CAH). The problem is that only 1 in 8 fetuses will be female AND affected, so 7 out of 8 possible fetuses will receive unnecessary treatment. Steroids, while effective, do cause side effects in pregnant women and some children. Our research aims to target dexamethasone treatment to only female fetuses at high risk of having CAH.

The purpose of this research study is to develop a simple prenatal test that will use blood samples from a pregnant woman and her partner. The pregnant woman's blood will be used to determine if the fetus is male or female using cell-free fetal DNA testing. This can be done as early as 7 weeks following the first day of the last menstrual period. If both parents have different mutations, the partner's blood will be used to test for the presence of his mutation in the

(Continued on page 11)

CAH Study at UNC

Chapel Hill, North Carolina
by Karen Jane Loechner, M.D./Ph.D.

Prenatal Diagnosis Project (Continued from page 10)

pregnant woman's blood, which was inherited by the fetus.

Please note that this is a research study. In the first phase of the study, we cannot release results of fetal gender testing to you. Thus, the results will not affect your clinical care. However, if we get enough patients enrolled, and our study is accurate, our hope is that in the near future this will transition to early fetal gender diagnosis that can be used clinically.

The study involves 1-2 blood samples from the pregnant woman and 1 sample from her partner. There is no travel required. We will arrange to have the blood drawn in your hometown.

To find out more information about participating in the study, please contact Barbara O'Brien, M.D. (bobrien@tufts-nemc.org) or Diana Bianchi, M.D. (dbianchi@tufts-nemc.org) at Tufts-New England Medical Center.

CF

We are recruiting children with CAH who are 6-12 years old (bone age <14 years), are still growing, and have not yet started puberty. Children will be enrolled in the study at the General Clinical Research Center at the University of North Carolina, Chapel Hill.

Although cortisol replacement (hydrocortisone, prednisone, or dexamethasone, for example) and florinef have virtually eliminated mortality, there are at least two reasons for continuing to try to perfect our treatment regimens: (1) the linear growth of children is suboptimal and the end result is an adult who is too short, and (2) over-treatment with glucocorticoids in children may increase risk of osteoporosis in later years.

Based on studies of the regulation of release of ACTH (that, in turn, drives the production of hormones from the adrenal cortex), we have found that we can decrease the amount of ACTH using calcium channel blockers (medications typically used to treat high blood

pressure, such as amlodipine). Amlodipine has been shown to be safe even in infants treated for a variety of medical reasons.

Our hypothesis is that the addition of amlodipine will allow us to decrease the amount of glucocorticoid medication that your child is currently taking to control his/her CAH. Such a decrease should translate into better growth and bone strength. This new medication would be added to your child's current CAH treatment program and evaluated in a double-blind/placebo-controlled crossover study.

For more information, please contact Dr. Karen Loechner at (919) 216-5946 (pager) or (919) 966-4435 ext. 224 (voice mail); fax (919) 966-2423; Roxanne Schock, CDE, Clinical Coordinator at (919) 966-0428 (voice mail). All visits, including laboratory testing, research medication, and parking will be paid for by this protocol. Limited "off-site" testing at local Pediatric Endocrinologist may be available subject to IRB approval.

CF

Participating in CLINICAL TRIALS

Reprinted with permission from the National Organization of Rare Diseases Newsletter

A study from the Rainbow Babies and Children's Hospital in Cleveland, Ohio reveals that parents of children who participate in clinical trials generally do not understand the concept of experimental treatments. The study of 137 parents of children with leukemia was published in *JAMA*.

When children were randomly assigned to an experimental treatment or the standard treatment, even though this was explained to them 50 percent of the parents did not fully understand the concept. Nor did they understand that the effectiveness of experimental treatments was unproven, nor that a

computer and not a doctor would make the decision about which treatment the child would get. Parents are supposed to give their informed consent before a child participates in research, but apparently many parents continue to expect that their child will benefit from the experimental treatment.

CF

What to Expect from CLINICAL TRIALS

When a person considers participating in a clinical trial, there are several things he or she should be aware of, in order to have realistic expectations and adequate knowledge of possible risks and benefits:

- What is the purpose of the trial? Is the intent to find a cure, or simply a treatment, or perhaps preventive measures or a diagnostic test? Don't sign up for a clinical trial unless your expectations are in line with the study's objective.
- Is an experimental clinical trial appropriate for you? Have you tried the standard treatments and failed to benefit from them? Are you willing to try a treatment for which little is known, rather than try a treatment that has risks and possible benefits that are well understood?
- What are the known risks and benefits of the experimental treatment? How many people have been in the experiment before you? Did they experience any harm?
- Is the experimental product only slightly different from other available treatments (e.g., a slightly revised version of an existing drug), or is it a completely new approach to treatment?
- Who is paying for the study? Is it supported by government funds or a commercial firm? Does your doctor have a commercial interest in the therapy (e.g., a patent, royalties, etc.)? Note: Some universities have "Conflict of Interest" committees who review these factors and decide whether a commercial conflict may

(Continued on page 15)

Reconstructive Surgery Act of 2003

by Congressman Mike Ross (D-AR)

For the last two years, I have introduced the *Reconstructive Surgery Act*. This year the bill number is H.R. 1499. The initiative would require health insurance plans to cover medically necessary corrective reconstructive surgery for congenital defects, developmental abnormalities, infection, trauma or disease, with no age limit. Examples of conditions for which this bill would apply are cleft lips and palates, skull deformities, burns, unformed ears, and missing pectoral muscles that cause chest deformities.

I introduced this measure after my constituent, Wendelyn Osborne, contacted me with the problem of getting her insurance company to cover her medically necessary surgical procedure. As an infant, Ms. Osborne was diagnosed with a rare congenital bone disease, Craniometaphyseal Dysplasia (CMD), which was depicted in the 1985 movie *Mask* starring Cher. CMD involves an overgrowth of craniofacial bone that never deteriorates. At the time of her diagnosis, Ms. Osborne was the sixteenth CMD case in medical history. Doctors told her parents that she would not live past the age of ten. With many surgeries, starting at the age of six, she is now in her thirties. The severity of Ms. Osborne's craniofacial anomalies precludes adequate treatment through a single procedure and requires continuous surgical procedures for the rest of her life.

This issue is important because it is about doing what is right for Ms. Osborne and people like her, regardless of their age. These patients did not ask to be born with a congenital disease or experience a traumatic, disfiguring event. There are too many families and patients with stories about trying to convince their insurers that surgeries to construct an ear or repair a cleft palate are not cosmetic but are in fact medically necessary.

The National Organization for Rare Disorders, the Children's Craniofacial Association, the American Society of Plastic Surgeons, Easter Seals, the March of Dimes, and the National Foundation for Facial Reconstruction endorse the *Reconstructive Surgery Act of 2003 (H.R. 1499)*. Please visit the public legislative information site, <http://thomas.loc.gov>, and contact my healthcare legislative assistant, Sylvia C. Brown, for more information about the measure.

CF

Congressman Mike Ross is serving his second term as representative of Arkansas's Fourth Congressional District. He can be contacted at: 314 Cannon House Office Bldg., Washington, DC 20003, Phone: 202-225-3772, Fax: 202-225-1314.

We are Vietnam's only parent and patient support group and We Need Help!

by Miss Lai Thi Quynh Giang, President, CAH Club

Our Vietnamese Congenital Adrenal Hyperplasia Club is the only parent and patient support group in this country. It was founded 5 years ago thanks to the great efforts of the Endocrinology Department at the National Hospital of Pediatrics (NHP) in cooperation with Assoc. Prof. Garry Warne. It was supported by a generous donation from Dot and Bryan Gronn - the Australian parents of two children with CAH - who attended the inaugural meeting in 1998. Mrs Dot Gronn was, at that time, the President of the Australian CAH Support Group. After 5 years of operation, the Club's financial resources are running out and we are in dire need of new donations. To assure you, potential sponsors, about the purposes of this charity appeal, this report will provide you with an overview of the Vietnamese CAH club and its vision for the short and long term.

The unceasing and vigorous efforts of medical staff at NHP and the Club itself have brought about several encouraging achievements:

- Improved knowledge about CAH amongst patients and their parents. They understand that CAH is a life long disease and that medicine is an essential part of their life, without which they would end up in misery, especially the female patients.

- Improved adherence to medication schedules and attendance for regular checkups at NHP.

- We have demonstrated our ability to use the money donated by Mr and Mrs Gronn effectively to pay

for medical examinations, subsidized medications, lunch, travel assistance and informative literature.

A problem we face is that many families are very poor (monthly income is at farmer level of \$25 to \$50 USD), but on average, each CAH patient whose dosage is 2 tablets a day has to spend \$15 a month on 3 packets of 25 tablets/10mg. In the countries where hydrocortisone is readily available, namely Australia or the USA, CAH patients only spend one-sixth as much money!

Another problem is that the number of patients being managed through the Endocrine Clinic at NHP has increased from 50 in 1998 to nearly 200 in 2003. This situation demands greater finance and human resource to make it workable. The Club's Chairmen have little experience in fundraising activity. CAH is a difficult condition to fund-raise for, because it involves sexual ambiguity, and is therefore an extremely sensitive and embarrassing condition in Vietnamese culture. This makes CAH patients reluctant to participate in annual meetings and Club activities, particularly when they are grown up.

More than 80 per cent of patients come from rural areas, some of which are remote and mountainous, where the efficiency of diagnosis and the public knowledge of CAH is limited, resulting in late diagnosis and the masculinization (of girls) that is too



strong to be reversed by operation and medicine. Second, the geographic distance causes great difficulties to the members to keep in touch with each other and with the Chairmen.

The most urgent and headache problem facing CAH patients in Vietnam now is how to find a cheap and continuous source of medicine. Hydrocortisone and Florinef have not been imported legally and officially, so prices are staggering and scarcity is usual. According to NHP statistics, 67.3% of CAH patients are under 3 months old, showing that diagnosis has improved, with the promise of a new generation of children who less severely affected by CAH. However, the high cost and scarcity of the best medicines would possibly force these infants' parents to resort to cheap, available alternatives like Prednisolone or Dexamethasone, which would have many long-term negative effects on the infant's physical development. Therefore, we, the Chairmen of the Vietnamese CAH Club, strongly appeal for charity from any organization in the world to relieve the suffering and economic difficulty of CAH patients and their families.

(Continued on page 14)

Vietnamese Club Needs Help

(Continued from page 13)

Taking all the aforementioned into consideration, we have set up a new action program to increase the efficiency and effectiveness of the club's activities and management. Since CAH is sensitive and embarrassing to many patients and their families, the strategic solution is to offer the members more benefits and conveniences so that they can realize the necessity of participation and involvement.

A. Improvement in quality of annual meeting:

1. *Accommodation.*

The vast majority of CAH patients live far from Hanoi so the Endocrine Department of NHP has had to provide accommodation for them the night before the meeting. However, with the aim to diversifying the activities, the chairmen would take the responsibility for holding a warm party at that night to motivate them and conduct a friendly and open discussion about life, medical condition, psychology, sufferings of CAH patients in order that they can share experience and receive advices from doctors.

2. *Diversify the activities in the formal meeting.*

Increase the period of feedbacks between doctors and patients, rather than just have one-way lectures. The focus is placed on the knowledge of parents and patients about CAH and consultation about psychological and behavioural abnormalities. In other words, members would play a more active role and participate more fully. As well, we can hold some contest or games between families to check their understanding of CAH and then

we will have awards for those who are best.

Awards would be presented to CAH patients who achieve good academic results, develop successful and appreciated social activities or who make a positive contribution to the CAH club.

If we had \$1,000 USD per year for the annual meeting, we would develop a social program so that parents and patients could get together and relax. We would hold trips or picnics around Hanoi. This kind of activity would have great positive influence on strengthening the relationship between the Club and its members.

If we would receive donated medicines, we would make an equal distribution at the annual meeting for each patient.

Overall, if CAH patients can enjoy the benefits of free or discounted medicines, the outdoor activities and active participation, we, the Chairmen, strongly believe that our club would be well supported and could develop.

CF

Footnote: Royal Children's Hospital International (Melbourne, Australia) will be offering financial and logistical support to this worthy group in 2004 but cannot provide everything that the group needs. We warmly encourage other friends to be co-sponsors. A small amount goes a long way here! Without help, the group could collapse. If you wish to offer a donation, contact me by email (garry.warne@rch.org.au).

Garry Warne, Director, RCH International & Senior Endocrinologist, RCH, Melbourne

Ask The Experts

Reprinted with permission from April
NORD on-line

NORD (National Organization of Rare Diseases) asks people with expertise on particular topics to answer questions related to living with a disorder or disability. For this issue, we asked NORD's registered nurse, Patti Kane-Carlsen, RN, MSN, to write about sources of help for people who are uninsured or underinsured since she receives many questions on that topic. Patti is in NORD's office three mornings a week. People who have questions related to caregiving or accessing services may contact her at RN@rarediseases.org or (203) 744-0100.

Q NORD has a Medication Assistance Program, but it doesn't cover my medication. What can I do? Also, I need medical insurance coverage... What about disability services?

A Since 1987, NORD has administered medication assistance programs to assist uninsured or under-insured individuals in securing life-saving or life-sustaining medications. NORD works closely with humanitarian-minded pharmaceutical and biotechnology companies to ensure that certain vital medications are available to those individuals whose income is too high to qualify for Medicaid but too low to pay for their prescribed medications. If your medication is not covered here, check directly with the manufacturer – they might be running their own program. Check with your pharmacist for the manufacturer name if you do not know it. Look for them on the Internet to see if they have a program. Of course, there are

(Continued on page 15)

Ask the Experts*(Continued from page 14)*

eligibility requirements that you would need to meet.

There are other prescription coverage programs available – you will have to do some research to find the one that works for you. Medicare has an excellent website that has a page devoted to “Prescription Drug and Other Assistance Programs”. The programs they cover are not necessarily for senior citizens only. Visit their website at <http://www.medicare.gov/default.asp>. If you do not have a computer, go to your local library and ask for help.

For medical insurance, you can start with your local social services department (listed in the government section of your phone book). You can also find your social services department on the Internet – use ‘your state name’ social services department as your keyword. You might also try your state insurance commissioner. To find yours, visit <http://www.aiadc.org/LinksResources/StateInsuranceCommissioners.asp>. You could also do a search using ‘your state name’ state insurance commissioner as your keyword. Look at the section for consumer services.

There is a federal program available for uninsured children. For information about the State Children's Health Insurance Program (SCHIP), visit <http://www.cms.hhs.gov/schip/> Adults might need to look into Medicare and/or Medicaid. For information about Medicare & Medicaid Services, visit <http://www.cms.hhs.gov/default.asp>.

What is available for children with disabilities? Check out <http://www.ssa.gov/pubs/10026.html> for a booklet entitled “Benefits for Children with Disabilities”. For information about Planning for your Disabled Child, visit http://www3.madd.org/nefe/home_sect/mh_planning.htm.

Children and/or adults with disabilities may be entitled to disability insurance. This is handled by the Social Security Administration. For information about SSI (Supplemental Security Income), including eligibility requirements and more, visit <http://www.ssa.gov/disability/>. A website that answers Frequently Asked Questions about Social Security Disability (which might help you through the process) is <http://www.nosscr.org/faqind.html>. Another excellent resource for programs for the disabled is <http://www.disabilityinfo.gov/>.

If you have no computer access, skills or desire to use one, you can find most of these services listed in the government section of the phone book. Call and ask for directions. If you are using a computer, you can also get more information by doing a search using the keywords most useful to you. Try sticking with the .gov and .org websites for this research.

Remember, all programs will have eligibility requirements. Find out what you can in advance, and then discuss options with a program administrator. Do not assume you are not eligible for a program based only on your research – call and ASK! Work to find the program that best fits your needs and eligibility.

Researching prescription coverage, medical insurance and

disability services can be a tedious process. Keep records of your work including dates of correspondence, information gathered, and the full name of the person who helped you (if phone contact). If you are researching on the Internet, bookmark the websites you visit for future reference. If you are using a computer at the library, print the information you need – make sure you note the web address on the printout. Make copies of any applications filed – you might need to refer to them later.

It might be frustrating at times, but stick with the process – it may well pay off in the end!

CF

What to Expect from Clinical Trials*(Continued from page 12)*

influence the outcome of a study.

- Is this a “placebo controlled” trial, or will the experimental treatment be compared to the standard treatment for your disease? Is there a chance that you will receive the placebo and if so, how much risk does this represent?
- If you are harmed by the experimental treatment, who will pay for your medical care?
- What will happen when the study is finished? Will you be able to continue using the treatment, or will it be stopped even though you feel you are benefiting from the product? Will the manufacturer promise you can have continued access?
- While the vast majority of experimental treatments are free, will you or your insurance be liable for any costs related to your medical care while you are participating in the clinical trial?

CF

Fundrai\$ing Corner

Texas Bowlathon

The CARES Bowlathon in Houston, Texas was a huge success. We all had a great time being together and bowling for a great cause!!! Even our little bowlers enjoyed themselves! It was nice to see the CAH families that came. I enjoyed catching up with them.



So far we have collected \$2,866.61 in donations!!! I wanted to thank **Krogers** for donating a \$50.00 gift card prize and yummy cookies for our bowlers. I also wanted to thank **Carrabas Restaurant** for donating dinner for two as another one of our Bowlathon prizes. Most of all, I wanted to thank all of our friends and family and friends of our friends who were so kind to give of their time, energy and money. I hope we can do it again next year!!!!

CF

Sandra Billings,
Texas Support Group

United Way Campaigns

A small bit of your time can go a long, long way...

We need your support!! CARES Foundation is looking to become a registered agency with your local United Way chapter. The United Way organization includes approximately 1,400 community-based United Way chapters. Each is *independent*, separately incorporated, and governed by local volunteers. We need volunteers to contact their local chapter and help fill out the forms necessary to get listed. Please consider taking the small amount of time it takes to help us in this worthwhile endeavor.

You can find your local chapter at the United Way national website: <http://national.unitedway.org/>. Just enter your zip code on their home page and it will give you the chapter that covers your community.

CF

Attention CALIFORNIA Members!!



CARES just received a very generous donation from **Cars 4 Causes** – a California non-profit public benefit corporation established in 1997 to raise money on behalf of schools, hospitals, religious institutions, social service agencies and other non-profit organizations. We received this donation because one of our members generously donated one of their vehicles to this organization. Cars 4 Causes will sell your donated vehicles (car, motorcycle, boat, etc.) and will make a cash donation from the proceeds to the non-profit charity of your choice.

Vehicles are accepted whether they are running or not. Cars 4 Causes will tow your vehicle away at no charge to you. They will provide you with a Kelly Blue Book evaluation to aid in the determination of the fair market value of the vehicle, a thank you letter to the donor, and a donation receipt to be used for taxes. For more information on how to participate, visit their website: <http://www.cars4causes.net>.

CF

Fundraising Guide

Interested in having fun and helping CARES? Here is your chance. CARES has a **Fundraising "How to" Guide** that can help you to help us. The Guide has over 20 ideas to entice your imagination, and "how to" explanations to guide you through the process. Not only does it explain how to run an event but it also has resources to assist you, template letters for the media and so much more! It is simple, comprehensive and has a convenient check list to help you organize and plan your event. If you are interested in holding a fundraising event and raising awareness in your community about CAH, please contact CARES to receive a copy of the Fundraising Guide. You can email: info@caresfoundation.org or call toll free: 866-227-3737.

CF

Want to run a **CARES Fundraiser**, but need some *fresh ideas*?

Check out this really COOL Website:

<http://www.fundraising-ideas.org/DIY/>

Thank You... Thank You!!!

To all of our wonderful supporters who have given so generously of their time and money and their willingness to reach out to their family and friends for donations.

We would especially like to thank Larie Hall, Stephanie Erb and Arthur Hanket from California, Krista Breeding from Pennsylvania, Jenny Hendricks from Indiana, and Sandra Billings from Texas, for their successful and creative fundraising efforts.

Larie Hall raised money for CARES through her Valentine's Day Goodies Sale and the Science Workshop for kids that she taught. Many thanks to the teachers and families at her children's school who contributed.

Stephanie Erb and Arthur Hanket donated their vehicle to Cars for Causes - a "charity that gives to



"A little CAH humor"

charities" (see pg. 16 for details).

Krista Breeding had CARES added to her school's Casual Dress fundraiser. Now CARES will be part of the rotation of charities that the Shikellamy school district will support each year.

Jenny Hendricks donated a portion of the profit she received from the Cody Cares Medical Alert Jewelry sales to CARES. (See the Cody Cares advertisement on back cover of this newsletter). This is an ongoing fundraiser, so please consider purchasing this jewelry to benefit your child as well as CARES.

Jenny is also planning a **Golf Outing to benefit CARES**. It will be held on **September 19, 2004 at the Valle Vista Golf Course in Greenwood, IN** at 1:00 pm. There will be 18 holes of golf, a luncheon and an auction. Prizes to be given away also. Indiana families willing to help can contact Jenny at contact@codycaresid.com.

Sandra Billings organized a wonderful Bowlathon in Houston, Texas (see page 16 for Sandra's article).

Thank you all so much for your continuing support and contribution to our mission.

CF

Parent Tip!!

(Solutions for Common Problems)

MEDIC ALERT BRACELETS

I've had trouble keeping my daughter's MedicAlert ID on her since she pulls at it until she gets it off. After many months of guilt over her not wearing it, I finally found a solution. I bought some "shoe charms" at Target. (These are charms that you attach to your shoelaces via a necklace-like clasp.) I took the clasps off of 2 of the charms and attached them to my daughter's MedicAlert ID. Then, I attached it to the shoelace on her tennis shoe. I check them every day or two to make sure they are still firmly clasped, but have used this for a month or so and it's worked great.



Karen Zappa
Mother of Miriam 17 mths SWCAH
Bloomington, IL

Have a great tip to share with other parents? Please email us and we will post it in our newsletter and on our website. See all our parent tips at: <http://www.caresfoundation.org/parent.html>

CF

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Legislative and Newborn Screening Update

Genetic Nondiscrimination Legislation

House Action Needed Now: Please Contact Your Representative to Support Federal Protections!

Last fall, the Senate approved the Genetic Information Nondiscrimination Act of 2003 (S1053) unanimously. The bill would provide a basic level of protection against genetic discrimination in health insurance and in the workplace. President Bush supports the legislation. While more than half of the Members of the House of Representatives have cosponsored similar legislation, action has stalled.

We need your help. The House must act soon to approve a basic level of genetic nondiscrimination protections. Your voice could make the difference. Please contact your Representative to urge him/her to support passage now of genetic nondiscrimination legislation.

Budget Resolution Conference Report

In January, President Bush proposed a budget for the National Institutes of Health that was only 2.6% above last year's level. Since inflation in the field of biomedical research is expected to exceed 4%, the request, if adopted by Congress, would mean that less research could be supported in the coming fiscal year than is being conducted during the current year.

Both houses of Congress have passed budget resolutions that will force significant cuts in domestic programs from the amounts requested by the President. The House is almost

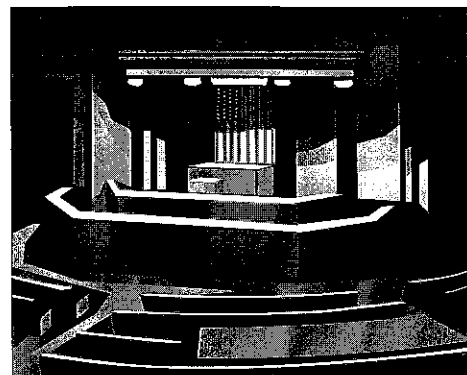
\$1.6 billion below the President's request for domestic spending and the Senate Resolution is \$2.3 billion below that of the House.

The Conference Committee that will resolve the difference between these two bills is continuing to meet, but there is little hope that they will produce a resolution that would allow spending for domestic programs to reach even the levels requested by the President. Please contact your Senator and Representatives to express your opposition to the Conference Committee Report. It is also imperative that you speak out in your community, visit with your colleagues and talk to the various patient advocate organizations who share our concern about domestic spending priorities. We are at a critical point in the federal budget process and we have one more chance to make our voices heard.

The **Federation of American Societies for Experimental Biology** website has a tool to fill in the blanks and generate email or hard copy letters to your legislators on **Genetic Discrimination** and on the **Budget Conference for NIH Funding**. Please visit the web site at <http://capwiz.com/faseb/issues/?style=D&> and spend five minutes making a difference. It couldn't be easier!

Family Opportunity Act

On Thursday, May 6, the Senate passed the *Family Opportunity Act* sponsored by Senator Chuck Grassley. It now goes to the House for consideration. The companion House bill, HR



1811, sponsored by Rep. Pete Sessions (R-TX), faces an uncertain future. The cost of the bill is estimated to be \$7 billion over 10 years and is "likely to face opposition from fiscal conservatives" (CQ Today, May 6). According to the *Kaiser Daily Health Policy Report*, Grassley plans talks with House leadership in the hopes of moving the bill to the House floor for a vote. Major provisions of the legislation include:

- Medicaid "buy-in" for families of disabled children or with special healthcare needs with annual incomes up to 250% of the federal poverty level;
- Medicaid demonstration project allowing a Medicaid "buy-in" for less severely disabled children or with special healthcare needs to keep them healthy enough to avoid the need for more expensive treatments;
- State authority to offer more services to children with psychiatric disabilities at home, instead of in an institution;
- Immediate access to Medicaid coverage for disabled children or with special healthcare needs who are "presumed eligible" for SSI;

(Continued on page 19)

(Continued from page 18)

▪ Funds for information and outreach centers in each state to serve families with disabled children or with special healthcare needs;

This legislation could benefit many of our members and allow them access to quality healthcare for their children. Please take the time to write your Congressional representatives about this legislation as well. You can find your Representative at www.house.gov.

Newborn Screening Update

California

Our members have bombarded the Governor's office with hot pink postcards!!! Over 10,000 postcards have been sent to Governor Arnold Schwarzenegger urging him to expand California's newborn screening program to include CAH and other disorders. While in the CA State House in March, I stopped in to the Governor's mailroom and asked whether they had been receiving any pink postcards. The mailroom manager, said "Oh yeah. CARES Foundation. They come in every day".

In addition, our members have visited the State House and spoken to legislators, as well as phoned and written and visited with their aides in their district offices. In March, members attended a symposium hosted by the Public Health Institute at which I spoke on grassroots activism in newborn screening, and members told their stories to the attendees. Alyssa Ackenheil, age 10 of Sacramento, CA, bravely got up before the audience and spoke about her life with CAH and how she never wanted to see another baby with CAH suffer or die. She made a

tremendous impact on the group and on Senator Dede Alpert of San Diego.

At the close of the symposium after hearing Alyssa and all of the parents' poignant stories, Senator Alpert agreed to introduce legislation dramatically expanding CA's newborn screening program and said she would work vigorously to get it passed quickly. She has been good to her word. Last month, the bill, SB142 passed through the Senate and is now moving through the Assembly. Moreover, the bill has been attached to the 2005 Budget and cannot be line-item vetoed if the Governor approves the budget (which he will). The legislation is now moving on 2 parallel paths at an amazing speed. All of our efforts this past year or so primed the legislature for and made them very receptive to this expansion. Many thanks to everyone who has helped so far. We need to keep up the effort. Please call the office or email us for more postcards.

Oklahoma

Oklahoma expected to begin newborn screening for CAH, cystic fibrosis, and MCAD on July 1, 2004. Unfortunately, they are delayed due to construction of a new lab. They now expect to begin in January 2005.

New Hampshire

The State is working on an expansion plan that will include CAH. However, it may be some time before the expansion is approved and implemented.

Other States

If you are interested in spearheading an effort in your state

to expand newborn screening, please call the office and talk to Kelly Leight, 1-866-227-3737.

CF



Kelly Leight, Alyssa Ackenheil & her grandmother at the California Symposium on Newborn Screening

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Cody Cares Medical ID Jewelry

4155 Carson Avenue
Indianapolis, IN 46227

317-783-7702 • contact@codycaresid.com
<http://www.codycaresid.com/> (new website!!)

Specializing in Custom Made and Engraved Medical
Jewelry. *Choose from:*

- ✓ ID Bracelets
 - ✓ Shoetags
 - ✓ ID Wristbands (*perfect for infants & sports*)
- New items added all the time... check the website!!*

*Don't wait... it could protect and save your child's life!
It saved Cody's life! We also carry many more items. A
portion of all profits will go to CARES Foundation, Inc.*

Calling all Artists!!

We are looking for someone to create a simple design for CARES Foundation. This design would be used as a symbol of support for CAH and the CARES organization. Our plans are to turn this design into a small piece of jewelry that we could sell as a fundraiser and as a way to raise awareness about CAH.

☞ *Have you recently moved, changed your phone number or email? Please make sure to let us know, so we can keep our information current. ☑*



CARES FOUNDATION, Inc.
189 Main Street
Millburn, NJ 07041

**DO NOT DELAY
MEETING NOTICE**

Address Service Requested