ACDA Notes

From the Alveolar Capillary Dysplasia Association

Volume 4, Issue 1 Winter 2002

Transition Underway. . .

Happy New Year to everyone! As many of you realize, Madonna has worked tirelessly over the last five years to further the awareness of ACD and to push for ACD research. Since her first letters to doctors asking them to forward her name to other families who have lost a baby to ACD, Madonna has put in countless hours and committed personal financial resources to comfort us in our grief and to solicit support from doctors and researchers around the world. ACDA has come a long way in five years under Madonna's leadership and will continue to grow. Madonna will continue to play a key leadership role, but many of the administrative tasks are transitioning to Donna and Steve Hanson, with Phil Tenney continuing to maintain the web-site. Madonna sends this letter:

Dear Friends,
Since early summer, my family has gone through some trying times. Many of you have been kind enough to offer comfort and support. Please know how much it has meant to us. I am sure an equal number of you have been wondering what has been happening. For many newer families, you have not received the support from me that many other families have come to expect because I have been so distracted.

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INSIDE THIS ISSUE

Check Out the Web-Site

Check the ACDA web-site (www.acd-association.com) later this month for current articles. Phil Tenney, who is generously maintaining the site, will be adding articles published in 2001. If you have any comments on the web-site, please pass them along to Phil or us. It is there to serve you!!!

Baylor College of Medicine Update
By
Dr. Partha Sen

As many of you know, Baylor College of Medicine in Houston was severely affected by tropical storm Allison. Most of the research buildings had no power for almost a week because of the flood. This has caused tremendous loss of research material for most laboratories. Fortunately, we were not as affected as many of our colleagues. While our laboratory lost power for about 24 hours, we were able to move all our samples (ACD and others) to refrigerators that were running on back-up power. During the recovery period in late June, we tested all the samples we had, and confirmed that there was not any damage to the biological samples. We are pushing ahead with our research plan.

Our list of participating families has gone up to twenty-seven. Families from all over the world are participating. We are still looking for families with more than one affected child. Families with two or more affected children increase the power of the genetic analyses and provide a unique opportunity to identify the ACD gene. If you are aware of such families, please encourage them to contact us. We need their help.

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After a great deal of thought as well as conversations with Jeff, Steve and Donna, we have come up with what we believe will be a better system that will provide better support, organization and communication within the group. Each family in the group has been so very important. That is why we will be making the following changes.

Welcoming and initial contact will now be managed by Donna Hanson. Newsletters will be created by Steve and Donna and hopefully be available online. They will also continue their work with keeping up with the articles and the directory. I will be shifting my focus to work within the medical community. I hope to incorporate the group, create a medical database, network with researchers and physicians, and work with other support organizations, like PHA, NORD, etc.

Please continue to share your family news with everyone through our newsletters. You can now send those additions to Donna Hanson. All address, phone and e-mail changes should also be sent to her (see below).

I will do my very best to keep you as up to date as possible on what is happening in the medical community. If you have any questions or just need an ear, know that I am still here for each and everyone of you.
All my best in the New Year,

Madonna

We alone cannot make ACDA a successful organization. **We need your help!** We hope that all of you will find some way to assist us in memory of your precious child (children) that you have so tragically lost. See the article on page 3 on ways you can help.

We look forward to working with you towards our ultimate goal of a cure for ACD.

Fondly,

Donna and Steve Hanson
sdhanson@flash.net
5902 Marcie Court
Garland, TX USA 75044-4958
(972) 414-7722

Join a Chat Room

Allen and Starlynn Markiewicz have started a chat room for our ACDA families. This is a place for families to share messages, photos and ideas in a private webpage. Please contact them at justicenav@aol.com if you are interested. ꝏ

Please Welcome Our New Families…

It is with mixed emotions that we'd like to introduce the families that have joined ACDA since the last newsletter. Please take the time to introduce yourself, offer support and share the story of your child (children).

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[redacted]

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[redacted]

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[redacted]

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[redacted]

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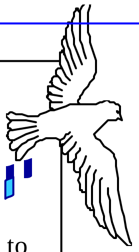
[redacted]

If you are a new family and do not see your name mentioned here, it is because we do not have your registration form. Please register on the web-site. ꝏ

Remembering your baby!...



During the last four years, we have found it healing to remember Eric in our daily lives. Here are two suggestions that you might consider:

- Have a stone engraved with your child's name, birthday and/or a special phrase and place it in your garden;
- Make a donation in your baby's name to the hospital where he/she was treated.

If you have found a unique way to celebrate your child's life and would like to share it with the ACDA, please e-mail us at sdhanson@flash.net.

How Can You Help?

ACDA is managed by families who have lost children to ACD. They volunteer their time and money to assist in the goal of providing support to other families and finding a cause and cure for ACD. Here are some ways for you to assist in this effort.

- Participate in the ACD study being conducted by Baylor College of Medicine
- Contact other families who have lost a baby (or babies) to offer and receive support during such a devastating time
- Contact the doctors who treated your child and inform them of ACDA, or if you prefer, send your doctor's names/addresses to ACDA and we will contact them
- Search the internet and other medical sources for new research on ACD
- Provide legal or financial expertise for our efforts to become a non-profit organization
- Submit an idea(s) for an ACDA logo
- Assist with the development of a new brochure for ACDA
- Assist in creating a medical database with the ECMO organization
- Attend the American Thoracic Society Conference in Atlanta in May to promote ACD awareness (contact Madonna).

We welcome new ideas and suggestions about the organization so please, get involved and let's work together for an ACD cure. ∞

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We have hired a full-time research associate to work on this project. She has been able to isolate good quality DNA from the paraffin blocks. We are now able to use DNA from the paraffin blocks reliably and consistently. We are in the process of testing some candidate genes for ACD. To date, we have not found any mutations, but we will keep trying, and you will be the first to know. We are also preparing a manuscript that describes the clinical findings in ACD to make other physicians aware of this condition.

Our laboratory is now listed on GeneTests® (www.genetests.org), a web site funded by the National Library of Medicine of the NIH and Maternal & Child Health Bureau of the HRSA aimed at helping genetic counselors and geneticists to identify research and clinical laboratories that study inherited conditions. We have already been contacted by a genetic counselor, who referred a family to us. We have encouraged the counselor to tell the family about the ACD Association and about the tremendous support that this family can get from talking to various members of the ACDA. ∞

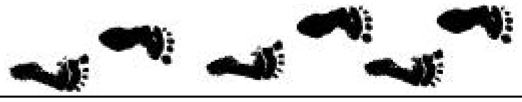
Safe Arrivals

Congratulations to the following families who have been blessed with another baby:

[redacted]

[redacted]

[redacted]



Want to contribute financially to ACD research?

The ACDA is *not* a non-profit organization yet, so we are unable to provide a tax-deductible receipt for donations. However, we welcome small cash donations to support printing, mailing and administrative expenses.

If you want to make a significant tax-deductible contribution to ACD research, this may soon be possible through the National Organization for Rare Disorders (NORD). Please contact Madonna (by e-mail) who is coordinating with NORD to establish an ACD fund. Information about NORD's grant program is provided below:

NORD'S RARE DISEASE CLINICAL RESEARCH PROGRAM POLICY

The National Organization for Rare Disorders (NORD) represents more than 25 million Americans with over 6,000 rare "orphan diseases." Because each disease affects a small number of people, there generally is little commercial interest in performing research or developing new products to diagnose or treat these diseases. Moreover, academic scientists believe it is easier for them to obtain government research grants for the study of more prevalent diseases, so they sometimes avoid applying for grants to study rare diseases.

NORD's mission is to promote the diagnosis, treatment, and cure of rare disorders through programs of education, research, advocacy, and service to families and health professionals.

NORD's ***Research Grant Program*** provides small "seed money" grants to academic scientists studying new treatments or diagnostic tests for rare diseases. The small clinical trials supported by NORD's research grants provide preliminary data indicating that a treatment (drug, device, or medical food) may be safe and effective when used for a larger number of patients. Researchers can then use the preliminary data to apply for larger multi-year government grants, or to attract a commercial sponsor who will manufacture the orphan product and get it approved for marketing by the Food & Drug Administration (FDA).

DONATING FOR RARE DISEASE RESEARCH

Donors may specify that their gift to NORD be used for general research and related activities, or they may restrict their gift to research on a specific disease.

Disease Specific Research

Gifts restricted to a single disease will be used to support clinical research on that disease, including new treatments, diagnostic tests, or genetic studies. However, a Request for Proposals (RFP) will not be issued to the scientific community until the disease-specific fund reaches $35,000 to $50,000. This is because scientific research is very expensive, and scientists cannot accomplish meaningful results unless they have enough money to support their work.

Out of each grant sum restricted to specific rare diseases, NORD uses only $5,000 to cover the direct costs of the RFP, which include advertisements in leading medical journals and a mailing to every academic research facility in the United States. Additionally, research institutions in Canada and Europe may also be notified of the RFP. After the grant is awarded, NORD monitors the progress of the research, processing biannual reports to NORD's Medical Advisory Committee. In the past, when donors gave small sums restricted to research on specified diseases, the gifts languished unspent for several years because the sums were too low to support medical research. Therefore, NORD's Board of Directors decided if the funds donated to a specific disease do not exceed $1,000 within two years, the gifts will be transferred to NORD's general research account.

Exceptions can be made if NORD sees continued donor activity for a specific disease, indicating that there is a determined effort to reach the minimum $35,000 goal. In many instances, families will launch fundraising campaigns in their communities that raise several hundred dollars at a time. NORD encourages these activities and recognizes that it can take several years to reach the necessary sum that triggers a request for research proposals.

General Research Fund

Donors may direct their gift to NORD's General Research Fund which ensures the funds will be spent on medical research and related activities that encourage increased research, but are not restricted to one disease. Again, the fund must reach a minimum of $35,000 before a Request for Proposals will be issued.

Selection of Grantees

The initial Request for Proposals asks scientists to submit a brief description of their proposed research project along with their qualifications and a draft budget. Once that information is received, NORD's Medical Advisory Committee (composed of leading academic scientific experts) reviews all of the applications and selects finalists. The finalists are invited to submit a full grant application describing their proposed project in detail. NORD's Medical Advisory Committee then reviews the full grant applications (peer review) and ranks them through a scoring system. The highest scoring grant applications are recommended for funding to NORD's Board of Directors who vote on the final grant awards.

NORD's grant review process is based on the same peer review system utilized by the National Institutes of Health (NIH). Scoring of grants is done individually by each medical expert, and scores are added together to determine the final ranking of each proposal. Any reviewer with a conflict of interest is excused from voting on a specific proposal.

By encouraging research applicants to compete against each other, NORD's Research Grant Program funds only the best proposals that are most likely to result in research breakthroughs that can be applied to the diagnosis or treatment of people with rare disorders. Donors who wish to support research on rare diseases are encouraged to contact NORD's Department of Development to find out more about this unique program. ꝏ