HOPE for families and friends of children with Neuroblastoma

An informative newsletter to educate, support, and increase awareness

CHILDREN ARE OUR FUTURE! THEY ARE THE RAINBOW THAT MAKE OUR DAYS BRIGHT

HOPE for more tomorrows for children with Neuroblastoma is the theme of the Neuroblastoma Children’s Cancer Society. HOPE stands for the following:

Help for Children and their Families
Oncology Research
Public Awareness
Education

In Memory of... Eden Rae Adams

State: Ohio
Family: Father Rourke, Mother Lisa, Brother Riley
Pets: Dog Bo
Favorite place: Home!
Favorite thing: High School Musical and Hannah Montana
Favorite movie: Lord of the Rings Trilogy
When I grow up, I want to be: A doctor

The Neuroblastoma Children’s Cancer Society is a group of volunteers, many with children or relatives who have been both victims and survivors of the disease. The Organization is an advocate for the children and their families and is dedicated to providing support. The primary focus of the Organization is to raise money to assist local research in neuroblastoma cancer and to bring the fight to a national level to try and focus additional research and funding until a cure can be found!
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Those areas in bold will be the focus of our quarterly newsletter. Please send in your ideas, articles, requests, special stories and pictures to: The Neuroblastoma Children’s Cancer Society P. O. Box 957672 • Hoffman Estates, IL 60195

GUIDE TO INTERNET CONNECTIONS
AMERICAN BRAIN TUMOR ASSOCIATION HOME PAGE: www.abta.org
AMERICAN SELF-HELP CLEARINGHOUSE, SELF-HELP SOURCE-BOOK ON-LINE: Information on national and model self-help support groups, clearinghouses, organizations, and resources — www.cmhc.com/self-help/
CANCER KIDS WEBSITE: www.cancer.org
CANCERNET and PDQ: This website is managed by the National Cancer Institute and provides information on cancer treatment, supportive care, and advocacy — www.cancernet.nci.nih.gov
CANDLELIGHTERS CHILDHOOD CANCER FOUNDATION: Links to other information and resources on childhood cancer. www.candlelighters.org
CHILDREN’S MEMORIAL HOSPITAL www.childmmc.edu/cmhweb/default.htm
UNIVERSITY of CHICAGO COMER CHILDREN’S HOSPITAL www.uchicagokidshospital.org
HISTIOCYTOSIS ASSOCIATION OF AMERICA: The goals of this organization include public and professional education, patient and family support, and stimulation and support of research — www.histio.org
KIDS WITH CANCER — www.kidswithcancer.com
NATIONAL CHILDHOOD CANCER FOUNDATION (NCCF): Describes NCCF and offers a listing of CCG clinical cooperative group protocols — www.nccf.org/
NATIONAL HEALTH INFORMATION CENTER (NHIC): This website is managed by the NHIC, a government sponsored health information referral service. Offers direction to other organizations and resources — http://nhic.hc.gov
NEUROBLASTOMA CHILDREN’S CANCER SOCIETY: Provides information on neuroblastoma for families of children diagnosed with this form of cancer — www.neuroblastomacancer.org
PEDIATRIC ONCOLOGY GROUP (POG): www.pog.ufl.edu/main.html
http://cancerguide.org/bonemarrow.html

Note from the Editor
The views contained in the articles of this newsletter are the opinions of the authors. The editor and Neuroblastoma Children’s Cancer Society are not in any way affiliated and make no attestation in support (or against) the information supplied in these articles.
March 25, 2009

Neuroblastoma Children's Cancer Society

I wanted to share a recent editorial that Dr. Sam Volchenboum and I wrote for the *Journal of Clinical Oncology* to summarize recent progress in defining and treating high-risk neuroblastoma. While modest improvements have been made in the treatment of high-risk neuroblastoma, there is still much to be done to ensure that children with a neuroblastoma diagnosis—whether biologically favorable or high-risk—survive with less toxicity than with current therapies.

Dr. Volchenboum is a talented young investigator with expertise in pediatric cancers and blood disorders, and has been at the University of Chicago Comer Children's Hospital since July 2008. In addition to caring for pediatric cancer patients, Dr. Volchenboum studies neuroblastoma in the laboratory, using sophisticated tools to measure the amounts of proteins found in these tumors. He is also pursuing several research projects in bioinformatics, a field that harnesses tools from the world of computer science to solve complex issues, such as genetics, in both adult and pediatric cancers. He is a great asset to our clinical and research teams, and I look forward to introducing you to him, if you have not yet met.

Enjoy the editorial and I hope you will all contact me personally if you have any questions about the editorial or would like more information about our ongoing laboratory work.

Sincerely,

Susan L. Cohn, MD
Professor and Director of Pediatric Clinical Sciences
Section of Pediatric Oncology, Department of Pediatrics
University of Chicago

*Neuroblastoma HOPE*
HIGH RISK NEUROBLASTOMA (continued from page 3)

13-cis-retinoic acid (cis-R) versus no additional therapy. The initial results of the study, reported almost 10 years ago, demonstrated significantly better 3-year event-free survival (EFS) for the group randomly assigned to myeloablative therapy and ABMT and for patients randomly assigned to cis-R. With additional follow-up, patients randomly assigned to the more intensive arm (myeloablative therapy and AM=BMT) continue to have significantly higher 5-year EFS. Similar results have been reported by European groups, although survival still remains poor. In the COG study, 30% ± SE4% of the patients randomly assigned to the superior arm of therapy were event-free at 5 years. Although overall survival (OS) in the COG study was found to be significantly higher for each randomization at 5 years using a test of the log(−log(.)) transformation, a significant advantage for OS was not observed in the European studies. Thus, the majority of the patients are not cured with intensive treatment strategies that include myeloablative therapy, including delays in growth and development, hearing loss, renal and cardiac dysfunction, learning problems, and treatment-related leukemia and second cancers.

The study by Canete et al focuses on the outcome of infants younger than 12 months of age with high-risk disease treated on a International Society of Pediatric Oncology European Neuroblastoma (SIOPEN) clinical trial. In this cohort of 35 infants, 2-year EFS was 29% (SE = 0.07) and OS was 30% (SE = 0.08) after treatment with intensive multimodality therapy. In the COG, infants with high-risk NB are treated on the same clinical trial as older patients with high-risk disease. Because of small numbers, international collaboration will likely be needed to determine if the clinical behavior of high-risk tumors diagnosed in infancy differs from high-risk tumors in older children.

To ensure that patients with a low- or intermediate-risk clinical phenotype are spared toxic, dose-intensive, high-risk treatment regimens, accurate risk-group classification is critical. Historically, an age cutoff of 12 months has been used for risk stratification. However, recent analysis of a large series of 3,666 patients has demonstrated statistical evidence for increasing the age cutoff to 15 to 19 months. On the basis of these results, COG has modified eligibility criteria for its intermediate-risk clinical study to include toddlers, age 12 to 18 months, with favorable biology tumors. Similarly, an age cutoff of 18 months has been included in the INRG classification system. This new age cutoff will shift approximately 10% of patients previously classified as high-risk to a lower-risk group. Newly designed intermediate-risk clinical trials will need to be closely monitored to verify that high survival rates are maintained in this cohort of patients, despite therapy reduction.

Janoueix-Lerosey et al investigated the value of using a genetic approach to risk-stratify patients more precisely. In this study, the prognostic significance of genomic profiles was initially evaluated in 224 NB samples using array-based comparative genomic hybridization and then validated on a second series of 269 patients. In both cohorts, no disease-related death was observed in patients with whole chromosome copy number variations, whereas segmental chromosome alterations were associated with significantly worse outcome. In this series, genomic classification was found to be more powerful for predicting relapse than individual genetic markers. In addition, patients classified as low or intermediate risk with segmental alterations had significantly worse outcome than those lacking genetic aberrations. Similar results have been seen in other array-based studies. Although genetic profiling is not routinely performed by the large cooperative groups at the present time, it is likely that global genetic data will replace individual genetic markers in future risk-stratification schemas.

All four NB studies published in this issue of Journal of Clinical Oncology stress the need for new therapeutic approaches for high-risk NB. Building on the promising response rates in refractory NB with iodine-131-metiodobenzylguanidine (131I-MIBG), Matthay et al conducted a phase I study using double-infusion 131I-MIBG with autologous peripheral stem-cell rescue. The therapy was tolerated well and activity of 131I-MIBG was demonstrated. A randomized study will be required to determine if outcome is improved with double-infusion versus single-infusion. In an effort to further increase response rates, studies testing the combination of chemotherapy and 131I-MIBG in refractory NB are ongoing. COG is also developing a pilot study that will test 131I-MIBG in combination with myeloablative therapy and hematopoietic stem cell rescue in newly diagnosed high-risk patients.

Although the studies in this issue of Journal of Clinical Oncology highlight the progress that has been made in stratifying and treating children with high-risk NB, outcomes remain dismal for this cohort of patients. To further improve survival, it is evident that novel treatment strategies targeting the biologic pathways responsible for driving the high-risk NB phenotype will be needed. Genomic studies have led to the discovery of a number of putative molecular targets. Recently, germline mutations in the anaplastic lymphoma kinase (ALK) gene have been identified in patients with familial neuroblastoma, and somatic ALK mutations have been detected in a subset of primary NBs and cell lines. Functional studies show that many of the ALK mutations represent gain-of-function alleles that can sustain key signaling pathways and are therefore likely to be valid therapeutic targets for ALK inhibitors. Therapies, like ALK inhibitors, that are tailored to individual patients are now emerging, and early-phase pediatric clinical trials incorporating targeted therapies are ongoing. This approach may herald a paradigm shift in how patients with high-risk NB are treated, and hopefully, will also lead to higher rates of cure.
The 8th Annual "Friends of Michael Williams" Fund Raiser raised close to $25,000, making it another huge success. "Friends of Michael Williams" is a chapter of The Neuroblastoma Children's Cancer Society. NCCS matched the proceeds in the amount of $25,000.

Jim and Dori Sexton of The Neuroblastoma Children's Cancer Society were pleased to announce that Dr. Susan Cohn, in connection with the University of Chicago Comer Children's Hospital, was the recipient of our annual research grant for neuroblastoma research totaling $50,000!

NCCS appreciates Dr. Cohn's dedicated work and support of neuroblastoma research. It is our hope that this grant will give Dr. Cohn the financial means to support the research that will lead to a cure for neuroblastoma!
NCCS would like to offer you the opportunity to recognize your friends and loved ones and help NCCS at the same time. We have honorary and memorial cards available to send out on your behalf.

Contributions may be given in memory of a loved one or in honor of special occasions such as birthdays, graduations, holidays, anniversaries, etc. Call the NCCS office for more information …(800) 532-5162.

In Honor or Memory Of…

We have received many contributions in memory of children lost (but not forgotten) to neuroblastoma and in honor of children in the battle of treatment with neuroblastoma. It is these children that inspire our relentless effort to find a cure.

We also receive donations for special occasions, in honor or memory of family members and loved ones. Rest assured this money will be put to meaningful research. It is in honor and memory of our children that we continue the battle for a cure!

In Memory of:

Eden Rae Adams

Our sincere gratitude to and acknowledgement has been sent to Family and Friends.

RECOGNIZE A BIRTHDAY, ANNIVERSARY, OR SPECIAL OCCASION WITH A GIFT OF LOVE! (You can find this page on our web site!)

Memorial or Honor Gift Request

Your Name: _____________________________
Address 1: _____________________________
Address 2: _____________________________
City: _____________________________ State: ________ Zip: ________
Phone: _____________________________

☐ In Memory Of  ☐ In Honor Of  ☐ On the Occasion Of ______________

Name: _____________________________

I would like an acknowledgement gift card sent to:
Name: _____________________________
Address 1: _____________________________
Address 2: _____________________________
City: _____________________________ State: ________ Zip: ________

How do you wish the card to be signed? _____________________________

Enclosed Gift Amount: _____________________________

Send form and payment to: NCCS, P. O. Box 957672 • Hoffman Estates, IL 60195

Donations also can be made online through PayPal. Go to our web site at www.neuroblastomacancer.org.

United Way is not just about giving … it's about caring

You can designate that your contribution be given to NCCS.

Call us at our office for details (800) 532-5162.

NATIONAL CANCER INSTITUTE
CANCER INFORMATION SERVICE
1-800-4-CANCER
(1-800-422-6237)
☐ Tips to prevent cancer
☐ Informational Materials
☐ Answers to questions about cancer
☐ Other Resources
www.cancer.gov/publications
Thank You!

We recognize and give a “big hug” and thank you to our recent contributors:

- Bruce & Kenna Anderson
- Sharon Bending
- Mrs. Roger Bielinski
- Dr. Charles Boyajian
- Mr. & Mrs. Larry Cohen
- Dr. & Mrs. Sidney Cohen
- John & Cathy Curielli
- Dynamic Manufacturing
- Ennes & Associates, Inc.
- Marshall Gordon
- Gynecological & Obstetric Associates
- Ronald Herring, CPA
- Kevin & Kathy Irving
- Christopher Kalmus
- Brian & Grace Klimes
- John Krzeski, IMAC Motion Control
- Mr. Larry Malinowski
- Mr. & Mrs. Dan Nagle
- Mary Partipilo
- Tony & Sue Partipilo
- R. B. Rooper
- The Sexton Family
- Fred & Jeanne Stevens
- Mike Urban
- Dr. Sheehy, Bielinski & Griffin, Urology, Ltd.
- Robert & Patricia Wheeler
- Lou & Renee Williams
- Woodlawn Engineering Co., Inc.

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**Pediatric Resources**

**Cure Search**—800-458-6223; info@curesearch.org; www.curesearch.org
—Information about pediatric cancer and treatment.

**Candlelighters Childhood Cancer Foundation**—800-366-2223; staff@candlelighters.org; www.candlelighters.org—Publications and support groups for families with children with cancer.

**SuperSibs!**—866-444-7427; info@supersibs.org; www.supersibs.org—Free age-appropriate support for 4 to 18-year-old siblings of young cancer patients, including journals, trophies, mail, and events. Written guides are available for siblings and others for adults.

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**The Neuroblastoma Children's Cancer Society On-line Resource Survival Handbook**

This handbook has accumulated a resource of information of facts about neuroblastoma and related treatments, national and local resources available to families, patient history and treatment forms, health claim forms, pamphlets, etc.

This book was prepared and dedicated in honor of the life of Michael James Sexton, whose determination and purpose in his fight against neuroblastoma has given many the courage and spirit to continue the battle.

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- Patient's History and Other Important Information
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- Calendars
- Treatment Journal
- Children's Memorial Hospital Chicago Family Survey Form

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- Dr. Sheehy, Bielinski & Griffin, Urology, Ltd.
- Robert & Patricia Wheeler
- Lou & Renee Williams
- Woodlawn Engineering Co., Inc.
New NCCS Website Coming Soon

Current Features Expanded
- Links to Vital Resources
- Legislation Update
- Fund-raising Opportunities
- Resource Handbook

New Features Include...
- Contact with Medical Advisors
- Postings of Current Research and Treatment
- You Tube Video of Our Special Kids
- Facebook and Family Communications

Plus Much More!

http://www.neuroblastomacancer.org

Wall of Fame Sponsors

Please help us by sponsoring one of our children and build our WALL OF FAME!! The response to our survey and WALL OF FAME has been overwhelming. We have had over 200 families respond with pictures, drawings, surveys, etc. and they are still rolling in. Come visit our internet WALL OF FAME being built and see our little celebrities, our children. Now we need your help to complete this project. The wall is now complete and we have over 200 children who represent the thousands of U.S. families affected by neuroblastoma. Please help by sponsoring or finding sponsors for these children. If we can raise $2,500 for each child, we will have raised over $250,000, 100% of which will be used for a neuroblastoma research grant.

Let us build this WALL OF FAME and provide HOPE for these children! Together we can make a difference!

My tax deductible contribution is enclosed:
☐ $25,000 ☐ $2,500 ☐ $1,000 ☐ $500 ☐ $100 ☐ Other $ ________________
☐ Research Sponsor ☐ Child Sponsor ☐ Honor ☐ Memory

Child's Name ________________________________

Donations can be made online through PayPal. Go to our web site at www.neuroblastomacancer.org.

For all donations: My name is ________________________________
Address ________________________________________________
City __________________________ State ______ Zip ______