In this issue

Two Issues for Pennsylvania Patients with Bleeding Disorders. . .One Solved in 2005! One to Go in 2006!

2005 was certainly challenging for 1,700 Pennsylvania patients with bleeding disorders represented by the two Chapters of the National Hemophilia Foundation! Here's the "report out" on both issues as we close another year of service to our community!!

Issue #1
For blood clotting factor therapies used to treat hemophilia, the Pennsylvania Department of Public Welfare (DPW) indicated its intent to implement a "Preferred Drug List" (PDL) for Medicaid hemophilia patients. DPW would designate a single product (factor), or perhaps only a few factor therapies as "preferred products." These preferred product designations would be based primarily on price. Other "non-preferred" products would be available to Medicaid patients only after strict, "fail-first" prior authorization criteria had been satisfied. This decision by DPW was of tremendous concern for the Pennsylvania hemophilia community.

OUR POSITION:

Lack of therapeutic interchangeability – Blood clotting factor therapies are neither pharmaceutically nor therapeutically equivalent, and are therefore not interchangeable. Each blood clotting factor therapy has unique characteristics that result in varying effectiveness and tolerability from individual to individual. There are no generics.

Equity with privately insured patients – Designation of a single preferred product for Medicaid recipients would create a significant disparity in treatment options from those individuals with hemophilia who are fortunate enough to have private insurance. All private insurers in the Commonwealth make the full range of therapies available to patients without prior authorization requirements. Timely access to the blood clotting factor that a physician

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The Winning Spirit

Winter 2006

Volume 13

Number 1

Quarterly Newsletter of the National Hemophilia Foundation Delaware Valley Chapter

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The Winning Spirit is published quarterly by the National Hemophilia Foundation, Delaware Valley Chapter.
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Sending Our Kids to Camp
A Dad's Perspective

By Art Stinger

When my wife Sue and I were starting our family, we read books on raising children. Each book had different perspectives on the subject and the only thing we agreed with was a saying "the job of a parent is to give your children roots, then give them wings!" This made sense to us. It was easy to understand and we applied this philosophy to our first son successfully.

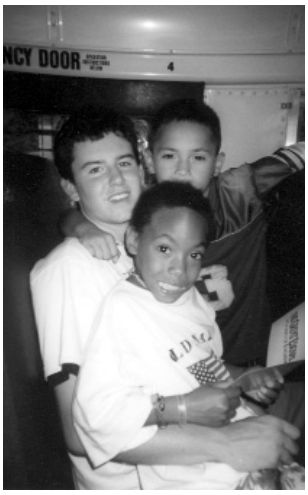
When our second son, Matt, was born with severe hemophilia (no family history), the part about giving him "wings" was a lot more difficult. But we were determined to let Matt be as independent as possible. We were encouraged by our treatment center and by parents from our local hemophilia organization, the Delaware Valley Chapter.

As we looked around, the older kids with hemophilia who seemed best adjusted were the kids that were independent with their care and had no limit in their geographic boundaries. That was our goal…to get Matt independent!

When Matt was five years old, we sent him off to hemophilia camp. That was easy for him and difficult for us as parents. We knew that the camp was staffed with people who had as much or more experience with hemophilia than we did, but it was the "letting go of Matt" that was so difficult.

When Matt returned from camp that first year, he told us it was "better than Christmas" and he couldn't wait to go back next year! He was finally around kids with similar problems and issues. They shared stories and he learned to infuse himself (a few summers later) at camp. He came home that first year, a changed kid, with a better understanding and acceptance of hemophilia and a sense of independence.

As the years went by, Matt never missed a session as a camper. He attended Brandywine Camp in Pennsylvania, Hole in the Wall Gang Camp in Connecticut and finally Hole in the Woods Camp in New York. When he was old enough and for the next five summers, he was a counselor at Woods Camp, helping other little kids with bleeding disorders have a great experience as well. His role as a counselor took him from a teenager to a young adult.



Matt Stinger, camp counselor, with some young campers.

He has made many lifelong friends through his hemophilia camp experiences and he travels from Canada to Florida to visit people who have shared his "life walk and challenges."

Matt graduated from Seton Hall University in New Jersey last year. He lived away from home for all four years as a normal college student who just happened to have hemophilia. His future is bright and there are no boundaries in his world. So our suggestion to other parents is to give your children love and roots, but don't forget those "wings."

We Gratefully Acknowledge...the pledge of support from the following manufacturers for 2006

American Red Cross
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Hemofil M
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Novo Nordisk
NovoSeven™

ZLB Behring
Monoclate-P
Mononine
Helixate FS
Humate-P
Stimate
Gammar-P I.V

Calendar

March
? Moose Night!!

April
8 Fashion Show!!
? Moose Night!!

May
9 Family Dinner!!

July
3-8 Woods' Camp #1!

August
4-9 Woods' Camp #2!
17-23 Dragonfly Camp!

September
15-17 Family Camp!!

Plan to join the Carlino's and the DiMichele's and all of their friends and employees of Carlino's Specialty Foods in Ardmore for **"2006 Moose Night"** (March or April date to be determined) at Our Lady Good Counsel in Southampton. Lot's of surprises and tons of fun!! Great food, music, dancing.....an informal night with supporters of the Delaware Valley Chapter!! Call the DVC office for more information!! 215-885-6500! Check our website for updated information.

2005 Family Camp Weekend
Misty Brunnmeier, National Champion in Tae Kwon Do, Woman with vWD

2005 Family Camp, September 16, 17 and 18, 2005, was a great weekend again at the Variety Club Camp in Worcester, PA!! Good weather, lots of fun, fabulous food and a chance to be together!! What a recipe for a weekend together!! Thanks to our friends at ZLB Behring (especially Janet Riemund), for bringing "Getting' in the Game" to us once again! Misty Brunnmeier, National Champion in Tae Kwon Do and a person with vWD showed us that you can be successful even though you have a bleeding disorder!! And Kogee came too!! NEW FAMILY CAMP LOCATION FOR NEXT YEAR *(see page 8 for more information!!)*

The DVC Golf Classic, chaired by Mark Marra, was a successful day for the DVC. It was held at the Meadowlands country Club in Blue Bell, PA on Monday **September 19th!** Many thanks to Mark for organizing this event, to ZLB for being the event sponsor and for the many golfers and volunteers who came out to support the mission of our Chapter!!

DVC 2005 Golf Classic

Oktoberfest 2005, sponsored by Wyeth, was held at the Hilton Hotel on City Avenue on Friday, **October 21, 2005**....and it was a smashing success! What a great night of food, music, gaming events and a terrific live auction! Many thanks to Bob Romano, Megan McEnroe (Wyeth) and everyone who helped with this great night for our Chapter. Just spectacular!!

Wyeth Family Day on Saturday, **October 15th** at the Wyeth corporate campus in Collegeville, Pennsylvania was a great time for meeting other families who share the same challenges of living with a bleeding disorder while having fun, fun, fun and some education too!!! We met the folks from Wyeth and heard about ways to navigate the insurance nightmare! There were lots of games and prizes for everyone!! Did you ever wrap yourself up like a mummy? Hmmmmmmmm. We sure had a great day!! A great big thanks to our friends at Wyeth for letting us be a part of the fun!!

At the **57th NHF Annual Meeting, October 27, 28 and 29, 2005 in San Diego, California,** George Levy (DVC board member) was honored as NHFs "Advocate of the Year" for his leadership with HB 1705! Way to go, George!! Jill Abrams, RN, nurse coordinator at St. Christopher's Hospital for Children Hemophilia Program received a "Chapter Volunteer" award for her support of our local Chapter (she really deserved this honor!!)..... And the DVC received a check from the NHF for $25,000, as a grant recipient supporting our legislation project resulting in HB 1705!! We are very proud of our work, to say the least!! And there's still so much more to do!!

left: Dr. Alan Kinniburgh, NHF CEO and George Levy "2006 Advocate of the Year" right: Dawn Rotellini, George Levy and Ann Rogers at the NHF Annual Meeting, San Diego, CA.

Santa arrived early at the **DVC Annual Holiday Party** on **Saturday, December 5, 2005!** Everyone had a great time and there were presents for all!! Thanks to everyone who helped make this a special day for the families!! We have some new ideas for next year's "Holiday Bash!!"

With Spring just around the corner, get ready for the **"Annual Fashion Show and Luncheon"** which will be held on **Saturday, April 8, 2006** at the beautiful Drexelbrook in Drexel Hill, Pennsylvania. This event has raised more than $1 million in support of our important work. Please mark your calendars now and plan to be with us. You'll never guess who the honoree will be this year!! Shhhhhhhh!!!

Annual Family Dinner scheduled for Tuesday, May 9, 2006!! Plan to be with us!!!

continued from page 1

Two Issues for Pennsylvania Patients with Bleeding Disorde

deems is most appropriate is essential to ensuring the continued health and well-being of all individuals with hemophilia. The Commonwealth should not take a step that results in different treatment options based upon an individual's economic resources.

Pennsylvania would be the first state to designate a single "preferred product." Most states that have created PDLs have not considered the blood clotting factor class of therapies for inclusion. In fact, several states have excluded blood clotting factor therapies from the PDL and prior authorization requirements for patients with hemophilia. Other states have automatically included most, if not all, of these life-saving therapies on their PDL. We do not believe that Pennsylvania should be the first to take a step that would have such serious consequences on the lives of individuals with a life-threatening chronic condition.

NHF MASAC Recommendation – The Medical and Scientific Advisory Council (MASAC) of the National Hemophilia Foundation (NHF) sets the standard of care for the treatment of hemophilia around the world. The scientists, physicians and other treatment professionals who comprise MASAC are internationally regarded as experts in the broad field of bleeding disorders research and care. MASAC has issued a recommendation, #159, "Recommendation Regarding Factor Concentrate Prescription and Formulary Development and Restrictions" which states that each of the available products is unique in its formulation, and as a result, may have a unique biological indicator for a specific patient. We do not believe that DPW, or its contractor has the expertise to be making any clinical decisions regarding hemophilia and that DPW policies should adhere to the MASAC Recommendation.

Solution to Issue #1

On December 14, 2005, the Pennsylvania Pharmacy and Therapeutic Committee reviewed hemophilia and von Willebrand therapies and made a recommendation to the Department of Public Welfare that ALL FACTOR THERAPIES SHOULD BE AVAILABLE TO MEDICAID PATIENTS WITH BLEEDING DISORDERS. That recommendation was accepted by DPW. All factor therapies will be included on the "preferred drug list." Medicaid patients will continue to have access to the factor therapies that are most effective for them.

It was the combined efforts of the Chapters, the patients and their families, our treatment center physicians and staff and much help from the National Hemophilia Foundation that solved this serious, serious issue. We are grateful to each of you who worked for more than six months each and every day to make sure our voices were heard in Harrisburg! We are especially grateful to Frank Shafer, MD, Medical Director, St. Christopher's Hospital for Children Hemophilia Program for his participation on the Pharmacy and Therapeutic Committee that made this important recommendation. Dr. Shafer's preparation and testimony played a key role in the final recommendation. Thank you, Dr. Shafer! We couldn't have done this without you!

Issue #2

For the last two years, Pennsylvania patients with bleeding disorders have been experiencing restricted access to factor therapies and medical services due to changing practices of insurance companies. Some insurance companies have been authorizing payment for only certain brands of factor or only allowing patients to have their coagulation studies done at certain labs. Other problems include insurance companies determining the amount of factor patients are allowed to have on hand at home, which pharmacy they can use or not paying for quality home support services.

OUR POSITION:

Patients with bleeding disorders need to maintain access to proper treatment and factor therapies in order to stay healthy and productive. Insurance companies should not determine which medicine we can take, what amount we can keep at home, which pharmacy will provide our therapies to us and whether or not we need homecare support.

ers . . . One Solved in 2005! One to Go in 2006!

We know, as a community, that these decisions must be made by us with our treatment center team. It is inappropriate for an insurance company to determine what we need.

Solution #2
In response to a growing concern about patient access to factor therapies, hemophilia treatment centers and home supportive services, the two Pennsylvania NHF Chapters joined forces to educate the state House of Representatives about bleeding disorders and our need to maintain access to care and medicines through legislation.

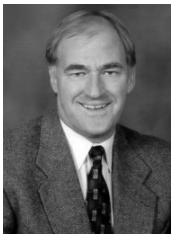
THE RESULT!!
On June 21, 2005, Representative Roy Baldwin (R-97) introduced HB 1705

"The Hemophilia Health Care Act"

This legislation was written to protect access to life-sustaining therapies and care for 1,700 patients with bleeding disorders in Pennsylvania. Over the last six months, the Pennsylvania NHF Chapters, treatment center staff and patients have met with members of the PA House of Representatives, written letters and participated in media events to talk about access issues that are negatively affecting the lives of patients with bleeding disorders.

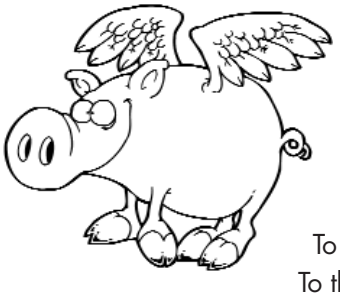
In the end, it was the power of their stories that won the support of the majority of House members for HB 1705. We anticipate its passage early in 2006 and are confident that this legislation will be a model for other states to use in addressing changing insurance practices nationwide for patients affected by hemophilia and related bleeding disorders. We are very grateful to Representative Baldwin for being such a strong leader, believing in us and standing by our issues despite strong objections to the bill from the insurance industry.

Stay Tuned for Part 2 of HB 1705 in 2006.....



Representative Roy Baldwin (R-97) introduced HB 1705

Just a Thought



The Optimist's Creed

Promise Yourself.....
To be so strong that nothing can disturb your peace of mind.
To talk health, happiness and prosperity to every person you meet.
To make all your friends feel that there is something in them.
To look at the sunny side of everything and make your optimism come true.
To think only of the best, to work only for the best and to expect only the best.
To be just as enthusiastic about the success of others as you are about your own.
To forget the mistakes of the past and press on to the greater achievements of the future.
To wear a cheerful countenance at all times and give every living creature you meet a smile.
To give so much time to the improvement of yourself that you have no time to criticize others.
To be too large for worry, too noble for anger, too strong for fear and too happy to permit the presence of trouble.

Research Explores Causes of Inhibitor Development

The following is a compilation of abstracts from recent research articles in the area of hemophilia

For reasons that aren't fully understood, about 30% of people with severe hemophilia A develop antibodies to therapeutic factor VIII. Inhibitors are less common in milder forms of the disorder and much less frequent in other coagulation disorders. In fact, inhibitors are 10 times more likely to form in hemophilia A than any other inherited coagulation disorder. Understanding the how's and why's of inhibitors would be very helpful in the development of treatments as well as knowing what to tell individuals about their risks.

A group of researchers at the Blood Research Institute in Milwaukee, Wisconsin and University of North Carolina Chapel Hill sought to explain why some people with hemophilia develop inhibitors and some don't. Clearly, inhibitors formed in some people because their body recognizes factor VIII as a foreign protein. Since a person with hemophilia doesn't produce clotting factor during embryonic development, the growing body doesn't have a chance to get used to the protein.

Why do some people with hemophilia escape inhibitors? Part of the reason is that some people make factor proteins that are defective–not complete enough to work or be detected as clotting factor, but with enough of a protein to be recognized by the immune system during embryonic development.

Armed with information about a person's genetic and immunologic factors, it may be possible to predict the risk of inhibitor formation. But what, researchers want to know, does one do with that information?

Environmental Factors

Genetics explains some, but not all, of inhibitor development. Studies with twins, for example, show that these traits are not always shared. Clearly, some other factors are at play, which trigger the formation of inhibitors. Among the environmental factors that have been suggested as culprits are the type of factor VIII product used for therapy, treatment during infections or vaccination and treatment during severe bleeding or surgery.

In the *British Journal of Haemotology*, a group of researchers in Italy conducted a study of 108 children with severe to moderately severe hemophilia A to discern the influence of environmental factors in the development of inhibitors–60 with inhibitors and 48 inhibitor-free controls. To reduce confounding effects, all of the participants were treated with recombinant factor VIII.

A family history of inhibitors and certain mutations in the factor VIII gene were more common in people with inhibitors. On the other hand, no link was seen between inhibitor development and such factors as amniocentesis/villocentesis, prematurity or cesarean birth, breastfeeding, factor treatment during infections or vaccinations, surgical procedures or episodes of severe bleeding.

A further analysis revealed that participants who started prophylaxis at an early age had a lower risk of inhibitors than those treated on demand. "The protective effect on inhibitor development shown by prophylaxis may represent an additional advantage prompting its use in haemophilic children," the researchers concluded.

(White GC, Kempton CL, Grimsley A, et. al.: Cellular immune responses in hemophilia: Why do inhibitors develop in some, but not all hemophiliacs? Journal of Thrombosis and Haemostasis *2005;3: 1676-1681)*

(Santagostino E, Mancuso ME, Rocino A, et. al.: Environmental risk factors for inhibitor development in children with haemophilia A: A case-control study. British Journal of Haemotology *2005; 130:422-427)*

Suppressing a Nonsense Mutation

A number of different types of genetic defects can lead to hemophilia, but one particular kind–a nonsense mutation that causes a premature stop in the encoding of a protein–is responsible for about 9% of the cases.

In a recent issue of the journal *Blood,* a group of researchers report on a proof-of-concept trial using the aminoglycoside antibiotic gentamycin to correct the misread of the gene that produces clotting factor. Aminoglycoside antibiotics work by interfering with the normal activity of ribosomes, causing them to disregard a premature termination and continue the translation of the gene product.

Research suggests that this mechanism may be potentially exploited for the treatment of inherited diseases, such as cystic fibrosis, muscular dystrophy and the tragically debilitating neurological disorder ataxia-telangiectasia.

A small study was conducted with five volunteers–three with hemophilia A and three with hemophilia B. The participants underwent extensive lab testing, including factor and inhibitor studies, serum creatinine, electrolytes and liver enzyme and function tests. Each patient received three consecutive days of gentamycin at a dose of 7 mg/kg intravenously per 24 hours. The participants were watched closely for bleeds.

Testing revealed that two patients showed an increase in their factor levels and a reduction in their aPPT times. For the other three patients, there was no change in factor levels or aPPT, but one showed an increase in factor antigen level.

Although gentamycin is too toxic for an effective treatment, the study provides "a proof-of-principle suggesting that ribosomal interference with a less toxic agent may be a potential therapeutic mechanism for severe hemophilia patients with nonsense mutations."

(James PD, Raut S, Rivard GE, et. al.: Aminoglycoside suppression of nonsense mutation in severe hemophilia. Blood, *2005; 3: 1307).*

Finding the Source of Factor

One of the mysteries of clotting factor is where in the body it is produced. Medicine has known for a long time that factor is made by the liver, but narrowing it down to specific cells or tissue has remained elusive. Recent studies by scientists at Albert Einstein School of Medicine and the University of Pennsylvania indicates that factor is actually generated by endothelial cells–in the liver and perhaps elsewhere in the body.

Researchers studied "knock-out" mice that lack the gene for factor VIII. Liver cells were transplanted into the abdominal

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We appreciate the leadership of the National Hemophilia Foundation in supporting patient access to hemophilia therapies in Pennsylvania.

MASAC Document # 166

MASAC RESOLUTION REGARDING PREFERRED DRUG LISTS

The following resolution was approved by the Medical and Scientific Advisory Council (MASAC) of the National Hemophilia Foundation on October 29, 2005, and adopted by the NHF Board of Directors on October 30, 2005.

The Pennsylvania Department of Public Welfare (DPW) is considering the implementation of a "Preferred Drug List" (PDL) for clotting factor concentrates for hemophilia patients on Medicaid. This proposed policy of the DPW, which may designate only a few factor products as "preferred products" and which requires a strict "fail-first" prior authorization, poses grave concerns for consumers with hemophilia and other bleeding disorders. The lack of therapeutic interchangeability of products, in terms of efficacy, tolerance, and adverse drug reactions, could result in life-threatening bleeding. Moreover, limitations recommended by Medicaid on products authorized by insurers, use of mail-order pharmacies, limitations on use of specialty labs, and limits on number of outpatient visits and inpatient hospitalization days may further threaten optimal health outcomes in those with bleeding disorders. Clotting factor products are licensed by the FDA based on specific indications. Implementation of a PDL could require evidence that such a policy results in no poorer medical outcomes. Finally, the lack of equity with privately insured patients will result in substandard access to treatment in a group of patients with substantial pre-existing economic barriers to optimal health care.

For these reasons, MASAC finds a PDL to be unethical and in direct conflict with good medical care. The characteristics of each product and the resultant product choice for an individual patient require a complex decision-making process, with the ultimate product being agreed upon by the patient and his/her healthcare provider. Institution of a PDL would lead to increased costs and predictable deterioration of health in a group of patients whose health and quality of life have improved in the last decade due to the availability of a variety of safe clotting factors which work best when prescribed on an individualized basis.

This material is provided for your general information only. NHF does not give medical advice or engage in the practice of medicine. NHF under no circumstances recommends particular treatment for specific individuals and in all cases recommends that you consult your physician or local treatment center before pursuing any course of treatment.

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Research Explores Causes of Inhibitor Development

cavity of each animal. Some animals received a mix of hepatocytes, which comprise about 60% of the liver, along with liver sinusoidal epithelial cells (LSECs), Kupffer cells and hepatic stellate cells. Other animals received fractionated hepatocytes or epithelial cells, as well as pancreatic islet-derived islet cells.

Animals who received the mix of cells and pure LSECs showed levels of factor VIII, while mice that received just hepatocytes had none. Similar finds were noted for animals that received pancreatic epithelial cells. None of the animals showed detectable levels of factor circulating in the bloodstream.

The research may settle the question of where in the liver factor proteins originate. In addition, LSECs may provide a target for cellular or gene therapies for hemophilia.

Kumaran V, Benten D, Follenzi A, et. Al.: Transplantation of endothelial cells corrects the phenotype of hemophilia A mice. Journal of Thrombosis and Haemostasis *2005; 3: 2022-2031.*

Delaware Valley Chapter Hosts

Inhibitor Patient Education Summit

November 5 and 6, 2005, Philadelphia

Made possible by a generous grant from Novo Nordisk, The DVC hosted the first consumer meeting in the United States focused on the serious issue of inhibitors for patients with hemophilia.

Individuals with hemophilia and inhibitors, their family members, and healthcare providers attended a much-needed meeting dedicated to making living with an inhibitor much easier for all those affected. With an agenda developed by individuals with hemophilia and inhibitors, advocates, and hemophilia treaters, the meeting helped to educate, generate ideas, and promote greater unity within the inhibitor community.

It seems early to be thinking about sending the kids off to bleeding disorders camp in the summer of 2006, but we wanted to give you lots of time to get organized so that you can be sure your child will have a spot for one of the camp sessions. Here's the buzzzzzzz!!

SPECIAL CAMP NEWS

AND NOW, FOR THE GOOD NEW NEWS!

Spending a week at camp this summer just got easier (and closer!!) for Philadelphia area children with serious conditions, including hemophilia and von Willebrand Disease!!

Dragonfly Forest
your place to soar

Dragonfly Forest is a camp located in Green Lane, Pennsylvania, on hundreds of acres right here in Montgomery County!! AND it has just achieved threshold status with The Association of Hole in the Wall Camps!! ARE YOU READY FOR THIS??!!.....we will be able to send 30 DVC area children with bleeding disorders to one session during the summer of 2006!! The camp has all the amenities of the Newman Camps and is staffed with professionals who understand what kids with bleeding disorders need!!

Once again summer of '06, DVC area kids with bleeding disorders will have an opportunity to attend The Double "H" Hole in the Woods Camp nestled in the mountains in Lake Luzerne, New York. This camp provides an outdoor Adirondack adventure for children with hemophilia and von Willebrand Disease (boys and girls!!) each summer. Modeled after actor Paul Newman's Hole in the Wall Gang Camp in Connecticut, the Double "H" Ranch is a place where children with bleeding disorders can participate in a full range of activities and learn leadership skills, as well. The Delaware Valley Chapter will provide FREE transportation for the following two sessions:

SESSION #1: JULY 3 TO JULY 8, 2006
SESSION #2: AUGUST 4 TO AUGUST 9, 2006

FOR AN APPLICATION, CALL THE DVC OFFICE AT 215-885-6500 OR ASK YOUR TREATMENT CENTER TEAM. THE DEADLINE FOR APPLICATION IS APRIL 1, 2006 AND SPACES ARE VERY LIMITED!!

dragonfly forest session for DVC area kids, august 17–23, 2006

If you are interested in sending your child to Dragonfly Forest for this one session (whether or not they attend a session during 2006 at The Double "H" Hole in the Woods Camp), call the Chapter office right away (215-885-6500)!! We only have room for 30 children so you need to let us know right away!! There may be a charge for each child attending this local camp and all parents must find transportation for their child. We will not have a bus.