

*Education  
Support  
Research*

*Fall 2009*

# Aplastic Anemia & Myelodysplasia Association of Canada

# NEWSLETTER

## President's Message

Well it's been an extraordinarily cool and wet summer in Toronto while the West is dealing with a heat wave and numerous forest fires. Throughout the unusual weather, AAMAC has continued to be active and busy.

We just recently launched our new, redesigned website – the website address is the same, [www.aamac.ca](http://www.aamac.ca). The work of maintaining and refining our website takes a tremendous amount of energy and time and we owe a huge debt of gratitude to volunteer Paul Sue. He has contributed many hours of work in redesigning our site and he deserves much credit for a job well done. I would also like to acknowledge the important contribution of Chris Meyer and Silvia Marchesin for help expanding the website content.

The patient support group that was organized in Ottawa this past June has held a couple of well attended meetings. Support for the meetings is being provided by both physicians and caregivers from the Children's Hospital of Eastern Ontario and from the Ottawa General Hospital. Volunteer Bob Ross is doing a wonderful job of organizing the meetings and taking charge of the communications and logistics with AAMAC support provided by Ontario Chapter Coordinator, Andy May. We can use the knowledge gained in setting up the Ottawa group to begin similar support groups in other areas. Several contacts have been made in the Montreal area and we hope to initiate a meeting of interested patients and local physicians and support worker in the near future. Regular support group meetings are an excellent opportunity for patients to share their experiences and provide another layer of support and community. Support groups are also an opportunity for AAMAC to gain better contact with patients that we represent and be able to respond quickly to their needs. If anyone has interest in starting a support group in their area please contact AAMAC at 1 (888) 840-0039 or [info@aamac.ca](mailto:info@aamac.ca). We are more than willing to provide support, guidance, information – whatever help that is required to get things started.

A considerable amount of our resources are being deployed in advocating for access to new drugs. Some exciting new therapies are coming to market and there is a need to make the politicians, bureaucrats, and the public at large aware of the potential for these new drugs to make a dramatic impact on the lives of patients and give physicians new tools in the battle against bone marrow failure diseases.

We recently met with Ontario Ministry of Health and Long-term Care representatives to discuss some problems with cyclosporine access for aplastic anemia patients and some excellent solutions were discussed and implemented which will certainly improve the process for patient access to cyclosporine. Helen Stevenson (Executive Officer, Ontario Public Drug Programs) and her staff are to be commended for their willingness to give audience to physicians and rare disease patient groups in a team effort to provide better care for patients. This approach can serve as a model to other provinces on recognizing the special needs of rare diseases.

Sincerely,  
Stan Chmelyk  
President, AAMAC



**Aplastic Anemia  
&  
Myelodysplasia**  
ASSOCIATION OF CANADA

The Aplastic Anemia and Myelodysplasia Association of Canada (AAMAC): provides information about aplastic anemia, myelodysplasia and PNH to the public; operates a nationwide support network for patients, families and medical professionals; supports Canadian Blood Services blood programs and the Unrelated Bone Marrow Donor Registry; and raises funds for medical research.

#### **BOARD OF DIRECTORS 2008**

Rolla Bahsous, Newsletter Editor  
Reanne Booker  
Anna Chamrai, Treasurer  
Stan Chmelyk, President  
Janice Cook  
Robert MacDonald  
Silvia Marchesin  
Andy May  
Chris Meyer  
Doug Mylie  
Trevor Thompson  
Pam Wishart, Secretary

#### **MEDICAL AND SCIENTIFIC ADVISORY COMMITTEE**

Dr. S. Couban  
Dr. Y. Dror  
Dr. A. Eaves  
Dr. V. Gupta  
Dr. L. Larratt  
Dr. K. Schultz  
Dr. R. Wells, Chair

#### **CHAPTER CO-ORDINATORS**

Janice Cook/Pam Wishart, BC  
Doug Mylie, Alberta  
Andy May, Ontario  
Robert MacDonald, Atlantic

#### **OTHER**

Lois Henderson, Admin Assistant

The Aplastic Anemia & Myelodysplasia Association of Canada Newsletter is published 3-4 times a year. The contents are not intended to provide medical advice, which should be obtained from a qualified health professional. No part of this publication may be used or reprinted without written permission. For submissions, inquiries or comments, please contact us at:

Aplastic Anemia and Myelodysplasia  
Association of Canada  
11181 Yonge Street, Suite 321  
Richmond Hill Ontario L4S 1L2  
(905) 780-0698 or 1 (888) 840-0039  
info@aamac.ca www.aamac.ca

Charitable Registration Number  
87557 2265 RR001

## **Alberta Chapter Update**

*By Doug Mylie*

The last few months have been busy with an increase in recently diagnosed patients and family members looking for support and information from AAMAC. We were able to match these individuals with a peer support person in every instance. That support is such an important resource for people who are just starting to navigate through the process of dealing with these illnesses. The listening ear and friendly voice of someone who may not be part of the medical intervention, but someone who understands the issues from experience, can be very comforting.

In early June we had an informal support meeting in Sherwood Park. This was an opportunity for those in attendance to meet other patients and family members. There was lots of discussion and shared stories. This meeting went very well and another is being planned for Sunday, October 4, 2009 at 1:00 p.m. in Sherwood Park. If anyone is interested in attending please contact me at [alberta@aamac.ca](mailto:alberta@aamac.ca).

If anyone would be interested in hosting a similar meeting in their community, please contact me at the above e-mail and I would be happy to help with getting that to happen.

## **Ontario Chapter Update**

*By Andy May*

Here in Ontario we are wondering what happened to summer. Actually even with the weather being up and down it is still a wonderful time to spend with family and friends.

**Ottawa Support Group...**by the time this article gets out there will have been several support group meetings in Ottawa. Congratulation to all involved in helping this group get off the ground. If anyone wishes to

participate, you can contact Bob Ross at [bob@sand-box.ca](mailto:bob@sand-box.ca) or Katherine Knoll at [asi@sympatico.ca](mailto:asi@sympatico.ca). After attending the initial support group meeting with AAMAC's President Stan Chmelyk it was easy to see that this group, being full of enthusiasm, will be a great success. Bob Ross indicates that there are already just over 25 participants that include patients, medical staff, family and friends.

**Spring Swing 2010...**Mark your calendars...it's never too early to start planning for next year's Spring Swing fundraiser. Spring Swing will be at the Meridian Banquet Hall again on Saturday, April 17, 2010. If anyone is interested in volunteering please let me know at [themays@rogers.com](mailto:themays@rogers.com) or contact this year's event co-chair Caroline Laughlin at [wclaughlin@rogers.com](mailto:wclaughlin@rogers.com). In order for the fundraiser to be successful we require silent auction items. If you wish to donate an item we would really appreciate it, please don't wait. Please contact Caroline or myself.

**Reminder...**AAMAC depends on financial support from individuals like you. Please visit our web site and click on the Donate Now link to make a contribution today.

**Donation from Kirkland Lake...**a big "Thank You" goes out to Melissa and Joe Coupland who made a \$500.00 donation on behalf of their daughter Taylor to AAMAC after hosting a local fundraiser. Kirkland Lake and surrounding friends held a concert in support of Taylor. Taylor is fighting aplastic anemia and we wish her and her family all the best.

**In closing...**if you are able please give blood. Visit Canadian Blood Services at [www.blood.ca](http://www.blood.ca).

If you have any comments, concerns or events you would like posted in the next newsletter or on our web site, please contact me.

## BC Chapter Update

By Janice Cook

As I write this most people in BC are sweltering under a record breaking heat wave, 38 degrees with high humidity and smoky air today where I live. A dragonboat team from Victoria is actually training in these conditions, and this year they plan to carry our banner on their boat. The team has chosen AAMAC as their charity of choice. Lisa Ross and her boyfriend Doug Bacon are teaching the team about bone marrow failure disease and PNH in particular and raising money for us by holding a garage sale, dinner and auction, and all will be wearing Lifeblood Bracelets during the team's races this summer. So if you have any dragonboat festivals in your area of BC this summer why not get out there to meet and thank the team for their efforts, and cheer them along.

Our best wishes go out to Cindy Bell on her wedding day in August. Cindy was BC Chapter Coordinator for fifteen years and is well known by many BC chapter members. She remains active in the chapter, and is currently involved in advocacy for Exjade.

We are waiting to hear of the BC government's decision on funding of Exjade for those who experience iron overload as a result of blood transfusion. The BC chapter of AAMAC has written two letters supporting funding, and if you wish to help by sharing your story about life with Exjade or Desferal please contact me at [cookjan@telus.net](mailto:cookjan@telus.net) or [bc@aamac.ca](mailto:bc@aamac.ca).

## Atlantic Chapter Update

By Gwen Barry

Atlantic Chapter has held several extra meetings in the past few months in preparation for Education Day. Facilities and speakers have been finalized. Posters about Education Day have been distributed far and wide throughout Atlantic Canada.

Our Chapter meets regularly at 3 p.m. on the last Wednesday of each month, from September to June, in the VON offices in the Halifax Shopping Centre. All those in Atlantic Canada with MDS, AA, and PNH and their families and caregivers are welcome to join us. For further information, contact Robert MacDonald at [rob.jack@ns.sympatico.ca](mailto:rob.jack@ns.sympatico.ca) or (902) 443-1615.

## 2009 Aplastic Anemia/Myelodysplasia Nursing Grant

By Reanne Booker

The Canadian Nurses Foundation (CNF) is a national non-profit charitable organization that was founded in 1962 by the Canadian Nurses Association. Through the support of nursing education and research, the association endeavors to promote health and quality care for Canadians. The CNF has more than 25 research partners throughout Canada and provides support to each province via scholarships for nurses at the baccalaureate, masters and doctoral levels.

AAMAC has supported a CNF nursing grant since 2000. The grant is awarded by the CNF to individuals pursuing graduate nursing studies who specialize in oncology/hematology or who have oncology/hematology as the focus of their research.

The 2009 AAMAC Nursing Grant Recipient is Ms. Sonia Joannette. Ms Joannette began her nursing career in pediatric oncology in 1999. She has taken advanced educational courses on radiation oncology and has a special interest in geriatric oncology. Ms. Joannette is also a lecturer at the School of Nursing at the University of Sherbrooke. She is involved in an array of professional activities including participation on the board of directors of the Quebec Association of Oncology Nurses and as well has been involved with the Centre intégré de cancérologie de la Montérégie (CICM). Her academic goals include the completion of her master's degree

before proceeding on to pursue a PhD in nursing.

## Revlimid Approval in Alberta!

By Stan Chmelyk

As the newsletter goes to press we have just received notice that Revlimid has now been added to the formulary for MDS patients in Alberta. Alberta has agreed to reimburse Revlimid using the same criteria in Ontario, and this should lead to the broadest possible availability for MDS patients with deletion of the 5q chromosome.

Public reimbursement is now available in Alberta, British Columbia, Quebec and Ontario. Combined with the various private insurance companies that provide reimbursement, more than 90% of Canadians in need of Revlimid for MDS should now have access. However, there are still six other provinces that need to come on side to complete the picture and our advocacy efforts will need to focus on them.

We also understand that Vidaza, a new drug shown to increase survival for patients suffering with Intermediate 2 high risk MDS, may soon be available in Canada. Once again, the advocacy efforts of AAMAC will be essential to ensure that patients in need across Canada will be provided with access to this important new therapy. Stay tuned for further information and we hope to count on all of you for your ongoing support in our advocacy efforts.

## Wellspring Program Focuses on Financial Planning

By Chris Meyer

In early June, Pamela Bowes and Ilene Shiller from Wellspring, a network of centres providing cancer support, presented "Where Will the Money Come From if I'm Too Sick to Work." This was a very interesting overview of the income sources available to Ontarians who are unable to work any longer due to a serious illness.

We sincerely appreciated Pamela and Ilene taking the time to speak to our small group. Special thanks also to Lynn and Mark Buchanan who volunteered to pull together a wonderful spread for the supertime presentation and to Wellspring's Sunnybrook location for hosting the event.

Ilene explained how different government programs work and when you might access each. She also talked about short-term and long-term disability programs from employers and the use of personal assets for income since available programs have limitations.

Pamela and Ilene shared that Wellspring recently launched a Money Matters program in downtown Toronto that allows patients to book an individual appointment to discuss their situation and the options available to them. In addition to reviewing potential income resources, they can help create an individualized financial plan, advocate with government programs and refer patients to other programs and services. Staff will help patients fill out government forms and applications including those for the Trillium Drug Program and Canada Pension Plan. To find out more about Money Matters, visit [www.wellspring.ca](http://www.wellspring.ca) or contact (416) 323-6400, ext. 4240.

Although the Money Matters program is new and currently offered only in select Toronto area locations, Wellspring offers many other helpful programs. I would encourage patients to contact their local Wellspring or other cancer support centre to find out if there are programs that may help them cope with their bone marrow failure disease. There is a connection to cancer as some patients with aplastic anemia, MDS or PNH can develop leukemia. As well, many of the issues and treatments our patients face are similar to those faced by cancer patients. Wellspring has opened its doors to Toronto area AAMAC members for more than five years as one of our primary support group venues and as a result many

patients have benefitted from the organization's generosity.

There are locations across Ontario in Toronto, Brampton, Oakville, London, and Niagara, and satellite programs in Newmarket and Alliston. As well, a new location just opened in Calgary and locations are planned in Ottawa, Halifax and Edmonton. Programs vary by site. For details contact Wellspring on its toll-free line at 1 (877) 499-9904.

## Action Day on Parliament Hill for YOU

*By Caroline Laughlin*

On April 30, 2009 Stan Chmelyk, President of AAMAC, Patti Chmelyk, Caroline Laughlin and Bill Laughlin spent the day meeting with Members of Parliament (MPs) advocating for you and other patients with rare disorders to receive better access to urgently needed drugs. Organized by the Canadian Organization for Rare Disorders (CORD), we and other concerned citizens spoke to MPs regarding the urgent need for an Orphan Drug Policy here in Canada. Canada is the only country in the developed world that does not have such a policy.

According to CORD, nearly three million Canadians are affected by one of the 6,000 known rare disorders, most of which are severe and debilitating, leading to early death or disability. Canadian patients with rare disorders have delayed access to treatment or cannot access drugs available to patients elsewhere due to regulatory hurdles. Only a fraction of drugs approved for these illnesses in Europe (less than 1/3) and in the US (less than 1/5) are brought to Canada. Criteria for access are more stringent in Canada than anywhere else: so many patients cannot access treatment. Current programs such as Health Canada's Special Access Programme, do not adequately address the medical needs of Canadians with rare disorders.

After fighting for some action since the 1980s, a motion was passed by the House of Commons on May 7, 2008. The House unanimously approved Liberal Don Bell's Motion 426 calling on the government to 'respond specially to the challenges faced by Canadians with rare diseases and disorders.' No action has been taken thus far.

So on April 30, we advocated to Canadian MPs to change this situation so that all Canadians will have equal access to the drugs that will make their lives easier and healthier. I met with two MPs and one Senator who all agreed in principle that something needed to be done, however all said that there are many other issues that are equally or more important. This should not be an acceptable response to Canadians who are in desperate need of special drugs.

AAMAC is committed to helping others win this fight for a National Orphan Drug Policy and we will win.

For more information see [www.raredisorders.ca](http://www.raredisorders.ca).

## Cross Canada Educational Calls

*By Chris Meyer*

In June, we held our first educational telephone call on the topic of iron chelation with Dr. Rena Buckstein. Patients simply phoned a toll-free telephone number, entered a code using the numbers on their touch tone phone and listened to the presentation.

There was ample opportunity for questions and Dr. Buckstein gave patients practical suggestions to discuss with their doctor – from medications that can reduce some side effects of chelation therapy to the kinds of regular tests that patients should be receiving while taking chelation medication.

The call was a huge hit judging by the feedback from the patients who joined the phone call from Alberta, Ontario

and Nova Scotia. Thank you Dr. Buckstein for such an informative presentation!

For those who missed the call, Dr. Buckstein will be speaking on the topic at our upcoming Education Day in Halifax, Nova Scotia.

We hope to offer more of these educational calls in the future because there are a number of benefits.

- There is no cost to patients who phone a toll-free phone number.
- The cost for the association to host the call is very low.
- People can listen to the presentation no matter where in Canada they live.
- Patients with neutropenia or severe fatigue can participate from the comfort of their own home.
- Future presentations can be recorded and played back from our website for anyone who wants to listen at another time.

If you have any suggestions for future topics, please let us know.

## Join the “Marrow Movers” Walking for AAMAC

*By Susan McVeigh and Chris Meyer*

Sunday September 27, 2009 is the 20<sup>th</sup> Scotiabank Toronto Waterfront Marathon – fast, flat and festive! AAMAC is one of the official charities that will use the five kilometre walk/run as a fundraiser. It is the first time we will be participating, as the ‘Marrow Movers’.

Beginning at 10:20 a.m. inside the Princes’ Gate of the Canadian National Exhibition, the short and scenic distance finishes at Toronto City Hall’s Nathan Phillips Square in time to meet friends for brunch. A brisk walk several times a week is usually enough to get you prepared for a 5K route. This is an ideal event for the whole family to support — there are no age restrictions!

You can register online for the walk, and even the fundraising. Donations

can be made online or you can download a pledge form from either the race website or our own site, [www.aamac.ca](http://www.aamac.ca).

Let’s make this a cross-country event! Online pledging means you can support this fundraising effort by rounding up support for any of the walkers/runners registered to support AAMAC. You don’t have to be in Toronto to participate!

Join the ‘Marrow Movers’ today!

- Each Marrow Mover receives a red and white “Lifeblood” bracelet.
- Raise \$250 or more and we’ll cover your entry fee for the 5K walk.
- Walk with your family – the first 12 children age 13 or under who join the walk will each receive one Webkinz® pet courtesy of GANZ. (Just email or phone Susan with your child’s name.)
- The Marrow Mover who raises the most funds for AAMAC this year will receive a \$50 movie gift certificate.
- Let us know if you are walking in honour or memory of someone and we’ll include their name in our national newsletter.

Register at:

[www.torontowaterfrontmarathon.com](http://www.torontowaterfrontmarathon.com) and contact volunteer Susan McVeigh at 416-251-9552 or [susanbmcveigh@hotmail.com](mailto:susanbmcveigh@hotmail.com) if you have any questions.

## Many Ways to Give

*By Silvia Marchesin*

The association is fortunate to be supported by a dedicated group of individual donors. A number of members have chosen to include the association in their estate planning. Recently the association was named as the beneficiary of a charitable remainder trust. This trust was established by Mrs. Lorraine Hill of Ottawa and we spoke with her briefly about this.

**AAMAC:** What prompted you to make this gift to AAMAC?

**LH:** My husband Don suffered from MDS for a number of years. I had never heard of your organization and it was only after his death, while reading the obituaries, that I learned your organization existed.

I was in the process of establishing a foundation in the memory of my husband.

It (MDS) is such a frustrating disease. There is still a great deal of research to be done. So many other organizations are better funded and known. Our organization [AAMAC] needs more exposure so that others can learn of it.

**AAMAC:** How did you go about establishing this gift?

**LH:** I dealt with my bank with the assistance of my lawyer

**AAMAC:** Is there anything else you’d like to say?

**LH:** I would encourage others to consider leaving money in their will.

I am pleased knowing that the money will go to meet unmet needs. At the time, there was very little that could be done for my husband. Hopefully someday there will be more options for MDS patients.

The conversation ended with Lorraine extending her best wishes to the association.

Research, education and support are the foundation of what we do at AAMAC. Generous donations such as this allow us to not only continue the work of the association, but hopefully to expand the services we provide to patients, and to fund significantly more research into bone marrow failure disease.

There are many options when it comes to making gifts to AAMAC. If you are interested supporting the mandate of the association, please speak with your lawyer to determine the type of gift that works best for you.

## **What is a Charitable Remainder**

**Trust?** A charitable remainder trust is a powerful planning tool for people who are wishing to make a charitable bequest. It is a legal agreement that specifies how investments placed in the trust will be managed by a trustee. A charitable remainder trust is an effective way to provide you with an income for life and know that upon your passing, or after a specified period of time, the capital will go to the charities of your choice. This irrevocable gift can be funded with cash, real estate or securities.

**How does it work?** Basically, a trustee manages your investments. The trust agreement outlines how you will receive income from the investment during your lifetime, or for a specified period. It also outlines which charities the remainder will go to upon your death.

There are numerous advantages of this type of arrangement. The main one is that you get a tax receipt from the charity once the trust is established which can result in an immediate tax savings.

There are a number of websites that describe the charitable remainder trust. One that you can refer to is <http://rulelaw.blogspot.com/2008/01/charitable-remainder-trusts.html>.

## **Influenza A H1N1 And You**

*By Janice Cook*

Influenza A H1N1, also known as human swine influenza, is something that has been in the news since spring 2009. We'll be hearing much more about it as fall approaches, because fall/winter is flu season in the northern hemisphere. This new virus has spread around the world much quicker than other influenza viruses and is being closely watched by Canadian public health, and by world health organizations.

H1N1 has symptoms similar to common influenza: high fever, cough, headache, body aches, fatigue, eye

pain, loss of appetite, and shortness of breath. Less common symptoms are runny nose, sore throat, nausea, vomiting and diarrhea. Most people who get H1N1 will be managed at home without seeing a doctor, and recover within a week. A few get very sick and need hospital admission. Influenza is a viral illness and does not respond to antibiotics; most cases do not need antiviral drugs. H1N1 is spread person to person through coughing/sneezing by people infected with the virus. It can also be spread by touching something with the virus on it (eg., doorknob, counters) and then touching your eyes, nose or mouth.

The precautions that people are being told to follow to help reduce the chances of catching H1N1, or any other virus spread by droplet or airborne, are basic precautions that should really be always followed by those with chronic illness (including bone marrow failure disease) and by those who are immune-compromised (those on Cyclosporine, chemotherapy, post-BMT patients, and those with neutropenia).

Basic precautions include:

- 1) Wash hands frequently with soap and warm water, especially after a cough or sneeze, or after touching surfaces.
- 2) Use hand sanitizer when you can't wash your hands (carry your own).
- 3) Cover nose and mouth with tissue when coughing or sneezing. Throw used tissues into the trash immediately, and wash or hand sanitize right away. Remind others to do the same.
- 4) Use your upper arm or sleeve if no tissue is available.
- 5) Avoid touching eyes, nose and mouth.

People with bone marrow failure disease should ask their hematologist about getting a flu shot. Their families and friends should, as always, stay

away if they have any of the symptoms of influenza. In BC, health care workers are being told to stay away from others for seven days from the start of symptoms or until the fever is gone, whichever is longer. If you come in contact with someone who has H1N1, you should monitor yourself for symptoms, including fever and cough, and contact your doctor if these develop. In many communities there is or will be a nurse line or even a "flu" line where you can call if you are unsure, but your own doctor knows you best.

Even without H1N1 in the picture, people with bone marrow failure disease have to sometimes go to labs, clinics and even hospital emergency rooms for tests or treatment. If you are immune-compromised, always carry hand sanitizer, and inform the staff as you arrive so that you can be given a mask to wear. In some places you'll need to take your own. In these days of crowded emergency waiting rooms and long waits to be seen, it is especially important for the triage nurse to know your health situation so she can try and arrange where you will be best placed. And always remember to frequently wash your hands with soap and water because everyone else might not. Listen for updates on H1N1 in Canada and follow public health guidelines.

## **Calling Corporations**

Celgene Canada has challenged corporations across Canada to support AAMAC's Spring Swing event in April 2010. Celgene will match other companies that sponsor or donate to the event up to a total of \$25,000. If you know a company that might make a donation for Celgene to match please contact us.

## **News of Note**

**Attention pediatric patients and parents at the Hospital for Sick**

**Children, Toronto:** Volunteer Caroline Laughlin will be attending Dr. Dror's Monday morning clinics once a month. Please come and talk to her as she will be open to listening and answering any questions regarding aplastic anemia or myelodysplasia.

## Mark Your Calendars

**AAMAC Education Day 2009, Halifax, Nova Scotia:** This year's AAMAC National Education Day will be hosted by the Atlantic Chapter, and will be held in Halifax on October 24. It is an excellent opportunity for those with MDS, AA, and PNH, and their families and caregivers, to learn about these diseases from the experts.

Registration is free, and some financial reimbursement is available for travel costs. Education Day will be held in the Atlantica Halifax Hotel (formerly the Holiday Inn Select). A special room rate of \$135 per room per night is available for attendees. The deadline for room reservations at this reduced rate is Sept 23. The hotel can be reached at 1 (888) 810-7288 or locally (902) 423-1161. Halifax hotels are very busy during the fall conference season, so no matter where you plan to stay, reservations should be made early.

**Further details on the Education Day agenda, and the registration form can be found in this issue of the AAMAC Newsletter.**

*See Chapter Updates for local events.*

## **Soliris™ (Eculizumab), the First and Only Proven Therapy for the Treatment of Patients with PNH, is now available in Canada**

Until recently, patients with paroxysmal nocturnal haemoglobinuria (PNH)—an ultra-rare, progressive, and life-threatening blood disorder—had no therapeutic options to address this devastating disease. But now, Soliris™ (eculizumab), a new treatment option for PNH, is available in Canada.

Soliris is the first and only approved medication proven to reduce haemolysis, the underlying cause of the clinical manifestations of PNH. PNH is a progressive and destructive disease that can lead to thrombosis, end organ damage, impaired quality of life—independent of anaemia/transfusion requirements—and mortality.

A genetic mutation in PNH patients leads to the creation of abnormal blood cells that do not have terminal complement inhibitors, making the cells sensitive to destruction by the protein complement. The destruction and loss of these oxygen-carrying red blood cells results in anaemia, as well as fatigue, difficulty in functioning, pain, dark urine, shortness of breath and blood clots.

The safety and efficacy of Soliris in PNH patients has been demonstrated in three multi-national clinical trials: TRIUMPH, SHEPHERD, and E05-001, a long-term extension study. The studies determined that patients with PNH who received Soliris experienced a number of benefits including an immediate and sustained reduction in chronic haemolysis<sup>1,1</sup>, fewer blood clots, significant improvements in fatigue levels and overall quality of life<sup>1,1</sup> and a significant reduction in the need for transfusions<sup>1</sup>.

In January 2009, Soliris was approved under priority review by Health Canada. Soliris has also been recognized on the international stage for scientific innovation and the positive impact the drug is having on the lives of patients with PNH. Soliris received both the 2009 Prix Galien International Award in the category of medicines for rare diseases and the 2008 Prix Galien USA Award for Best Biotechnology Product.

Any patient who is diagnosed with PNH will have access to Alexion Pharma Canada's OneSource™ program, a personalized service for patients with PNH and their health care providers. Each patient who enrolls in OneSource is assigned a specific OneSource Case Manager who supports the patient immediately and over time, providing a broad range of services from disease education to coordinating care with the physician's office. Case managers also work with patients and physicians to secure access to Soliris and identify funding options when needed. All case managers are registered nurses who have extensive experience caring for patients.

For more information please contact 1 888 SOLIRIS (888-765-4747) or email Alexion Pharma Canada at [AlexionPharmaCanada@alxn.com](mailto:AlexionPharmaCanada@alxn.com).

<sup>1</sup> Hillmen P, Young NS, Schubert J, Brodsky RA, Socie G, Muus P, et al. The complement inhibitor eculizumab in paroxysmal nocturnal hemoglobinuria. *N Engl J Med* 2006 Sep 21;355(12):1233-43.

<sup>1</sup> Brodsky RA, Young NS, Antonioli E, Risitano AM, Schrezenmeier H, Schubert J, et al. Multicenter phase 3 study of the complement inhibitor eculizumab for the treatment of patients with paroxysmal nocturnal hemoglobinuria. *Blood* 2008 Feb 15;111(4):1840-7.

<sup>1</sup> Hillmen P, Young NS, Schubert J, Brodsky RA, Socie G, Muus P, et al. The complement inhibitor eculizumab in paroxysmal nocturnal hemoglobinuria. *N Engl J Med* 2006 Sep 21;355(12):1233-43.

<sup>1</sup> Brodsky RA, Young NS, Antonioli E, Risitano AM, Schrezenmeier H, Schubert J, et al. Multicenter phase 3 study of the complement inhibitor eculizumab for the treatment of patients with paroxysmal nocturnal hemoglobinuria. *Blood* 2008 Feb 15;111(4):1840-7.

<sup>1</sup> Brodsky RA, Young NS, Antonioli E, Risitano AM, Schrezenmeier H, Schubert J, et al. Multicenter phase 3 study of the complement inhibitor eculizumab for the treatment of patients with paroxysmal nocturnal hemoglobinuria. *Blood* 2008 Feb 15;111(4):1840-7.

## Cyclosporine Access for AA and PRCA Patients

*By Stan Chmelyk*

AAMAC recently became aware that there were some delays and difficulties in getting approval from the Ontario Ministry of Health's Exceptional Access Program for cyclosporine for aplastic anemia (AA) and pure red cell aplasia (PRCA) patients. A meeting was arranged with Ministry officials and the issue was discussed. The following is the text of a letter that Dr. Vikas Gupta drafted and AAMAC sent out to about 165 hematologists in Ontario as a result of action items that arose from that meeting. We are grateful for Dr. Gupta's assistance.

12 July 2009

### **Re: Approval of cyclosporine for patients with aplastic anemia (AA) or pure red cell aplasia (PRCA) through Exceptional Access Program (EAP)**

Dear Colleague,

It has been brought to our attention that there have been recent difficulties in obtaining the approval of cyclosporine for patients with AA or PRCA. The main reasons for these delays or rejections have been because the manufacturer of cyclosporine has not submitted clinical evaluation of the drug (CED) for the above indications. Therefore, at present the drug is only available through the compassionate review program. The paperwork for compassionate review program is time consuming and complex.

On behalf of the Aplastic Anemia and Myelodysplasia Association of Canada (AAMAC), we met with director Brenda Kritzer and managers of the Exceptional Access Branch (EAB) on June 16 to discuss the issues regarding EAP turnaround times for AA and PRCA. Various ways were discussed to avoid the time consuming paper work and the unnecessary delays. It was agreed that including the following information with the original letter from the treating physician would allow for a more expedited turnaround time.

Therefore, we suggest including the following information and supporting documents in your request for cyclosporine for AA and PRCA.

#### **For both AA and PRCA:**

- Bone marrow aspirate/biopsy
- Serial CBC and retic count
- Is patient transfusion-dependent?
- Past and current treatment plan
- If the patient has been on CsA, what was their response (i.e. before and after CBC, was there decreased transfusions)?

#### **For AA, also include, if possible:**

- PNH screen and autoimmune tests

#### **For PRCA, also include:**

- Past drug history and if possible, autoimmune tests

We sincerely hope that this process will allow for approval of cyclosporine in a timely manner. If you experience any difficulties, please contact us.

Yours sincerely,



Vikas Gupta, MD, MRCP, FRCPath  
AAMAC Medical and Scientific Advisory Committee Member  
Assistant Professor, Department of Medicine  
Staff Physician, Leukemia/Blood & Marrow Transplant Program  
Princess Margaret Hospital, Toronto



Stan Chmelyk  
President, AAMAC



**Aplastic Anemia and Myelodysplasia Association of Canada  
Education Day, October 24, 2009  
Preliminary Program**

**8:00 a.m. – 4:00 p.m.**

Atlantica Hotel Halifax, 1980 Robie Street, Halifax, Nova Scotia

**Registration & Breakfast**

<b>Welcome</b>	<i>Stan Chmelyk, AAMAC President</i>
<b>Overview of Myelodysplasia</b>	<i>Dr. Stephen Couban, Queen Elizabeth II Health Sciences Centre, Halifax</i>
<b>Patient Testimonial</b>	
<b>Overview of Blood and Marrow Transplantation</b>	<i>Dr. Stephen Couban, Queen Elizabeth II Health Sciences Centre, Halifax</i>
<b>Role of Nurse Practitioner</b>	<i>Jo-Anne Edwards, Queen Elizabeth II Health Sciences Centre, Halifax</i>
<b>Pediatric Perspectives on Childhood Marrow Failures</b>	<i>Dr. Conrad Fernandez, IWK Health Centre, Halifax</i>

**Annual General Meeting & Lunch**

<b>Patient Testimonial</b>	
<b>Overview of Aplastic Anemia in Adults</b>	<i>Dr. Andrea Kew, Queen Elizabeth II Health Sciences Centre, Halifax</i>
<b>Should I be Worried about Iron?</b>	<i>Dr. Rena Buckstein, Sunnybrook Health Sciences Centre, Toronto</i>
<b>PNH Overview</b>	<i>TBA</i>
<b>One Match Program</b>	<i>Sue Smith, Canadian Blood Services, Halifax</i>
<b>Wrap up</b>	

**Platinum Sponsor:**



**Gold Sponsor:**



**Gold Sponsor:**



# Registration Form - Education Day

Saturday, October 24, 2009  
8:00 a.m. – 4:00 p.m.

Atlantica Hotel\*, 1980 Robie Street, Halifax, NS



## Contact Information

Surname:	First name:
Title:	
Institution/Organization:	
Address:	City:
Province:	Postal code:
Telephone (Res.):	Telephone (Bus.):
Fax:	Email:

## Profile (please check all applicable)

<input type="checkbox"/>	Patient, family member or friend <input type="checkbox"/> Aplastic Anemia <input type="checkbox"/> Myelodysplasia (MDS) <input type="checkbox"/> PNH
<input type="checkbox"/>	Representative of patient organization Name of organization: _____
<input type="checkbox"/>	Physician
<input type="checkbox"/>	Health professional
<input type="checkbox"/>	Industry representative

Breakfast, coffee breaks, and lunch will be provided.

There are a limited number of travel sponsorships available to a maximum of \$300 per person. If you require financial assistance to attend Education Day, please indicate this below. The purpose of the sponsorship is to assist patients and families from outside of Halifax to attend. We will let you know as soon as possible if your sponsorship is approved. You will be required to bring receipts to Education Day for reimbursement at the event.

## Travel Sponsorship

<input type="checkbox"/>	Yes, I require a travel sponsorship.
--------------------------	--------------------------------------

**There are no fees to register for the Education Day, however you must pre-register.** The deadline for registration is **October 15, 2009**. Please submit registration forms to:

Janice Cook 8538 Cade Barr Street Mission, BC V2V 6Y2	E-mail: cookjan@telus.net Phone: (604) 826-7222
---	--

For more information, contact:

Janice Cook, Registration Coordinator, (604) 826-7222, cookjan@telus.net, or  
Robert MacDonald, Education Day Co-chair, (902) 443-1615, rob.jack@ns.sympatico.ca

\* formerly the Holiday Inn Select