AAMAC
ACAAM

Aplastic Anemia & Myelodysplasia Association of Canada

Association canadienne de l'anémie aplasique et de la myélodysplasie

# Newsletter - Spring 2024

***for patients living with AA, MDS, PNH and the caregivers who support them***

## Message from the Executive Director

Welcome to another news update, we have some wonderful articles to share in this newsletter.

AAMAC has always been involved in research on MDS, Aplastic Anemia and PNH and we are happy to announce two new research projects. It is always exciting to see new research that will look to improve the lives of our patients. Dr. Philip Eappen, the Chair of our Research Committee outlines these projects in his update.

Plans are in place for our three spring meetings Toronto on April 20th, Vancouver, and Halifax on May 4th. We will have a talk on MDS at each of these meetings; however, for the second speaker we will be focusing on how to live life well with a chronic disease. There will be breakout sessions for patients and care partners. Feedback from our 2023 meetings told us you wanted to hear from patients and their stories, so at each meeting we will be hosting a patient panel. There will be lots of opportunity to ask questions and meet one another to talk. Keep an eye out for fall dates in Edmonton, Winnipeg, and St. Johns, Newfoundland. We will be announcing those dates soon.

Another project that we have been working on is Educational Books on the three diseases. AAMAC has never published our own books and we are excited to be able to have these important resources for patients. They should be ready to launch in the spring. They will be available in hard print and electronically. We hope to have them available at the spring meetings and of course you can always order one and we will send it out to you.

Have you attended one of our online support groups? They are a wonderful way to learn more through shared experiences with other patients and care partners. Details are available on the website about time and registration.
I hope you enjoy this issue of our newsletter and if you have suggestions for future articles, please let us know info@aamac.ca

Cindy Anthony

## IN THIS ISSUE

# PATIENT INPUT SHAPES CADTH REVIEW OF ELTROMBOPAG

Patients with severe aplastic anemia (SAA) will be pleased to hear the latest draft recommendation from CADTH, Canada's Drug and Health Technology Agency. CADTH's Formulary Management Expert Committee (FMEC) was asked by publicly funded drug plans to conduct a reimbursement review of the drug, eltrombopag. As part of its review process, the FMEC receives input from people with lived experience with the drug, the pharmaceutical industry, clinicians, and the administrators of public drug plans.

AAMAC assisted in the review by asking board member, Philip Veness, to share his experience with the drug. Philip spoke to the committee via Zoom, providing information about his journey with SAA over the past three years and the role eltrombopag has played in his well-being.

As a result of the review, the FMEC recommends eltrombopag in combination with immunosuppressive therapy (with horse antithymocyte globulin and cyclosporine) be reimbursed in previously untreated patients with severe or very severe AA, if clinical conditions are met. The recommendation will be finalized once the final review period closes.

Philip was very pleased to hear the draft recommendation and expressed gratitude that he was able to play a small part in the successful review. He encourages other patients to take advantage of opportunities to share their lived experiences: "It is very important that we come forward to tell our story in these situations."

You can read the draft recommendation and find out more about the review timeline here: *https://www.cadth.ca/eltrombopag-1**

# PROFILE OF AN AA SURVIVOR – ASHLEY

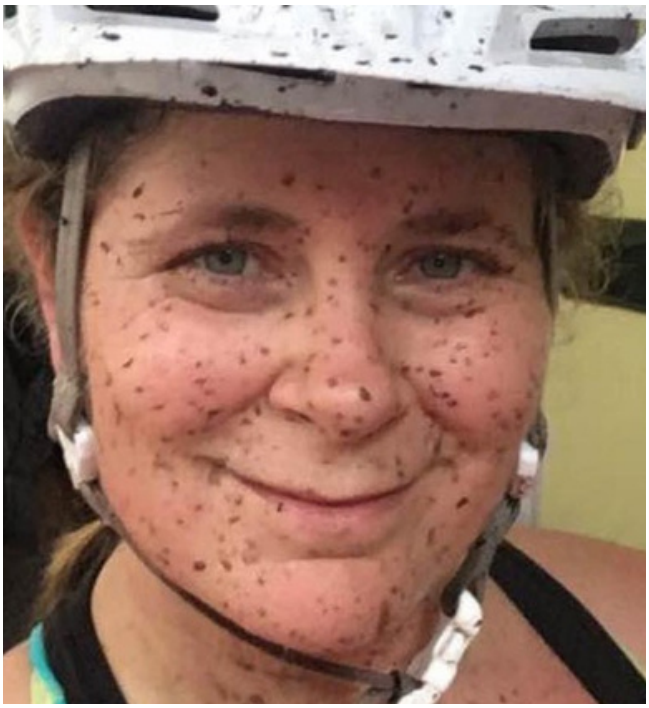
**Taking the long view with Aplastic Anemia**

Patients who are recently diagnosed and treated for Aplastic Anemia (AA) may wonder how their condition will affect them into the future. To get some insight into that question, we connected with Ashley Oakes, an AAMAC board member, who was diagnosed with AA in 2010 when she was pregnant with her son Cohen. Her story of diagnosis and treatment was featured in our Fall 2018 newsletter and is available on the AAAMAC website.

* https://www.cadth.ca/eltrombopag-1

**How is your health now?**
There has been no change with my AA; I am still in remission. All my blood counts are in the normal range. I am still on a maintenance dose of cyclosporine and will likely be on this for the rest of my life. I've been on it for 13 years already. Most people come off it much earlier but when I did so in 2013, my AA flared up again.

**Are you still followed closely by a hematologist?**
Yes, I see a hematologist every 6 months and will for as long as I'm on cyclosporine. I also have monthly bloodwork and monitor my results closely.



Ashley Oakes

**Do you have any long-term side effects from your treatment?**
I struggle with issues with my kidney and liver counts; however, this is not impacting me on a daily basis. I make careful lifestyle choices to prevent unnecessary aggravation of my condition. For example, I limit my alcohol intake, eat healthy meals, and stay active every day.

**Have doctors given you much information about what you can expect in the future?**
I know it is not typical for AA patients to be on cyclosporine so long. Cyclosporine can increase the risk of cancer so I make sure I do all the cancer screenings for which I am eligible. We monitor for MDS and other cancers. I have a small clone for PNH that we are watching.

**What concerns do you have about your health as you look to the future?**
It's hard to unpack AA from other concerns with aging. I'll only know aging as a person with AA.
I don't dwell on AA; I make choices that support my health. AA is not the thing that stops me from being active. The rest will be as it is. Having this condition has lent me more empathy as to how people navigate life with chronic health conditions and hidden disabilities. I have a better understanding of gaps in health insurance, drug coverage, etc.

**Do you find that having AA weighs on you emotionally?**
My anxiety spikes when I get a weird blood test. That will always be there. I still have to disclose my condition when I interact with the health care system. For example, in preparation for a recent surgery, the surgeon was concerned about my platelets. I need to know my numbers so I can reassure health care professionals in these situations.

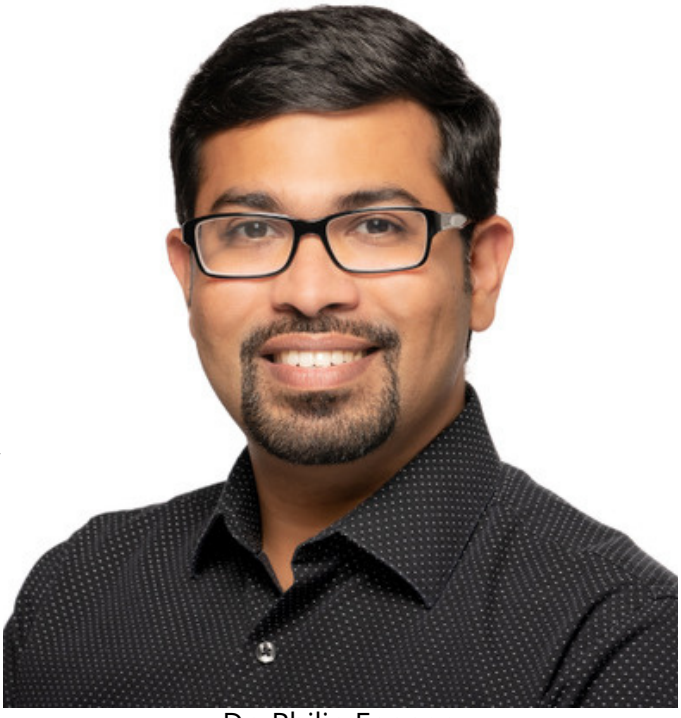
AA is a big part of my identity but in a positive way. I've been able to use the experience to make a difference. I was on the National Liaison Committee of Canadian Blood Services, representing AAMAC. We do policy work and I am able to share voices of the recipient
community to support changes for better outcomes. Recently I advocated for inclusion practices for gay men and other donors. I enjoy bringing hope to people who are recently diagnosed in Canada. Online communities are mainly American, where the health care system is so different. My participation in international conferences has shown me how good we have it in Canada. I'm honored to be able to share my experience with others from all over the world.

**Any concluding words?**
I live a full life. I make a difference in people's lives. I work full-time, I travel. AA doesn't stop me from doing the things I love.

## RESEARCH PROJECT HIGHLIGHTS

**Update on AAMAC-funded Research**
**Submitted by Dr. Philip Eappen, Chair, Research Committee**

The Aplastic Anemia and Myelodysplasia Association of Canada (AAMAC) funds research on bone marrow failure diseases such as Aplastic Anemia, PNH, and MDS. AAMAC recently started supporting Dr. Yigal Dror, MD, FRCPC, a hematologist and oncologist at the Sick Kids, to further study therapeutic approaches and the outcome of children with AA/MDS/PNH. AAMAC is supporting Dr. Dror in a crucial initiative aimed at advancing research and improving outcomes for pediatric patients with acquired aplastic anemia (AA),


Dr. Philip Eappen

myelodysplastic syndrome (MDS), and paroxysmal nocturnal hemoglobinuria (PNH) in Canada.

The Canadian Aplastic Anemia and Myelodysplastic Syndrome Study (CAAMS), headed by the research team, is the only pediatric registry for these disorders in Canada and has a consortium of researchers, healthcare professionals, and institutions collaborating to address these rare conditions. Dr. Dror's research aims align with AAMAC's goals, and funding from AAMAC will help sustain and expand this precious and unparalleled work for advancing the field of AA/MDS/PNH.

The principal aims of CAMS are comprehensive and focused on expanding knowledge, understanding clinical characteristics, and deciphering the pathobiology of AA, MDS, and PNH. This is a comprehensive and multidimensional research initiative that addresses both clinical and biological aspects of AA, MDS, and PNH. Integrating epidemiological data, clinical research and cutting-edge biological analyses in this work demonstrates a holistic approach to understanding and improving outcomes for patients with these rare and challenging disorders.

AAMAC also supports another hematologist, Dr. Signy Chow, MD, FRCPC, at Sunnybrook Health Sciences Centre, to further study Paroxysmal Nocturnal Hemoglobinuria (PNH), which is indeed a rare hematologic disorder characterized by abnormal complement activation and hemolytic anemia. Iron overload can be a concern in patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) who are treated with complement inhibition, particularly with medications like eculizumab. Complement inhibition helps manage the hemolysis associated with PNH, but it may also impact the normal clearance of damaged red blood cells and, consequently, iron recycling. Clinical chart review and subsequent investigation of iron dysregulation in patients with PNH is well-structured and aims to provide valuable insights into the relationship between iron balance, treatment characteristics, and clinical outcomes. Dr. Chow's proposed retrospective review is a valuable initiative to understand better the extent of iron overload in patients with PNH who are receiving anti-complement therapy, mainly focusing on treatment, hemolytic indices, iron indices, imaging for liver iron overload, and chelation therapy. This study, supported by AAMAC, contributes significantly to the knowledge base, and can create better clinical management practices.

In summary, AAMAC's support for these research initiatives reflects a commitment to addressing the complexities of AA, MDS, and PNH. The combination of clinical studies, biological analyses, and collaborative efforts positions these initiatives to make meaningful contributions to the field, ultimately benefitting patients with these rare hematologic disorders.

# THE STRESSES OF WATCHFUL WAITING WITH MDS

Many individuals diagnosed with myelodysplastic syndrome (MDS) are familiar with the term watchful waiting. This is a phase of the disease in which no active treatment is prescribed, with a patient assessment conducted every 3-6 months. The assessment includes blood tests and a review of any signs or symptoms of infection, bleeding, and anemia.[1] Although this approach is well-supported by evidence,[2] it can be a stressful time for both patients and care partners. A recent study of the experiences of caregivers of patients with higher -risk MDS found that stress peaked during the watch and wait period.[3]

There are several factors that make watchful waiting stressful. The first is the fear that doing nothing will allow the disease to progress. Accepting that this may not be the case requires some faith in the health care team. You may find it difficult to have that faith if the team appears to lack knowledge or expertise about MDS.

Watchful waiting can also lead to feeling powerless in the face of the disease. You be equating delaying treatment with giving in to the disease. Another stressor is the need to always be on alert to symptoms that may indicate the disease has progressed. Being watchful means the disease is always on your mind even if symptoms are minimal. Being asymptomatic has its own stresses as you adjust emotionally to the diagnosis while looking "well" to family and friends. You may lack emotional support at a time when it is needed most.

So, how can you deal with the stress of watchful waiting? It can be helpful to be familiar with the evidence about treatment of MDS (see, for example, the articles referenced below or talk to your hematologist). This can give you and your family reassurance that watchful waiting is the right course of action. Of course, it is important to follow health team guidelines for regular follow up. While the "head in the sand" approach may appeal to some, knowing the health team is monitoring the disease can be reassuring as any changes can be quickly addressed. Taking a holistic view of your health is also wise. By paying attention to nutrition, exercise, rest, and social support, you can maximize your well-being at this stage of your illness.

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1 MDS Clearpath: https://www.mdsclearpath.org/ClearPath.aspx

2 Fenaux, P., Haase, D., Santini, V., et al. Myelodysplastic syndromes: ESMO Clinical Practice Guidelines for diagnosis, treatment and follow-up. Annals of Oncology (2020).

3 Frank, P., Olshan, A., Iraca, T. et al. Experiences and Support Needs of Caregivers of Patients with Higher-Risk Myelodysplastic Syndrome via Online Bulletin Board in the USA, Canada and UK. Oncol Ther (2023). https://doi.org/10.1007/s40487-023-00253-4

You can also take steps to address the worrying that may accompany watchful waiting. Mindfulness techniques can help you stay in the present moment. Talking to others who are going through a similar experience can also help. AAMAC offers peer support programs and patient support groups that can connect you with other patients. Finally, share information with friends and family so that they understand the phases of the illness and the type of emotional support you may need at each phase.

Watchful waiting is a phase of MDS which can be highly stressful even though the disease may be "laying low". Be compassionate with yourself during this phase and take active steps to alleviate some of the stress.

We will be discussing the psycho-social aspects of living with bone marrow failure at our in-person patient education meetings in the spring. Patients and care partners are encouraged to register via the AAMAC website.

## CALLING FOR BOARD MEMBERS!

AAMAC has openings for a new volunteer Board Member to join us to execute on our goals of providing an integrated support network for every patient, family member, friend and health care professional affected by aplastic anemia, myelodysplasia, or paroxysmal nocturnal hemoglobinuria.

If you or someone you know has an interest and time they would like to share with us, please reach out (or have them reach out) to info@aamac.ca. We are currently looking for individuals with experience in any of the following areas: legal, medical, government affairs, scientific research, fundraising, marketing, or communications. Bilingualism would be a great asset. Previous experience in governance not required.

# RARE DISEASE DAY

This year Rare Disease Day falls on that rarest of days: February 29th.


Rare Disease Day is the official international awareness-raising campaign for rare diseases. The primary goal of this campaign is to increase awareness among the general public and decision-makers about rare diseases and their influence on the lives of families living with these conditions.

Did you know?

- 300 million people worldwide live with a rare disease.
- There are over 6000 different rare diseases.
- 72% of rare diseases are genetic.
- 70% of these genetic rare diseases begin in childhood.

You can raise awareness about rare diseases and their impact on people's lives by sharing your personal experience of living with a rare disease or caring for someone who does. Submit your written or video testimony on the *Rare Disease Day website** and explore stories already shared by others!

# 2024 PATIENT TRACKERS


AAMAC's 2024 Progress Trackers are available. This booklet helps you keep track of your important contact information, appointments, test results and treatments. To order this or any of our other materials please:

- Call 1-888-840-0039;
- Email info@aamac.ca; or
- Go to https://aamac.ca/resource-books-available/

* https://www.rarediseaseday.org/share-your-story/

# IN - PERSON MEETINGS

Visit ***AAMAC.CA*** for all meeting and event details and registration

## Upcoming Spring In-person Patient Education Meetings

### TORONTO

Date: Saturday April 20, 2024
Time: 830AM - 1 PM
Location: Novotel Toronto North York

**Speakers:**

- Dr Rena Buckstein – Overview of MDS – New Treatment Options
- Pamela J. West, NP - Living Well with a Chronic Disease
- Patient Experience Panel

### HALIFAX

Date: Saturday May 4, 2024
Time: 830AM - 1 PM
Location: Hotel Halifax

**Speakers:**

- Dr. Amy Trottier – Overview of MDS – New Treatment Options
- Celeigh Barber-Russell - Living Well with a Chronic Disease
- Patient Experience Panel

### VANCOUVER

Date: Saturday May 4, 2024
Time: 830AM - 1 PM
Location: Holiday Inn Express Vancouver-Metrotown

**Speakers:**

- Dr. Wendy Davis – Overview of MDS - New Treatment Options
- Laura Brace, RN BSN - Living Well with a Chronic Disease
- Patient Experience Panel

## Virtual Patient Support Group Meetings

AAMAC offers monthly virtual patient support group meetings. Join us from the comfort of your home - patients and care partners welcome!

### French Speaking Patient Support Group

Date: Wednesday March 6
Time: 7PM ET
This meeting will be held on the first Wednesday of each month

### Central Canada Patient Support Group

Date: Wednesday, March 13
Time: 6pm ET
This meeting will be held on the second Wednesday of each month

### Western Canada Patient Support Group

Date: Monday, March 18
Time: 6PM PT
This meeting is held on the third Monday of each month

### Atlantic Patient Support Group

Date: Thursday, March 21
Time: 7pm AT
This meeting is held on the third Thursday of each month

## DONATE

You can help someone living with AA, MDS & PNH by making a donation. Patient support meetings, resources and programs are made possible by your thoughtful and generous donation. Thank you.

There are many ways to donate to AAMAC:

Call the National Office to donate by phone.

Click the 'Donate Now' button on our website

Visit CanadaHelps.org

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