AAMAC
ACAAM

Aplastic Anemia & Myelodysplasia Association of Canada

Association canadienne de l'anémie aplasique et de la myélodysplasie

Newsletter - Fall 2026

for patients living with AA, MDS, PNH and those who support them

Message from the Executive Director

It is August already; I hope all of you have had an enjoyable and restful summer. AAMAC has lots of plans in place for meetings for you to participate in both online and in person. We are excited to be hosting our first meeting in Saskatchewan in November of this year. Details are on the last page of the newsletter along with information on our monthly online support groups and other meeting information.

AAMAC has always been involved in research projects in Canada and this year one of the project we assisted with was the MDS Research Project through Sunnybrook Hospital in Toronto. In this issue Dr. Rena Buckstein talks about this important project.

Our Peer to Peer Support Program is so important to our patients, especially those who are newly diagnosed. Patients sharing their experiences with other patients and caregivers gives much needed reassurance as patients start to navigate their treatment. We thank Jill Buckley for sharing her story and for being such an inspiration. Jill is a regular participant in our Wednesday Online Support Group and also offers her support through the Peer to Peer program.

Thank you to Fiona Lewis for another great newsletter. We hope you enjoy this issue and we look forward to seeing many of you this fall.

Cindy Anthony

IN THIS ISSUE

CANADA'S RARE DISEASE STRATEGY

Since 2024, Canada has been investing in a National Rare Disease Strategy. With an initial infusion of $1.5 billion over three years, the federal government has signed bilateral funding agreements with all 13 provinces and territories. Funding is also being provided to Indigenous, First Nations and Inuit people.

The strategy focuses on 4 key areas:

1. Support patient outcomes and sustainability
2. Invest in innovation
3. Seek national consistency
4. Collect and use evidence

The inter-governmental agreements aim to improve access to treatments and drugs for rare diseases by focusing on:

- Collecting data
- Making informed decisions
- Expanding coverage of drugs for rare diseases
- Improving screening and diagnostics for rare diseases
- Exploring how to improve availability of drugs for rare diseases

According to the Canadian Organization for Rare Disorders (CORD), the strategy is helping Canada build capacity in genomics-based research and diagnosis, clinical trials, collection of real-world evidence, data monitoring, and cell and gene therapy development. CORD also reports that the national strategy is enabling greater provincial and territorial coordination and collaboration.

The Government of Canada recently confirmed ongoing funding of up to $500 million per year for future phases to help people with rare diseases access needed drugs.

CORD is calling upon the government to extend the current bilateral agreements before they expire and to protect continuity of treatment for patients already benefitting from the strategy. AAMAC is a CORD affiliate and supports CORD in its advocacy efforts.

Read more about CORD's advocacy on this topic at **www.raredisorders.ca**

UPDATE ON THE MDS RESEARCH PROGRAM AT SUNNYBROOK

AAMAC has recently awarded additional funding to the MDS Research Program at Sunnybrook Hospital. We spoke to Dr. Rena Buckstein, the Head of Hematology Clinical Trials at Sunnybrook, to learn more about the project.

Can you give us an overview of the MDS Research Program?

I started the MDS-Can registry about 20 years ago, with the initial plan to document the epidemiology of MDS in Canada, because it didn't exist before. It evolved over time to evaluating how MDS impacts quality of life, for example. We started initially just at Sunnybrook. Once we worked out all the kinks about what elements are important and not important, we then started asking other sites across Canada to join the registry. It started with patient characteristics and quality of life, then with the guidance of a geriatrician from Princess Margaret hospital, Dr Shabbir Alibhai, we began considering the impact of patient related factors like frailty, disability and comorbidity on clinical outcomes, not just the disease characteristics including chromosomes, blasts, etc. We wanted to know more about how the soil (the patient as host), not just the seed (the disease), impacts patient outcomes.

Until then, prognostic models for survival have relied only on disease characteristics, rather than patient characteristics including other health problems, level of fitness, and resilience. Therefore, we added to the registry the measurement of several geriatric domains including frailty, disability, and then co-morbidity, using validated instruments and followed the patients to see how they did. We were able to not only validate the importance of MDS characteristics for predicting survival but also were able to refine and improve on these prognostic models by incorporating patient-related characteristics, most importantly frailty. We are trying to spread the word internationally about how important the consideration of frailty and comorbidity is. And we are internationally recognized for this work.

When a patient joins the registry, they must be within a year of their diagnosis, based on a bone marrow biopsy. At baseline, we ask them to fill out several quality-of-life questionnaires, one of which was developed specifically for MDS patients and validated with the assistance of the registry (called the QUALMS).

A research assistant will then measure how fast they walk 4 metres, time them standing up from a chair and sitting down unassisted 10 times, and we measure their grip strength. That's the physical performance testing. Then we evaluate their frailty using the Rockwood frailty scale and an MDS-CAN developed frailty scale. We have them fill out a disability scale. All these tests take just minutes.

Dr. Rena Buckstein, MD, FRCPC

Now, what we're working on today is finalizing the evaluation of quality of life (QOL) over time – what are the elements that predict for improvements or decrements in quality of life? Is it disease related: are they becoming transfusion dependent or independent; are they on certain medications; does their risk score matter? What is emerging over and over is the importance patient related-factors play in predicting QOL score directions. So, patients that are frail or have a disability have inferior QOL scores over time, independent of their disease characteristics such as their hemoglobin or their transfusion dependence. We are emphasizing the importance of considering patient-related factors in the evaluation of quality of life in clinical trials since they may impact the QOL scores. This is yet another example of how the Canadian registry is contributing to the literature.

The next step in the journey of managing MDS patients is to come up with a nimbler and easier to use QOL instrument that will capture the symptom and function components that are most important to MDS patients. Currently, we use generic instruments used in oncology that ask questions about symptoms that may be less relevant to MDS patients. Furthermore, many of these instruments are complicated and lengthy, leading to survey-fatigue. We are working to come up with a 10-element instrument that can be used in real life and clinical trials to understand the impact of our therapies. We hope to have AAMAC representation at a meeting we are convening in the fall with international MDS experts to help address this issue and finalize the questions we will ask. Once the tool is validated, we would seek FDA approval so that it can become the de-facto MDS symptom instrument deployed in clinical trials.

Finally, we are providing 'real world' analysis of drug effectiveness. Does Luspatercept achieve a 38% response rate in patients who are dependent on transfusions? At what dose? How long do they remain on treatment? It is important to study this because, when patients go on clinical trials, they are the fittest of the fit and do not really resemble 'real-life'. People get excluded for a number of reasons: impaired kidney or liver function, poor functional status, to name a few, so we don't always replicate the results of clinical trials in the real world.

By interrogating our registry, we can then see if our results match those found in the clinical trial. This is important data that the government needs for their economic modelling and but also for us in terms of what we can counsel our patients.

Anything else you wish to share about your research program?

Yes! We are piloting the OURA smart ring in a 12-week study in 60 patients with MDS, 30 who are dependent on red blood cell transfusions and 30 who are not. The OURA ring is a convenient 'wearable' that measures a patient's biometrics continuously. Patients can wear it 24/7 through all activities. I'm interested to see if the biometrics of someone with MDS has prognostic information (ex. inactivity, heart rate variability, sleep quality, etc.). I also want to compare the biometrics of those that are transfusion dependent with those that are independent, those that are severely anemic with those that are not, those that are fatigued with those that are not. How does sleep impact daily function? Our pilot study is enrolling patients at Sunnybrook. If it looks promising, I hope to get funding to deploy it across the country in more patients. It's objective, not subjective, like quality-of-life questionnaires. We still have 21 more slots, so I'm happy to have patients be referred to this study.

This article was edited for length. You can read the full interview on our website: **Update on MDS Research**

PATIENT STORY - JILL BUCKLEY

When I first met Jill Buckley at a Zoom patient support meeting, she was going through a rough time. She was calling in from her hospital room recovering from a bone marrow transplant to cure her myelodysplastic syndrome (MDS). But this was no ordinary transplant – if there is such a thing. Jill's transplant had not fully taken so she was given a boost of donor lymphocytes.

This can often trigger graft versus host disease (GVHD) and in Jill, it triggered a severe response which included GVHD of the liver and intestinal tract requiring high doses of prednisone. Prednisone can weaken bones, leading Jill to fracture her back in 3 places prior to her discharge. Five or six weeks into that, she was feeling better and out walking when she experienced horrible pain which turned out to be a broken hip. She was again admitted to hospital.

In addition to a blood infection, the hip was also found to be infected, so the femoral head was removed and Jill had to remain motionless for the next 31 days. Eventually, she was able to sit in a wheelchair for one hour twice a day. It would take nearly 3 months for the infection to clear, allowing her to have surgery to replace the hip, followed by rehab before finally being discharged home. All of this meant spending 6 months in hospital, one stretch being 133 days straight.

I was curious about how Jill found the inner strength to get through so many difficult days. She says that when she finally came out of the hospital, it took her a long time to function again, leading her to conclude that her brain, “just shut down when I was in there, so that I could deal with it.” With no visitors except her husband and the constant interruptions for care, she just focused on getting through each day. She notes that she has a strange sense of humour which leads her to simply laugh at things that others might find distressing.

When she did get home, she spent a lot of time lying in bed, not doing anything due to the deconditioning of the lengthy hospital stay. It took her 8 months to get back to things she liked, such as artwork. She started making bookmarks as a small project that she could take on and this led her back to more demanding creative projects.

In addition to dealing with GVHD, Jill was still experiencing MDS symptoms. Eventually she found out that, despite the transplant being successful, she had a new and rare version of MDS, called Donor Derived MDS.

She was told that she was now at very high risk and would probably transition to AML if her MDS was not treated. She went back on Azacitadine and developed infections, one of which went to her heart. She had to spend 6 weeks self-administering antibiotics 3 times a day through a pic line. At that point, her team was concerned she would not survive a second transplant and recommended supportive care.

She was surprised by this news but accepted it. A second team was then asked for their opinion. After their assessment, she was deemed a good candidate and offered a second transplant. After all she had gone through with the first transplant, how did she approach the tough decision to have another? Jill says she looked at her alternatives as well as the poor statistics regarding survival of a second transplant. Ultimately, she didn't feel like she had a choice:

"The decision was almost instant for me. I talked it over with my husband as it would impact him as well – he said he would support whatever I decided. The team doesn't sugarcoat anything or give you false hope. I saw it as a second chance that I had to take."

Bookmarks Made by Jill Buckley

In her mind, she framed it as chance for living versus waiting to die. When she had doubts, she would go back to that fundamental choice.

I asked Jill if she agreed with my characterization of her as a person with considerable inner strength and determination. She responded, "I would say the word is stubborn. I have always been very determined. If someone says I can't do something, I try darn hard to prove them wrong. It's just my nature."

That nature may have saved her life more than once, although Jill credits a few other things to her survival: drug cost coverage, educating herself about her disease, learning to advocate for herself, and of course the outstanding health care she has received. She is extremely thankful to the blood and stem cell donors who make treatment possible.

> "I've been fortunate. It's taught me that I can handle a lot more than I thought I could handle. I used to have panic attacks and I don't have them anymore. Keep telling yourself it's temporary. Knowing that the health care team has a plan is very reassuring to me. I don't live my life thinking about the mortality stats the doctors gave me. I try not to give that information a lot of weight."

Approaching 18 months post-transplant #2, Jill is doing extremely well and feeling so much better than she had imagined she would. She assists other patients by participating in AAMAC's patient support groups and sharing her experiences and knowledge. Her story not only provides practical information to fellow patients but also demonstrates that we are often stronger than we realize.

MEETINGS & EVENTS

Visit **AAMAC.CA** for all meetings, event details, and registration.

Upcoming Webinar

BLOOD 101

Date: Monday, November 2, 2026
Time: 7 PM (ET)
Speaker: Dr. Matthew Yan, MD, FRCPC, MSc

This webinar is presented in partnership with the Canadian Blood Services.

Upcoming In-Person Patient Education Conferences

HALIFAX

Date: Saturday, October 3, 2026
Time: 9:00 AM - 1:00 PM
Venue: Delta Hotels Halifax Downtown, 1990 Barrington Street
Speakers:
Dr. Amy Trottier – MDS
Dr. Rachelle Blackman – PNH

SASKATOON

Date: Saturday, November 21, 2026
Time: 9:00 AM - 1:30 PM
Venue: Saskatoon Inn and Conference Centre, 2002 Airport Drive
Speakers:
Dr. Mohamed Elemary – MDS
Dr. Hadi Goubran-Messiha – PNH
Dr. Fei Fang – Aplastic Anemia

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!

Daytime Patient Support Group

Date: Monday, October 12
Time: 4 PM (AT), 3 PM (ET), 1 PM (MT), or 12 PM (PT)
This meeting will be held on the second Monday of each month

Wednesday Patient Support Group

Date: Wednesday, October 14
Time: 7 PM (AT), 6 PM (ET), 4 PM (MT), or 3 PM (PT)
This meeting will be held on the second Wednesday of each month

Monday Patient Support Group

Date: Monday, October 19
Time: 6 PM (PT), 7 PM (MT), 9 PM (ET), or 10 PM (AT)
This meeting is held on the third Monday 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' button on our website

Visit CanadaHelps.org

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@AAMACanada @aamac1989 @AAMAC1989 @Aplastic Anemia & Myelodysplasia Association of Canada