Mission

The Aplastic Anemia & MDS International Foundation is the world's leading nonprofit health organization dedicated to supporting patients and families living with aplastic anemia, myelodysplastic syndrome (MDS), paroxysmal nocturnal hemoglobinuria (PNH), and related bone marrow failure diseases. The Foundation provides answers, support, and hope to thousands of patients and their families around the world.
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Answers, Support and Hope
MESSAGE OF HOPE

Dear AAMDSIF Community,

As our 40th anniversary year comes to a close, we are filled with gratitude for the unwavering commitment of our community in the face of many challenges. This Annual Report for AAMDSIF highlights the profound impact of our patient and health professional programs, as well as our commitment to advancing research in bone marrow failure diseases.

Your stories, resilience, and the collaborative spirit of our community fueled by support from our generous donors and corporate partners have been the driving force behind the success of our initiatives. Despite the complexities of the healthcare landscape, AAMDSIF remains steadfast in its dedication to providing answers, support, and hope to those affected by bone marrow failure.

Our patient programs have continued to empower individuals and families and our health professional initiatives have fostered a network of expertise, ensuring that the latest developments in science are translated into compassionate and effective care. Investments in scientific research made through our competitive grants and patient registry programs are a testament to our community’s efforts to discover promising new possibilities for the future of bone marrow failure treatment.

As we navigate the challenges ahead, let this annual report serve as a source of inspiration and motivation. Together, we have achieved significant milestones, and together, we will continue to forge a path towards a brighter and healthier future for all those touched by bone marrow failure.

Thank you for being an integral part of the AAMDSIF community.

www.aamds.org
I am so proud of him! His strength of character bowls me over. He inspires me every day. Especially the days when I find it hard to get on with my life, after keeping so much fear, panic and trauma inside for so long. But I’m ready to trust life again, with him by my side.

With all my love and courage to Aplastic Anemia parents. Some days will be harder than others, on you and on your child, but calmer winds will come. Stay confident.

-Marie-Eve

My name is Marie-Eve. I’m the mother of three talented hockey players. In March 2021, I’d been noticing marks on my oldest son’s body. Then, one morning, he left for the hockey rink, stopping up blood from his nose and gums, for no apparent reason. We didn’t know it yet, but that day would mark a turning point in his life.

The next morning, he was sent for an emergency bone marrow aspiration at Ste-Justine’s hospital in Montreal. As we waited for what seemed like an eternity, I tried to shoot him a few smiles, but it was tearing my heart out. They were about to go digging into my son’s bones, and there was nothing I could do to protect him. The results came back late that afternoon. A bunch of words spilled out of the doctor’s mouth — severe, aplastic, anemia, bone marrow failure, transfusions, transplant, isolation. I heard them all, but my brain refused to process them. I was in complete shock.

After weeks of blood tests, transfusions, and appointments, the next big step was in sight: a stem cell transplant from an anonymous donor. After 41 days in the hospital, Milan was allowed to come home to recover and be with his brothers. He started training again, then skating, and that helped him stay positive. In March 2022, almost a year to the day of his diagnosis, he went back to school in person and returned on the ice to play with his elated teammates. By September 2022, Milan’s life as a student athlete was in full swing again as he left home for a prep school in Ontario.
Patient Programs

In 2022, we achieved remarkable milestones in our patient programs: hosting impactful conferences, engaging webinars, and support groups, all uniting and empowering individuals facing rare diseases.

**Patient and Family Conferences**

**IMPACT:** In 2022, our organization demonstrated the transformative impact of our 5 patient and family conferences, uniting communities with 40 leading medical experts. Through these events, we’ve shattered access barriers, empowering individuals with vital disease and treatment education.

“I had never met another aplastic anemia patient until I attended a conference. It was the best feeling to talk to talk with someone like me. My family and I had lots of questions. The medical experts were so great and patient. I left the conference with hope for a future with my family.”

**-P&F Conference Attendee**

**Interactive Webinars**

**IMPACT:** In 2022, our webinars addressed the challenges faced by patients with invisible, life-threatening rare diseases. Over 1,800 participants engaged in live sessions, while 741 accessed recorded webinars, ensuring vital medical information reached all who needed it.

“I love the webinars. I can participate at home and still get to ask my questions. Thank you for recording the webinars. I can’t always attend the live program. It’s nice to know I am not missing anything.”

**-Webinar Participant**

**Online Support Groups**

**IMPACT:** In 2022, our 84 virtual support groups, hosted worldwide, connected 996 participants from 16 different countries. This global outreach, offering frequent and flexible opportunities, underscores our commitment to inclusivity, ensuring that all patients and families, regardless of location, can find the support they need.

“When I attended my first support group, I was nervous and didn’t know what to expect. The group leader was so knowledgeable and made me feel at home. I look forward to attending the group each month. It gives me something to look forward to. Everyone gets what I’m going through.”

**-Support Group Member**

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Health Professional Programs

2022 Scientific Symposium

The Eighth AAMDSIF International Bone Marrow Failure Scientific Symposium was held on May 19-20, 2022 in Bethesda, Maryland. Our Symposium brought together many of the world’s leading experts on the biology and treatment of aplastic anemia, MDS, AML, PNH, and related disorders. We were pleased to have 120 participants in person and 563 more registered virtually, representing 25 nations. The hybrid format this year enabled more new investigators and those from distant countries to participate, again making it a global event.

More than 30 presentations by the internationally respected faculty stimulated discussion and provided new insights for future studies. This ongoing collaborative effort of academia, government, private industry, and AAMDSIF demonstrates the mutual commitment to the discovery of new treatments for patients, and ultimately, cures for bone marrow failure diseases.

AAMDSIF also recorded interviews with several symposium presenters to share the key highlights of the program for patients. These interviews were shared on social media and the AAMDSIF YouTube channel.

IMPACT: Our symposium united experts and fueled discussions on advances in bone marrow failure diseases, reaching 25 nations through hybrid participation and digital outreach.

“On behalf of patients, thank you for helping us, for continuing to work for us to find not just newer but better treatments, that are more successful, with fewer side effects, that improve quality of life.

You choose to dedicate your lives to these diseases, and without you I don’t know where all of us would be - but I do know that it would be a far, far worse situation.

We truly appreciate all you do - from the research, to seeing patients, to working with local hematologists, to sharing your knowledge and participating in event like this to share knowledge so we can advance progress on the research together.”

-Tamir Orbach, Patient Advocate, speaking to researchers at 2022 Scientific Symposium
Research

Funding the early stage of innovative approaches to studying bone marrow failure has been a hallmark of the AAMDSIF Research Grant program since the first grant was awarded in 1989. New investigators are challenged to receive grant support to test novel concepts early in their careers, so the seed funding from an AAMDSIF award allows them to initiate their research and demonstrate the need for more extensive study.

We are proud to have awarded more than $5.5 million in research grants to 100 researchers since 1989 to advance the study of bone marrow failure. Over 90% of the 100 AAMDSIF research grantees have devoted their careers to bone marrow failure research. Many are now globally recognized as leaders in the field, leading their own labs and advancing the knowledge of aplastic anemia, MDS and PNH through medical journal publications, scientific meeting presentations, and research collaborations.

Introducing Our 2022 Grantees

In 2022, AAMDSIF achieved another milestone by awarding grants to two additional grantees. Guided by the AAMDSIF Medical Advisory Board, grants are carefully bestowed based on research quality, alignment with our goals, applicant expertise, and study facility caliber. This ensures our resources catalyze impactful discoveries and drive progress.

David Beck, MD, PhD

Institution: NYU Grossman School of Medicine

Study Title: Advanced genomic and molecular investigation of acquired bone marrow failure syndromes

Sushant Kumar, PhD

Institution: University of Pennsylvania Perelman School of Medicine

Study Title: The mechanisms of immune evasion by PNH clones in aplastic anemia
Global PNH Patient Registry

The Global PNH Registry, launched by AAMDSIF in 2021, is an opportunity for PNH patients to advance research by sharing their experience with PNH. A patient registry is an online data system to collect, store, and retrieve patient data for analysis in research studies. The primary aim of the Global PNH Patient Registry is to conduct a prospectively-planned and efficient natural history study that will result in a more comprehensive understanding of PNH and how it progresses over time.

In addition, the Registry serves to:

- Provide a convenient online platform for participants (or caregivers) to self-report cases of PNH
- Characterize and describe the PNH population as a whole
- Assist researchers studying the pathophysiology of PNH
- Support the design of clinical trials that explore new PNH treatments

Information collected in the Registry includes demographics, diagnostics, medical history, treatment and disease progression, management of care, and quality of life.

To learn more visit: https://pnh.iamrare.org/

The Impact of Our Research

Dr. Audrey Lasry of NYU Langone Medical Center concluded her two year AAMDSIF grant project that explored the potential of using immunotherapy, a method that targets the immune system instead of cancer cells, to treat MDS and AML. Her team identified a "gene signature" of MDS and AML that allowed them to develop a risk score to identify patients who would benefit from more aggressive treatment. Dr. Lasry's work has paved the way for further research collaborations with other institutions.

Meanwhile, Dr. Daria Babushok has now established her own lab at the University of Pennsylvania and is mentoring new investigators, including 2022 AAMDSIF Grantee Sushant Kumar. Dr. Babushok's research focuses on using the genetic make-up of individual bone marrow to improve the diagnosis and disease surveillance of aplastic anemia patients. Her work aims to pave the way for personalized treatment strategies.

AAMDSIF has been instrumental in supporting both Dr. Lasry and Dr. Babushok's research programs, which promise to improve patient risk stratification and provide novel therapeutic targets for MDS and AML patients, and bring Precision Medicine to the field of aplastic anemia.
Development

Giving Tuesday

Giving Tuesday is a global generosity movement! People worldwide join together each year the Tuesday after Thanksgiving, known as Giving Tuesday, to support the causes they care about most. AAMDSIF is grateful for our dedicated supporters who show up for us year after year on this global day of giving.

In 2022, a board member generously offered a $10,000 match, which AAMDSIF was able to unlock and subsequently raise over $23,000 with help from our supporters. The funds raised will enable AAMDSIF to support patients with bone marrow failure and their families.

IMPACT: Giving Tuesday isn’t just about raising funds; it’s about the power of community. In 2022, AAMDSIF had supporters from all over the world contribute to celebrate Giving Tuesday. There is strength in numbers, and when our community pulls together, AAMDSIF can significantly impact our patients and families.

“I give on Giving Tuesday to ensure more patients have access to the same critical resources the Foundation has provided me. I was diagnosed with Aplastic Anemia in May 2021 and have had atypical treatment needs. The Foundation has been an excellent resource for staying abreast of the latest treatments, and the information I’ve received from conferences, seminars, and research has empowered me to feel more knowledgeable and confident about my treatment options.”

-Carmen Hall, AAMDSIF donor

March for Marrow

In 2022, over 900 patients, family members, friends, health professionals, corporate partners and advocates participated in March for Marrow events to raise money and awareness for rare bone marrow failure disease. There were marches nationwide—in Michigan, California, New Jersey, New York, Texas, Nevada, and Virginia. Their selflessness truly inspires us.

IMPACT: Due to the incredible efforts of our supporters, the March for Marrow events raised nearly $80,000 for AAMDSIF. These funds will directly impact patients and families living with rare bone marrow disease failure. The generosity of our supporters and corporate partners enables AAMDSIF to continue to fund key programs like patient education, support groups, research and other patient resources.
2022 Audited Financial Information

**REVENUE**

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<td>Corporations</td>
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<td>Individuals</td>
<td>598,015</td>
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<td>Family Foundations &amp; Estates</td>
<td>599,180</td>
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<td>Investments</td>
<td>(173,020)</td>
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<td><strong>Total Revenue</strong></td>
<td><strong>$3,056,484</strong></td>
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**EXPENSES**

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<td>Research</td>
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<td>Management &amp; General</td>
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<td><strong>Total Expenses</strong></td>
<td><strong>$2,599,946</strong></td>
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AAMDSIF is a 501(c)(3) nonprofit charitable organization.
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A Glimpse At 40

In a world where questions often outnumber answers, where the journey of health can be riddled with uncertainties, the Aplastic Anemia & MDS International Foundation (AAMDSIF) has stood as a beacon of unwavering support for patients and families for four decades. As we celebrate our 40th anniversary, this annual report serves as a testament to our enduring commitment to provide not only answers but also a resolute shoulder to lean on, and above all, a boundless wellspring of hope.

Scan to Learn More:

www.aamds.org