HAPPY HOLIDAYS FROM THE ISAAC FOUNDATION

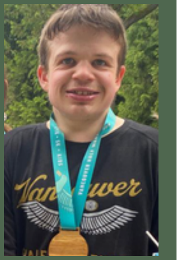
Holiday newsletter and update from 2025

FUNDRAISING

The More family and friends hosted their annual "**More than a Spiel**" curling bonspiel this fall at the Palmerston Curling Club, raising over $6000!

Kamie's mom and board member Parise Robichaud raised over $1600 this summer at their **10th annual Softball Tournament** in Grand-Digue, NB. This brings their total raised to over $40,000!

The Purcell family and friends raised more than $6000 in the **Scotiabank 5k and half marathon** in Vancouver, BC. This event was particularly special as it included Trey and some of his siblings who ran to raise funds for Trey's rare disease, MPS II.



The Hoskins family raised $16,500 at their **Hoedown for Hope** on May 10th in Victoria, BC. All funds raised go toward our MPS IV fund.

FOUNDATION NEWS

We are excited to have gradually ramped up fundraising again after the few years of strange pandemic times in which we hesitated to host in-person events with vulnerable kids...and in fact, many of our kids are becoming young adults and we're still working through what fundraising looks like over the next decade. Our son Isaac, after whom our charity was named at 2 years of age, is now finishing university and getting ready to start a career in computer sciences. We are so grateful that he is at this stage of life we were told we'd likely never see. Every day is a gift when your child has a progressive disease, and our fundraising over the years has helped get him to where he is now (more on that below). However, we continue to see the effects of MPS on Isaac and the many others with rare diseases who are part of our lives. And we know that there is much more work to do.

Since starting our charity, we've hosted galas, patient symposia, golf tournaments, bonspiels, baseball tournaments, runs for a cure, and more. To date, we've given **more than 2 million dollars** to research. The past decade has seen many new treatments and clinical trials launched, and we are proud that several of these trials have been supported by The Isaac Foundation. However, challenges treating MPS diseases persist - existing therapies don't reach the brain, spinal cord, bones, and heart valves; gene therapy is only available for patients without antibodies and is slow to progress through clinical trials; and individuals with MPS continue to have a reduced life expectancy. So the fundraising goes on and we appreciate your continued support along the way.

Happy Holidays, with love from the McFadyen family

RESEARCH NEWS

In 2025, we're thrilled to have given over a half million dollars to MPS research that includes a novel approach to treating lysosomal storage disorders. Work being done by Kommodo Therapeutics is looking at memory T (Tm) cells engineered to express IDUA as a systemic enzyme replacement therapy in an MPS I mouse model. A single infusion has shown ability to sustain enzyme activity for up to 22 weeks, reduce urinary GAGs, and restore tissue GAG levels across multiple organs, with modest cognitive improvement. These findings support engineered Tm cells as a promising platform for durable, cell-based enzyme replacement in MPS I and related disorders. We're very excited about this work, funded in collaboration with the Morquio and MPSII Fund!

Recently, the Isaac Foundation's MPS VI and MPS IV funds collaborated to provide an additional $50,000 USD to Dr. Ellen Fung and team at UCSF Benioff Children's Hospital in Oakland, California to complete their study exploring the safety and efficacy of Voxzogo® (vosoritide) for the treatment of growth deficits in MPS IVA and VI. The results are promising and they will be wrapping up in 2026: https://clinicaltrials.gov/study/NCT05845749?cond=mps&rank=1

Several of our Canadian MPS VI patients (Isaac included) continue annual follow-up as part of the gene therapy trial in Italy. After receiving a one-time infusion of the liver-targeted AAV8 vector with a correct copy of the gene that is defective in these MPS VI patients, they continue to make some of their own enzyme (the goal of gene therapy) several years later. Researchers will further increase the gene therapy dose and move into Phase 3 in 2026.

UPCOMING EVENTS

In 2026, we'll be starting up our cross-country **Run for a Cure** again, and hope to participate in races in Quebec, Ontario, and BC (would love to add more!). We will host a fundraising platform on The Isaac Foundation website and will be looking for walkers/runners to join our teams from across the country. More to come!

On May 30th, 2026, the Hoskins family will host their 2nd annual **Hoedown for Hope** at the Saanich Fairgrounds in Victoria, BC. We'll also continue with our annual softball tournament, curling bonspiel, and other events in the new year and hope you can join us at some of these!

To donate, please go to:
theisaacfoundation.com/#donate

TREATMENT BREAKTHROUGH

We're very proud to share that a project first funded by The Isaac Foundation has been making international news over the past month! We were proud to support the early work by Dr. Brian Bigger to help make this potential new, curative treatment possible.

Three-year-old Oliver Chu has become the first person in the world to receive a pioneering gene therapy for Hunter syndrome, a rare and devastating genetic disorder. Hunter Syndrome prevents the body from producing a vital enzyme, leading to progressive physical and cognitive decline and a shortened life expectancy.

In the clinical trial in Manchester, UK, doctors modified Oliver's own stem cells to include a working copy of the faulty gene before reintroducing them into his body. Since the treatment, Oliver has shown remarkable improvement in his speech, agility, and cognitive development, and no longer requires weekly enzyme infusions.

While doctors remain cautiously optimistic and need to monitor long-term results, the success offers hope for other children with similar metabolic diseases. Oliver's progress is being hailed as a potential turning point in treating previously incurable genetic conditions.

CONTACT:
Ellen Buck-McFadyen
ellen@theisaacfoundation.com

Andrew McFadyen
mcfadyena@me.com
(613) 328-9136