CARDIOMYOPATHY Australia New Zealand

NEWSLETTER | JULY 25

Dear CMANZ Community,

It has been some time since our last newsletter, but with a fabulous new volunteer on board, we will be bringing you regular news from here on in (thank you Georgie! And thanks Nicole for the layout). It's a big edition! We are pleased to introduce you to our new Board Member Gordon Thoms, who shares his very personal and tragic reason for becoming involved with the organisation.

We are proud to share pics of our two recent support groups, along with a link to the talk by Dr Kathryn Waddell Smith, who kindly spoke on Genetics in Cardiomyopathy – Now and In the Future at our Adelaide meeting.

The landscape of cardiomyopathy care is ever evolving, and we bring you some recent developments which show both the promise and the challenges of advancing treatment options.

It was disappointing to learn that the trial of Mavacamten for Hypertrophic Cardiomyopathy (HCM) patients without obstruction did not show significant benefit, resulting in the research trial being ended early. Other trials for non-obstructed patients are under way which are showing more promising data, and we provide some recent results for Aficamten. And in technological advancements, it is fascinating to see the progress being made with ever-smaller pacemakers, and the very exciting news of successful implantations of Total Artificial Hearts.

We are very pleased to provide information on gene therapy trials for certain HCM and Arrhythmogenic Cardiomyopathy (ACM) genes. I never thought we would be talking about human trials for genetic therapies for cardiomyopathy in my lifetime, and it is incredible to see advancements which could change the trajectory of care in the future.

Our Perth Support Group was held in April, hosted by Vikki Collins and attended by guest speaker Cassie Greer, Genetics Counsellor

It's important to note these are trials, and there are many, many steps over a number of years until we have results of the trials, and if successful, approvals and availability. However, we include information here so that our community can see that research is occurring.

We also share excellent and comprehensive research about recommendations for exercise in inherited cardiomyopathies from Dr Eleanor Rye and Prof. Diane Fatkin at The Victor Chang Cardiac Research Institute, Sydney.

I'm sorry to share some sad news. Many of our Victorian community members will have known Reva Blowfield, the wife of Tim Blowfield, our former Treasurer. Sadly Reva passed away earlier this month. Reva lived with Cystic Fibrosis and cardiomyopathy, and was almost 83 when she passed. We send our sincere condolences to Tim and all who knew and loved Reva.

I hope you find this newsletter informative, hopeful and interesting reading. As always, we welcome your feedback and any comments or ideas for future newsletters, to info@cmanz.org.au

Keep well this winter,

Leigh

Leigh Bell
President, Cardiomyopathy Australia New Zealand

GET INVOLVED

Do you have Transthyretin Amyloid Cardiomyopathy (ATTR-CM)?

There is an opportunity to participate in online market research by completing a patient preference survey. This survey will help researchers better understand the ATTR-CM patient journey and let them know what is most important to you. **Your time will be reimbursed.** If you're interested in participating, please email survey@hawkpartners.com for further details.

Welcome Gordon Thoms

Gordon joined the Board of CMANZ in late 2024 following the tragic and devastating loss of his 23-year-old daughter, Alexandra to an undiagnosed Arrhythmogenic Cardiomyopathy in 2023. His younger son, Charlie, also has the same heart disease and has had a heart defibrillator installed as well as being on daily heart medication.

Through his involvement with CMANZ and other related organisations, his strong motivation is simply to work with others to build awareness, research capability and advocacy - in the hope that other families will not have to live through his family's tragic experience. Gordon has had a successful career in financial services, having worked in senior roles with a number of global investment banking firms before establishing his own advisory business which he has recently sold. He serves as a board member and director of a number of private companies and charitable foundations.

Non-Obstructive HCM Trials

Mavacamten's ODYSSEY-HCM Trial: A Sobering Result

In an unexpected outcome, Bristol Myers Squibb announced that its Phase 3 ODYSSEY-HCM trial of Mavacamten (Camzyos) in non-obstructive hypertrophic cardiomyopathy (nHCM) did not meet its dual primary endpoints. The two end points assessed were patient-reported benefit, and peak oxygen consumption however, unfortunately based on these measures the medication didn't show benefit. The data will be released in the coming months. I know this is extremely disappointing news for those hoping this might be a new treatment option for non-obstructed patients, especially given the success of the drug for the majority of those who are obstructed.

While the drug remains a valuable option in obstructive HCM, this result highlights the urgent need for more tailored treatments for nHCM and reminds us that medical progress often moves in steps—not leaps. There are at least three other drugs being assessed for nHCM at this time (that I know of), and we maintain hope that with ongoing research, more treatments will become available.

I've spoken with Bristol Myers Squibb (BMS, the Pharma company who make the drug) to understand more, including whether there are options for ongoing compassionate access for those who were on the trial and believed they saw benefit. If you participated in the trial and believe you saw benefit, you may like to get in touch with Leigh via info@cmanz.org.au, to hear about options for compassionate access.

Non-Obstructive HCM: Aficamten Studies Show Promise

While some recent progress in hypertrophic cardiomyopathy (HCM) treatment has focused on the obstructive form, developments are now emerging for people living with non-obstructive HCM (nHCM)—a form of the condition with fewer treatment options.

Phase 2 REDWOOD-HCM (Cohort 4) Study

In a recent clinical trial, 41 people with non-obstructive HCM were given a new medication called Aficamten, a type of cardiac myosin inhibitor. Over 10 weeks, researchers found that Aficamten helped improve symptoms and reduce heart stress markers (like NT-proBNP levels), without causing serious side effects. This gives hope that Aficamten may help people with nHCM feel better and possibly avoid future complications.

Phase 3 ACACIA-HCM Trial Now Underway

Based on the promising Phase 2 results, a larger Phase 3 trial—called ACACIA-HCM—is now underway. This study is testing how well Aficamten improves symptoms and heart function over 36 weeks in people with non-obstructive HCM. Results are expected in the next year or two and may support wider approval of the drug for this form of cardiomyopathy. Read more about the ACACIA HCM trial here: ACACIA HCM trial

Have you joined our private Facebook Group yet? If you use Facebook and would like to connect with others in the cardiomyopathy community, you're welcome to join via this link. You can also follow our public Facebook page here.

Gene Therapy Trial for Genetic Hypertrophic Cardiomyopathy

Tenaya Therapeutics has shared encouraging updates on its gene therapy trial, called MyPEAK-1, which is testing a potential one-time intravenous treatment (TN-201) for people with hypertrophic cardiomyopathy (HCM) caused by mutations in the MYBPC gene. Early results from the first group of patients show that the treatment appears mostly well tolerated with few adverse effects, and some participants experiencing improvements in symptoms and heart health markers. Based on these positive signs, the trial is now moving to a higher dose group with more patients being enrolled. The company expects to share more results later in 2025. If successful, this gene therapy could offer a new approach for treating HCM at its genetic source, rather than just managing symptoms. **Read more about Gene Therapy Trials here**

Arrhythmogenic Cardiomyopathy (ACM)

Researchers are exploring gene therapy as a potential future treatment for ACM—particularly for people with changes in the PKP2 gene. Variants in the PKP2 gene are one of the most common genetic causes of ACM. Human trials are under way in the United States of a gene therapy for those with PKP2-ACM. **Read more about these studies here**

Exercise in Inherited Cardiomyopathies

We see a lot of discussion about exercise in our forums – how much is safe vs how much might be causing harm. New research from The Victor Change Cardiac Institute (Dr Eleanor Rye and Prof. Diane Fatkin et al) offers insights into finding the right exercise for everyone with cardiomyopathy.

FIVE TIPS FOR GETTING ACTIVE

Talk to your team first: Your doctor can help you understand your personal risks and what activities are most suitable for you. They can provide advice that is tailored to your specific condition, symptoms, and fitness level.
Start low and slow: A great way to begin is with short, 5-10-minute walks. As you feel stronger, you can gradually increase the time. Some activity is always better than none.
Warm up and cool down: Always spend five minutes warming up with gentle movements before you exercise and five minutes cooling down with stretches afterwards. This helps your body adjust.
Listen to your body: You will have good days and bad days. If you feel more tired or breathless than usual, it's okay to take a rest day.
Choose activities you enjoy: You are more likely to stick with an activity if you find it fun. This could be walking, gardening, dancing, or cycling. Consistency is key!

Read a great summary for patients here and for those who like to delve into the science, read the full paper in the American Heart Association Journal.

Pacemakers for Infants

A groundbreaking medical device, the world's smallest pacemaker, has been developed for infants with heart defects. Comparable in size to a grain of rice, this dissolvable device is powered by light and can be implanted non-invasively via a syringe. It addresses the need for temporary heart support in infants, particularly in low-resource regions. Traditional pacemakers for adults involve invasive procedures with significant post-surgery risks, but this new device mitigates these issues by dissolving over time, eliminating the need for removal surgery.

The minuscule pacemaker safely dissolves over time. Credit: John Rogers / Northwestern University

The device, developed by a team from Northwestern University, Illinois, replaces the original pacemaker's radio antenna with a light-based data transmission system and a galvanic cell which allows the pacemaker to be extremely small, measuring just 1.8 mm wide and 3.5 mm long, while still providing adequate electrical stimulation.

The primary motivation for this development was to provide a safe and effective solution for children with heart issues, reducing the risks associated with traditional pacemakers. Source: here

Australia's First Total Artificial Heart Implant

St Vincent's has once again cemented its reputation as a global leader in cardiac care by successfully implanting Australia's first BiVACOR Total Artificial Heart. This groundbreaking procedure, performed late last year, marks a new era in heart transplantation and offers new hope to patients suffering from heart failure. The latest breakthrough saw a team led by St Vincent's Hospital Sydney's Dr Paul Jansz implant the revolutionary BiVACOR Total Artificial Heart, a titanium device with a single moving part, no valves, and a no-contact suspension system, designed to eliminate mechanical wear. Initially developed as a bridge to transplantation, the long-term vision for this device is to serve as a permanent replacement for a human heart.

Under the expert care of Professor Chris Hayward and his dedicated team, the patient was discharged in early February, making the patient the first person in the world to leave a hospital with the BiVACOR Total Artificial Heart. After more than 100 days – the longest period for a patient with this implant – they successfully received a donor heart transplant in early March and are now recovering well. This latest success reaffirms St Vincent's role as a global leader in heart health, offering renewed hope to patients and their families while shaping the future of cardiac treatment. Source: here

Dr Daniel Timm, inventor of the BiVACOR Artificial Heart

Global Heart Hub Key Messages

The Global Heart Hub Cardiomyopathy Council has developed a set of Key Messages which can be used worldwide by patient organisations. This will help ensure our various awareness raising activities tell a consistent story. There are 6 Key Messages covering a variety of cardiomyopathy facts. We shared the first message in our last newsletter, and we'll continue to share one each newsletter.

KEY MESSAGE TWO: Understand your family's heart health.
Knowing if a family has a history of cardiomyopathy is crucial because:

- Inherited forms are common: Many cardiomyopathies are genetic, meaning they can be passed down through families.
- Early diagnosis is vital: Early detection through screening and awareness can lead to timely treatment, potentially preventing serious complications like heart failure or sudden cardiac death.
- Family members are at risk: If one family member has an inherited cardiomyopathy, other relatives may also be at risk, and knowing this can prompt them to get tested.
- The Council encourages individuals to have open conversations about heart health within their families and to seek medical advice if they have concerns or a family history of cardiomyopathy.

Get involved: Volunteer with us!

We need you! As our community grows, so does our need for passionate volunteers. If you have skills in social media, especially on platforms like Facebook, Instagram, or LinkedIn, or experience in fundraising, we'd love to hear from you. Whether you can contribute just a few hours a month or take on a more active role, your time and talent could make a meaningful impact on the lives of heart patients across Australia and New Zealand.

Interested in helping out?
Please reach out to us at info@cmanz.org.au

Have questions, feedback, article ideas, or a story to share? We'd love to hear from you, just send us an email and we'll be in touch.

Our Adelaide support group listened to a wonderful talk by Dr Kathryn Waddell Smith on Genetics in Cardiomyopathy – now and into the future. This was recorded; you can watch via this link to Kathryn's talk on the homepage of our website.

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