Cardiomyopathy Association of Australia Ltd

MAY 2024 – THE LATEST IN BIG-HEARTED NEWS

AT A GLANCE

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Letter from the President

Hello everyone,

We are pleased to bring you our third newsletter for the year. This edition, we are excited to introduce our new Medical Advisory Committee (MAC). It's important that we ensure we offer reliable, credible, recent, and reviewed information, and we are grateful to have such a high-calibre team of experts to assist us.

May is a big month for cardiomyopathy medication advancements with two new medications for treating different forms of cardiomyopathy being listed on the Pharmaceutical Benefits Scheme (PBS). Camzyos® (mavacamten) for adults with obstructive HCM, and Vyndamax® (tafamidis) for transthyretin amyloid cardiomyopathy (ATRR-CM) were listed from May 01. This means these medications will receive a government subsidy, and the most patients will pay per prescription is the same amount as for all other PBS drugs, which is $31.60, or $7.70 with a concession card.

We continue our series on genetic therapies, this month bringing you information about approaches and effects. Our Research Spotlight continues the genetics theme; read about a new gene-editing human trial for transthyretin amyloidosis cardiomyopathy (ATTR-CM).

For those with an ICD, please take note of the Royal North Shore Hospital's ICD online education session on Tuesday 28 May, where A/Professor Belinda Gray, Cardiologist and Director of Sports Cardiology RPA, speaks about Exercise and Safety of Sport for People with ICDs.

I recently had the opportunity to speak about all things cardiomyopathy at my local Cooroy-Pomona Lions Club (photo below). It was terrific to see they have just obtained an AED for their clubhouse. We

were grateful to receive a generous donation. And finally, I have just been appointed Chair of the Global Heart Hub Cardiomyopathy Council. The Global Heart Hub is a not-for-profit based in Ireland, with a goal to bring together an international community of heart patient organisations to elevate the voice of patients. The Cardiomyopathy Council has 24 member organisations from 14 countries, and I'm honoured to have the opportunity to take on the Chair role.

Until next time. I hope you have a great couple of months, and look forward to writing again in July, when we hope to be able to share exciting news about some initiatives we have in the pipeline.

If you'd like to get in touch, please do. Our e-mail is info@cmaa.org.au.

Meet our Medical Advisory Committee

We are proud and honoured to introduce our Medical Advisory Committee (MAC). This impressive team of advisors will offer professional guidance to our team, including providing insights to clinical advancements, and ensuring we are disseminating recent and correct information. We are grateful for their support. The MAC are featured on our website - check it out by clicking here.

Prof Christian Hamilton-Craig is an expert in cardiovascular imaging and diagnostics, particularly Echocardiography, Cardiac CT, and Cardiac MRI. He is an Honorary Staff Cardiologist at the Prince Charles Hospital and the Director of Noosa Hearts Cardiology, Sunshine Coast. His academic

appointments are Professor Faculty of Medicine & Centre for Advanced Imaging at the University of Queensland, and Professor at the School of Medicine Griffith University Sunshine Coast.

He was the inaugural chair of the Conjoint Committee for Certification in Cardiac MRI across Australia-NZ, is the chief guideline writer on Coronary Artery Calcium Scoring and Non-Invasive Coronary Imaging, and the regional co-Chair of the Society of Cardiovascular Computed Tomography. His research interests are translational imaging research, cardiomyopathies, lipidology. He has published over 150 scientific papers including citations in the AHA/ACC Valve Guidelines. He collects guitars and likes to surf.

Dr Andris Ellims is a Cardiologist and Cardiac Imaging Specialist at Epworth Freemasons, The Avenue Hospital and the Alfred Hospital.

After graduating with First Class Honours as Top Student at the University of Melbourne, Dr Ellims trained at the Royal Melbourne Hospital. He completed Advanced Training in Cardiology with a Fellowship in Cardiac Imaging (including echocardiography, cardiac CT and cardiac MRI) at the Alfred Hospital.

Dr Ellims was awarded a Doctor of Philosophy (PhD) for his work at Baker IDI Heart & Diabetes Institute involving the use of cardiac imaging to evaluate cardiomyopathies. His PhD also involved an investigation of novel risk factors contributing to coronary artery disease. Dr Ellims is regarded as a national expert in these areas. He has published extensively in major scientific journals and has presented original research at both international and local scientific congresses.

Dr Ellims extended his research into clinical practice by establishing Victoria's first specialised clinic for Hypertrophic Cardiomyopathy at The Alfred Hospital.

Dr. Mark Ryan is a long term supporter of CMAA. He is a well qualified cardiologist with more than three decades of experience in the medical field. He graduated from the University of Sydney in 1987, where he obtained a Bachelor of Medicine and Surgery.

In 1994, Dr. Ryan earned a Fellowship of the Royal Australasian College of Physicians from The Royal Australasian College of Physicians, Australia. He is an esteemed member of the organization and has been actively involved in various medical research projects throughout his career. Dr. Ryan's expertise lies in internal medicine, cardiology, and critical care medicine. His commitment to patient care and his knowledge in these areas have earned him numerous recognitions and accolades.

Dr. Ryan’s goal is to make a positive impact on his patients' lives and help them achieve optimal health. Besides his proficiency in English, Dr. Ryan is fluent in German. This additional language skill allows him to effectively communicate with a broader range of patients.

Dr. Ryan practices in Nowra, New South Wales, Australia, primarily affiliated with Nowra Private Hospital. He is a respected and trusted physician within the medical community, known for his expertise, professionalism, and commitment to delivering the highest quality of care to his patients.

Associate Professor Jodie Ingles is Head of the Clinical Genomics Laboratory, Garvan Institute of Medical Research and Co-Director of the Garvan Genomic and Inherited Diseases Program. She is a cardiac genetic counsellor in the Department of Cardiology, Royal Prince Alfred Hospital with more than 20 years' experience working with families with inherited heart diseases. She is a Heart Foundation Future Leader Fellow, receiving the Shirley E. Freeman award for innovation in 2022. In

2018 she received an NHMRC Excellence Award for the top-ranked Career Development Fellowship application.

Jodie and her team are team are focused on finding ways to use cardiac genomics and genetic counselling to improve diagnosis, management and care for families with inherited cardiovascular diseases.

Linda Moloney is a Cardiology Nurse Specialist with extensive experience in the UK, Australia, and New Zealand, where she is currently engaged in cardiac clinics at Ascot Cardiology.

Linda has completed Post Graduate study at Greenlane Hospital, Auckland and Imperial College, London, as she was based there as a Senior Sister at the Royal Brompton Hospital. At the Royal Brompton, she developed a passion for working with patients with chronic cardiac conditions and this has been highlighted throughout her career.

She has more than two decades of experience with all cardiac devices (LVAD,BIVAD, CRT-d, ICD and Pacemaker) and her international experience includes roles as Pacemaker Nurse Liaison and Shift Leader at Royal North Shore Hospital, Sydney.

Having worked as a Clinical Nurse Lead at the Heart Foundation of New Zealand, Linda understands the importance of patients having access to information and support, and has experience with developing Heart Foundation resources, campaigns and protocols. She is passionate about patients receiving access to appropriate care and treatment and the benefits of patients and their whanau being supported through their diagnosis journey.

New PBS Medication Subsidies

Mavacamten

Mavacamten (brand name Camzyos®) was listed on the PBS on May 01. The medication is the first and only TGA approved treatment to specifically address HCM. It is approved for adults with symptomatic obstructive HCM It is called a myosin inhibitor, and works by reducing the strength of the contraction of the heart muscle. Mavacamten has been available for two years in the US, where it has seen excellent symptomatic relief and reduction of symptoms. We are so pleased there is another tool in the cardiologists toolbox for the treatment of Obstructive HCM. There is a current trial for non-obstructive patients here in Australia and around the world, and we will bring you more information about that as soon as results are published.

Tafimidis

Tafimidis (Vyndamax®) was also listed on May 01. It is the first and only subsidised treatment for adults with wild-type pr hereditary transthyretin-mediated amyloidosis (ATTR-CM) in Australia. The medication is a selective stabiliser of transthyretin (TTR), slowing the amyloidogenic process.It provides an option for patients, based on factors such as disease stage, symptoms, and individual patient characteristics. It is wonderful to have a targeted therapy for this typeof amyloid cardiomyopathy.

Both medications may be prescribed by a cardiologist or a consultant physician experienced with the condition.

Gene Therapy - approaches and effects

We continue our education series about genetic therapies. Genetic therapies have been approved for other conditions, and clinical trials to treat cardiomyopathy are underway overseas. Gene therapy offers hope for genetic cardiomyopathy patients, and we think it's important that we keep our community up to date with developments, so that when trials or treatments potentially reach Australia, we have all been along for the journey.

What is Gene Therapy?

Gene therapy is a way of treating or preventing conditions caused by genetic mutations.It aims to address the underlying cause of disease, such as changes in our genes. If genes are like the blueprint to our body, gene therapy can fill in missing parts or correct errors in the drawings.

DNA therapy is the use of DNA that codes for the production of a specific RNA or protein to treat a disorder. To have a therapeutic effect, the DNA must be delivered to the nucleus of a cell, where it can then be used by the cell to affect protein expression. This gene will now live in the nucleus which gives a greater chance of being permanent and is typically only given one time.

RNA therapy is the use of shorter sequences of genetic material in RNA format to treat or prevent a disease. There are many different types of RNA therapy because there are so many different types of RNA sequences that can affect cell functions. These types of therapies are delivered using viral vectors, or other non-viral vehicles such as lipid nanoparticles. They often need repeat dosing to maintain a therapeutic (good) effect since the DNA is not being altered or supplemented.

Cell therapy is the transfer of a specific cell type(s) into a patient to treat or prevent a disease. Depending on the cell therapy, the cells can come from either the affected individual or an unaffected donor. Some cell therapies are more common, like a hematopoietic stem cell (blood forming cells) transplant. Depending on the treatment, conditioning to prepare the body to receive the biological material is done to reduce the risk of an immune response and help the body successfully accept the cells. There are many FDA approved cell therapies.

Three common effects of gene therapies in cells are:

- **Gene addition** adds in a working gene that has the instructions for the cell to make more of the specific protein needed. Vectors, which are often viruses, are used to deliver the working gene to the cell's nucleus, where the DNA is stored. This gene will now live in the nucleus which gives a greater chance of being permanent and is only given one time. Sometimes the therapy is designed for the new gene to insert itself into the main DNA storage while other times it will stay *next to* the main DNA storage, like an extra set of instructions.
- **Gene silencing** is where the delivered genetic material prevents or inhibits the activity of a gene that is already present in a cell. Gene silencing often decreases the amount of a specific protein being made.
- **Gene Editing** corrects pieces of DNA by changing or deleting the information *within* the affected individual's gene. Genetic material is sent to directly edit or change pieces of DNA already located within a cell to correct the protein being made by that DNA. Gene editing uses technology that is highly precise to make these types of changes.

Visit The American Society of Gene and Cell Therapy (our source) to learn more.

Research Spotlight

The first U.S. heart patient involved in a study for a new gene-editing therapy has successfully been treated at MedStar Washington Hospital Center. The Phase 3 MAGNITUDE clinical trial is evaluating the safety of a gene editing treatment for patients with transthyretin amyloidosis cardiomyopathy

(ATTR-CM). The study is expected to enrol 765 participants worldwide. There are participating entries in Australia and New Zealand. For further information, e-mail us at info@cmaa.org.au.

Transthyretin amyloidosis is a protein disorder that typically affects the heart by stiffening the muscle and making it harder for the heart to pump blood throughout the body. The treatment is designed to precent the production of an abnormal form of a protein called transthyretin that causes the disease.

Source - MedStar Washington Hospital Center Facebook Page

Upcoming Events

The Royal North Shore Hospital free ICD Education Session

Online via Zoom

Date: Tuesday 28th May 6pm-7pm

Speaker: A/Professor Belinda Gray, Cardiologist and Director of Sports Cardiology, RPA

Topic: Exercise and Safety of Sport for People with ICDs: Updated guidelines

If you would like to attend, please register via the link

https://us02web.zoom.us/meeting/register/tZUvf-qppjIqH9JtfQPwpd5Uaid6PNMGof8X

Reach Out

For questions, feedback, article ideas, or story contributions, email info@cmaa.org.au, **and we'll be in touch.**