Consumer and Community Involvement Program

WAHTN
Western Australian Health Translation Network

HOW MOLECULAR MEDICINE CAN IMPROVE THE LIVES OF PEOPLE WITH RARE DISEASES

COMMUNITY CONVERSATION REPORT

Prepared By

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DECEMBER 2022

Murdoch University | Centre for Molecular Medicine + Innovative Therapeutics

JOINT RESEARCH WITH THE PERRON INSTITUTE

Contents

ACKNOWLEDGEMENTS

Acknowledgement of Country

The Western Australian Health Translation Network (WAHTN) and Consumer and Community Involvement Program (CCIProgram) acknowledges the Aboriginal people of the many traditional lands and language groups of Western Australia. We acknowledge the wisdom of Aboriginal Elders both past and present and pay respect to Aboriginal communities of today.

Acknowledgement of Lived Experience

We acknowledge the importance and expertise of the lived experience voice of health consumers and carers. We recognise their involvement in making a difference in supporting health research and impacting the health and wellbeing of our communities.

Other Acknowledgements

We would like to express our sincerest gratitude to all the consumers and community members who attended the Centre for Molecular Medicine and Innovative Therapeutics (CMMIT) Community Conversation; your time, energy and contributions are all greatly appreciated.

Lastly, we would like to thank the team from CMMIT along with the team from the CCIProgram for their diligent work in planning, promoting and successfully facilitating the Community Conversation.

BACKGROUND

The Consumer and Community Involvement Program and the Centre for Molecular Medicine and Innovative Therapeutics have developed a partnership aimed at engaging and involving consumers and community members in research to ensure people's lived experiences and perspectives shape decisions about research priorities, policy and practice.

THE CONSUMER AND COMMUNITY INVOLVEMENT PROGRAM

The Consumer and Community Involvement Program (CCIProgram) is an enabling platform of the Western Australian Health Translation Network (WAHTN). The CCIProgram actively supports partner universities, health and medical research institutes, health service providers and non-Government organisations to bring together consumers and people with lived experience and connect them with health and medical researchers. The success of the CCIProgram comes through building and maintaining strong relationships with WAHTN and Partner Organisations.

Consumers and community members are an integral part of the research process, allowing for greater transparency, openness and accountability leading to research which is more relevant and impactful to the community. Our aim is for Consumer and Community Involvement to be standard practice in all health research in Western Australia. As such, our services, resources and training programs have been developed to support consumers and community members being involved at all stages of the research cycle.

CENTRE FOR MOLECULAR MEDICINE AND INNOVATIVE THERAPEUTICS - MURDOCH UNIVERSITY

A joint research centre between Murdoch University and the Perron Institute, the Centre for Molecular Medicine and Innovative Therapeutics (CMMIT) brings together researchers and specialist clinicians from a range of disciplines who have a common focus on developing precision medicine solutions to combat diseases affecting society today. Researchers from the Centre for Molecular Medicine and Innovative Therapeutics (CMMIT) believe precision medicine has the potential to transform healthcare on a scale equivalent to the way antibiotics transformed the fight against infectious diseases.

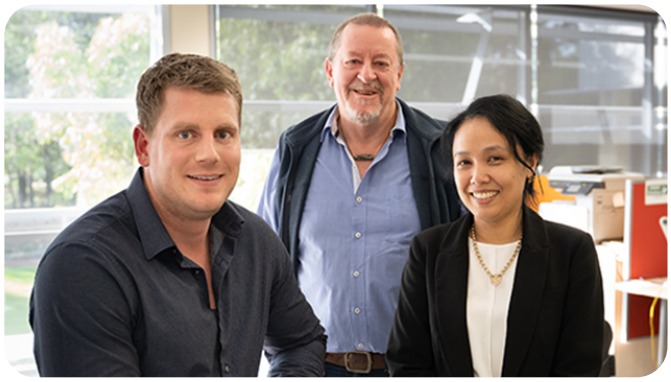
Established in 2019, CMMIT has made substantial progress towards its long-term goal of evolving into multi-disciplinary research centre with the range of skills to enable it to address some of the bigger issues facing healthcare today.

CMMIT aims to be adaptable responding to needs as they change. This is reflected in the success of multiple COVID-19 projects that have led to new screening techniques and new therapeutic approaches.

The core research areas within CMMIT are working on topics as diverse as genetic therapies, genomics, motor neurone disease, neurodegenerative disorders, including Parkinson's disease, myositis, multiple sclerosis, cognition and exercise, health economics, sepsis, mathematical modelling of biological processes and wound healing.

Molecular Therapy (Wilton/Aung-Htut)

As one of CMMIT's core research areas, Professor Steve Wilton and Dr May Aung-Htut lead a group that focuses on developing novel therapeutic strategies to treat rare inherited or the much more common acquired human diseases by designing genetic drugs (antisense oligonucleotides) to modify gene expression as necessary: suppressing or by-passing disease-causing mutations, switching isoforms or increasing expression.



Throughout the collaborative partnership between CMMIT and the CCIProgram, ongoing conversations were had around how to best capture an informed, lived experience perspective around rare disease and molecular medicine. These discussions eventually led to the proposal of running a Community Conversation specifically facilitated to gather information from people with lived experience and of individuals with rare health conditions. This event was designed and developed to obtain information from the attendees with the hope that findings could direct and aid in prioritising future research projects and initiatives.

WHAT IS A COMMUNITY CONVERSATION?

A Community Conversation is an event using an abridged version of the Word Café Method[1] and allows for the facilitation of informal, open conversations around a specific topic of importance. This method allows researchers to informally obtain a range of communal ideas from a group of people with lived experience around a particular topic specified prior to the event. [2],[3] Additionally, a Community Conversation provides an opportunity for attendees to reflect upon their own relevant experiences and contribute in meaningful discussions within a safe and comfortable space.

This Community Conversation was designed to promote discussion surrounding living with rare disease and suggestions for future research directions. Ultimately, the objective of this Community Conversation was to highlight and capture the collective voice of people with lived experience of rare diseases and use the emerging themes from the event to influence and direct future projects.

[1] Brown, J., & Isaacs, D. (2005). The World Cafe´: Shaping our futures through conversations that matter. Barrett-Koehler
[2] Chieh-Ling Yang, Delphine Labbé, Brodie M. Sakakibara, Janneke Vissers & Marie-Louise Bird (2022) World Café- a community conversation: a Canadian perspective on stroke survivors needs for community integration, Topics in Stroke Rehabilitation, 29:5, 392-400.
[3] Carter, E. W., Schutz, M. A., Gajjar, S. A., Maves, E. A., Bumble, J. L., & McMillan, E. D. (2021). Using Community Conversations to Inform Transition Education in Rural Communities. The Journal of Special Education, 55(3), 131–142.

THE COMMUNITY CONVERSATION TEAM

Deb Langridge, Head of the CCIProgram:

Deb has worked in the public health and prevention space at all levels of government – Federal, State and Local – and not for profit sectors to contribute to the health and well-being of communities. She has worked to capture the voices of all parts of community including access and inclusion, Aboriginal and Torres Strait Islander people, culturally and linguistically diverse communities, children and young people, mental health, health and community services. Deb has been the Chair of Advisory Groups in both NSW and WA. She has always worked connecting community, government, and community with a well-being focus, and was a representative on WA Sustainable Health Review with this in mind.

Deb's role leading the Consumer and Community Involvement Program as a platform of the Western Australia Health Translation Network is to connect researchers in WA Universities, Medical Research Institutes, Government, and Health Service Providers with people with lived experience to support and encourage best practice research. Deb is passionate about ensuring research can impact policy, practice and wellbeing of communities and enjoys bringing people together to make a difference.

Professor Steve Wilton AO – Director, Centre for Molecular Therapy and Innovative Therapeutics:

Steve Wilton AO is Foundation Professor of Molecular Therapy at Murdoch University and has the dual roles of Director of Murdoch's Centre for Molecular Medicine and Innovative Therapeutics (CMMIT) and Director of the Perron Institute for Neurological and Translational Science. His research has led to the development of life-changing antisense oligonucleotide-based drugs for the treatment of Duchenne muscular dystrophy. Working with the US company, Sarepta Therapeutics, Steve's group showed that Eteplersin (Exondys 51) slows disease progression in Duchenne patients. This compound was given accelerated approval by the US Food and Drug Administration (FDA) in 2016 and is now in use in patients. Two further compounds, Golodirsen (Vyondys 53) and Casimersen (Amondys 45) targeting different mutations that cause Duchenne has since reveived FDA approval. The three drugs – Exondys 51, Vyondys 53 and Amondys 45 – provide clinicians with the tools to treat over a quarter of Duchenne cases. Steve is currently extending his research on the therapeutic applications of antisense oligomers to other inherited and acquired diseases.

Dr May Aung-Htut – Co-Head of Molecular Therapy Laboratory

May Aung-Htut is the Co-Head of the Molecular Therapy Laboratory at the Centre for Molecular Medicine and Innovative Therapeutics (CMMIT). A PhD graduate from the University of New South Wales, May previously held postdoctoral positions at the UNSW and University of South Australia working on cellular aging and transcriptional regulation of programmed cell death before moving to Perth. Her current research focuses on developing therapeutic antisense oligonucleotides for various diseases, including multiple sclerosis, congenital muscular dystrophy and Pompe's disease. Dr. Aung-Htut's ultimate goal is to extend this research to other diseases, including rare inherited diseases and cardiovascular disease.

Dr Craig McIntosh - Postdoctoral Scientist, Molecular Therapy Group, CMMIT

Craig McIntosh is currently working as a postdoctoral researcher under the guidance of Professor Steve Wilton and Dr May Aung-Htut. His current research aims to develop a novel therapeutic platform within CMMIT, assessing a novel mechanism to increase gene and protein expression, with the use of the proven safety and translational history of gene patches developed for Duchenne muscular dystrophy. Dr McIntosh was awarded the prestigious "City of Perth 2020 Aspire Award" to attend an international conference of his choosing and represent Western Australia in the neuromuscular field. In under two years as a Postdoc Dr McIntosh has been involved in funding, totally in excess of $1.6 million, while being the PI on grants worth over $250,000.

Thankyou to the CCIProgram facilitators: Kerry Mace, Kat Stewart for their assistance in facilitating the conversation with the attending community members.

ABOUT THE COMMUNITY CONVERSATION

Approach

CMMIT recognises the unique perspective consumers and people with lived experience can contribute regarding their specific rare health condition. In partnership with the CCIProgram, a Community Conversation was planned to gather and collect the community's thoughts and attitudes towards new, cutting edge medical technologies.

The session was designed to:

- Have a better understanding of the community's current awareness of CMMIT's presence and research work in rare diseases.
- Assess the group's thoughts and level of understanding regarding the use of gene therapy technologies.
- Discuss and capture suggestions of ways research institutes could better engage with consumers and/or people with lived experience relevant to their research.

Promotion

The CCIProgram worked closely with the CMMIT team to share promotional communications across multiple channels:

1. Consumer and relevant and/or related health service provider networks
2. Social media posts (Facebook, Instagram, LinkedIn & Twitter)
3. Flyers displayed around hospitals & Murdoch University
4. Promotional video about the event and what to expect (shared on multiple media platforms).

Attendees were encouraged to register their interest to attend.

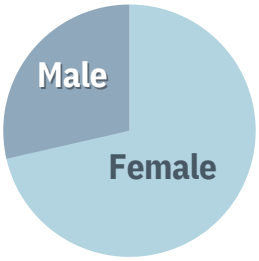
To best promote this involvement opportunity, the CCIProgram produced a video featuring the CMMIT researchers providing detail around the upcoming Community Conversation. This promotional video was shared amongst the CCIProgram and CMMIT's social networks.

https://youtu.be/eUp1YBxdeKs

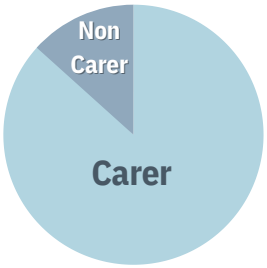
ATTENDEES

In attendance at the Community Conversation were 15 community members with a lived experience of a rare disease, 5 members of the CCIProgram and 3 researchers from CMMIT joined the Community Conversation.

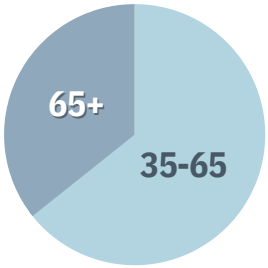
Demographic snapshot



Gender



Identifies as a carer



Age

How Molecular Medicine can improve the lives of people with rare diseases - A Community Conversation

Tuesday, 23rd August 2022 – 6pm to 8pm

Agenda

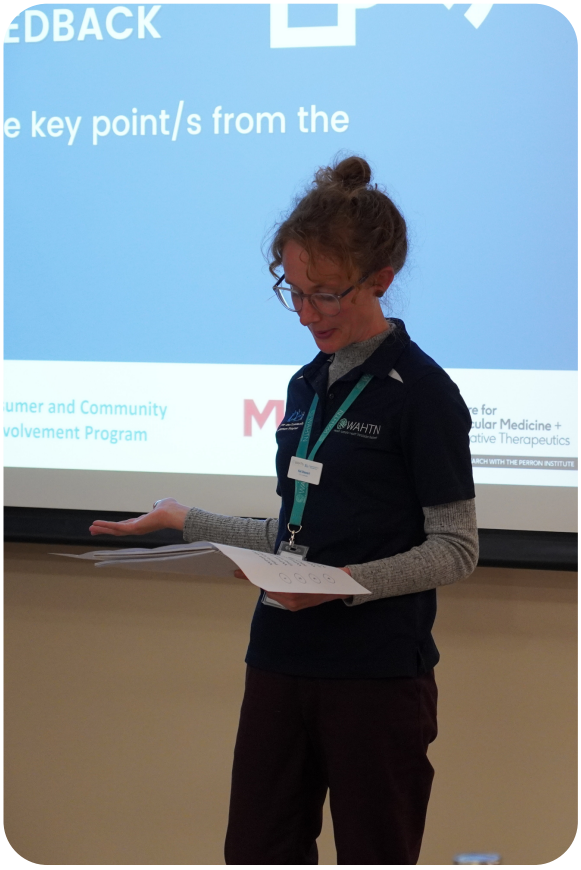
5.45pm	**Registration & refreshments**	**All**
6.00pm	**Welcome** • Acknowledgement of Country • Welcome to the workshop • Introductions	**Deb Langridge**
6.10pm	**Presentation by CMMIT Team**	**Prof. Steve Wilton and Dr Craig McIntosh**
6.30pm	**Process of the evening**	**Deb Langridge**
6.35am	**Question 1:** What do you know of the research CMMIT has been doing into Rare Diseases in WA? **10min** **Question 2:** What are your thoughts regarding the use of RNA therapies for the treatment of rare diseases? **20min** **Question 3:** What would you like to know before potentially starting a new RNA therapy? **20min** **Question 4:** What are some ways that research institutes like CMMIT could engage with consumers/people with lived experience relevant to their research? **10min**	**All** **All** **All**
7.35pm	Table facilitator feedback	**Table facilitators**
7.50pm	Next steps and questions	**Deb, Prof. Steve Wilton and Dr Craig McIntosh**
7.58pm	Evaluation and Staying in touch	**All**
8.00pm	**Close**	

STRUCTURE AND PROCESS

Held at Murdoch University within CMMIT, an earlier start was offered for those attendees who were interested in attending in a guided tour of the lab facilities led by one of the key researchers.

Following tours and arrival of all attendees, the event commenced with a welcome and Acknowledgement of Country by Deb Langridge, Head of the CCIProgram. A brief introduction was given to provide an overview of the session agenda.

Postdoctoral Scientist in the Molecular Therapy Laboratory, Dr Craig McIntosh, provided a contextual background around CMMIT's research direction and areas of current investigation. He also presented an excellent overview of gene therapy technology to better prepare attendees for the breakout groups and provide a framework of shared knowledge amongst the group of attendees.



Attendees were then split between two breakout tables. Each table had a facilitator from the CCIProgram to help guide the discussion and a scribe from Murdoch University to collect all attendee feedback.

Facilitators posed 4 questions in total to the group (see Table 1); each individual question was allotted 20 minutes for discussion. The comments, feedback and suggestions were all captured on butcher paper by the table scribe and are presented in the following pages of this report.

COMMUNITY CONVERSATION FINDINGS

Attendee Insights

Question 1

What do you know of the research CMMIT has been doing into Rare Diseases in WA?

An emerging theme was the lack of awareness about the centre and even less knowledge around the work that is currently conducted at the centre. Additionally, some attendees spoke more generally about the misunderstanding and lack of awareness and knowledge around navigating the healthcare sector/system and where to go to find reliable/correct information. It was mentioned that even health care professionals (GPs) are unaware of the work that is being done through CMMIT.

Those individuals that did comment that they were aware of CMMIT and its research were informed from their involvement in consumer and advisory groups, or other advocacy work in the rare diseases space.

This word cloud represents direct quotes captured during the breakout session in response to Question1.

Question 2

What are your thoughts regarding the use of RNA therapies for the treatment of rare diseases?

"Concept RNA gives hope to people with rare diseases"

Responses to this query were mixed between two schools of thought. One school of thought reported feelings of hope and/or encouragement in relation to RNA therapies. The other school of thought shared feelings of frustration, concern, and fear. Interestingly, both groups shared the common perspective that there is a need for increasing awareness and knowledge around the use and accessibility of RNA therapies.

Numerous suggestions were made around how to raise awareness and educate the greater community about RNA therapies and individualised medicine. Several comments around engaging Parliament and State government to not only broaden the reach of Rare Disease knowledge and RNA therapies/technology, but also to allow for CMMIT to be viewed under a "big picture" lens, showcasing the fantastic work that is being done in our very backyard.

Question 3

What would you like to know before potentially starting a new RNA therapy?

Among all the numerous responses collected regarding this query, three themes were consistently shared amongst all attendees. The three key themes were:

Access:

- Who would be able to receive this type of medical treatment?
- Is it reserved for a particular patient population only?

Cost:

- What is the cost for individualised medicine?
- Does the government and/or private health insurance provide funding for this type of medical treatment?

Side Effects:

- Are there any known side effects to this treatment?
- Would I have the side effects forever?

Although not as prevalent as the above themes, it should be noted that the process of administration of the therapy was another topic that several attendees expressed wanting additional information about prior to proceeding with such treatment.

Question 4

What are some ways that research institutes like Centre for Molecular Medicine and Innovative Therapeutics (CMMIT) could engage with consumers/people with lived experience relevant to their research?

Attendees responded consistently to this question and their answers were unanimous. Firstly, consumers strongly felt that by involving and engaging with established consumer and/or advocacy groups, CMMIT could form lasting relationships with individuals living with relevant health conditions within the rare disease space.

Furthermore, consumers expressed that CMMIT should actively engage with external networks and related health and research professionals around Perth (and Australia) for purposes of collaboration, again to highlight and increase their footprint in this niche sector of molecular medicine.

Fostering these outside partnerships will aid in expanding CMMIT's reach, ultimately increasing access to potential consumers and/or people with lived experience.

TABLE 1:
The below table captures the main themes, issues and potential opportunities identified during the Community Conversation for each topic discussed.

Topic	Issues	Opportunities Identified
Existence & presence of CMMIT	• Lack of awareness • No current knowledge around what CMMIT is and what they do • Limited knowledge of CMMIT within Perth medical community	
RNA therapies as treatment for rare diseases	• Encouraging prospect for some • Concern and fear for others • Accessibility?	• More education and awareness around RNA therapy as a viable treatment option • Engage and collaborate with Parliament to expand CMMIT's footprint and profile
Pre-requisites for agreeing to RNA therapy	• Funding • Accessibility • Cost • Side-effects	• Broaden the wider community's knowledge around RNA therapy as a treatment for rare disease • Publicly highlight successes to date
Consumer involvement & engagement	• Existing consumer and advocacy groups • Collaborate and engage with external stakeholders within the medical research community	• Research update days • Networking between relevant stakeholders within the rare disease space

Event Summary

Event Video

The CCIProgram captured the responses of attendees pre and post Community Conversation. Utilising this footage, a highlight reel of the evening was created using attendee feedback to capture the overall success of the CMMIT Community Conversation.

https://youtu.be/58eOVaWvmrU

Event Highlights

Evaluation and feedback from attendees were collected through the CCIProgram following the closing of the Community Conversation. The consensus from all who attended was that the Community Conversation was extremely informative, interactive, and engaging. Attendees were pleased they were given the opportunity to share their lived experience and have their voices heard by the CMMIT research team.

The above graphic represents direct quotes captured from attendees following the CMMIT Community Conversation.

WANT TO KNOW MORE?

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