

“Working together, seeking a cure”

REPORT FROM THE CHAIR

Dr Simon Gibbs



“Working Together, Seeking a Cure”



Objects

The objects of the Company are to pursue the following charitable purposes:

- (a) to provide training and education of medical professionals, patients and their carers regarding the diagnosis (including accurate sub-typing), treatment and management of amyloidosis of every type;
- (b) to undertake and promote research into the causes, diagnosis, treatments and management of amyloidosis and publishing the results of that research;
- (c) to advocate, in partnership with patients with amyloidosis and medical professionals, to ensure equitable access to the best possible treatments for all persons suffering amyloidosis of any type in Australia and for there to be a greater understanding of what it is like to live with amyloidosis; and
- (d) to enhance awareness and to promote the multi-disciplinary and collegiate management of the diagnosis and treatment of amyloidosis patients, including supportive care, to enhance the quality of life of patients of amyloidosis and related diseases.

***RACE* TO A CURE - *R*ESearch, *A*DVOCACY, *C*LINICAL CARE AND *E*DUcATION**

Structure of the AAN - Our Board



Dr
Simon Gibbs

Chairman
Founding member of the AAN

Medical Director Victorian and
Tasmanian Amyloidosis Service



Associate Professor
Hasib Siddiqi

Treasurer
Founding Member of the AAN

Medical Director Fiona Stanley
Hospital Amyloidosis Service, Perth



Associate Professor
Peter Mollee

Founding member of the AAN

Medical Director Queensland
Amyloidosis Service



Dr
Fiona Kwok

Founding member of the AAN

Medical Director Westmead
Amyloidosis Services, Sydney



Mrs
Pat Neely

Company Secretary
Founding member of the AAN

Patient Advocate and medical
social worker



Ms
Merran Brookman

Joined the Board in June 2022

CPA, Financial and Accounting
services

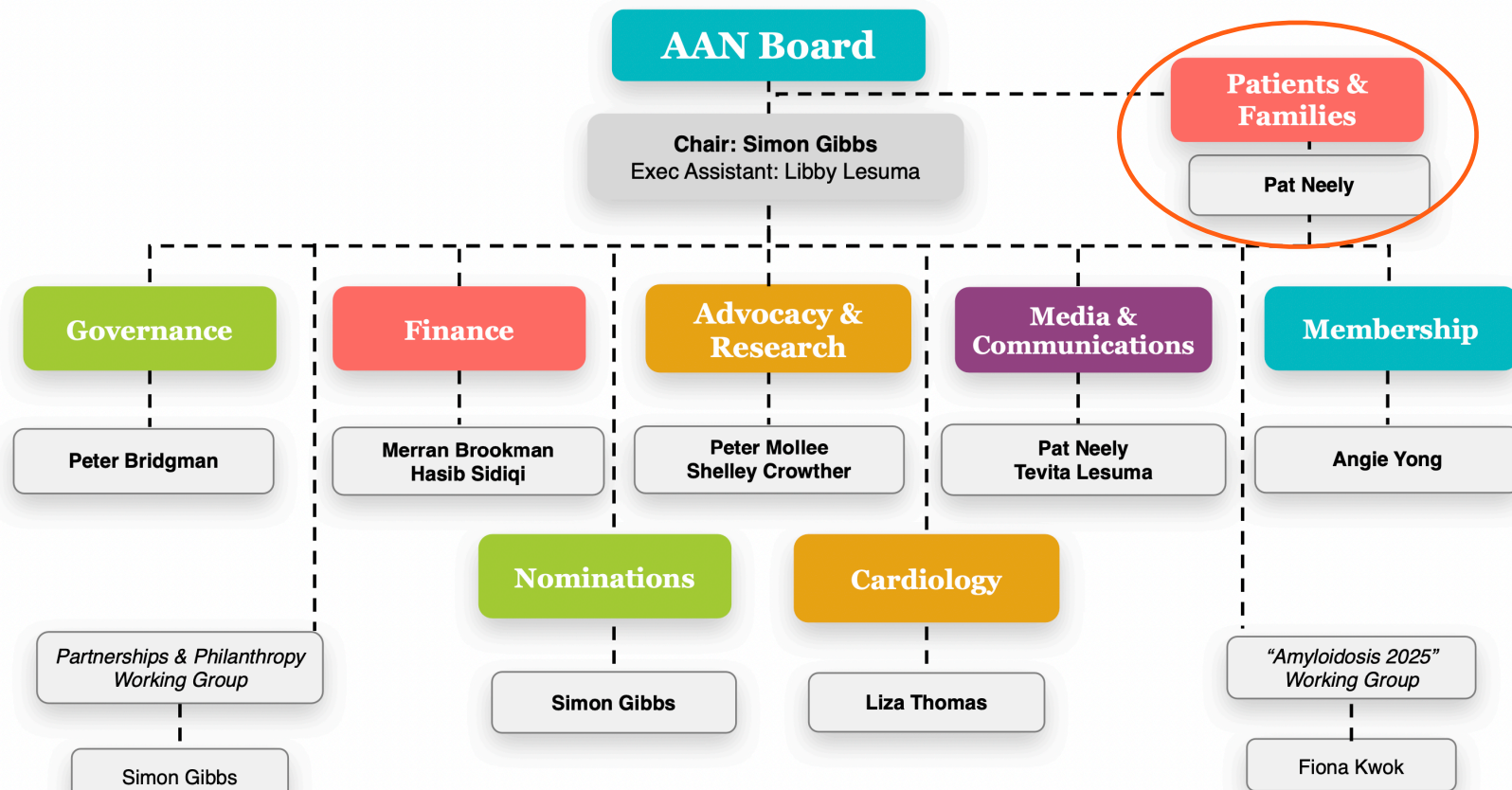


Mr
Peter Bridgman

Joined the Board in July 2022

Barrister specialising in public
policy and corporate governance

Subcommittees and Working Groups



Australian Amyloidosis Network Ltd

EDUCATION - Amyloidosis 2023 Workshops



- *Brisbane, Sydney, Melbourne, Adelaide, Hobart, Canberra (and Perth)*
- >1000 health care professionals, patients and families
- 4 international Speakers, >25 local speakers



**Prof Murielle Rousell (France),
Isabelle Lousada (USA)
Prof Martha Grogan (USA)**



Prof Ed Gane of NZ, discussing CRISPA



**Dr Michelle Ananda-Rajah MP introducing
the patient session in Melbourne**

EDUCATION - www.aan.org.au



The screenshot shows the homepage of the Australian Amyloidosis Network (AAN). At the top is a navigation bar with links: Home, Patients and Carers, Health Professionals, Our Stories, News, Events, Contact Us, and a search icon. Below the navigation bar is a large purple banner with the text "Hello and welcome." and a description of the AAN as an amalgamation of four Centres of Excellence. It includes a "Find out more about us >" link and a "Contact Us" button. Below the banner is a section titled "Here to help." with a link to "Click through to our tailored portals to find information and resources for patients & carers or health professionals". There are two buttons: "Patients & Carers" (green) and "Health Professionals" (red). Below this are three columns: "Latest News." with a link to "Additional Resources Now Available for Home Care.", "Our Stories." featuring a photo of Vince O'Donnell and his story, and "Upcoming Events." listing three events: "Special Clinic - South Australia", "National Conference", and "Visiting Specialist". At the bottom is a red ribbon banner for "Amyloidosis Day 8th May". The footer contains the AAN logo, links for "Contact Us", "Privacy Policy", "Site Map", and "Site glossary", and a statement of financial support from the Neely family.

Latest News.

Additional Resources Now Available for Home Care.

February 22nd, 2019
Read More >

On the 25th of April this year the federal government has agreed to setup a review of funding across all departments [...]

Our Stories.

Vince represents Australian patients

Hi I am Vince O'Donnell. I have a slow but fatal genetic condition known as hereditary ATTR (ATTRv) Ttr60Ala [...]

Upcoming Events.

Special Clinic - South Australia
Date: April 26 Time: 9:00 am - 5:00 pm

National Conference
Date: April 29 Time: 8:00 am - 5:00 pm

Visiting Specialist
Date: April 30 Time: 9:00 am - 5:00 pm

View All Events

Amyloidosis Day 8th May

Footer:
AAN logo | Contact Us | Privacy Policy | Site Map | Site glossary
This web site is financially supported by the Neely family in memory of Dr Marvyn Neely, surgeon, who died of AL amyloidosis in 1996. © 2019 Australian Amyloidosis Network

Practical resource for Australian doctors, patients and carers

- Information about amyloidosis
- Details of AAN services:
 - Where we are
 - Who we are
 - How to refer
- Section on **Clinical Trials**
 - Regularly updated
- Upcoming Educational events
- Videos of past Workshops
- Patient Stories
- Donations page

Special thanks to Don Francis and Fiona Kwok

ALSO.....

- **Quarterly Virtual Patient Education sessions, coordinated by Pat Neely**

RESEARCH - *Clinical Trials*



- **ATTR**

- Acoramidis (AG10-301/304)
- Eplerotersen (Cardio TTRansform)
- Patisiran (Apollo-B)
- Vutisiran
 - Helios-A for ATTRv
 - Helios-B for ATTRwt
- Tafamidis OLE
- Inotersen - ATTRv neuropathy

- **AL**

- CAEL-101 monoclonal antibody
- Birtamimab monoclonal antibody (AFFIRM)
- Pomalidomide and isatuxumab (MM24 - [ALLG and IFM](#))
- Zentalis - novel BCL2-inhibitor
- Ascentage - pomalidomide with BCL2 inhibitor, lisafoclax
- Iberdomide and venetoclax (VIBER - [ALLG](#))



First patient in the world to receive AT-02

ATTRALUS AT02 study recruiting in Melbourne, Brisbane, Adelaide and Perth

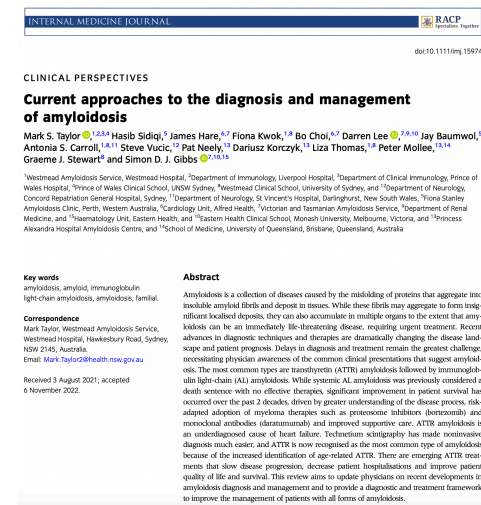
→ first in human pan-amyloid removal study, well tolerated

→ **AAN enrolled the first 20 patients in the world!**

RESEARCH - Publications



1. Ferkh A, Tjahjadi C, Stefani L, Geenty P, Byth K, De Silva K, Boyd AC, Richards D, Mollee P, Korczyk D, Taylor MS, Kwok F, Kizana E, Ng ACT, Thomas L.
 - **Cardiac "hypertrophy" phenotyping: differentiating aetiologies with increased left ventricular wall thickness on echocardiography.**
Front Cardiovasc Med. 2023 Jul 3;10:1183485
2. Chong YP, Lim SM, Loh TP, Mollee P, Wijeratne N, Choy KW.
 - **Screening for and diagnosis of monoclonal gammopathy**
J Clin Pathol. 2023 Aug 21. Online ahead of print.
3. Taylor MS, Sidiqi H, Hare J, Kwok F, Choi B, Lee D, Baumwol J, Carroll AS, Vucic S, Neely P, Korczyk D, Thomas L, Mollee P, Stewart GJ, Gibbs SDJ.
 - **Current approaches to the diagnosis and management of amyloidosis.**
Intern Med J. 2022 Dec;52(12):2046-2067
4. Wechlekar AD, Cibeira MT, Gibbs SD, Jaccard A, Kumar S, Merlini G, Palladini G, Sanchowala V, Schonland S, Venner C, Boccadoro M, Kastiris E.
 - **Guidelines for non-transplant chemotherapy for treatment of systemic AL amyloidosis: EHA-ISA working group**
Amyloid 2023 Mar;30(1):3-17
5. Maurer, *et al.* "Primary Results from APOLLO-B Open-label Extension Study of Patisiran in Patients with Transthyretin Cardiac Amyloidosis"
6. Shah, *et al.* "Impact of Patisiran on Health Status and Quality of Life in Patients with Transthyretin Cardiac Amyloidosis"
7. Saha, *et al.* "Baseline Characteristics of Patients with Transthyretin Cardiac Amyloidosis Enrolled in the Patisiran Expanded Access Program"



AAN Guidelines for the Diagnosis and Management of Amyloidosis in Internal Medicine Journal

Cardiac Amyloidosis Guidelines

led by Prof Liza Thomas, just accepted for publication

RESEARCH - *International Conferences*



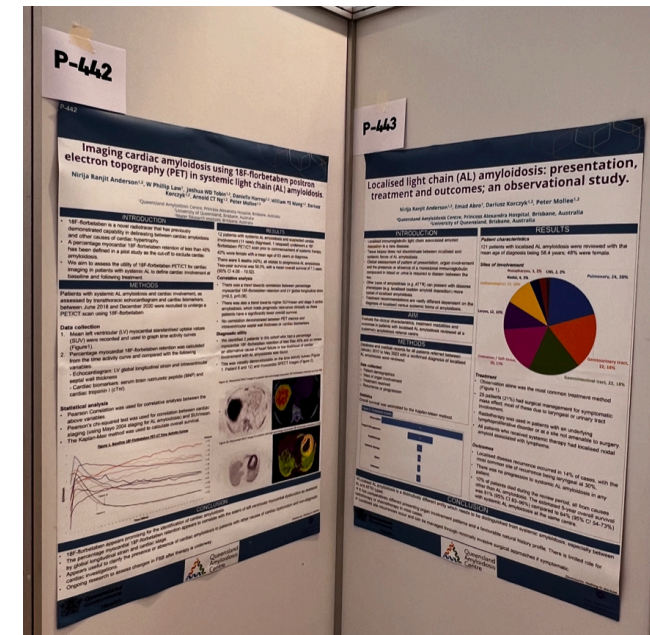
European Society of Cardiology

1. Gillmore J, Cappelli P, Garcia-Pavia P, Gibbs S, Grogan M, Hanna M, *et al* “

Efficacy and Safety of Acoramidis in Transthyretin Amyloid Cardiomyopathy: Results of the ATTRIBUTE-CM Trial

American Society of Hematology

2. "First Report on the Effects of Lisoftoclax (APG-2575) in Combination with Novel Therapeutic Regimens in Patients with Relapsed or Refractory Multiple Myeloma (R/R MM) or Immunoglobulin Light-Chain (Amyloid Light-Chain [AL]) Amyloidosis"
3. "Identifying Early Suboptimal Hematological Response in Patients with AL Amyloidosis Treated with Bortezomib-Based Chemotherapy"
4. "Outcomes of Second Line Therapy for Light Chain Amyloidosis after Initial Therapy with a Bortezomib Based Regimen"



4 abstracts by Dr Nirija Anderson
International Myeloma Workshop

CLINICAL CARE - AAN Services



1. Advice on diagnosis and management

- Histology reviews and MDMs
- Monthly Virtual National Grand Rounds

2. Expanding Access AAN Service

- Welcome our newest AAN Centre - *St Vincent's Hospital, Sydney*
- Welcome our first Affiliate Service - *Royal Adelaide Hospital*

3. Drug Access

- Diflunisal by Special Access Scheme
- Tafamidis by Pfizer Bridging Scheme

4. Diagnosing hereditary disease

- Genotyping at Westmead Hospital

5. Mass spectrometry service

- Queensland Amyloidosis Service
- Awaiting NATA accreditation

ADVOCACY - *Pharma and Government*



Advocating on Zoom or in person

- International Myeloma Workshop, Athens; ATTR Workshop, Zurich
- American Society of Hematology, San Diego

Janssen

- Supported PBAC submission for **daratumumab** → **NOW AVAILABLE on PBS for new AL patients**
- Lobbying for teclistamab Australian-European collaborative study

Pfizer

- Supported PBAC submission for **tafamidis** → **SOON AVAILABLE on PBS for ATTR patients**
- Proposal for an elrantomab trial in AL amyloidosis → *Declined sadly*

Alnylam

- Supporting PBAC submission for **patisiran** → **hopeful for PBS listing for ATTRv patients soon**
- Now TGA licensed but expensive \$\$\$ - pushing for compassionate access program

BridgeBio

- Steering committee for AG10 (acoramidis) trials
- Success in advocating for ACT-EARLY trial to be brought to Australia

BMS

- Advocating for an iberdomide study in R/R AL amyloidosis

Zentalis and Ascentage

- BCL-2 inhibitors in clinical trials for t(11;14) positive AL amyloidosis

Intellia

- Bringing CRISPA studies in ATTR to Australia in 2024

Attralus

- Advocating for every mainland state in Australia has AT02 trial available
- Advocating for an **AT01 imaging study** to come to Australia



The AAN in Parliament House

Other Milestones for the AAN



- **Huge Growth in Membership**
 - Now almost 350 members!
 - 78 specialists
 - 48 trainees/nurses
 - 208 patients and families
- **Healthy Financial gains**
- **Growth of Philanthropic Giving**
 - *Thank you!*
- **Appointment of an Executive Assistant**
 - Mrs Libby Lesuma

What are our plans for 2024?



- **AAN Board and PFAC Meetings**
 - Joint meeting 7th December
 - *How can we improve the patient journey?*
- **Strategy Day - late January 2024**
 - Ideas welcome !
 - Long term vision
 - Our own clinical trials? Eg. Supportive care
 - AAN Nurse for Patient Education and Support?
 - How can we best collaborate with other groups ?
 - Myeloma Australia, Heart Foundation, CSANZ, ISA, ALLG, IFM, EMA etc
- **Orderly Board Changes**
 - Dr Simon Gibbs -> New York sabbatical in ATTR Amyloidosis 2024
 - A/Prof Peter Mollee to assume Chair
 - Welcome Dr Olga Motorna, Melbourne
 - Mrs Pat Neely to retire after >20 years of service
 - ***Need a new Patient Advocate and a new Company Secretary***

What are our plans for 2024?



- **Research**

- Continue and expand our Clinical trials portfolio and international collaboration
- Collaborate more with basic scientific research in Sydney
- Look for grant opportunities for funding
- Travel Grants for Young Investigators to attend conferences and promote research
- Amyloidosis Fellow (*through donations*) and PhD candidate at Eastern Health

- **Advocacy**

- Continue to lobby for compassionate access to novel therapies and government funding

- **Clinical Care**

- Look for other collaborative services to join as affiliates to increase excellent local patient care
- Engage less involved specialties - Neurology, Gastroenterology, Specialist nursing, Dietitians
- Ensure holistic and supportive care, especially for ATTR patients - *not just a tafamidis script*

- **Membership and Donation Drive**

- Continue HCP and Patient Newsletters and Educational Events
- Aim to increase membership to >100 health care professionals, 300 patients and carers
- Expand and Improve the Partnership and Philanthropy Working Group
- Aim for a 25% increase in donations for 2023
- *Please continue to give for our ongoing research!*

Establish & Expand Amyloid Registries



- **Myeloma and Related Diseases Registry (MRDR)**
 - AL Amyloidosis now included
 - Special thanks to Dr Masa Lasica and A/Prof Peter Mollee
 - *We encourage physicians to list all AL amyloidosis patients*
- **ATTR Amyloidosis Registry**
 - Led by Dr James Hare
 - Hopefully to open in late 2024
 - Agreed model with Monash Uni
 - Now seeking funding from
 - Donations
 - Government
 - Pharma