

Patient's Alliance September 2019

VINCE & TERRI O'DONNELL FOR
AUSTRALIAN AMYLOIDOSIS NETWORK

[HTTP://AMYLOIDOSIS.NET.AU/](http://amyloidosis.net.au/)

[HTTPS://ATTRMEETING.ORG/SCHEDULE/](https://attrmeeting.org/schedule/)

<https://attrmeeting.org/live-stream/>

(NOTE: please go to end of report for Video link includes lecture listings & times)



VENUE <https://www.langenbeck-virchow-haus.com/en.html>

2ND EUROPEAN MEETING FOR ATTR AMYLOIDOSIS FOR DOCTORS & PATIENTS REPORT by Vince & Terri O'DONNELL
 vnt1955@icloud.com 0404460623 34 Blue Water Blvd Mulambin Queensland Australia (living the life in a van)



Wearing our Princess Alexandra Amyloidosis T's



Our NZ Friends Jaime & Aubrey



The Alliance - Board: President Jean Christophe-Fidalgo
 Treasurer Giovanni d'Alessio
 Secretary Koenraad Vwerhagen

Support: Francoise Pelcot (not on the Board)

The Alliance was created 18/6/2018 by three European founding members, France/The Netherlands/Italy, with the headquarters located in Marseille France. It was registered on 09/07/18 and published 21/07/18.

Australia is not a full country member of The Alliance, as we would need to be an incorporated patient group, with the majority of its members on the Board. However, we can be an Associate Member Country as a non-profit organisations

- which have a primary focus on Amyloidosis but are not patient owned
- Amyloidosis organisations which are less than 1 year old
- Patient Organisations representing a family of rare diseases in countries where there is no Amyloidosis Organisation to represent them
- Private or public organisations which are active in the health field and contributing to the objectives of the Amyloidosis Alliance by their mission and work.

At present Australia is listed as an Associated patients Group, along with South Korea and Spain. Candidate countries are New Zealand, UK, Canada, & Mexico. Dr Simon Gibbs & Dr Peter Mollee along with the AAN network have offered assistance for our future development.

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SUNDAY, SEPT. 1ST 2019

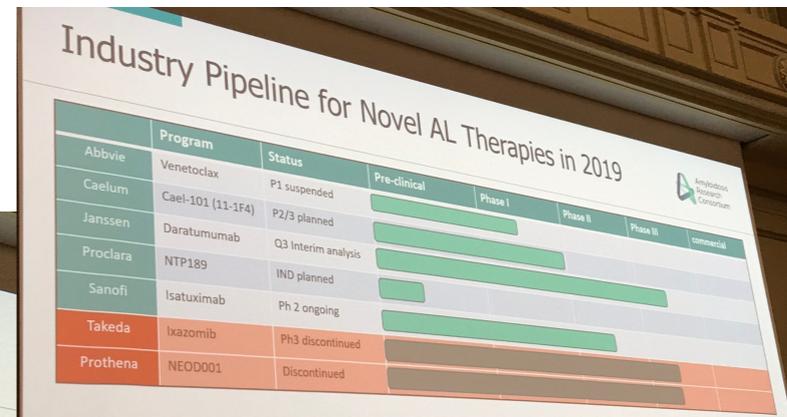
- 16:00 Registration
- 17:00 **Opening and Welcome** by *Roland Straube, Jean-Christophe Fidalgo and Hartmut Schmidt* on behalf of the organizing committee
- 17:00 – 21:00 Get-together

MONDAY, SEPT. 2ND 2019: SESSIONS 1 – 4 FOR PATIENTS

- 07:30 Registration
- 08:30 – 10:00 **SESSION 1: ALLIANCE ACTIVITIES AND TASK FORCE 1**
 Chair: *Koenraad Verhagen (Hilversum)*
 Open Session: Round Table with PO Presentation
 - Summary Overview of Alliance Activities Since Its Creation, *Jean-Christophe Fidalgo (Marseille)*
 - Task Force One: Website Alliance, *Tracey Deutsch (Pittsboro)* and *Dorien Müller (Zoetemeer)*
- 10:00 – 10:30 **Coffee Break**
- 10:30 – 12:00 **SESSION 2: TASK FORCES 2 – 7**
 Chair: *Jean-Christophe Fidalgo (Marseille)*
 - Task Force Two: Which Medication Available/Not-Available and Accessible in Which Countries, *Fabio Figuereido di Almeida (São Paulo)*
 - Task Force Three: Awareness and Diagnosis, *Jean-Christophe Fidalgo (Marseille)*
 - Task Force Four: Standard for Cure and Care, *Carlos Heras-Palou (Ashbourne)*
 - Task Force Five: Research and Trials, *Koenraad Verhagen (Hilversum)*
 - Task Force Six: Advocacy, *Giovanni d'Alessio (Bologna)*
 - Task Force Seven: AL Amyloidosis, *Ben Woltering (Venlo)*
- 12:00 – 14:00 **Lunch Break**
- 14:00 – 15:30 **SESSION 3: MANAGEMENT**
 Chair: *Giovanni d'Alessio (Bologna)*
 - New Medicines and Treatments Combating hATTR Polyneuropathy, *David Adams (Paris)*
 - New Medicines and Treatments Combating Cardiomyopathy, *Julian Gillmore (London)*
 - Nutritional Aspects, *Dr. Anna Hüsing-Kabar (Münster)*
- 15:30 – 16:00 **Coffee Break**
- 16:00 – 17:30 **SESSION 4: GENERAL ASSEMBLY OF THE ALLIANCE**
 Chair: *Jean-Christophe Fidalgo (Marseille)*
- 19:00 **Dinner**
 Evening at the Bode Museum including a tour and dinner (additional fee)

Berlin: The objectives and Priorities are Awareness; Diagnosis; Treatment & Care; Research; Communication & Mutual Support; Advocacy at International Levels; Support other countries in initiating patient groups. To update a Register of Centres of Expertise in Europe first & then the world. The setting for our Forum was at the Langenbeck-Virchos House in Berlin. It was a wonderful venue, and the organisation was first class. The days were full lecture, talks, networking and information sharing - that was as impressive as the PAH Amyloidosis Forum in May 2019.

Day One: The forum opened with each participant introducing themselves, their country & diagnosis. The Alliance task force reported back on the findings from the 2018 initiatives. There were many conversations from Task Force Two (Giovanni [Italy]; Susanne [Sweden]; Fabio [Brazil]; Carlos [UK]; Don [South Korea]; Doren [The Netherlands]; Maria [Spain]; Françoise [France] Koenraad [The Netherlands] & Vince [Australia]) re medications available and accessible in each investigated country, along with upcoming trials report. In 2019 new task forces started work on:- Standards for Cure & Care; Research & Trials; Advocacy; AL Amyloidosis. We like the fact that we are looking at AA & AL along with ATTR - in the conference.



- Key Takeways**
- Expanding treatment populations for recently approved ATTR drugs
 - Many trials for second generation drugs from pharmaceutical companies are upcoming / ongoing
 - Huge increase in diagnostic trials
 - Improvements needed in AL

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MONDAY, SEPT. 2ND 2019: SESSIONS 5 – 7 FOR DOCTORS

- 07:30 Registration
- 08:30 – 10:00 **SESSION 5 – WILDTYPE ATTR AMYLOIDOSIS – WHERE DO WE STAND?**
 Chairs: *Rodney Falk (Boston), Julian Gillmore (London)*
 • Diagnosis and Epidemiology, *Julian Gillmore (London)*
 • Real Life Monitoring of Cardiac Disease, *Ali Yilmaz (Münster)*
 • Therapeutic Perspectives, *Rodney Falk (Boston)*
- 10:00 – 10:15 Coffee Break
- 10:15 – 11:45 **SESSION 6 – LONGTERM EFFECT OF DISEASE MODIFYING THERAPY IN ATTR VARIANTS**
 Chairs: *Matthias Schilling (Münster), Giampaolo Merlini (Pavia)*
 • Tafamidis, *Marcia Waddington-Cruz (Rio de Janeiro)*
 • Patisiran, *David Adams (Paris)*
 • Inotersen and Beyond – A Comprehensive Antisense Therapeutic Strategy for All Forms of ATTR, *Sotirios Tsimikas (San Diego)*
- 11:45 – 12:15 Lunch Break
- 12:15 – 13:45 **Symposium I: Lunch Symposium for Doctors by Alnylam – How and When: An Expert Discussion on Management of hATTR Amyloidosis**
 Lunch will be provided by Alnylam
 • Welcome and Introduction by *Hartmut Schmidt (Münster)*
 • How Can We Capture the Patient Transition from Asymptomatic to Symptomatic and How Do We Evaluate the Whole Patient? *Lucia Galan Davila (Madrid)*
 • How Do We Define Disease Progression for hATTR Amyloidosis? *David Adams (Paris)*
 • What Can We Do to Monitor Disease Progression in Our Patients? *Fabian Knebel (Berlin)*
- 14:00 – 15:30 **SESSION 7: LIMITS IN TREATING ATTR**
 Chairs: *Marcia Waddington-Cruz (Rio de Janeiro), Michel Slama (Paris)*
 • Ophthalmologic, *Antoine Rousseau (Paris)*
 • Cardiac, *Ana Martinez Naharro (London)*
 • Neurologic, *Anna Mazzeo (Messina)*
- 15:30 – 16:00 Coffee Break
- 16:00 – 17:30 **Symposium II: Coffee Symposium for Doctors by Akcea – Multidisciplinary Care for Hereditary ATTR Amyloidosis: A 360 Degree Perspective**
 Chair: *Giampaolo Merlini (Pavia)*
 • *Diana Bonderman (Vienna)*
 • *Lucia Galan Davila (Madrid)*
 • *Hartmut Schmidt (Münster)*
 • *Vince Nicholas (Salisbury)*
 • *Koenraag Verhagen (Hilversum)*
 • *Theodora Weisz, Akcea Therapeutics (Switzerland)*
- 19:00 Dinner
 Evening at the Bode Museum including a tour and dinner (additional fee)

Day One (Cont'd)

The Alliance also welcomed the task force report re our patients friends with AL into the group. The key points they reported on were: More members of the Alliance required to assist with the task force: They will work to establish an inventory of methods for cure and care in the different countries and will further access trials. A considered part of their discussion focuses around the ongoing need to be included in the Alliance particularly given there are so many close parallels. Of particular interest to both Terri & myself was the Nutritional Aspects report by Dr Anna Husing-Klabar (Munster). To this end, both of us have asked to join the Lifestyle taskforce if the initiative is voted for investigation for 2020.

Dinner at the Bode Museum, with tour, was a great setting - having a standing dinner allowed for better networking and great conversations ensued. We had very fruitful conversations with Don Kim & Hyeran Park from South Korea; Aiko Miura from Alnylam & group translator; Chieko Kukinaka, Fusami Taniguchi, Masamitsu Okada & Yukio Ando from Japan; Aubrey & Jaime Christmas from New Zealand. The group, including ourselves Vince & Terri O'Donnell from Australia, is called THE ASIA-PACIFIC REGIONAL NETWORK - a subgroup being formed under the Amyloidosis Alliance. NB: Mrs Fusami Taniguchi (Masamitsu Okada, Japan) is also talking to the patients & other groups in Japan.

We would also like China, Thailand, Vietnam, India, along with other Asia Pacific countries in the hope they will join us.

The presentation by Professor Yukio Ando, pic on left here, and 2nd from the right. It was enlightening to us, & will help us going forward in choosing a drug trial, with Dr Peter Mollee.



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TUESDAY, SEPT. 3RD 2019: SESSIONS 8 – 11 FOR PATIENTS AND DOCTORS

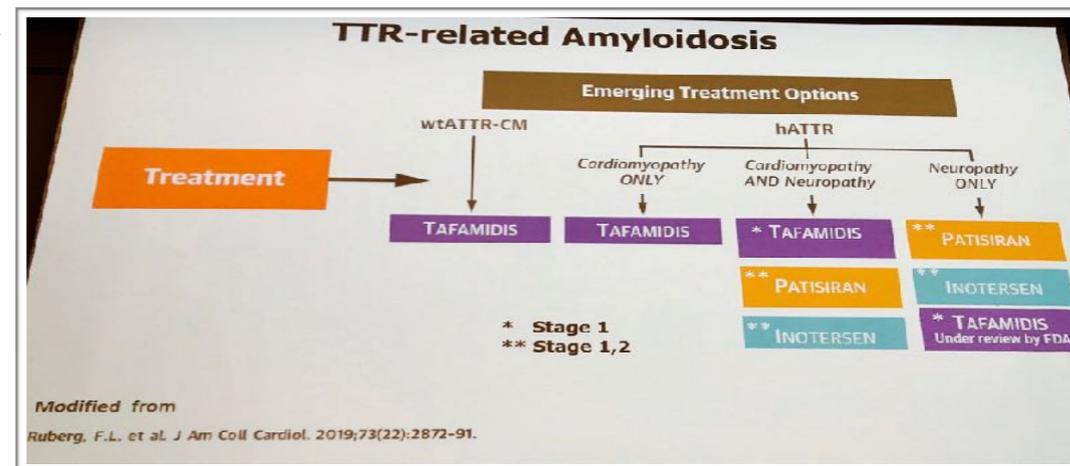
- 07:30 Registration
- 08:30 – 09:45 **SESSION 8 – MANAGING ATTR AMYLOIDOSIS: REAL LIFE IN EUROPE**
 Chairs: Koenraad Verhagen (Hilversum), Julian Gillmore (London)
 • Read out Doctors' Day, Julian Gillmore (London) and David Adams (Paris)
 • Read out Patients' Day, Carlos Heras-Palou (London)
 • Panel: Filling the Gap Between Cure and Care, Koenraad Verhagen (Hilversum), Jean-Christophe Fidalgo (Marseille), Tatjana Weiss (Münster), Julian Gillmore (London), David Adams (Paris)
- 09:45 – 10:30 **Coffee Break and POSTER SESSION I**
 Chairs: Per Westermark (Uppsala), Maria João Saraiva (Porto)
- 10:30 – 12:00 **SESSION 9: IDENTIFYING AND CARING OF ATTR IN THE FUTURE**
 Chairs: Jean-Christophe Fidalgo (Marseille), Ole Suhr (Umeå)
 • AG10 Update, Julian Gillmore (London)
 • Gastroenterologic Challenges Including Nutrition, Jonas Wixner (Uppsala)
 • Strategies for Identifying Symptomatic Patients, Hans Nienhuis (Groningen)
 • ATTR in Bulgaria, Ivailo Tournev (Sofia)
 • ATTR in Cyprus, Savanna Andreou (Nicosia)
 • ATTR Epidemiology in Europe, Yeşim Gülşen Parman (Istanbul)
- 12:00 – 13:00 **SESSION 10**
 Chairs: Teresa Coelho (Porto), Michel Slama (Paris)
 • Tafamidis on the Long Term Use in Real Life, Teresa Coelho (Porto)
 • TTR Stabilizer in Cardiac Patients, Claudio Rapezzi (Bologna)
- 13:00 – 14:00 **Lunch Break**
- 14:00 – 15:30 **SESSION 11: WHEN TO TREAT – WHEN TO SWITCH – WHEN TO STOP**
 Chairs: David Adams (Paris), Claudio Rapezzi (Bologna), Julian Gillmore (London), Isabelle Lousada (Newton)
 • Neurological View, Yukio Ando (Nagasaki)
 • Cardiological View, Diana Bodermann (Vienna)
 • Current Status on Ongoing Studies in the Field of Amyloidosis, Isabelle Lousada (Newton)
- 15:30 – 16:15 **Coffee Break and POSTER SESSION II**
- 16:15 – 17:45 **SESSION 12 – PERSPECTIVES OF AMYLOIDOSIS CENTERS: IMPLEMENTING PRECISION MEDICINE**
 Chair: Violaine Planté-Bordeneuve (Paris), Isabelle Lousada (Newton)
 • Emerging Diagnostics for Earlier Diagnosis: Genetic Screening in ATTR Amyloidosis, Laura Obici (Pavia)
 • The Integration of Data Across Amyloidosis Expertise Centers, Fernando Exposto and Finlay MacDougall (London)
 • Accelerating Diagnosis by Innovative Health Technology, Edmund Druage and Finlay MacDougall (London)
 • CRISPR-Mediated Therapy for Amyloidosis, Mark McKee, Vice President, Clinical Development Intellia Therapeutics (Boston)
 • Panel: Managing an Amyloidosis Expert Centre – Which Disciplines? Reimbursement? Referrals? Research? Home Care?

Day Two: Doctors aren't always sure either...

It was major revelation, not shocking, but certainly thought provoking when, on Prof Julian Gillmore posed the questions When to treat? When to switch? When to Stop? <https://attrmeeting.org/live-stream/> go to 4:44:19 to view.

We are beginning to realise that GP's may be the group in dire need of being educated re Amyloidosis. Ideally, every medical school across all Primary Health Care should be educated going forward, and we know Australian Amyloidosis does this in some capacity, patients need to be involved in this - at the very least aware of where this is happening. Perhaps we could look at being guest speakers at the universities for future doctors. In this pilot study most GP's DID NOT refer, even though the alert showed, re possible Amyloidosis diagnosis alert popped up on their computer screen <https://attrmeeting.org/live-stream/> Pilot: Using Electronic Health Record Algorithm & **Best Practice Alert** to identify patients with systemic Amyloidosis 1:05:00 to view.

It was a nice surprise to be one couple, of many, to be video interviewed on Day Two by Theodora Weisz - Akcea Therapeutic (Switzerland). We know we left heaps out during the session, it's very nerve racking being a video stars LOL. Theodora will send through a copy to us, unknown timeframe, which we will share on the FB and AAN. With the idea we would advocate for ourselves re access to trials. Hard to do if we don't know the info. Love this slide.



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TUESDAY, SEPT. 3RD 2019: FINAL CLOSURE

17:45 – 18:00 **ANNOUNCEMENT OF POSTER PRIZES AND FINAL CLOSURE OF THE MEETING**
Maria João Saraiva (Porto), Per Westermark (Uppsala), David Adams (Paris), Hartmut Schmidt (Münster), Julian Gillmore (London)

WEDNESDAY, SEPT. 4TH 2019: SATELLITE WORKSHOP

08:30 – 12:45 **SATELLITE WORKSHOP FOR PATIENTS:
HOW TO START AND MATURE A PATIENT ORGANISATION**
by PASComp (Patients Associations Support Company) sponsored by Akcea

08:30 – 10:00 Steps to Set up a Patient Association, PASComp

10:00 – 10:15 Break

10:15 – 10:45 How to Use Social Media, Theodora Weisz

10:45 – 11:00 Break

11:00 – 11:45 How to Mature a Patient Association, PASComp

11:45 – 12:00 Break

12:00 – 12:45 Fundraising Issues and Ideas, PASComp

**2ND EUROPEAN ATTR AMYLOIDOSIS MEETING
FOR PATIENTS AND DOCTORS**

WHEN 01.-04.09.2019
WHERE Langenbeck-Virchow-Haus, Luisenstraße 58/59, 10117 Berlin
CONTACT Patientenverband Familiäre Amyloid Polyneuropathie (FAP e.V.)
info@attrmeeting.org
Tel.: 0049 (0)251 924 511 55
For detailed information see
[HTTPS://ATTRMEETING.ORG](https://attrmeeting.org)

A survey for patients with ATTR amyloidosis will be available at the desk of the FAP e.V. on level 1. Any patient is welcome to take part. Please contact us at the desk if language barriers exist.

Last Day

It was unfortunate that we didn't realise in time that this day was added, luckily for us Aubrey and Jaime Christmas, from NZ, sent information. See last written section in this report. We probably would have learnt heaps from Theodora who presented Workshops for Patients: How to use social media by Theodora Weisz, Akcea Therapeutics. (See last page)

Our main takeaways from the forum: Advancements are to be applauded, achievements across the whole sector; ensuring greater education and information, education availability to Primary Health carers, GPs, Specialists and the general community, as well as access to medications across the whole world. The next Doctors and Patients Conference will be held in London in 2021.

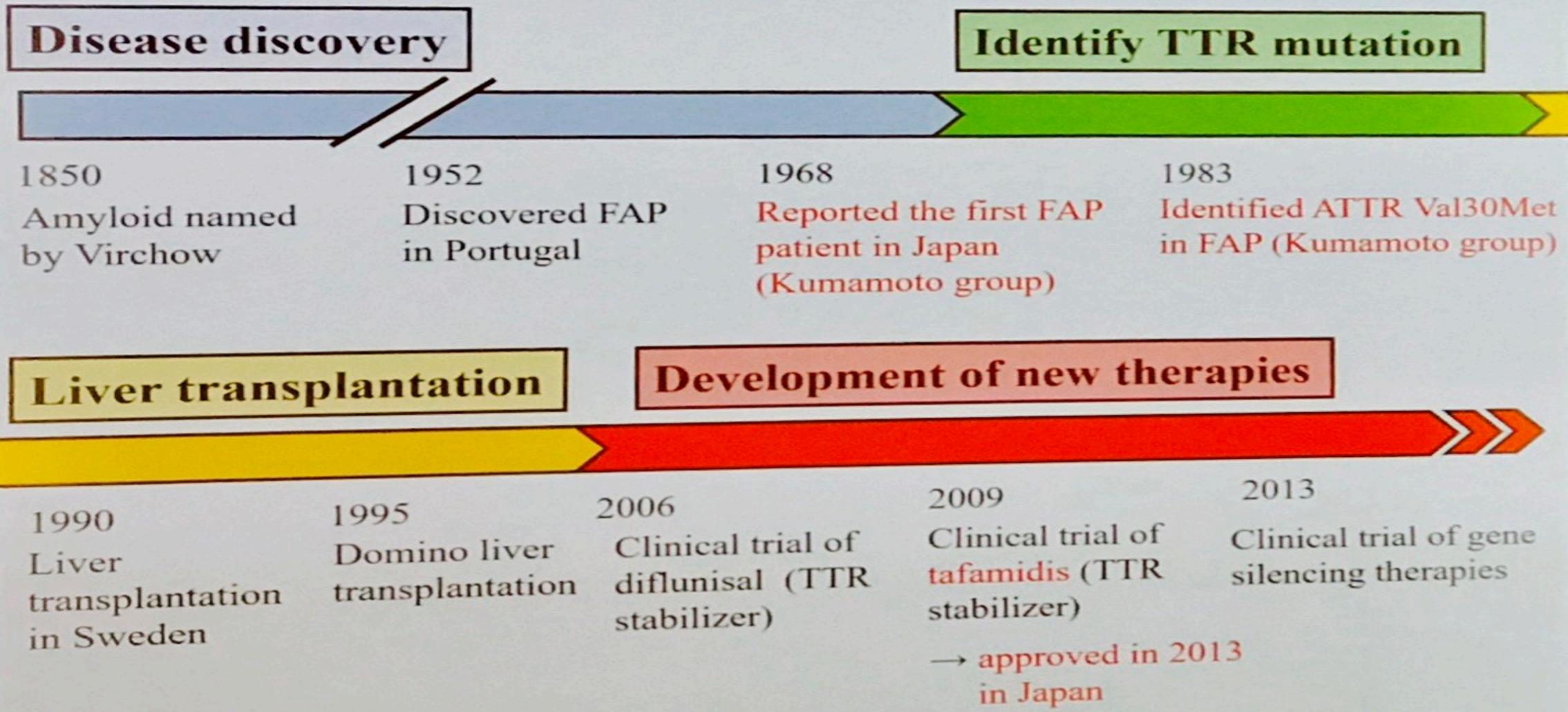
For the Australian scene, our ongoing work for us (Vince & Terri) will include the following:

Continue the work of the work plan developed following the Paris gathering in 2018. This includes what status Australia's formal membership should eventually adopt. To work with the AAN to become incorporated so as to formally join the World Alliance - Voice of Patients as a member country.

From 2019 Forum - to work with our newly formed Asia-Pacific Amyloidosis Regional Network. An initial meeting was held in Berlin and we have commenced work on the aims, goals, etc.

Within the above two points, to continue to seek others who would like to work with us to fly the flag for all people living with Amyloidosis and their carers/partners in Australia.

Brief history of ATTR amyloidosis research



<https://attrmeeting.org/live-stream/>

Please find subject list, and approximate times they appear on the video, e.g., 1hr 5mins & 0secs would look like 1:05:00 :-

New Treatments 33:45

Gastro Challenges 41:17

Pilot: Using Electronic Health Record Algorithm & Best Practice Alert to identify patients with systemic Amyloidosis
1:05:00 (we found this extremely alarming, as GP's mostly didn't refer - we feel we should be educating medical students)

ATTR Bulgaria 1:12:35

ATTR V30M Amyloidotic neuropathy in Cyprus 1:26:00

ATTR In Israel 1:37:20

ATTR Epidemiology in Europe 1:49:38

Tafamidis on the Long Term Use in Real Life. 2:06

TTR Stabiliser In Cardiac Patients 2:32:05

Neurological View. Recent Therapeutic Trial in ATTRv Amyloidosis 3:55:35

Clinical Trials Status Update 4:21:35

Eye Opener. When to treat? When to switch? When to Stop? 4:44:19 (Humbling, for us, to see & brave of them to share)

Song my Amyloidosis 5:30:06 (Highly Recommended) - we have the 2018 songs as well, on CD)

Pre-symptomatic Testing for adult onset hereditary diseases 6:15:00

Integration of data 6:37:15

Medicated Therapy for ATTR 7:12:00

Poster Presentations 7:48:10



Thank you NZ Amyloidosis Network for the notes on the Power of Social Media

by Theodora Weisz 4/9/19 Berlin

- Disclosure:
 - Conflict of interest disclosure: Akcea employee - I state that I am here in my personal capacity, not representing the company.

Theodora presented a slideshow going through the various choices and explained the importance of this assisting in getting your story across. The biggest takeaway from session was that Social Media is very important:-

- Social Media Platforms: Facebook; Instagram; Nextdoor - Neighbourhood App; Pinterest; Google DRIVE; YouTube; LinkedIn; Twitter; & Blogs alongside Web Pages!
- You should post
 - Facebook 3-10 times per week
 - Twitter at least 2-5 times a day
 - LinkedIn 2-5 times per week
 - Pinterest 5-15 times per day
 - Blog - it depends on your goal



Without even mentioning YouTube, which we will have to learn to utilise.

