

4TH INTERNATIONAL ATTR Amyloidosis meeting for patients and doctors

ORGANIZED BY



**AMYLOIDOSIS
ALLIANCE**

THE VOICE OF PATIENTS

WITH THE COLLABORATION OF



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November, 2-3 2023

Venue:

**ILUSTRE COLEGIO OFICIAL
DE MÉDICOS DE MADRID**

Santa Isabel, 51. 28012 Madrid

#ATTRAmyloidosis2023



PROGRAM

attramyloidosis2023.com

MADRID

ORGANIZED BY



**AMYLOIDOSIS
ALLIANCE**

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WITH THE
COLLABORATION OF



Hospital Universitario
Puerta de Hierro
Majadahonda



European
Reference
Network

for rare or low prevalence
complex diseases

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ORGANIZING COMMITTEE

Prof. Pablo Garcia-Pavia

University Hospital Puerta de Hierro Majadahonda, Madrid

Dr. Esther Gonzalez-Lopez

University Hospital Puerta de Hierro Majadahonda, Madrid

Prof. Julian Gillmore

National Amyloidosis Centre, University College London, London, UK

Prof. David Adams

Centre de référence des Neuropathies amyloides familiales
et autres neuropathies rares France

Prof. Thibaud Damy

Centre de référence des Amyloses Cardiaques France

Dr. Laura Obici

Amyloidosis Research and Treatment Centre,
IRCCS Fondazione Policlinico San Matteo, Pavia, Italy

Jean-Christophe Fidalgo

President of the Amyloidosis Alliance

Catilena Bibiloni

ABEA Asociación

Agnès Farrugia.

Director of the Amyloidosis Alliance

David Gregory

Patients organization (Ukatpa)



PROGRAM



PATIENTS ASSOCIATION CHANNEL

Thursday November 2nd

08:00 - 08:30 am

REGISTRATION

08:30 - 09:00 am

**Introduction by a spanish representant
of the Health ministry**

09:00 - 09:45 am

Amyloidosis Alliance Introduction and Actions

Agnès Farrugia (Marseille, France) and Jean-Christophe Fidalgo (Gemenos, France)

09:45 - 10:45 am

DAILY LIFE WITH AMYLOIDOSIS

Chairs: Agnès Farrugia (Marseille, France)
and Jean-Christophe Fidalgo (Gemenos, France)

Physical training for Amyloidosis Patients

Agnes Morier (Paris, France)

Occupational Therapy and hATTR Amyloidosis

Aina Gayà (Palma de Mallorca, Spain)

Complementary treatments. The French experience

Julie Pompougnac (Paris, France)

Dietary issues: nutrition

Jonas Wixner (Umeå, Sweden)

10:45 - 11:30 am

COFFEE BREAK

11:30 - 01:00 pm

HOW TO BECOME AN INVOLVED PATIENT?

How to comunicate with Doctors?

David Gregory (Shrewsbury, UK)

Educational program to learn more about your disease?

Andoni Echaniz (Paris, France)

Understanding your medical tests

Christine Chiti (London, UK)

Role of the patients Organisations to support patients

Catilena Bibiloni (Palma de Mallorca, Spain)

01:00 - 02:30 pm

LUNCH TIME

PATIENTS ASSOCIATION CHANNEL

Thursday November 2nd

02:30 - 3:30 pm

A vision toward a more holistic approach to care in hereditary ATTR amyloidosis (Alnylam)

Catilena Bibiloni (Palma de Mallorca, Spain)

Older adults with rare diseases – barriers and solutions for equitable access to care (Pfizer)

Aldona Zygmunt (Warsaw, Poland)

3:30 - 5:00 pm

CAREGIVERS SUPPORT NETWORK?

Chair: Laura Obici (Pavia, Italy)

Importance of self-care (physical, mental & emotional wellbeing)

Jaime Christmas (Auckland, New Zealand)

When to seek help as a caregiver and where to find help

Mervyn Carr (Ontario, Canada)

How to be empowered in the role of caregiving

Rocío Eiros (Salamanca, Spain)

Being resilient no matter the outcome

Lucinda Nicholas (London, UK)

5:00 - 5:30 pm

COFFEE BREAK

5:30 - 6:30 pm

DARE TO SPEAK ABOUT

Chair: Thibaud Damy (Paris, France)

Palliative care, what it means and what it does not mean, the myth and the truth

Kenneth Lång (Piteå, Sweden)

Genetic testing for family members genetic counseling

Inés Losada (Palma de Mallorca, Spain)

Discussion about sex and the impact of Amyloidosis

Ferrán Martínez (Madrid, Spain)

9:00 pm

DINNER

DOCTORS CHANNEL

Thursday November 2nd

08:00 - 08:30 am

REGISTRATION

08:30 - 09:00 am

**Introduction by a spanish representant
of the Health ministry**

09:00 - 09:45 am

**Precision Therapeutics With CRISPR-Based Technology:
Preparing for a New Era of Medicine** (Intellia Therapeutics)

Welcome and Introduction

Pablo García-Pavía (Madrid, Spain)

The Road to Precision Medicine

Pablo García-Pavía (Madrid, Spain)

**Advances in Gene Editing: From a Nobel Prize–Winning
Publication to Applications in Clinical Practice**

Rodolphe Barrangou (North Carolina, USA)

**Preparing for the Future: How Clinicians Can Include
Precision Medicine in the Management of ATTR
Amyloidosis**

Marianna Fontana (London, UK)

Q&A

09:45 - 10:45 am

LATEST ADVANCES IN PATHOPHYSIOLOGY

Chairs: Laura Obici (Pavia, Italy) and Julian Gillmore (Barnet, UK)

How do fibrils start? Vittorio Belloti (Pavia, Italy)

How do fibrils grow? Lorena Saelices (Dallas, US)

How do fibrils dissapear? Philip Hawkins (London, UK)

10:45 - 11:30 am

COFFEE BREAK



11:30 - 01:00 pm

LATEST DATA FOR PATIENTS DIAGNOSIS IN A CHANGING WORLD

Chairs: Mazen Hanna (Ohio, UK) and Isabel Conceicao (Lisboa, Portugal)

State of the art. Julian Gillmore (Barnet, UK)

Abstract session

- [99MTC] TC-HYDROXYDIPHOSPHONATE UPTAKE IN SOFT TISSUE REFLECTS AMYLOID LOAD IN SUBCUTANEOUS ABDOMINAL FAT TISSUE AND HARBORS PROGNOSTIC VALUE IN WILD-TYPE TRANSTHYRETIN AMYLOIDOSIS PATIENTS
Tingen, Hendrea Sanne Aletta; Tingen, Hendrea S.A.; Groothof, Dion; Tubben, Alwin; Houwerzijl, Ewout J.; Muntinghe, Friso L.H.; Van Der Meer, Peter; Hazenberg, Bouke P.C.; Slart, Riemer H.J.A.; Nienhuis, Hans L.A.
- ENABLING THE DEVELOPMENT OF SERUM TTR AS A BIOMARKER FOR TREATMENT OF ATTR AMYLOIDOSIS
Gillmore, Julian D.; Täubel, Jörg Täubel; Gane, Ed; Pilebro, Björn; Maitland, Michael L.; Rocha, Ricardo; Olbert, Joy; Leung, Adia; Smith, Derek; Pickard, Michael D.; Boiselle, Carri; Xu, Yuanxin; Zhu, Peijuan; Gutstein, David; Walsh, Liron; Adams, David
- FIFTY YEARS OF EXPERIENCE WITH HEREDITARY TRANSTHYRETIN AMYLOIDOSIS FIRST REPORT FROM SVEATTR
Wixner, Jonas; Stenberg, Lotta; Anan, Intissar; Pilebro, Björn; Suhr, Ole; Wixner, Jonas
- LONGITUDINAL ANALYSIS OF SERUM NEUROFILAMENT LIGHT CHAIN LEVELS AS MARKER FOR NEURONAL DAMAGE IN HEREDITARY TRANSTHYRETIN-RELATED AMYLOIDOSIS
Berends, Milou; Brunger, Anne Floor; Kroesen, Bart Jan; Drost, Gea; Lange, Fiete; Teunissen, Charlotte; In 't Veld, Sjors; Vrancken, Alexander Fje; Gans, Rijk Ob; Hazenberg, Bouke; Van Der Zwaag, Paul A; Nienhuis, Hans La
- RESULTS OF A POPULATION-BASED GENETIC SCREENING PROGRAM FOR HEREDITARY TRANSTHYRETIN AMYLOIDOSIS.
De Castro Campos, Daniel; De Frutos Seminario, Fernando; Caraballo Ramos, Isabel; Martinez Chaves, Victoria; Corral Azor, Adoración; Berchid Débdi, M. Solimán; Ochoa, Juan Pablo; González-lópez, Esther; Dominguez, Fernando; Cabrera-romero, Eva; Urbano-moral, Jose Angel; García-pavía, Pablo
- SKIN BIOPSY AS A DIAGNOSTIC TOOL FOR ATTRV NEUROPATHY IN THE UK
O'Donnell, Luke; Carganillo, Roy; Laura, Matilde; Gilbertson, Janet; Rowczenio, Dorota; Razvi, Yousuf; Gillmore, Julian; Fontana, Marianna; Skorupinska, Mariola; Rossor, Alexander; Reilly, Mary M

DOCTORS CHANNEL

Thursday November 2nd

01:00 - 02:30 pm

LUNCH TIME

02:30 - 03:30 pm

Stemming the multisystemic progression of hATTR amyloidosis – how do we turn the tide? (Alnylam)

Welcome and introductions

Julian Gillmore (London, UK)

Turning the tide: Evolution of the patient care pathway

Mazen Hanna (Ohio, UK)

Turning the tide: Therapeutic innovation in hATTR amyloidosis

Isabel Conceição (Lisboa, Portugal)

Ask the experts

All speakers

Summary and close

Julian Gillmore (London, UK)

This is an Alnylam-sponsored promotional symposium; organised and funded by Alnylam Pharmaceuticals. It may contain information on Alnylam medicinal products; for healthcare professionals only.



DOCTORS CHANNEL

Thursday November 2nd

03:30 - 5:00 pm

LATEST DATA ABOUT PATIENTS' TREATMENT IN A CHANGING WORLD

Chairs: Esther González-López (Madrid, Spain) and Thibaud Damy (Paris, France)

State of the art. Andoni Echaniz-Laguna (Paris, France)

Abstract session

- ANTI-PEG ANTIBODIES REDUCE THE THERAPEUTIC EFFECT OF PATISIRAN IN PATIENTS WITH HEREDITARY TRANSTHYRETIN AMYLOIDOSIS

Pilebro, Björn; Wixner, Jonas; Olsson, Malin; Anan, Intissar

- CARDIAC AND NEUROLOGICAL STABILITY IN ATTRV PATIENTS ON GENE SILENCING THERAPY

Razvi, Yousuf; O'donnell, Luke; Carganillo, Roy; Laura, Matilde; Skorupinska, Mariola; Rossor, Alexander; Rowczenio, Dorota; Gilbertson, Janet; Hawkins, Philip; Fontana, Marianna; Reilly, Mary; Gillmore, Julian

- CLINICAL CONSEQUENCES OF BETA-BLOCKER WITHDRAWAL IN TRANSTHYRETIN AMYLOID CARDIOMYOPATHY

Cabrera-Romero, Eva; Serrao-faria, Leidy-Alexandra; Mora-Ayestarán, Nerea; Peiró-Aventin, Belén; De Castro, Daniel; Domínguez-Rodríguez, Fernando; González-López, Esther; García-Pavía, Pablo

- PHASE 3 NEURO-TTRANSFORM STUDY: EFFICACY AND SAFETY OF EPLONTERSEN AT WEEK 85

Waddington Cruz, Marcia; Weiler, Markus; Conceicao, Isabel; Chao, Chi-chao; Marques Jr, Wilson; Parman, Yesim; Wixner, Jonas; Berk, John L.; Dasgupta, Noel R.; Obici, Laura; Jung, Shiangtung W.; Chen, Jersey; Schneider, Eugene; Viney, Nicholas J.; Gertz, Morie R.; Coelho, Teresa

- TRANSITIONING FROM INOTERSEN TO EPLONTERSEN IN PATIENTS WITH HEREDITARY TRANSTHYRETIN AMYLOIDOSIS POLYNEUROPATHY

Conceicao, Isabel; Weiler, Markus; Kowacs, Pedro; Dasgupta, Noel; Khella, Sami; Chao, Chi-chao; Attarian, Shahram; Jung, Shiangtung W.; Chen, Jersey; Schneider, Eugene; Viney, Nicholas J.; Gertz, Morie; Masri, Ahmad; Waddington Cruz, Marcia; Coelho, Teresa; Berk, John L.

- EPLONTERSEN IMPROVES AUTONOMIC NEUROPATHY SYMPTOMS IN HEREDITARY ATTR: AN ANALYSIS FROM NEURO-TTRANSFORM

Wixner, Jonas; Berk, John; Adams, David; Polydefkis, Michael; Conceicao, Isabel; Attarian, Shahram; Gilmore, Julian D.; Dyck, P. James B.; Folkvaljon, Folke; Zhou, Wunan; Chen, Jersey; Schneider, Eugene; Viney, Nicholas J.; Kwok, Jesse; Coelho, Teresa; Waddington Cruz, Marcia

05:00 - 05:30 pm

COFFEE BREAK

DOCTORS CHANNEL

Thursday November 2nd

05:30 - 06:30 pm

LATEST TOOLS IN THE FIELD

Chairs: Marianna Fontana (London, UK) and Christoph Rocken (Magdeburg, Germany)

Neurofilaments. Mary Reilly (London, UK)

PET tracers. Mazen Hanna (Ohio, UK)

Nerve imaging. Chiara Briani (Padova, Italy)

06:30 - 07:15 pm

Improving outcomes in ATTR amyloidosis: clinical and real-world perspectives (Pfizer)

Stabilizing the tetramer: an established strategy

Laura Obici (Pavia, Italy)

Optimizing outcomes in ATTR-CM with early diagnosis and management

Thibaud Damy (Paris, France)

ATTR-CM in clinical practice: suspicion, management, and beyond

Esther González-López (Madrid, Spain)

Panel discussion and Q&A

9:00 pm

DINNER



DOCTORS AND PATIENTS
CHANNEL

Friday November 3rd

08:10 - 09:00 am

TRIBUTE TO PR RAPEZZI

Chairs: Agnès Farrugia (Marseille, France)
and Pablo García Pavia (Madrid, Spain)

Elena Biagini (Bologna, Italy)

Giovanni D'Alessio (Italian patient)

Mathew S. Maurer (New York, US)

**Taking Action to Improve the Patient Journey With ATTR:
Shared Perspectives From Patients and Providers**

(PeerVoice - supported by an educational grant from
AstraZeneca)

Welcome and introduction

Laura Obici (Pavia, Italy)

**Panel discussion: Seeing ATTR Through One Another's
Eyes: Opportunities to Optimise the Patient-Physician
Partnership**

09:00 - 09:45 am

Laura Obici (Pavia, Italy), Lucía Galán (Madrid, Spain) and
David Gregory (Shrewsbury, UK)

**Panel discussion: Taking the Next Step Together:
Action Items in ATTR for Providers and Patients**

Laura Obici (Pavia, Italy), Lucía Galán (Madrid, Spain) and
David Gregory (Shrewsbury, UK)

**Audience Questions, Faculty Discussion, Final
Remarks, and Conclusion**

No treatments will be discussed at the symposium

09:45 - 10:45 am

HOW TO MANAGE SYMPTOMS FOR?

Chairs: Marianna Fontana (London, UK) and Lucía Galán (Madrid,
Spain)

Dyspnea/heart failure. Esther González López (Madrid, Spain)

Arrhythmia. Peter Van der Meer (Utrecht, Netherlands)

GI manifestations. Jonas Wixner (Umeå, Sweden)

**DOCTORS AND PATIENTS
CHANNEL**

Friday November 3rd

10:45 - 11:30 am

COFFEE BREAK

11:30 - 01:00 pm

HOW TO MANAGE SYMPTOMS FOR?

Chairs: Andoni Echaniz-Laguna (Paris, France) and Mary Reilly (London, UK)

Peripheral neuropathic manifestations

Marcia Waddington Cruz (Rio de Janeiro, Brasil)

Central neurological manifestations and pain

Isabel Conceição (Lisboa, Portugal)

Synovial manifestations

Oscar Westin (Copenhagen, Denmark)

Ocular complications

Angelo Minnella (Rome, Italy)

01:00 - 02:00 pm

LUNCH TIME

02:00 - 02:45 pm

BridgeBio's commitment to the ATTR community.

(Bridge Bio)

Julian Gillmore (London, UK)

Panel discussion and Q&A

Julian Gillmore, Mark McGovern and Pedro Pina



DOCTORS AND PATIENTS
CHANNEL

Friday November 3rd

02:45 - 04:15 pm

ATTR TREATMENTS IN 2023 AND BEYOND

Chairs: Pablo Garcia-Pavia (Madrid, Spain) and Julian Gillmore (Barnet, UK)

Current treatments available for H-ATTR. Latest info

Thibaud Damy (Paris, France)

Current treatments available for WT-ATTR. Latest info

Jose Nativi-Nicollau (Jacksonville, Florida)

Ongoing Clinical Trials

Francesco Cappelli (Florence, Italy)

Combined specific therapies. Current and future role

Mathew S. Maurer (New York, US)

From innovation to patient, how to ensure that patients benefit from treatments

Speakers and chairs

04:15 - 04:30 pm

Conclusion



POSTERS

SERUM NEUROFILAMENT LIGHT CHAIN AND AUTONOMIC NEUROPATHY IN HEREDITARY TRANSTHYRETIN-RELATED AMYLOIDOSIS

Berends, Milou; Brunger, Anne Floor; Tubben, Alwin; Tingen, Hendrea Sa; Bijzet, Johan; Van Der Zwaag, Paul A; Hazenberg, Bouke Pc; Noordzij, Walter; Slart, Riemer Hja; Nienhuis, Hans La

THE CHALLENGES OF PRE-SYMPTOMATIC TESTING FOR ATTRH AMYLOIDOSIS: LESSONS LEARNED FROM OVER 25 YEARS OF GENETIC COUNSELLING PRACTICE IN PORTUGAL

Paneque, Milena; Méndes, Álvaro; Parente Freixo, João; Sequeiros, Jorge

BASELINE CHARACTERISTICS OF PATIENTS WITH ATTR AMYLOIDOSIS ENROLLED INTO THE CONTRIBUTE REGISTRY

Silvester, Chloe; Polydefkis, Michael; Leonardi, Luca; Echaniz-laguna, Andoni; Gillmore, Julian D; Knebel, Fabian; Pareyson, Davide; Drachman, Brian; Nienhuis, Hans; Shah, Zubair; Sole, Guilhem; Svahn, Juliette; Bender, Shaun; Moffitt, Colleen; Summers, Catherine; Capocelli, Kelley; Maurer, Mathew S

SYSTEMATIC LITERATURE REVIEW (SLR) OF THE EPIDEMIOLOGY AND CHARACTERISTICS OF TRANSTHYRETIN AMYLOIDOSIS (ATTR)

Delgado, Diego; Shridharmurthy, Divya; Shivappa, Nitin; Dabbous, Firas; Mazhar, Faizan; Wittbrodt, Eric; Järbrink, Krister

RNAI THERAPY IN ATTRV AMYLOIDOSIS WITH POLYNEUROPATHY (HELIOS-A): PATIENT-REPORTED EXPERIENCES AND PREFERENCES

Asokan, Nathiyah; Obici, Laura; Gonzalez-duarte, Alejandra; Galan, Lucia; Berk, John; Bender, Shaun; Jain, Siddharth; Kumar, Varun; Capocelli, Kelley; Polydefkis, Michael

TOLERANCE AND SAFETY OF DIFLUNISAL IN THE TREATMENT OF TRANSTHYRETIN CARDIAC AMYLOIDOSIS

Peiró Aventín, Belén; Mora Ayestarán, Nerea; Cabrera Romero, Eva; De Castro, Daniel; De Frutos, Fernando; Briceño Hinojo, Ana; González-lópez, Esther; Domínguez, Fernando; Cobo-marcos, Marta; López-azor, Juan Carlos; Gómez-bueno, Manuel; Segovia, Javier; García-pavía, Pablo



POSTERS

PEOPLE WITH UNTREATED TRANSTHYRETIN AMYLOID CARDIOMYOPATHY EXPERIENCE A SIMILAR BURDEN ACROSS COUNTRIES

Bodkin, Jennifer; Damy, Thibaud; Ponti, Lucia; Hsu, Kristen; Villacorta, Eduardo; Verheyen, Nicolas; Keohane, Denis; Wang, Ronnie; Ines, Monica; Kumar, Nisith; Munteanu, Carmen; Cappelli, Francesco

THE JOURNEY TO DIAGNOSIS OF ATTRWT AMYLOIDOSIS: BURDEN OF EARLY DISEASE

Silvester, Chloe; Hanna, Mazen; Puls, Mathilde; Weijers, Laure; Finkel, Muriel; Schmitt, Paula; Merkel, Madeline P.; Kochman, Fran M.; Moffitt, Colleen; Summers, Summers; Karam, Chafic

UNDERSTANDING YOUR MEDICAL TESTS

Chiti, Bwalya Christine Mwila

OCCUPATIONAL THERAPY IN PATIENTS WITH HEREDITARY TRANSTHYRETIN AMYLOIDOSIS: MORE THAN AN INTERVENTION.

Gayá Barroso, Aina Isabel; González Moreno, Juan; Rodríguez Rodríguez, Adrián; Cisneros Barroso, Eugenia; Losada López, Inés



GENERAL INFORMATION



GENERAL INFORMATION

VENUE



ILUSTRE COLEGIO OFICIAL DE MÉDICOS DE MADRID

Santa Isabel, 51.28012 Madrid

Phone: +34 915 385 100

DATES

November 2-3, 2023

LANGUAGE

English: English-Spanish translation available

REGISTRATION

	Before 30/09/2023	From 30/09/2023
General registration	350 €	400 €
Fellow / Nursery*	150 €	200 €
Streaming (only online)	125 €	175 €
Patients	30 €	30 €
Patients with dinner	60 €	60 €
Patients (online only)	Free	Free
Industry	400 €	450 €

Registration fee includes: attendance, certificate, coffee breaks (Thursday and Friday), Lunch (Thursday and Friday) and dinner (except Patients**) VAT Included

* To get this fee It is compulsory to send the secretariat a certificate acreditating that the attendee is fellow or nurse.

Technical Secretariat can receive **new registrations until 27th October 2023**. After this date registrations should be manage at the venue, once the congress had started.

Cancellation policy

Until 30/09/2023	100% refund
From 01/10/2023	No refund

For more information:
www.attramyloidosis2023.com

ACCOMMODATION

	Double (single use)	Double
Hotel NH Nacional	200 €	230 €

Accommodation fee includes breakfast. VAT Included.

Cancellation policy

Until 20/09/2023	80% refund
Until 30/09/2023	50% refund
From 01/10/2023	No refund



Hotel NH Nacional

Paseo del Prado, 48
28014 Madrid
Tel. 914 29 66 29

For more information:
www.attramyloidosis2023.com

DINNER

RESTAURANTE NUBEL. MUSEO REINA SOFÍA

Address: Argumosa 43, 28012, Madrid

CERTIFICATES

Certificates will be emailed 7 days after the meeting

REGISTRATION AND INFORMATION

www.attramyloidosis2023.com

TECHNICAL SECRETARIAT



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