

ONLINE EVENT

14th 18th SEPTEMBER 2020

CENTRAL EUROPE SUMMER TIME (CEST)

isa

XVII INTERNATIONAL
SYMPOSIUM ON
AMYLOIDOSIS

PRESIDENT

Joan Bladé

Hospital Clínic, Barcelona, Spain

SCIENTIFIC PROGRAM

ISA INTERNATIONAL SOCIETY
OF AMYLOIDOSIS



www.isa2020.org

MONDAY 14TH SEPTEMBER

14:00 - 14:15 **Welcome**
Opening Remarks
 Joan Bladé, Barcelona, Spain
 Giovanni Palladini, Pavia, Italy

14:15 - 14:35 **OPENING LECTURE**

Amyloidosis: Classification and Epidemiology

Chair: Joan Bladé, Barcelona, Spain
Speaker: Per Westermark, Uppsala, Sweden

14:35 - 15:10 **GIAMPAOLO MERLINI AWARD AND LECTURE**

Giampaolo Merlini: Aiming for the Cure of Amyloid Disease

(Introduction by Robert A. Kyle)

15:10 - 15:20 **Break**

15:20 - 16:50 **PLENARY SESSION 1**

Basic Science: Amyloid Fibril Formation, Deposition and Clearance

Chairs:
 John Berk, Boston, MA, USA
 Francesca Lavatelli, Pavia, Italy

Amyloid fibril structures using cryo EM and ssNMR

Marcus Fändrich, Ulm, Germany

Structural basis of amyloidogenicity

Marina Ramírez-Alvarado, Rochester, MN, USA

Drivers of amyloid organ tropism and deposition

Gunilla Westermark, Uppsala, Sweden

Tissue based diagnosis and classification of amyloidosis by mass spectrometry-based proteomics

Ahmet Dogan, New York, NY, USA

Proteotoxicity and organ damage

Francesca Lavatelli, Pavia, Italy

Intrinsic mechanisms of amyloid tissue clearance

Marianna Fontana, London, UK

Development of amyloid disruptors for ATTR amyloidosis

Mitsuharu Ueda, Kumamoto, Japan

16:30 - 16:50 **Discussion**

16:50 - 17:00 **Break**

17:00 - 18:30 **INDUSTRY SPONSORED SYMPOSIUM 1 - Pfizer**

A Deeper Look at ATTR-CM: An Under-recognized and Life-threatening Illness

Chair: Pablo García-Pavía, Madrid, Spain

Mechanisms and Patterns of Cardiac Deposition in Amyloidosis

Yukio Ando, Kumamoto, Japan

Recognition and Diagnosis of ATTR Cardiomyopathy

Claudio Rapezzi, Bologna, Italy

Management of ATTR Cardiomyopathy

Pablo García-Pavía, Madrid, Spain

Panel Discussion and Q&A

18:30 - 18:40 **Break**

18:40 - 19:50 **PLENARY SESSION 2**

AL amyloidosis: Diagnosis and Management in 2020

Chairs:
 Ashutosh Wechalekar, London, UK
 Stefan Schönland, Heidelberg, Germany

Diagnosis work-up and typing

Angela Dispenzieri, Rochester, MN, USA

Red-flags for early diagnosis

Ute Hegenbart, Heidelberg, Germany

New prognostic markers

Efstathios Kastiris, Athens, Greece

Cytogenetics in AL amyloidosis

Stefan Schönland, Heidelberg, Germany

Hematologic and organ response criteria

Giovanni Palladini, Pavia, Italy

19:30 - 19:50 **Discussion**

TUESDAY 15TH SEPTEMBER

14:00 - 15:00

PLENARY SESSION 3

ATTR amyloidosis: Genetics and Basic Science

Chairs:

Merrill D. Benson, Indianapolis, IN, USA
Laura Obici, Pavia, Italy

Molecular mechanisms of ATTR amyloidosis

Maria João Saraiva, Porto, Portugal

Driving forces in ATTR amyloidosis

Vittorio Bellotti, London, UK and Pavia, Italy

Genetic signatures associated with hereditary ATTR amyloidosis

Joel Buxbaum, La Jolla, CA, USA

Factors involved in increased susceptibility to TTR amyloidogenesis

Teresa Coelho, Porto, Portugal

14:40 - 15:00

Discussion

15:00 - 15:10

Break

15:10 - 16:10

SELECTED ABSTRACT PRESENTATIONS I

Chairs:

Mitsuharu Ueda, Kumamoto, Japan
Tomás Ripoll-Vera, Palma de Mallorca, Spain

BASIC SCIENCE I

OP01

High resolution cryo-EM structure of a transthyretin-derived amyloid fibril from a patient with hereditary val30met ATTR amyloidosis

Matthias Schmidt, Ulm, Germany

OP02

Defining the cardiac amyloid proteome and its association with patient clinical characteristics and outcomes

Taxiarchis Kourelis, Rochester, MN, USA

OP03

Immunogenetic profile of purified pathological plasma cells of patients with light chain amyloidosis

Isabel Cuenca, Madrid, Spain

OP04

From protein-protein interaction to protein co-expression networks: a systems biology-based perspective to investigate amyloidosis diseases

Dario Di Silvestre, Milano, Italy

OP05

Targeting deubiquitylating enzymes USP14 and UCHL5 in systemic immunoglobulin light chain (AL) amyloidosis

Mario Nuvolone, Pavia, Italy

OP06

Membrane and soluble b-cell maturation antigen (BCMA) in systemic light-chain amyloidosis

Ping Zhou, Boston, MA, USA

15:52 - 16:10

Discussion

16:10 - 16:20

Break

16:20 - 17:50

INDUSTRY SPONSORED SYMPOSIUM 2 - Eidos Therapeutics

Wild-type Transthyretin Amyloidosis – An epidemic hiding in plain sight

Chair:

Pablo García-Pavía, Madrid, Spain

When the bright side of TTR breaks the heart

Maria João Saraiva, Porto, Portugal

Diagnosis and management of wild type TTR amyloidosis

Julian Gillmore, London, UK

Where the Wild-Type Ones Are

Esther González-López, Madrid, Spain

17:50 - 18:00

Break

18:00 - 19:00

PLENARY SESSION 4

Organ Transplantation in Systemic Amyloidosis

Chairs:

Claudio Rapezzi, Bologna, Italy
Pablo García-Pavía, Madrid, Spain

Heart transplantation in AL amyloidosis

Arnt V. Kristen, Heidelberg, Germany

Heart transplantation in ATTR amyloidosis

Mathew Maurer, NY, USA

Liver transplantation in hereditary ATTR amyloidosis

TBC

Kidney transplant in AL amyloidosis and monoclonal immunoglobulin deposition disease: who and when?

Nelson Leung, Rochester, MN, USA

18:40 - 19:00

Discussion

19:00 - 19:10

Break

19:10 - 20:10

PLENARY SESSION 5

Experts' Discussion on ASCT in AL amyloidosis: burning questions

Chair:

Francesca Lavatelli, Pavia, Italy

Panelists:

Vaishali Sancharawala, Boston, MA, USA
Heather Landau, New York, NY, USA
Hasib Siddiqi, Rochester, MN, USA
Carlos Fernández de Larrea, Barcelona, Spain
Eli Muchtar, Rochester, MN, USA

Topics:

- Patient selection
- Decreasing transplant-related mortality
- Pretransplant induction
- ASCT in patients with renal function impairment
- Any role for consolidation or maintenance?
- Is it time for CAR-T cell therapy in AL amyloidosis?

WEDNESDAY 16TH SEPTEMBER

13:00 - 14:00 **ISA Members Meeting**

14:00 - 15:30 **SELECTED ABSTRACT PRESENTATIONS II**

Chairs:

Joel Buxbaum, La Jolla, CA, USA
Violaine Plante-Bordeneuve, Créteil, France

ATTR AMYLOIDOSIS

- OP 07 **Skin biopsy in hereditary transthyretin amyloidosis with polyneuropathy in France**
TBC
- OP08 **Long-term safety and efficacy of patisiran: Global open-label extension 24-month data in patients with hereditary transthyretin-mediated amyloidosis**
David Adams, Le Kremlin Bicêtre, France
- OP09 **Long-term impact of tafamidis in patients with late-onset hereditary transthyretin amyloidosis with stage I polyneuropathy**
Roberta Mussinelli, Pavia, Italy
- OP10 **External validation of the national amyloidosis center score in an international cohort of patients with transthyretin cardiac amyloidosis**
Adrián Rivas-Pérez, Madrid, Spain
- OP11 **Evaluation of patisiran with concomitant or prior use of transthyretin stabilizers in patients with hereditary transthyretin-mediated amyloidosis**
Hollis Lin, Cambridge, MA, USA
- OP12 **Open-label study of patisiran in patients with hereditary transthyretin-mediated amyloidosis with polyneuropathy post-orthotopic liver transplant**
Julian Gillmore, London, UK
- OP13 **High resolution nerve ultrasound as a diagnostic tool for differential diagnosis and progression recognition in TTR-related familial amyloidosis**
Natalie Winter, Tübingen, Germany
- OP14 **Origin of val30met in familial amyloid polyneuropathy (TTR-FAP) in Portugal: a walk through the mutational path**
Carolina Lemos, Porto, Portugal
- OP15 **99mTc-DPD scintigraphy predicts amyloid fibril type in hereditary transthyretin amyloidosis**
Jonas Wixner, Umeå, Sweden

15:03 - 15:30 **Discussion**

15:30 - 15:40 **Break**

15:40 - 17:10 **INDUSTRY SPONSORED SYMPOSIUM 3 - Janssen**

Multidisciplinary treatment approach in the management of patients with AL amyloidosis

Chair:

Giovanni Palladini, Pavia, Italy

Diagnostic pit-falls and risk stratification in AL amyloidosis

Efstathios Kastiris, Athens, Greece

Monoclonal antibody treatment for AL amyloidosis

Ashutosh Wechalekar, London, UK

Novel targets and drugs for AL amyloidosis

Giovanni Palladini, Pavia, Italy

17:10 - 17:20 **Break**

17:20 - 18:20 **PLENARY SESSION 6**

Experts' discussion on the Treatment of Patients with AL amyloidosis non-eligible for ASCT: burning questions

Chair:

Morie A. Gertz, Rochester, MN, USA

Panelists:

Shaji Kumar, Rochester, MN, USA
Monique C. Minnema, Utrecht, the Netherlands
Paolo Milani, Pavia, Italy
Maria Teresa Cibeira, Barcelona, Spain
Arnaud Jaccard, Limoges, France
Maria Gavriatopoulou, Athens, Greece

Topics:

- Best initial therapy for fit patients
- Best initial therapy for unfit patients
- When to start therapy at relapse or progression
- Treatment at first relapse
- Treatment at later relapses or refractory disease
- Best novel emerging agents
- Role of anti-amyloid therapy

18:20 - 18:30 **Break**

18:30 - 19:20 **SELECTED ABSTRACT PRESENTATIONS III**

Chairs:

Matthias Schmidt, Ulm, Germany
Francesca Lavatelli, Pavia, Italy

BASIC SCIENCE II

- OP16 **Hepatic expression of mutant transthyretin remodels proteostasis machinery in hereditary ATTR amyloidosis**
Richard Giadone, Boston, MA, USA
- OP17 **Diagnostic potential of a novel RT-QPCR-based assay to measure CCND1MRNA expression levels in bone marrow plasma cells from AL amyloidosis patients**
Alice Nevone, Pavia, Italy
- OP18 **Machine learning predicts immunoglobulin light chain toxicity through somatic mutations**
Maura Garofalo, Bellinzona, Switzerland
- OP19 **Drosophila melanogaster as a model organism for ATTR amyloidosis**
Xiaohong Gu, Uppsala, Sweden
- OP20 **Eleven different amyloid types identified in cutaneous amyloidosis by proteomics-based typing**
Surendra Dasari, Rochester, MN, USA
- 19:05 - 19:20 **Discussion**

THURSDAY 17TH SEPTEMBER

14:00 - 15:00 **SELECTED ABSTRACT PRESENTATIONS IV**

Chairs:

Raymond Comenzo, Boston, MA, USA
Isabel Krsnik, Madrid, Spain

AL AMYLOIDOSIS I

- OP21 **New organ response criteria for light chain amyloidosis: An international validation study**
Eli Muchtar, Rochester, MN, USA
- OP22 **The quest for indicators of profound hematologic response in AL amyloidosis: Complete response remains the optimal goal of therapy**
Paolo Milani, Pavia, Italy
- OP23 **Minimal residual disease positivity by multiparameter flow cytometry hinders organ involvement recovery in AL amyloidosis patients in complete response**
Giovanni Palladini, Pavia, Italy
- OP24 **In systemic light-chain amyloidosis the best hematologic response for long-term survival is iFLC < 10mg/L**
Amandeep Godara, Boston, MA, USA
- OP25 **Comparison of measures of complete hematologic response after high dose melphalan and autologous stem cell transplantation for AL amyloidosis**
Shayna Sarosiek, Boston, MA, USA
- OP26 **The impact and importance of post-renal transplantation hematological response assessment in AL amyloidosis**
Oliver C. Cohen, London, UK

14:42-15:00 **Discussion**

15:00 - 15:10 **Break**

15:10 - 16:20 **PLENARY SESSION 7**

Hereditary ATTR Amyloidosis: Clinical Features and Follow-up

Chairs:

Rodney H. Falk, Boston, MA, USA
Lucía Galán, Madrid, Spain

Clinical features of polyneuropathy in hereditary amyloidosis

Yukio Ando, Kumamoto, Japan

Red-flags for early diagnosis in hereditary amyloidosis

Yoshiki Sekijima, Matsumoto, Japan

The global prevalence of ATTR amyloidosis

Hartmut Schmidt, Münster, Germany

Other manifestations in ATTR amyloidosis

Jonas Wixner, Umeå, Sweden

Follow-up, polyneuropathy detection, de novo manifestations and treatment after domino liver transplantation

Laura Obici, Pavia, Italy

16:00 - 16:20 **Discussion**

16:20 - 16:30 **Break**

16:30 - 18:00 **INDUSTRY SPONSORED SYMPOSIUM 4 - Akcea Therapeutics**

18:00 - 18:10 **Break**

18:10 - 19:30 **PLENARY SESSION 8**

AA and other forms of Amyloidosis

Chairs:

Martha Skinner, Boston, MA, USA
Julian Gillmore, London, UK

AA amyloidosis: current incidence and clinical presentation

Alberto Martinez-Vea, Tarragona, Spain

AA amyloidosis: management

Luís Quintana, Barcelona, Spain

AA amyloidosis associated with autoinflammatory diseases

Helen Lachmann, London, UK

Localized amyloidosis

Eli Muchtar, Rochester, MN, USA

Hereditary non-transthyretin amyloidosis

Julian Gillmore, London, UK

LECT2-associated renal amyloidosis

Tamer Rezk, London, UK

19:10 - 19:30 **Discussion**

FRIDAY 18TH SEPTEMBER

14:00 - 15:20 **SELECTED ABSTRACT PRESENTATIONS V**

Chairs:

Giovanni Palladini, Pavia, Italy
Carlos Fernández de Larrea, Barcelona, Spain

CARDIAC AMYLOIDOSIS AND OTHER FORMS

- OP27 **Prevalence and survival impact of atrial fibrillation in patients with transthyretin cardiac amyloidosis. Analysis from a large international cohort**
Adrián Rivas Pérez, Madrid, Spain
- OP28 **Impact on survival of N-terminal Pro-B-type natriuretic peptide (NT-proBNP) increase after diagnosis for cardiac transthyretin amyloidosis**
Silvia Oghina, Créteil, France
- OP29 **Diagnostic value of subcutaneous abdominal fat tissue aspirates in cardiac amyloidosis**
TBC
- OP30 **Describing the echocardiographic phenotype of transthyretin cardiac amyloidosis - What are the predictors of prognosis?**
TBC
- OP31 **Cardiac transthyretin wild type amyloidosis (ATTRwt): A prospective study of 400 patients followed at the Italian referral center**
Paolo Milani, Pavia, Italy
- OP32 **Regional cardiac uptake of 99-Tc-DPD is a novel powerful and independent prognostic marker in cardiac ATTR wild type amyloidosis**
Paolo Milani, Pavia, Italy
- OP33 **Finnish gelsolin amyloidosis causes significant disease burden but does not affect survival**
Sari Atula, Helsinki, Finland
- OP34 **Excellent outcomes of isolated renal transplantation for hereditary Fibrinogen (AFib) amyloidosis**
TBC
- 14:56 - 15:20 **Discussion**
- 15:20 - 15:30 **Break**

15:30 - 16:30 **SELECTED ABSTRACT PRESENTATIONS VI**

Chairs:

María Teresa Cibeira, Barcelona, Spain
Rámon Lecumberri, Pamplona, Spain

AL AMYLOIDOSIS II

- OP35 **A phase II study of isatuximab (SAR650984) (NSC-795145) for patients with previously treated AL amyloidosis (SWOG S1702; NCT#03499808)**
Terri Parker, CT, USA
- OP36 **Ixazomib-dexamethasone versus physician's choice in relapsed/refractory systemic AL amyloidosis: Results from the phase 3 tourmaline-AL1 trial**
Giampaolo Merlini, Pavia, Italy
- OP37 **Subcutaneous daratumumab + cyclophosphamide/bortezomib/dexamethasone in newly diagnosed AL amyloidosis: Updated safety run-in results of ANDROMEDA**
Vaishali Santhorawala, Boston, MA, USA
- OP38 **Assessment of minimal residual disease using multiparametric flow cytometry in treated patients with AL amyloidosis**
Andrew Staron, Boston, MA, USA
- OP39 **One-year evaluation of the incidence and distribution of amyloidosis diseases in Germany: National Clinical Amyloidosis Registry**
Ute Hegenbart, Heidelberg, Germany
- OP40 **Localised laryngeal amyloid - A series of 100 cases**
Helen Lachmann, London, UK
- 16:12 - 16:30 **Discussion**
- 16:30 - 16:40 **Break**

16:40 - 18:10 **INDUSTRY SPONSORED SYMPOSIUM 5 - Alnylam**

ATTR Amyloidosis: Unlocking the potential of RNAi therapeutics

Chair:

Mathew Maurer, New York, USA

Mechanisms of organ damage in ATTR amyloidosis

Julian Gillmore, London, UK

Controlling gene expression with RNAi in ATTR amyloidosis

Laura Obici, Pavia, Italy

Interfering with hereditary ATTR amyloidosis using RNAi

David Adams, Paris, France

18:10 - 18:20 **Break**

18:20 - 19:05 **HOT TOPICS IN AL AMYLOIDOSIS**

Chair:

Giampaolo Merlini, Pavia, Italy

Panelists:

Stefan Schönland, Heidelberg, Germany
Vaishali Santhorawala, Boston, MA, USA
Arnaud Jaccard, Limoges, France
Bouke Hazenberg, Groningen, The Netherlands
Bruno Paiva, Pamplona, Spain
Ramón Lecumberri, Pamplona, Spain

Topics:

- When to suspect AL amyloidosis during MGUS follow-up?
- New response criteria needed?
- Is there a role for MRD assessment?
- Are we curing AL amyloidosis in 2020?
- Amyloid deposition in organ transplant recipients?

19:05 - 19:15 **Break**

19:15 - 20:00 **HOT TOPICS IN ATTR AMYLOIDOSIS**

Chair:

Hartmut Schmidt, Münster, Germany

Panelists:

Ole Suhr, Umeå, Sweden
Violaine Plante-Bordeneuve, Créteil, France
Esther González-López, Madrid, Spain
Joel Buxbaum, La Jolla, CA, USA
Juan González-Moreno, Palma de Mallorca, Spain
Philip Hawkins, London, UK

Topics:

- What is the real prevalence of wild type ATTR amyloidosis?
- What are the critical endpoints in ATTR polyneuropathy?
- Best treatment approach at lack of response to patisiran or inotersen?
- Is it time for combination therapy trials?
- What is the best approach to ATTR mutant carriers?

20:00 - 20:15

The next ISA Symposium



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(+34) 91 372 02 03
e-mail: isa2020@doctaforum.com
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