ONLINE EVENT

14th 18th SEPTEMBER 2020

CENTRAL EUROPE SUMMER TIME (CEST)

XVII INTERNATIONAL SYMPOSIUM ON AMYLOIDOSIS

PRESIDENT

Joan Bladé
Hospital Clínic. Barcelona, Spain

SCIENTIFIC **PROGRAM**

ISA INTERNATIONAL SOCIETY OF AMYLOIDOSIS

FUNDACIÓN PETHEMA

ISA INTERNATIONAL SOCIETY OF AMYLOIDOSIS

FUNDACIÓN PETHEMA

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 Joan Bladé)

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

Cardiac amyloid regression by CMR in AL and ATTR amyloidosis
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 Kastritis, 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
Julie Heimbach, Rochester, USA

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:
Efstathios Kastritis, Athens, Greece

Panelists:

Vaishali Sanchorawala, Boston, MA, USA
Heather Landau, New York, NY, USA
Hasib Sidiqi, 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

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

14:56 – 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 Kastritis, 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 myloidosis 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 inicial 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 proteostasismachinery 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

Hereditary Transthyretin Amyloidosis

Chairs:
María Teresa Cibeira, Barcelona, Spain
Ole Suhr, Umeå, Sweden

Multidisciplinary management and quality of life of patients with hereditary TTR amyloidosis with polyneuropathy
Violaine Planté-Bordeneuve, Créteil, France

Potential predictors of progression and response to treatment of hereditary TTR amyloidosis
Teresa Coelho, Porto, Portugal

Treatment of the polyneuropathy of hereditary TTR amyloidosis with antisense agents
Carlos Casasnovas, Barcelona, Spain

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**
Hans Nienhuis, Groningen, The Netherlands

OP30 **Describing the echocardiographic phenotype of transthyretin cardiac amyloidosis - What are the predictors of prognosis?**
Liza Chacko, London, UK

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**
Hugh Goodman, Hamilton, New Zealand

14:56 – 15:20 **Discussion**

15:20 - 15:30 **Break**

15:30 - 16:30 SELECTED ABSTRACT PRESENTATIONS VI

Chairs:
María Teresa Cibeira, Barcelona, Spain
Ramón 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**
Angela Dispenzieri, Rochester, MN, USA

OP37 **Subcutaneous daratumumab + cyclophosphamide/bortezomib/dexamethasone in newly diagnosed AL amyloidosis: Updated safety run-in results of ANDROMEDA**
Vaishali Sanchorawala, 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 Sanchorawala, 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**

SPONSORS

Platinum

AKCEA THERAPEUTICS
A subsidiary of Ionis Pharmaceuticals, Inc.

Alnylam PHARMACEUTICALS

janssen Oncology
PHARMACEUTICAL COMPANIES OF Johnson&Johnson

Pfizer

Gold

eidos
a bridgebio company

Thanks to the unrestricted grant of:

Celgene | Bristol Myers Squibb Company

ORGANIZED BY

ISA INTERNATIONAL SOCIETY OF AMYLOIDOSIS

FUNDACIÓN PETHEMA

ACCREDITATION

1credit assessed by the Commission on Continuing Education of Health Professionals of the Community of Madrid.

Continuing Medical Education Credits requested to the European Board for Accreditation in Hematology (EBAH).

EBAH
European Board for Accreditation in Hematology

VENUE

Online Event
www.isa2020.org

SYMPOSIUM SECRETARIAT

doctaforum
MEDICAL MARKETING SPECIALISTS

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