TARRAGONA SPAIN

01st 05th MARCH 2020

XVII INTERNATIONAL SYMPOSIUM ON AMYLOIDOSIS

PRESIDENT

Joan Bladé

Hospital Clínic. Barcelona, Spain

ISA INTERNATIONAL SOCIETY OF AMYLOIDOSIS

FUNDACIÓN PETHEMA

www.**isa2020**.org

SUNDAY 1ST MARCH

15:30 **Registration Palau de Congressos de Tarragona**
Also open during meeting days

17:45 - 20:00 **Welcome reception**

MONDAY 2ND MARCH

08:00 - 08:10 **Welcome**
Opening Remarks
Joan Bladé, Barcelona, Spain
Josep Maria Campistol, Barcelona, Spain

08:10 - 08:30 OPENING **LECTURE**

Amyloidosis: Classification and Epidemiology
Chair: Joan Bladé, Barcelona, Spain
Speaker: Per Westermark, Uppsala, Sweden

08:30 - 10:15 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

Intrinsec mechanisms of amyloid tissue clearance
Marinna Fontana, London, UK

Development of amyloid disruptors for ATTR amyloidosis
Mitsuharu Ueda, Kumamoto, Japan

10:15 - 10:45 **Coffee break**

10:45 - 12:00 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

12:00 - 13:15 OPENING **CEREMONY**

Giampaolo Merlini Award and Lecture

13:15 - 14:30 **Lunch** Floor -2
Nomenclature Committee Meeting
(Meeting Room. Floor -1)

13:15 - 14:30 POSTER **VIEWING.** PM001-PM133. Floor -1

14:30 - 16:00 INDUSTRY SPONSORED **SYMPOSIUM 1 - Pfizer**

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

16:00 - 17:00 **PLENARY SESSION 3**

ATTR amyloidosis: Genetics and Basic Science

Chairs:
Merrill D. Benson, Indianapolis, IN, USA
Lawreen H. Connors, Boston, MA, USA

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

17:00 - 17:30 **Coffee break**

17:30 - 19:00 **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

Epidemiology and discovery in broader cardiovascular populations
Esther González-López, Madrid, Spain

19:05 - 19:50 **ISA Board Meeting** Meeting Room. Floor -1

TUESDAY 3RD MARCH

07:00 - 08:00 **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**
Mathias Schmidt, Ulm, Germany

OP02 **Defining the cardiac amyloid proteome and its association with patient clinical characteristics and outcomes**
Angela Dispenzieri, 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

08:00 - 09:00 **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
Bo-Göran Ericzon, Huddinge, Sweden

Kidney transplant in AL amyloidosIs and monoclonal immunoglobulin deposition disease: who and when?
Nelson Leung, Rochester, MN, USA

09:00 - 10:30 **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**
Luca Leonardi, Rome, Italy

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

0P13 **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

10:30 - 11:00 **Coffee break**

11:00 - 12:30 **INDUSTRY SPONSORED SYMPOSIUM 3 - Janssen**

Multidisciplinary treatment approach in the management of patients with A dosis

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

12:30 - 14:00 **Lunch** Floor -2
International Kidney MG working Group
(Medusa Meeting Room. Floor -1)

12:30 - 14:00 **POSTER VIEWING.** PT001-PT135. Floor -1

14:00 - 15:15 **PLENARY SESSION 5**

Experts' Discussion on ASCT in AL amyloidosis: burning questions

Chair:
Raymond Comenzo, Boston, MA, USA

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?

15:15 - 15:45 **Coffee break**

15:45 - 17:15 **INDUSTRY SPONSORED SYMPOSIUM 4 - Akcea Therapeutics**

Hereditary Transthyretin Amyloidosis

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

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

17:15 - 18:05 **SELECTED ABSTRACT PRESENTATIONS III**

Chairs:
Rodney H. Falk, Boston, MA, USA
Laura Obici, 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 CCND1 MRNA 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

WEDNESDAY 4TH MARCH

07:00 - 08:00 **SELECTED ABSTRACT PRESENTATIONS IV**

Chairs:
José Sarrà, Tarragona, Spain
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 cytometr 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 haematological response assessment in al amyloidosis**
Oliver C. Cohen, London, UK

08:00 - 09:15 **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

09:15 - 10:35 **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**
Oghina Silvia, Créteil, France

OP29 **Diagnostic value of subcutaneous abdominal fat tissue aspirates in cardiac amyloidosis**
Dion Groothof, 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**
Atula Sari, Helsinki, Finland

OP34 **Excellent Outcomes of Isolated Renal Transplantation for Hereditary Fibrinogen (AFib) Amyloidosis**
HJB Goodman, Hamilton, New Zealand

10:35 - 11:00 **Coffee break**

11:00 - 12:30 **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

12:30 - 14:00 **Lunch** Floor -2

12:30 - 14:00 **POSTER VIEWING.** PW001-PW139. Floor - 1

12:30 - 13:15 **ISA Members Meeting** Auditorium

13:15 - 14:00 **Taylor & Francis (managing editor of Amyloid)** Medusa Meeting Room. Floor -1

14:00 - 15:15 PLENARY **SESSION 7**

Hereditary ATTR Amyloidosis: Clinical Features and Follow-up

Chairs:
Ernst Hund, Heidelberg, Germany
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

15:15 - 16:45 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

THURSDAY 5TH MARCH

08:00 - 09:00 SELECTED ABSTRACT **PRESENTATIONS VI**

Chairs:
María Teresa Cibeira, Barcelona, Spain
Lourdes Escoda, Tarragona, 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**
Raymond L. Comenzo, 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

09:00 - 09:45 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
Rámon 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?

09:45 - 10:30 **HOT TOPICS IN ATTR AMYLOIDOSIS**

Chair:
Matthias Schmidt, Ulm, Germany

Panelists:
Ole Suhr, Umeå, Sweden
Violaine Plante-Bordeneuve, Créteil, France
Esther González-López ,Madrid, Spain
Joel Buxbaum, La Jolla, CA, USA
Juan González, Palma de Mallorca, Spain

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?

10:30 - 11:00 **Coffee break**

11:00 - 12:00 **PLENARY SESSION 9**

Clinical trials in Progress in Systemic Amyloidosis

Chair:
Joan Bladé, Barcelona, Spain

Front-line therapy in AL Amyloidosis
Giovanni Palladini, Pavia, Italy

Relapse/refractory therapy in AL Amyloidosis
Stefan Schönland, Heidelberg, Germany

Cardiac ATTR Amyloidosis
Pablo García-Pavía, Madrid, Spain

Neuropathic ATTR Amyloidosis
Yoshiki Sekijima, Matsumoto, Japan

12:00 - 12:15 **Symposium Overwiew and Future Prospects**

12:15 - 12:30 **The next symposium**

12:30 - 13:30 **Lunch and departures**

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ACCREDITATION

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

Continuing Medical Education Credits requested to the Spanish National Health System.

VENUE

Palau Firal i de Congressos de Tarragona
Arquitecte Rovira, 2,
43001 Tarragona, Spain

SYMPOSIUM SECRETARIAT

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