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
Mitsuhiko 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
Yuko 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 DiGaspare, 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

Discussion
**TUESDAY 14 SEPTEMBER**

**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

**Discussion**
14:40 - 15:00

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

**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
Tatiana Kourakis, Rochester, MN, USA

**OP03**
Immunogenetic profile of purified pathological plasma cells of patients with light chain amyloidosis
Isabel Cuerva, 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 Nostrine, Pavia, Italy

**Discussion**
14:57 - 16:10

**Break**
16:10 - 16:20

**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**
Julia Edinova, London, UK

**Where the Wild-Type Ones Are**
Esther González-López, Madrid, Spain

**Discussion**
15:52 - 16:10

**Break**
16:10 - 16:20

**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**
Matthew 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

**Discussion**
18:40 - 19:00

**Break**
19:00 - 19:10

**PLENARY SESSION 5**

**Experts’ Discussion on ASCT in AL amyloidosis: burning questions**

**Chair:**
Efstathios Kastritis, Athens, Greece

**Panellists:**
- Vaishali Sanchorawala, Boston, MA, USA
- Heather Landau, New York, NY, USA
- Helen Sobaj, Rochester, MN, USA
- Carlos Fernández de Larrinoa, 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?
**ISA Members Meeting**

13:00 - 14:00

**SELECTED ABSTRACT PRESENTATIONS II**

Chairs:
Joel Busbaum, La Jolla, CA, USA
Violaine Plante-Bordeneuve, Créteil, France

**ATR 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 concurrent or prior use of transthyretin stabilizers in patients with hereditary transthyretin-mediated amyloidosis
Holli 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
Natalia 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
Jöns Werner Uuuds, Sweden

14:56 - 15:30

**INDUSTRY SPONSORED SYMPOSIUM - Janssen**

Multi-disciplinary treatment approach in the management of patients with AL amyloidosis
Chair:
Giovanni Palladini, Pavia, Italy

**OP16**
Diagnostic pit-falls and risk stratification in AL amyloidosis
Efstathios Kastritis, Athens, Greece

**OP17**
Monoclonal antibody treatment for AL amyloidosis
Adithesh Wechalekar, London, UK

**OP18**
Novel targets and drugs for AL amyloidosis
Giovanni Palladini, Pavia, Italy

17:10 - 17:20

**PLENARY SESSION 6**

Experts’ discussion on the Treatment of Patients with AL amyloidosis non-eligible for ASCT: burning questions
Chair:
Mari A. Gertz, Rochester, MN, USA

Panelists:
Shaji Kumar, Rochester, MN, USA
Monique C. Minnema, Utrecht, the Netherlands
Paolo Miani, 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

18:05 - 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 ATR 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 Neveux, Pavia, Italy

**OP18**
Machine learning predicts immunoglobulin light chain toxicity through somatic mutations
Mauro Garofalo, Bellinzona, Switzerland

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 Machtzar, Rochester, MN, USA
  - OP22: The quest for indicators of profound hematologic response in AL amyloidosis: Complete response remains the optimal goal of therapy
    - Paulo Milani, Pavia, Italy
  - OP23: Minimal residual disease positivity by multiparameter flow cytometry hinders organ involvement recovery in AL amyloidosis patients in complete response
    - Gionvanna Palladini, Pavia, Italy
  - OP24: In systemic light-chain amyloidosis the best hematologic response for long-term survival is IFLC < 10 mg/L
    - Amanda Goodarzi, Boston, MA, USA
  - OP25: Comparison of measures of complete hematologic response after high dose melphalan and autologous stem cell transplantation for AL amyloidosis
    - Shyria 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

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

- **Hereditary ATTR Amyloidosis: Clinical Features and Follow-up**
  - **Chairs:**
    - Rodney H Fisk, Boston, MA, USA
    - Lucia García, 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

**14:00 - 15:00**
**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 Martínez-Res, Taragona, Spain
  - AA amyloidosis: management
    - Luis Quintana, Barcelona, Spain
  - AA amyloidosis associated with autoinflammatory diseases
    - Helen Lachmann, London, UK
  - Localized amyloidosis
    - Eli Machtzar, Rochester, MN, USA
  - Hereditary non-transthyretin amyloidosis
    - Julian Gillmore, London, UK
  - LECT2-associated renal amyloidosis
    - Tamer Rezk, London, UK

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

**15:10 - 15:20**
**INDUSTRY SPONSORED SYMPOSIUM 4 - Akcea Therapeutics**

- **Hereditary Transthyretin Amyloidosis**
  - **Chairs:**
    - María Teresa Echevarría, Barcelona, Spain
    - Ole Suy, Umeå, Sweden
  - Multidisciplinary management and quality of life of patients with hereditary TTR amyloidosis with polyneuropathy
    - Valérie Plante-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
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?
Lisa Chacks, 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
Sue Attila, Helsinki, Finland

OP34
Excellent outcomes of isolated renal transplantation for hereditary Fibrinogen (AFib) amyloidosis
Hugh Goodman, Hamilton, New Zealand

OP35

SELECTED ABSTRACT PRESENTATIONS VI

Chairs:
María Teresa Cabeza, Barcelona, Spain
Ramón Lacumberris, Pamplona, Spain

AL AMYLOIDOSIS II

OP35
A phase II study of ixazomib-dexamethasone versus physician’s choice in relapsed/refractory systemic AL amyloidosis: Results from the phase 3 tourmaline-AL trial
Angela Dispenzieri, Rochester, MN, USA

OP36
Subcutaneous daratumumab + cyclophosphamide/bortezomib/dexamethasone in newly diagnosed AL amyloidosis: Updated safety run-in results of ANDROMEDA
Vaishali Sanchorawala, Boston, MA, USA

OP37
Assessment of minimal residual disease using multiparametric flow cytometry in treated patients with AL amyloidosis
Andrew Staron, Boston, MA, USA

OP38
Localised laryngeal amyloid – A series of 100 cases
Helen Lachmann, London, UK

INDUSTRY SPONSORED SYMPOSIUM 5  - Alnylam

ATR Amyloidosis: Unlocking the potential of RNAi therapeutics
Chair:
Matthew Maurer, New York, USA

Mechanisms of organ damage in ATTR amyloidosis
Julian Gilmore, 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
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