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 Fibre 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
Marnia Kamiryo-Amarado, Rochester, MN, USA

Drivers of amyloid organ tropism and deposition
Gilda 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 Fantana, 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 Ardi, 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

18:20 - 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
Efthymios Kastritis, Athens, Greece

Cytogenetics in AL amyloidosis
Stefan Schönland, Heidelberg, Germany

Hematologic and organ response criteria
Giovanni Palladini, Pavia, Italy

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**

**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
Tanachai Koosakul, 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 Noe, Pavia, Italy

**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**
Julia Edhø, London, UK

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

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

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

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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**
Mathes Mauri, NY, USA

**Liver transplantation in hereditary ATTR amyloidosis**
TBC

**Kidney transplant in AL amyloidosis and monoclonal immunoglobulin deposition disease: who and when?**
Nelson Long, 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 Sanchorawala, Boston, MA, USA
Heather Landau, New York, NY, USA
Haido Sudh, Rochester, MN, USA
Carlo Fernandez de Larrada, 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

Selected Abstract Presentations II

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

ATR Amyloidosis

OP07
Skin biopsy in hereditary transthyretin amyloidosis with polyneuropathy in France
G. Berard, Lausanne, Switzerland

OP08
Long-term safety and efficacy of patisiran: Global open-label extension 24-month data in patients with hereditary transthyretin-mediated amyloidosis
David Adame, Le Kremlin-Bicêtre, France

OP09
Long-term impact of tafamidis in patients with late-onset hereditary transthyretin amyloidosis with stage I polyneuropathy
Robera Quereux, 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
Heiko 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
Jonas Warne, Umeå, Sweden

Basic Science II

OP16
Hepatic expression of mutant transthyretin remodels proteostasis machinery in hereditary ATR amyloidosis
Gianluca Pizzarelli, Pavia, Italy

OP17
Diagnostic potential of a novel RT-QPCR-based assay to measure CAND1/MIRNA expression levels in bone marrow plasma cells from ATR amyloidosis patients
Alice Neveu, Pavia, Italy

OP18
Machine learning predicts immunoglobulin light chain toxicity through somatic mutations
Mauricio Dassan, Basel, Switzerland

OP19
Drosophila melanogaster as a model organism for ATR amyloidosis
Xiaohong Gu, Uppsala, Sweden

OP20
Eleven different amyloid types identified in cutaneous amyloidosis by proteomics-based typing
Sciencia DiCaro, Rochester, MN, USA

Preliminary Session 6

Experts’ discussion on the Treatment of Patients with AL amyloidosis non-eligible for ASCT: burning questions
Chair:
M. W. 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

Discussion

Selected Abstract Presentations III

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

Basic Science II

OP16
Multidisciplinary treatment approach in the management of patients with AL amyloidosis
Chair:
Giovanni Palladini, Pavia, Italy

OP17
Diagnostic pit-falls and risk stratification in AL amyloidosis
Efthathios Kastritis, Athens, Greece

OP18
Monoclonal antibody treatment for AL amyloidosis
Ashutosh Wechalekar, London, UK

OP19
Novel targets and drugs for AL amyloidosis
Giovanni Palladini, Pavia, Italy

OP20
High resolution nerve ultrasound as a diagnostic tool for differential diagnosis and progression recognition in TTR-related familial amyloidosis
Natalia Winter, Tübingen, Germany

OP21
Origin of val30met in familial amyloid polyneuropathy (TTR-FAP) in Portugal: a walk through the mutational path
Carolina Lemos, Porto, Portugal

OP22
99mTc-DPD scintigraphy predicts amyloid fibril type in hereditary transthyretin amyloidosis
Jonas Warne, Umeå, Sweden

Discussion
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  
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  

18:10 - 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 Martínez-Vela, Tarragona, Spain  
AA amyloidosis: management  
Luis Quintana, Barcelona, Spain  
AA amyloidosis associated with autoinflammatory diseases  
Helen Lachmann, London, UK  
Localized amyloidosis  
Eli Muchtar, Rochester, MN, USA  
LECT2-associated renal amyloidosis  
Tamer Rezk, London, UK  

19:10 - 19:30 Discussion  

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14:00 - 15:00 SELECTED ABSTRACT PRESENTATIONS IV  
**AL AMYLOIDOSIS I**

Chairs:  
Raymond Comenzo, Boston, MA, USA  
Isabel Krusek, Madrid, Spain  

**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  
Amadeep Gospodar, Boston, MA, USA  

**OP25**  
Comparison of measures of complete hematologic response after high dose melphalan and autologous stem cell transplantation for AL amyloidosis  
Shanya Sarsilik, Boston, MA, USA  

**OP26**  
The impact and importance of post-rental transplantation hematological response assessment in AL amyloidosis  
Oliver C. Cohen, London, UK  

14:42-15:00 Discussion  

15:00 - 15:10 Break  

15:00 - 15:20 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  

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Oliver C. Cohen, London, UK  

14:42-15:00 Discussion  

15:00 - 15:10 Break  

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**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  
Sue Atula, Helsinki, Finland

**OP34**
Excellent outcomes of isolated renal transplantation for hereditary fibrinogen (AFib) amyloidosis  
TBC

**Discussion**

**14:56 - 15:20**

**15:20 - 15:30**

**Selected Abstract Presentations VI**

**Chairs:**
Maria Teresa Cebesaro, Barcelona, Spain  
Ramon Lozurdo, Pamplona, Spain

**Al Amyloidosis II**

**OP35**
A phase II study of istaiximab (SARE60984) [NSC 795145] for patients with previously treated AL amyloidosis (SWOG S17102; NCT#03499808)  
Tern Parker, CT, USA

**OP36**
Ixaiximab-dexamethasone versus physician’s choice in relapsed/refractory systemic AL amyloidosis: Results from the phase 3 tourmaline-AL1 trial  
Gianpaolo Merli, Pavia, Italy

**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

**Discussion**

**16:12 - 16:30**

**16:30 - 16:40**

**Localised laryngeal amyloid – A series of 100 cases**

**16:40 - 18:10**

**Industry Sponsored Symposium 5 - Alnylam**

**ATTR 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

**Break**

**20:00 - 20:15**

**The next ISA Symposium**
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