**MONDAY 14TH SEPTEMBER**

**10:00 - 10:15**

**Welcome**

Opening Remarks

Giovanni Palladini, Pavia, Italy

**10:15 - 10:25**

**OPENING LECTURE**

Amyloidosis: Classification and Epidemiology

Giovanni Palladini, Pavia, Italy

Joan Bladé, Barcelona, Spain

Speakers: Per Wexler, Uppsala, Sweden

**10:35 - 10:50**

**GIAMPAOLO MERLINI AWARD AND LECTURE**

Gianpaolo Merlini: Aiming for the Cure of Amyloid Disease

(介绍由 Robert A. Kyle)

**11:00 - 11:20**

Break

**11:20 - 11:50**

**PLENARY SESSION 1**

**Basic Science: Amyloid Fibril Formation, Deposition and Clearance**

**Chairs:**

John Berg, Boston, MA, USA

Francesca Lavatelli, Pavia, Italy

**Amyloid fibril structures using cryo EM and ssNMR**

Marcus Fändrich, Ulm, Germany

**Structural basis of amyloidogenicity**

Mauricio Martinez-Arias, Rochester, MN, USA

**Drivers of amyloid organ tropism and deposition**

Gottl/Verkman &, 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**

Maria Fontana, London, UK

**Development of amyloid disruptors for ATTR amyloidosis**

Marianna Fontana, London, UK

**12:00 - 12:30**

**AWARD AND LECTURE**

**13:00 - 14:00**

**PLENARY SESSION 2**

**AL amyloidosis: Diagnosis and Management in 2020**

**Chairs:**

Ashutosh Wechalekar, London, UK

Stefan Schenkenfeld, Heidelberg, Germany

**Diagnosis work-up and typing**

George Swerdlow, Rochester, MN, USA

**Red-flags for early diagnosis**

Ute Hegener, Heidelberg, Germany

**New prognostic markers**

Efstathios Kastritis, Athens, Greece

**Cyto genetics in AL amyloidosis**

Stefan Schenkenfeld, Heidelberg, Germany

**Hematologic and organ response criteria**

Giovanni Palladini, Pavia, Italy

**15:30 - 16:00**

**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**

Cristina Famiglietti, Bologna, Italy

**Management of ATTR Cardiomyopathy**

Pablo García-Pavía, Madrid, Spain

Panel Discussion and Q&A

**16:00 - 17:00**

**BREAK**

**17:00 - 18:30**

**PLENARY SESSION 3**

**Recognition and Diagnosis of AL Amyloidosis**

**Chairs:**

Ashutosh Wechalekar, London, UK

Stefan Schenkenfeld, Heidelberg, Germany

**Diagnosis work-up and typing**

George Swerdlow, Rochester, MN, USA

**Red-flags for early diagnosis**

Ute Hegener, Heidelberg, Germany

**New prognostic markers**

Efstathios Kastritis, Athens, Greece

**Cyto genetics in AL amyloidosis**

Stefan Schenkenfeld, Heidelberg, Germany

**Hematologic and organ response criteria**

Giovanni Palladini, Pavia, Italy

**19:30 - 19:50**

**Discussion**
14:00 - 14:20 | PRELIMINARY SESSION 3

ATTR amyloidosis: Genetics and Basic Science
Chairs: Merrill D. Benson, Indianapolis, IN, USA
Lawrence H. Conners, Boston, MA, USA
Molecular mechanisms of ATTR amyloidosis
Maria João Saravia, Porto, Portugal
Driving forces in ATTR amyloidosis
Vittorio Bellotti, London, UK and Pavia, Italy
Genetic signatures associated with hereditary ATTR amyloidosis
Josep Rodesaa, La Loba, CA, USA
Factors involved in increased susceptibility to TTR amyloidogenesis
Teresa Coelho, Porto, Portugal
Discussion
Break

15:00 - 15:10 | SELECTED ABSTRACT PRESENTATIONS 1

Chairs: Mitsuru Ueda, Kumamoto, Japan
Tomás Ropohl-Heiz, Patras, Greece
BASIC SCIENCE 1
OP01
High resolution cryo-EM structure of a transthyretin-derived amyloid fibril from a patient with hereditary wildtype TTR amyloidosis
Matthias Schmidt, Ulm, Germany
OP02
Defining the cardiac amyloid proteome and its association with patient clinical characteristics and outcomes
Angela Di Spinosa, Rochester, MN, USA
OP03
Immunogenetic profile of purified pathological plasma cells of patients with light chain amyloidosis
Isabel Coelho, Porto, Portugal
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 ubiquitinylating enzymes USP14 and UCHL5 in systemic immunoglobulin light chain (AL) amyloidosis
Mario Nostrone, Paris, Italy

16:20 - 16:50 | INDUSTRY SPONSORED SYMPOSIUM 2 - Eldos 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 Saravia, Porto, Portugal
Diagnosis and management of wild type TTR amyloidosis
Julian Gilmore, London, UK
Where the Wild-Type Ones Are
Esther González-López, Madrid, Spain

17:00 - 18:00 | SELECTED ABSTRACT PRESENTATIONS 2

Chairs: Maria João Saravia, Porto, Portugal
Vittorio Bellotti, London, UK
BASIC SCIENCE 2
OP06
Membrane and soluble b-cell maturation antigen (BCMA) in systemic light-chain amyloidosis
Ping Zhou, Boston, MA, USA
Discussion
Break

18:00 - 19:00 | PLENARY SESSION 4

Organ Transplantation in Systemic Amyloidosis
Chairs: Claudia Papaccio, Bologna, Italy
Pablo García-Pavía, Madrid, Spain
Heart transplantation in AL amyloidosis
Amit V. Krishen, Heidelberg, Germany
Heart transplantation in ATTR amyloidosis
Mathias Makris, NYC, 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 Laug, Rochester, MN, USA
Discussion
Break

19:10 - 20:10 | PLENARY SESSION 5

Experts’ Discussion on ASCT in AL amyloidosis: burning questions
Chair: Efthimos Kastritis, Athens, Greece
Panelists:
Vasileios Sanchorawala, Boston, MA, USA
Heather Landau, New York, NY, USA
Hudo Sofiy, Rochester, MN, USA
César Fernández de la Llave, Barcelona, Spain
Elie Muchtar, Rochester, MN, USA
Topics:
• Patient selection
• Decreasing transplant-related mortality
• Pretransplant induction
• ASCi in patients with renal function impairment
• Any role for consolidation or maintenance?
• Is it time for CAR-T cell therapy in AL amyloidosis?
13:00 – 14:00
ISA Members Meeting

14:00 – 15:30
SELECTED ABSTRACT PRESENTATIONS II

Chairs:
José Buscaino, La Jolla, CA, USA
Voltaire Plante-Bordeneuve, Créteil, France

ATTR AMYLOIDOSIS

OP 07
Skin biopsy in hereditary transthyretin amyloidosis with polyneuropathy in France
Lucia Leonardi, Rome, Italy

OP 08
Long-term safety and efficacy of patisiran: Global open-label extension
Maria Teresa Cibeira, Barcelona, Spain

OP 09
Long-term impact of tafamidis in patients with late-onset hereditary transthyretin amyloidosis with stage I polyneuropathy
Robertia Mussinelli, Pavia, Italy

OP 10
External validation of the national amyloidosis center score in an international cohort of patients with transthyretin cardiac amyloidosis
Adrián Rico-Pérez, Madrid, Spain

OP 11
Evaluation of patisiran in concomitant or prior use of transthyretin stabilizers in patients with hereditary transthyretin-mediated amyloidosis
Hélène Lozach, Cambrils, France

OP 12
Open-label study of patisiran in patients with hereditary transthyretin-mediated amyloidosis with polyneuropathy post-orthotopic liver transplant
Julian Gilmore, London, UK

OP 13
High resolution nerve ultrasound as a diagnostic tool for differential diagnosis and progression recognition in TTR-related familial amyloidosis
Natasha Warner, Tübingen, Germany

OP 14
Origin of val30met in familial amyloid polyneuropathy (TTR-FAP) in Portugal: a walk through the mutational path
Carolina Lemos, Porto, Portugal

OP 15
99mTc-DPD scintigraphy predicts amyloid fibril type in hereditary transthyretin amyloidosis
Jonas Wanner, Umeå, Sweden

15:00 – 15:30
Discussion

15:30 – 16:00
Break

15:40 – 16:00
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 Kastellinos, Athens, Greece

Monoclonal antibody treatment for AL amyloidosis
Ashutosh Wechselbar, London, UK

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

16:10 – 16:20
Break

18:30 – 19:20
SELECTED ABSTRACT PRESENTATIONS III

Chairs:
Rodney H. Falk, Boston, MA, USA
Matthias Schmidt, Ulm, Germany

BASIC SCIENCE III

OP 16
Hepatic expression of mutant transthyretin remodels proteostasismachinery in hereditary ATTR amyloidosis
Richard Giadone, Boston, MA, USA

DIAGNOSIS

OP 17
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

OP 18
Machine learning predicts immunoglobulin light chain toxicity through somatic mutations
Mirea Garofalo, Bellinzona, Switzerland

OP 19
Drosophila melanogaster as a model organism for ATTR amyloidosis
Xiaohong Gu, Upsalla, Sweden

19:05 – 19:20
Eleven different amyloid types identified in cutaneous amyloidosis by proteomics-based typing
Sandra Deleers, Leuven, Belgium

Discussion
14:00 - 15:00
SELECTED ABSTRACTS PRESENTATIONS IV

Chairs: José Sarría, Tarapacá, Spain
Isabel Krzysik, Madrid, Spain

AL AMYLOIDOSIS I

15:00 - 15:10
14:42-15:00
OP26
Shayna Sarosiek, Boston, MA, USA

15:00 – 16:20
15:10 - 16:20
OP25
Amandeep Godara, Boston, MA, USA

15:20 - 16:30
OP24
Giovanni Palladini, Pavia, Italy

16:00 – 18:00
INDUSTRY SPONSORED

16:00 - 18:00
HEREDITARY ATTR AMYLOIDOSIS: CLINICAL FEATURES AND FOLLOW-UP

Chairs: Ernst Huyn, Heidelberg, Germany
Lucia Gadea, Madrid, Spain

Clinical features of polyneuropathy in hereditary amyloidosis
Yaku Asada, Kanazawa, Japan

Red-flags for early diagnosis in hereditary amyloidosis
Yoshihiko Seki, Matsuura, Japan

The global prevalence of ATTR amyloidosis
Harald Stierli, Münster, Germany

Other manifestations in ATTR amyloidosis

Follow-up, polyneuropathy detection, de novo manifestations and treatment
after domino liver transplantation
Laura Obici, Pavia, Italy

Discussion

19:10 - 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-Vex, Tarragona, Spain

AA amyloidosis: management
Luis Quintana, Barcelona, Spain

AA amyloidosis associated with autoinflammatory diseases
Heidi Lachmann, London, UK

Hereditary non-transthyretin amyloidosis
Julian Gillmore, London, UK

LECT2-associated renal amyloidosis
Tamer Rezk, London, UK

Discussion
PRELIMINARY PROGRAM

14:00 - 15:20
SELECTED ABSTRACT PRESENTATIONS V

Chair: Giovanna Palladini, Pavia, Italy
Carlos Fernández de Lara, 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 Rosas 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 Góghina, Crețușani, Romania

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?
Lisa Chacko, London, UK

OP31
Cardiac transthyretin wild type amyloidosis (ATTRwt): A prospective study of 400 patients followed at the Italian referral center
Paola Milano, Pavia, Italy

OP32
Regional cardiac uptake of 99-Tc-DPD is a novel powerful and independent prognostic marker in cardiac ATTR wild type amyloidosis
Paola Milano, Pavia, Italy

OP33
Finnish gelsolin amyloidosis causes significant disease burden but does not affect survival
Sara Atula, Helsinki, Finland

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

Discussion

Break

15:30 - 16:30
SELECTED ABSTRACT PRESENTATIONS VI

Chair: María Teresa Cabeza, Barcelona, Spain

Louise Escoda, Tarragona, Spain

AL AMYLOIDOSIS I

OP35
A phase II study of ixazomib (SAR650984) (NSC-795145) for patients with previously treated AL amyloidosis (SWOG S1702; NCT#03499808)
Tenn Parker, CT, USA

OP36
Ixazomib-dexamethasone versus physician’s choice in relapsed/refractory systemic AL amyloidosis: Results from the phase 3 tualamine-ALi trial
Gianpaulo Merlani, 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

Assessment of minimal residual disease using multiparametric flow cytometry in treated patients with AL amyloidosis
Andrzej Stanisz, Boston, MA, USA

One-year evaluation of the incidence and distribution of amyloidosis diseases in Germany: National Clinical Amyloidosis Registry
Ute Hegenbarth, Heidelberg, Germany

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

Discussion

Break

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

Chair: Carlos Fernández de Lara, Barcelona, Spain

Prevalence and survival impact of atrial fibrillation in patients with transthyretin cardiac amyloidosis. Analysis from a large international cohort
Adrián Rosas Pérez, Madrid, Spain

Impact on survival of N-terminal Pro-B-type natriuretic peptide (NT-proBNP) increase after diagnosis for cardiac transthyretin amyloidosis
Silvia Góghina, Crețușani, Romania

Diagnostic value of subcutaneous abdominal fat tissue aspirates in cardiac amyloidosis
Dion Groothof, Groningen, The Netherlands

Describing the echocardiographic phenotype of transthyretin cardiac amyloidosis - What are the predictors of prognosis?
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Paola Milano, Pavia, Italy

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Finnish gelsolin amyloidosis causes significant disease burden but does not affect survival
Sara Atula, Helsinki, Finland

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

Discussion

Break

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

Chair: Gianpaulo Merlani, Pavia, Italy

Panelists:
Stefan Schützle, Heidelberg, Germany
Vaishali Sanchorawala, Boston, MA, USA
Andrew Isaac, Limoges, France
Bouke Hazenberg, Groningen, The Netherlands
Brkost Pavia, 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?

Break

18:20 - 19:15
The next ISA Symposium

Chair: Hartmut Schmidt, Münster, Germany

Panelists:
Ole Sufie, Umeå, Sweden
Violaine Plante-Bordeneuve, Créteil, France
Lüthi González-López, Madrid, Spain
Joel Buxbaum, Chicago, USA
Juan González-Moreno, 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?

Break

19:05 - 20:00
HOT TOPICS IN ATTR AMYLOIDOSIS

Chair: Bower Hazenberg, Groningen, The Netherlands

Panelists:
Mathew Maurer, New York, USA
Laura Obici, Pavia, Italy
Joel Buxbaum, La Jolla, CA, USA
Steve Pilot, Limoges, France
Ettore Guglielmi, Madrid, Spain

Topics:
- Controlling gene expression with RNAi in ATTR amyloidosis
- Interfering with hereditary ATTR amyloidosis using RNAi
- Mechanisms of organ damage in ATTR amyloidosis
- Future treatment strategies for ATTR amyloidosis

Break

20:00 - 20:15
The next ISA Symposium
PRELIMINARY PROGRAM

Platinum

Gold

Bronze

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