



## PROGRAMME FOR DOCTORS

**THURSDAY, 25 SEPTEMBER 2025**

**ROOM CARLO 2**

|             |                                                                                                                                                       |
|-------------|-------------------------------------------------------------------------------------------------------------------------------------------------------|
| 08.00-18.00 | Secretariat Opening Hours and Registrations                                                                                                           |
| 08.15-08.25 | <b>Welcome Address</b><br>Laura Obici (Italy)                                                                                                         |
| 08.25-08.45 | <b>Greetings from the Authorities</b>                                                                                                                 |
| 08.45-09.40 | <b>SESSION 1</b><br><b>DISEASE HETEROGENEITY: BRIDGING MOLECULAR EVIDENCE TO CLINICAL FEATURES</b><br><i>Chair: Bouke Hazenberg (The Netherlands)</i> |
| 08.45-09.05 | <b>Structural and composition heterogeneity of amyloid and development of new biomarkers</b><br>Lorena Saelices (USA)                                 |
| 09.05-09.25 | <b>One pathogenic protein dealing with a million of interactors</b><br>Christoph Roecken (Germany)                                                    |
| 09.25-09.40 | <b>Discussion</b>                                                                                                                                     |
| 09.40-10.25 | <b>Where evidence meets action: the role of data in ATTR cardiomyopathy clinical decision-making</b><br><i>This symposium is sponsored by PFIZER</i>  |
| 10.25-10.45 | <b>COFFEE BREAK</b>                                                                                                                                   |
| 10.45-11.10 | <b>LECTURE</b><br><b>Research at the Heart of Patient Care: A Personal Journey</b><br><i>Chair: Laura Obici (Italy)</i><br>Giampaolo Merlini (Italy)  |
| 11.10-12.25 | <b>SESSION 2</b><br><b>ATTR AMYLOIDOSIS: ADVANCES IN DIAGNOSIS AND DISEASE MONITORING</b><br><i>Chair: Isabel Conceicao (Portugal)</i>                |



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| 11.10-11.25 | <b>State of the art</b><br>Andoni Echaniz-Laguna (France)                                                                                                                                                                                                                                                                                                                                                                         |
| 11.25-12.25 | <b>ORAL COMMUNICATIONS</b>                                                                                                                                                                                                                                                                                                                                                                                                        |
| 11.25-11.37 | <b>Transthyretin as a Biomarker in Hereditary Transthyretin-mediated Amyloidosis with Polyneuropathy: Insights into Disease Progression</b><br><i>Catarina Falcão De Campos, Bernardo Machado Antunes, Mamede de Carvalho, Isabel Conceição</i>                                                                                                                                                                                   |
| 11.37-11.49 | <b>Radiographic spinal changes in amyloidosis-positive patients: comparative MRI and CT analysis of operated and non-operated levels</b><br><i>Loïc Van Oost, Karel Willems, Karl Dujardin, Arne Decramer</i>                                                                                                                                                                                                                     |
| 11.49-12.01 | <b>Serum neurofilament light chain in relation to autonomic neuropathy in hereditary transthyretin amyloidosis</b><br><i>Milou Berends</i>                                                                                                                                                                                                                                                                                        |
| 12.01-12.13 | <b>Cardiac phenotype in hereditary transthyretin amyloidosis: Correlation between fibril types and 99mTc-DPD uptake</b><br><i>Viktor Löfbacka, Jonas Wixner, Justina Damjanovic Vesterlund, Per Westermark, Intissar Anan, Björn Pilebro</i>                                                                                                                                                                                      |
| 12.13-12.25 | <b>Fast-tracking cardiac amyloidosis detection from 12-lead ECG: validating the deep learning model Willem AI platform</b><br><i>Pablo García-Pavía, Esther González-López, Iñigo Sánchez Rodríguez, Rubén Ruiz Zamorano, José María Lillo-Castellano, Manuel Marina-Breyse, François De Guio, Mariana Brandão, Lucía Perard, Nerea Mora-Ayestaran, Ruben Abbou, Rafael López García, Ángel de la Linde Valdés, Víctor Valdés</i> |
| 12.25-13.10 | <b>The Heart of the MATTeR: A time for change in ATTR-CM</b><br><i>This symposium is sponsored by BAYER</i>                                                                                                                                                                                                                                                                                                                       |
| 13.10-14.15 | <b>LUNCH AND POSTER SESSION</b>                                                                                                                                                                                                                                                                                                                                                                                                   |
| 14.15-15.00 | <b>Transforming lives through innovation, research and partnerships in ATTR</b><br><i>This symposium is sponsored by ASTRAZENECA</i>                                                                                                                                                                                                                                                                                              |
| 15.00-16.15 | <b>SESSION 3</b><br><b>NEW PERSPECTIVES IN ATTR AMYLOIDOSIS TREATMENT</b><br><i>Chairs: Thibaud Damy (France), Giovanni Palladini (Italy)</i>                                                                                                                                                                                                                                                                                     |



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| 15.00-15.15 | <b>State of the art</b><br>Olivier Lairez (France)                                                                                                                                                                                                                                                                                                                                                                                                                                                                 |
| 15.15-16.15 | <b>ORAL COMMUNICATIONS</b>                                                                                                                                                                                                                                                                                                                                                                                                                                                                                         |
| 15.15-15.27 | <b>Efficacy and safety of vutrisiran in transthyretin amyloid cardiomyopathy based on age: the HELIOS-B trial</b><br><i>Awaiz Sheikh, Z. Michael Miao, Brian Claggett, Pablo Garcia-Pavia, Emre Aldinc, Julian Gillmore, Scott D. Solomon, Marianna Fontana</i>                                                                                                                                                                                                                                                    |
| 15.27-15.39 | <b>Efficacy and safety of nexiguran ziclumeran, an investigational CRISPR/Cas9 gene editing treatment: 24-month follow-up from a phase 1 study in patients with hereditary ATTR with polyneuropathy</b><br><i>Julian Gillmore, Derek Smith, Jessica Kachadourian, David Lebwohl, Peijuan Zhu, Yuanxin Xu, Adia Leung, Alison Sonderfan, David E. Gutstein, Garen Manvelian, David Adams, Ed Gane, Jörg Täubel, Björn Pilebro, Andoni Echaniz-Laguna, Justin Kao, Safi Shahda, Alexandra Haagenzen, Liron Walsh</i> |
| 15.39-15.51 | <b>Timing of Mortality Benefit in Outcomes Trials in Transthyretin Amyloidosis</b><br><i>Scott D. Solomon, Brian L. Claggett, Muthiah Vaduganathan, Matthew S. Maurer, Julian D. Gillmore, Marianna Fontana</i>                                                                                                                                                                                                                                                                                                    |
| 15.51-16.03 | <b>Long-term efficacy and safety of eplontersen in patients with hereditary transthyretin amyloidosis with polyneuropathy: initial report from the open-label extension of the NEURO-TTRansform study</b><br><i>Marcia Waddington-Cruz, John Berk, Shiantung Jung, T. Jesse Kwoh, Pramod Reddy Patil, Teresa Coelho, Isabel Conceição, Morie Gertz, Sami Khella, Ahmad Masri, Mathew Maurer, Laura Obici, Jonas Wixner</i>                                                                                         |
| 16.03-16.15 | <b>HELIOS-B: 12-month results from the open-label extension period of vutrisiran in patients with transthyretin amyloidosis with cardiomyopathy</b><br><i>Pablo Garcia-Pavia, Ronald Witteles, Francesco Cappelli, Satish Eraly, Lili Yang, John Vest, Marianna Fontana, Caroline Morbach, Diego Delgado, Hiroaki Kitaoka, Mathew Maurer, Peter Van der Meer, Ali Yilmaz, Vincent Algalarrondo, Scott Solomon</i>                                                                                                  |
| 16.15-16.30 | <b>COFFEE BREAK</b>                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                |



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| 16.30-17.15 | <b>Treating ATTR amyloidosis as one disease by delivering rapid TTR knockdown with vutrisiran</b><br><i>This symposium is sponsored by ALNYLAM</i>          |
| 17.15-18.30 | <b>ROUND TABLE</b><br><b>Gaps in Management of ATTR AMYLOIDOSIS in 2025</b><br><i>Chairs: Eugenia Cisneros Barroso (Spain), Christoph Roecken (Germany)</i> |
| 17.15-17.30 | <b>Drugs availability in different countries</b><br>Marcia Waddington Cruz (Brazil)                                                                         |
| 17.30-17.45 | <b>Molecular histopathology: where we started, where we are and what we can achieve?</b><br>Francesca Lavatelli (Italy)                                     |
| 17.45-18.00 | <b>How to build an amyloidosis network?</b><br>Paolo Milani (Italy)                                                                                         |
| 18.00-18.15 | <b>Evaluation of disease progression and response to therapy</b><br>Isabel Conceição (Portugal)                                                             |
| 18.15-18.30 | <b>Discussion</b>                                                                                                                                           |



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**FRIDAY, 26 SEPTEMBER 2025**

**ROOM CARLO 1**

08.00-16.30 Secretariat Opening Hours and Registrations

08.30-09.15 **Precision Gene Editing in ATTR Amyloidosis: Driving CRISPR/Cas9 Solutions Forward**  
*This symposium is sponsored by INTELLIA*

09.15-09.30 **BREAK**

## JOINT PROGRAMME for DOCTORS and PATIENTS

09.30-10.30 **ROUND TABLE: BASIC AND CLINICAL RESEARCH IN AMYLOID DISEASES: WHERE ARE WE GOING?**  
*This session is supported by BridgeBio*  
Vittorio Bellotti (Italy), Giovanni D'Alessio (Italy), Julian Gillmore (UK)

10.30-10.45 **Holistic Management of ATTR: Addressing unmet needs**  
*This symposium is sponsored by ALNYLAM*

10.45-11.15 **COFFEE BREAK**

11.15-12.15 **SESSION 1  
CONTROVERSIES AND DEBATES: HOW WE MONITOR DISEASE PROGRESSION?**  
*Chairs: David Gregory (UK), Violaine Plante-Bordneuve (France)*

11.15-11.35 **Use of guidelines: pros and cons**  
Marianna Fontana (UK), Pablo Garcia-Pavia (Spain)

11.35-11.55 **Patient reported outcome measures**  
Hans Nienhuis (The Netherlands)

11.55-12.10 **Discussion**



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| 12.10-12.30 | <b>The psychology of patients with ATTR-CM, hurdles in daily life, fear of disease tactics to overcome</b><br><i>This symposium is sponsored by BAYER</i>                             |
| 12.30-13.30 | <b>SESSION 2<br/>SYMPTOMATIC MANAGEMENT OF ATTR AMYLOIDOSIS</b><br><i>Chairs: Agnès Farrugia (France), Simone Longhi (Italy)</i>                                                      |
| 12.30-12.45 | <b>Urologic aspects</b><br>Ricardo Pereira E Silva (Portugal)                                                                                                                         |
| 12.45-13.00 | <b>Gastrointestinal aspects</b><br>Jonas Wixner (Sweden)                                                                                                                              |
| 13.00-13.15 | <b>Cardiac aspects</b><br>Diana Bonderman (Austria)                                                                                                                                   |
| 13.15-13.30 | <b>Discussion</b>                                                                                                                                                                     |
| 13.30-14.30 | <b>LUNCH</b>                                                                                                                                                                          |
| 14.30-14.50 | <b>COLLABORATE Initiative (Empowering patients to address unmet needs of Adult-Onset Rare Disease through Shared Decision Making)</b><br><i>This symposium is sponsored by PFIZER</i> |
| 14.50-15.10 | <b>Presentation of Amylife Survey</b><br><i>Chair: Giovanni Palladini (Italy)</i><br>Thibaud Damy (France)                                                                            |



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| 15.10-16.40 | <b>ROUND TABLE</b><br><b>HOLISTIC APPROACH TO ATTR AMYLOIDOSIS: WHERE ARE WE GOING?</b><br><i>Chairs:</i> Keith Dares (Canada), Andoni Echaniz-Laguna (France), Scott D. Solomon (USA) |
| 15.10-15.25 | <b>Central nervous system manifestations</b><br>Luisa Sousa (Portugal)                                                                                                                 |
| 15.25-15.40 | <b>Eye involvement</b><br>Angelo Minnella (Italy)                                                                                                                                      |
| 15.40-15.55 | <b>Targeted approaches: present and future</b><br>Francesco Cappelli (Italy)                                                                                                           |
| 15.55-16.10 | <b>The role of geriatricians</b><br>Manuel Sanchez (France)                                                                                                                            |
| 16.10-16.25 | <b>Discussion</b>                                                                                                                                                                      |
| 16.25-16.40 | <b>Conclusions</b><br>Laura Obici (Italy), Giovanni D'Alessio (Italy)                                                                                                                  |