EURO-NMD WORKSHOP

ATTRv Neuropathy Treatments: From Molecular Innovation to Multistakeholder Access

Paris, 8-9/12/2025

WORKSHOP AIM and OBJECTIVES

This EURO-NMD Workshop aims to bring together key stakeholders to improve the clinical management, equitable access, and sequencing of therapies for hereditary transthyretin amyloidosis (ATTRv) neuropathy.
The core objectives include:
OBJECTIVE 1 - Clarify patient unmet needs and expectations
OBJECTIVE 2: propose a common diagnostic workflow
OBJECTIVE 3: propose minimal standards for treatment availability, delivery and follow-up
Besides the core objectives, we will explore strengthening early diagnostic strategies (including genetic and biochemical screening), reviewing the current landscape of disease-modifying and emerging therapies, and evaluating the real-world feasibility of treatment combinations and sequencing approaches. A central focus will be placed on discussing access across diverse health systems and on integrating patient and caregiver perspectives into care planning and research design.

WORKSHOP TARGET

The workshop is designed to convene all relevant stakeholders in the ATTRv field, including expert clinicians, basic and translational researchers, patient organisations, regulators, health technology assessors, and representatives from pharmaceutical and biotech companies developing or marketing therapies for ATTRv. By engaging all parties in a shared forum, the workshop seeks to identify practical short- and medium-term strategies to enhance care delivery and trial coordination across Europe.

WORKSHOP DISSEMINATION

A team of rapporteurs will summarise the key outcomes of the discussions. These findings will be synthesised into a consensus-driven summary paper, reviewed by all participants, and submitted for peer-reviewed publication within 6–12 months following the event. Where appropriate, working groups or follow-up initiatives will be launched to further develop proposals arising from the workshop.

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EURO-NMD WORKSHOP

Monday, December 8th, 2025

From Early Diagnosis to Therapy Selection

8:45-9:00 **Welcome and Introduction**

SESSION 1: ***Understanding Disease Evolution and the Diagnostic Window*** *(Moderators: Isabel Conceição and Vincent Algalarrondo)*

- **9:00-9:45 Keynote:** *Pathogenesis and phenotypic variability in ATTRv: Neuropathy, cardiomyopathy, and overlap syndromes (Isabel Conceição)*
- **9:45-10:15 Talk 1:** Genetic testing and variant interpretation: When and whom to test? (Violaine Planté-Bordeneuve)
- **10:15-10:45 Talk 2:** Cardiac involvement and biomarkers (Michel Slama)
- **10:45-11:15 Talk 3:** Emerging diagnostic and monitoring tools for AATRv neuropathy (Mary Reilly)

11:15-11.30 Break

- **11:30-12:30 Round Table 1:** *Patient unmet needs and expectations regarding diagnosis and treatments. Accelerating Early and Accurate Diagnosis Across Europe*
    - **Moderators:** *Jean Phillipe Plançon* **+ Panel (neurologists, cardiologists, patient reps, diagnostic industry):**
    - **Discussion points:**
        - *Setting the focus towards understanding and meeting patient unmet needs and expectations*
        - *Role of national genomic screening programs and family cascade testing*
        - *Diagnostic guidelines and the value of structured suspicion indices*

12:30-14:00 Lunch

SESSION 2: ***Overview of Current Therapies and Indications*** *(Moderators: Violaine Planté-Bordeneuve and Patient Representative)*

- **14:00-14:30 Talk 1:** The role of stabilisers: evidence base, indications, and limitations (Juan Gonzalez Moreno, Majorca)
- **14:30-15:00 Talk 2:** Gene-silencers: Efficacy, safety, and eligibility by phenotype (Teresa Coelho, Porto)
- **15:00-15:30 Talk 3:** Treatment response monitoring: clinical and paraclinical tools (Davide Pareyson, Milan)

15:30-15.45 Break

- **15:45-17:45 Round Table 2:** *Optimising Therapy Selection in Real-Life Patients*
    - *(Moderators: Violaine Planté-Bordeneuve and Patient Representative)* **+ Clinical leaders + Industry (1 per company):**
    - **Discussion points:**
        - *How to determine the best first-line therapy*
        - *Managing polyneuropathy progression*

17:45-18.00 Break

18:00-19:00 SESSION 3: ***Interactive Clinical Case Session***

- **Moderator: Teresa Coelho and Tanya Stojkovic**
    - Two clinical cases of different age and phenotype profiles (e.g., early-onset severe neuropathy vs. late-onset mixed phenotype)
    - Group discussion on diagnosis, treatment choice, and follow-up plans

20:00 Dinner at the restaurant "Le Train Bleu" within walking distance from the workshop venue

Tuesday, December 9th, 2025

From Treatment Sequencing to Access and Patient-Centred Care

SESSION 1: ***Sequencing of therapies, Combination Therapies, and What's Next*** ***(Moderators: João Costa and Mary Reilly)***

- ***9:00-9:30 Talk 1: Emerging therapies: ASO innovations, gene editing, antibodies (Laura Obici)***
- ***9:30-10:00 Talk 2: Combination therapy: Scientific rationale, feasibility, and potential synergy (Katrin Hahn, Berlin)***
- ***10:00-10:30 Talk 3: Switching or combining treatments (Catarina Campos, Lisboa)***

- ***11:00-12:00 Round Table 1: Scientific and Economic Feasibility of Treatment Sequencing***
    - ***Moderators: João Costa and Mary Reilly + Panel (neurologists, cardiologists, patient reps, diagnostic industry):***
    - ***Discussion points:***
        - ***Cost implications and modelling for long-term therapy***
        - ***Real-world endpoints and evidence gaps for combination strategies***
        - ***Cross-trial collaboration to define shared outcome sets***

12:00-13:30 Lunch

SESSION 2: ***Supportive Care and Patient-Centred Management***

- ***Moderators: Teresa Coelho and Michel Slama***
- ***13:30-14:00 Talk 1: Neuropathic pain, autonomic symptoms, motor disability, and physiotherapy integration (Lucia Galan, Madrid)***
- ***14:00-14:30 Talk 2: Cardiac management in mixed cases (João Agostinho, Lisboa)***
- ***14:30-15:00 Talk 3: Patient and caregiver needs: What is missing from current care? (Amyloidosis Alliance)***

15:00-15:15 Break

SESSION 3: ***Building the Road Ahead for ATTRv Research and Access***

- ***15:15-16:45 Round Table 2: Towards a European Collaborative Platform for ATTRv Neuropathy***
    - ***Moderators: João Costa and António Atalaia + Clinical leaders + Industry (1 per company):***
    - ***Discussion points:***
        - ***Coordinating registry-based and pragmatic trials across Europe***
        - ***Sharing natural history and post-approval data***
        - ***Joint, cross-stakeholder prioritisation of evidence needs***

16:45-17:45 SESSION 4: ***Interactive Clinical Case Session*** ***–***

- ***Moderators: Davide Pareyson and Isabel Conceição***
    - *Two additional cases focused on patients in later stages or with treatment failure.*

17:45-18:00 - Closing Remarks