A retrospective observational analysis of the realworld care pathway of people with hereditary transthyretin amyloidosis with polyneuropathy in Italy

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Study Finalised

Administrative details

EU PAS number

EUPAS1000000621

Study ID

1000000621

DARWIN EU® study

No

Study countries

☐ Italy

Study description

Background: This retrospective observational study described the epidemiology and the burden on the
Italian healthcare service (SSN) of patients with polyneuropathy (PN) associated with hereditary transthyretin
amyloidosis (ATTRv).
Research design and methods:
From the Fondazione ReS (Ricerca e Salute) administrative healthcare database (~5.5 million inhabitants in 2021), patients were identified as having ATTRv-PN in 2021 if they had received treatments for ATTRv-PN under SSN reimbursement (i.e. tafamidis, patisiran, or inotersen)
from 1 January 2014 to 31 December 2021 (index date in 2021). Demographics and comorbidities at the
baseline, healthcare resource consumption, and related direct costs reimbursed by the SSN throughout
the one-year follow-up were described.
Results: In 2021, 36 patients with ATTRv-PN (prevalence: 7.4/1,000,000) were identified (males were 83.3%; patients with ≥2 comorbidities were 61.1%; the mean age was 73 ± 8 years). During follow-up, of patients, 91.7% received drugs for ATTRv-PN; >50% received antiepileptics and acid suppressants; 22.2% were admitted to overnight hospitalizations; 30.6% accessed the emergency department; 97.2% received local outpatient specialist care.
Th e per patient mean annual cost was € 122,017; drugs forATTRv-PN accounted for 94.7% of the total expenditure. Conclusions: This study of real-world patients with ATTRv-PN showed a high rate of comorbidities, and substantial direct healthcare and economic burdens on the SSN.

Study status

Finalised

Research institutions and networks

Institutions

Fondazione ReS (Ricerca e Salute), CINECA partner

Italy

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Last updated: 01/10/2025

Institution | Not-for-profit | ENCePP partner

Contact details

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Study timelines

Date when funding contract was signed

Actual: 15/03/2024

Study start date
Actual: 15/04/2024

Date of final study report
Actual: 15/07/2024

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

This paper was funded by AstraZeneca SpA.

Regulatory

Was the study required by a regulatory body?

No

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Disease epidemiology

Healthcare resource utilisation

Data collection methods:

Secondary use of data

Study design:

Retrospective observational cohort study. Accrual period 01/01-31/12/2021. Index date: dispensation of specific drug (tafamidis 20 mg, patisiran, inotersen) from 2014 to 2021.
Baseline characteristics (demographics and comorbidities). 1-year healthcare resource utilization and related direct costs.

Main study objective:

This retrospective observational study of Italian administrative healthcare data aimed to identify people with ATTRv-PN in 2021, and describe their demographics, comorbidities, healthcare resource consumption and related direct costs reimbursed by the Italian National Healthcare Service (Servizio Sanitario Nazionale – SSN).

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medical condition to be studied

Polyneuropathy

Hereditary neuropathic amyloidosis

Population studied

Short description of the study population

This was a retrospective observational cohort study using administrative healthcare data for descriptive analysis of ~5.5 million inhabitants, which represent ~ 9% of the Italian population, and the age distribution of which is consistent with that obtained by the Italian Institute of Statistics.
People with ATTRv-PN were identified from 1 January 2021 to 31 December 2021 (accrual period) if at least one specific drug (ATC – Anatomical Therapeutic Chemical codes: N07×X08– tafamidis through its Italian marketing authorization code of the 20 mg box, N07×X12– patisiran, N07×X15– inotersen) was dispensed between 2014 and 2021.

Age groups

- **In utero**
- **Paediatric Population (< 18 years)**
  - Neonate
    - Preterm newborn infants (0 – 27 days)
    - Term newborn infants (0 – 27 days)
  - Infants and toddlers (28 days – 23 months)
  - Children (2 to < 12 years)

    - Adolescents (12 to < 18 years)
- **Adult and elderly population (≥18 years)**
    - Adults (18 to < 65 years)
        - Adults (18 to < 46 years)
        - Adults (46 to < 65 years)
    - Elderly (≥ 65 years)
        - Adults (65 to < 75 years)
        - Adults (75 to < 85 years)
        - Adults (85 years and over)

Study design details

Setting

Italian inhabitants or beneficiaries of the SSN. Overall observation period 2014-2022. Inhospital, local outpatient and primary care settings.

Summary results

From 5.5 million inhabitants of the ReS database in 2021, 36 patients with ATTRv-PN (prevalence: 7.4/1,000,000) were identified (males were 83.3%; patients with ≥2 comorbidities were 61.1%; the mean age was 73 ± 8 years). During follow-up, of patients, 91.7% received drugs for ATTRv-PN; >50% received antiepileptics and acid suppressants; 22.2% were admitted to overnight hospitalizations; 30.6% accessed the emergency department; 97.2% received local outpatient specialist care. The per patient mean annual cost was € 122,017; drugs for ATTRv-PN accounted for 94.7% of the total expenditure.

Documents

Study publications

Calabria S, Ronconi G, Dondi L, Dondi L, Dell'Anno I, Piccinni C, Esposito I, A...

Data management

ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

Data sources

Data source(s)

Database of Fondazione ReS

Data sources (types)

Administrative healthcare records (e.g., claims)

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Yes

Check completeness

Yes

Check stability

Yes

Check logical consistency

Yes

Data characterisation

Data characterisation conducted

Unknown