

Assessing the impact of Friedreich Ataxia in Italy: a stratified analysis based on patients' ambulatory status

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Objective

This study aimed to assess Friedreich Ataxia (FA) patient resource utilization in Italy, based on their mobility status: ambulatory vs non-ambulatory, from the perspective of the Italian Society

Conclusions

The loss of ambulation is associated with a greater consumption of resources which leads to an annual cost 4 times higher for non-ambulatory vs ambulatory patients from the perspective of the Italian Society

Introduction

- FA is a rare disease that primarily affects patients' mobility, spasticity and cardiac function
- FA poses a clinical, humanistic and economic burden on patients, caregivers, and healthcare services underscoring the need for targeted therapies and optimized resource allocation.

Results

- Among non-ambulatory patients, 56% had difficulties (score 3-4 in FA-ADL) with dressing/hygiene/feeding, 29% with swallowing/speech, 25.5% with bladder function, 37.3% with sitting posture
- Additionally, 45% had cardiomyopathy and/or scoliosis
- Specialist visits increased from 8.1 to 25.8 per year in ambulatory vs non-ambulatory patients.
- Formal caregiver and home nurse estimated rose from 30 to 273 days, while informal caregiving rose from 121 to 365 days annually.
- Assistive Support Instruments were used continuously, physiotherapy sessions remained constant at 170 per year.
- The estimated annual cost for ambulatory patients was €24,507. Non-ambulatory status was associated with total annual cost of €99,354 per patient and one-time cost of €26,917 per patient (Figure 1).

Materials and Methods

- Data from a multidisciplinary group of experts including 1 neurologist, 1 physiatrist, 1 cardiologist experienced in the treatment of FA were collected and used.
- Patients were classified as ambulatory (FA - Activities of Daily Living [FA-ADL] walking/falling scores 0-2) and non-ambulatory (score 3-4).
- Resource use was estimated across five FA-ADL domains (walking/falling, swallowing/speech, dressing/hygiene/feeding, bladder function, sitting position) and the prevalence of cardiomyopathy and scoliosis.
- The expert estimated typical resource use to each score across the 5 FA-ADL domains via an ad hoc questionnaire, basing on their knowledge
- Annual (specialist visits, formal/informal care, home health nursing, integrated home care (ADI), physiotherapy, emergency services) and one-time (Intensive Care Unit/hospitalization for pacemaker, wheelchair, Hoyer lift, speech devices, home/car modifications) resource utilization were estimated and adjusted for severity in other domains and complication prevalence to determine incremental needs.
- Costs were calculated using Italian National Healthcare Service tariffs for healthcare resources and literature or regional/national tariff for non-medical resources. Costs were expressed in €/patient/year by multiplying the proportion of users, frequency, and unit costs for ambulatory and non-ambulatory groups.

Resource Use	Unit Costs	Annual Frequency	
		Ambulatory	Non-Ambulatory
N° of specialist visits	€16,20	8,1	25,8
ICU + Hospitalization for pacemaker	€19.052,00	0,0	one-time
Days of formal caregiver	€62,91	30,0	272,5
N° of home health nurse	€28,00	30,0	272,5
Days of informal caregiver	€116,00	121,3	365,3
ADI (N° of months)	€1.269,00	0,0	12,0
Cardiomyopathy	€6.303,70	0,2	0,5
N° of emergency department visits	€350,00	0,5	1,0
Wheelchairs	€2.843,52	0,0	one-time
Hoyer lift	€443,40	0,0	one-time
Hospital bed	€7.423,86	0,0	1,0
Speech devices	€1.089,00	0,0	one-time
Car/home modification	€3.489,00	0,0	one-time
N° of physiotherapist session	€35,00	170,8	169,9

Table 1. Resource utilization by ambulatory and non-ambulatory patients. Estimated based off expert ascribed typical resource use

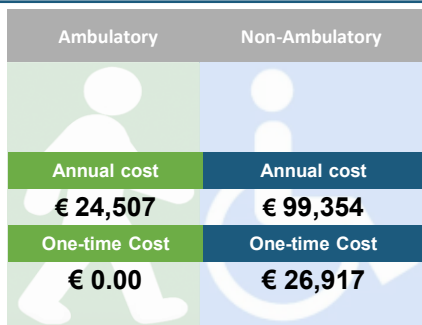


Figure 1. Estimated annual and one-time by ambulatory (green) and non ambulatory (blue) patients. Estimated based off expert ascribed typical resource use

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Reference: Pandolfo M. Arch Neurol. 2008 Oct;65(10):1296-303. doi: 10.1001/archneur.65.10.1296. Disclosures: SF: honoraria from Alexion, Biogen, Genpharm, Medpharma, Medison Pharma, Mylan, Neopharm Israel, Novartis, Roche, Sanofi, UCB, Zai Lab; compensation for Advisory boards or consultation fees from Alexion, Almiral, Amgen, Argenta, AstraZeneca, Aveks, Biogen, Dianthus, JohnsonJohnson, Leven, Novartis GmbH, Reata, Sanofi, UCB, Zai Lab; he is PI in clinical trials for Alexion, Argenta, Dianthus, Immunovant, Leditant, Leven, Novartis, Pitrisia, Remgen, Sanofi; PC: speaking honoraria from Biogen; CA: MerckSerono, Novartis; VF: Boehringer, AstraZeneca; KL, OC, SL: employees of and may hold stock in Biogen; MLG: Research Grant from Roche; Speaker's fees from Novartis; AIC: no conflicts of interest; CPA: speaking honoraria from Otsuka, Novartis, Roche, Bayer, Almiral and Sanofi; compensation for Advisory boards or consultation fees from Almiral, Roche, Sanofi and Daichi; Acknowledgments: This study was funded by Biogen Italia, Milan, Italy. (Copyright) ©2025 Biogen Inc. All rights reserved)