A Cost of Illness Study Evaluating the Healthcare and Societal Burden of Friedreich's Ataxia in the United Kingdom

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Objectives

- To estimate the annual cost of illness (COI) of all Friedreich's ataxia (FRDA) patients in the UK from a service provider (National Health Service, NHS) and societal perspective, and to identify major determinants of cost.

Background

- FRDA is a rare neurological disease with an estimated prevalence of 1/30–50,000, which is equivalent to ~2,000 individuals in the UK.[1]
- FRDA is an autosomal recessive, multi-system disorder, characterised by a range of severe and debilitating symptoms **(Figure 1)**.[2]
- Most notably, patients experience a progressive loss of coordination and mobility, resulting in the majority of patients eventually becoming wheelchair bound.[2]
- Currently, there are limited data on the economic burden of FRDA in the UK.[3] Thus, there is a need for additional studies to explore further the financial impact of this disease.

Methods

- A treatment pathway was developed based on published FRDA guidelines and expert clinical opinion.[4–6] Model inputs were obtained through a pragmatic literature review conducted in April 2016 in MEDLINE via the PubMed platform.
- Additional inputs were sourced from FRDA guidelines, Office of National Statistics (ONS) National Life Tables (Mid-2014), NHS Reference Costs (2014–15), Personal Social Services Research Unit (PSSRU, 2015), UK Genetic Testing Network and the British National Formulary (BNF, 2016).[4,6–11] Inputs were verified by two clinical experts and patient questionnaires.[12–14] Key model inputs are presented in **Table 1**.
- The model considered costs from two perspectives: NHS and societal, dividing costs into two categories:
  1. Direct costs – costs to the NHS for the treatment and management of FRDA;
  2. Indirect costs – costs that can be funded by the individual, social services or the NHS.
- A deterministic sensitivity analysis (DSA) was run at 10% to determine major cost drivers.

Table 1. Key inputs used in the model

Input	Value	Reference
Population inputs		
Prevalence of FRDA	0.000035	Schulz *et al.* 2009[15]
Incidence of FRDA	0.00000035	Assumption
Inputs for direct costs		
Proportion of patients with mobility disorders (to include dysmetria, dysdiadochokinesia and tremor)	100%	Assumption
Proportion of patients with cardiac symptoms	90%	FARA guidelines[4]
Proportion of patients with diabetes mellitus	10%	Expert opinion 2016[12,13]
Proportion of adult patients with diabetes requiring multiple daily injections	90%	Assumption
Proportion of patients requiring physiotherapy (of those with mobility disorders)	100%	Assumption
Cost of adult physiotherapy session	£51.72	NHS Reference Costs (2014–2015)[10]
Inputs for indirect costs		
Cost of extensive home improvements	£33,166.00	Giunti *et al.* 2013[3]
Travel cost to and from each appointment	£17.83	Patient Questionnaires 2016[14]
Proportion of patients incurring travel costs	100%	Assumption
Proportion of patients with a full time carer	5%	Assumption
Proportion of patients with loss to earnings	20%	Assumption
Proportion of patients requiring formal short-term or respite care	17%	Patient Questionnaires 2016[14]
Proportion of patients requiring home adaptations	100%	Assumption
Number of lost working days, per full-time carer, per year	261	Number of working days in a year
Number of appointments and consultations, (including medical appointments and gym sessions), per patient, per year	70	Patient Questionnaires 2016[14]
Number of carers, per patient	1	Assumption
Number of physiotherapy sessions, per patient, per year (excluding additional sessions for scoliosis patients)	12	Assumption
Number of extensive home improvements, per patient	1	Assumption

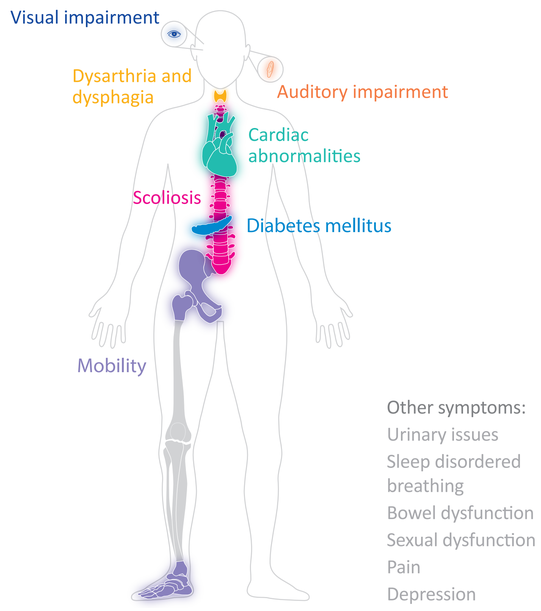
Key inputs defined as the twenty greatest cost drivers as identified by the DSA.

Results

The total direct cost to the NHS was £7,560,471.81 per year

- The average annual direct cost associated with the treatment and management of a FRDA patient in the UK was £3,344.03 **(Figure 2)**.

Figure 1. Symptoms of FRDA



- Stratifying direct costs by diagnosis and symptom groups determined the costs associated with loss of mobility and general follow-up to be the greatest contributors to direct costs **(Figure 3)**.

Indirect costs were estimated at £11,928,599.93 per year

- Indirect costs attributed to FRDA were considerably higher with an average cost per patient of £5,276.07 **(Figure 2)**.
- Income associated with lost working days for patients and carers, along with travel expenses, were shown to be the greatest contributors to indirect costs **(Figure 4)**.

The overall societal cost of FRDA was £19,489,071.73 per year

- The average total cost of FRDA was estimated at £8,620.10 per patient **(Figure 2)**.
- The DSA identified the prevalence of FRDA and the number of lost working days for a full-time carer as key cost drivers **(Figure 5)**.

Figure 2. Summary of results

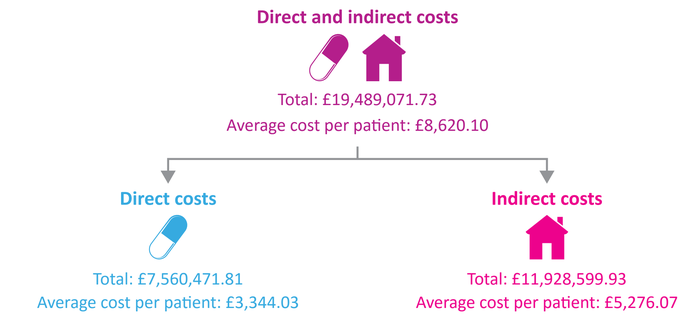


Figure 3. Direct costs of FRDA

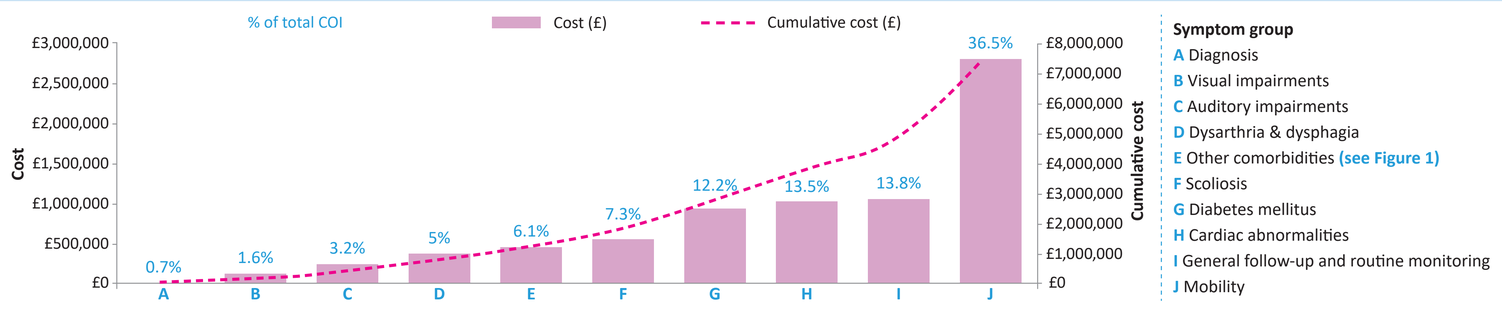


Figure 4. Indirect costs of FRDA

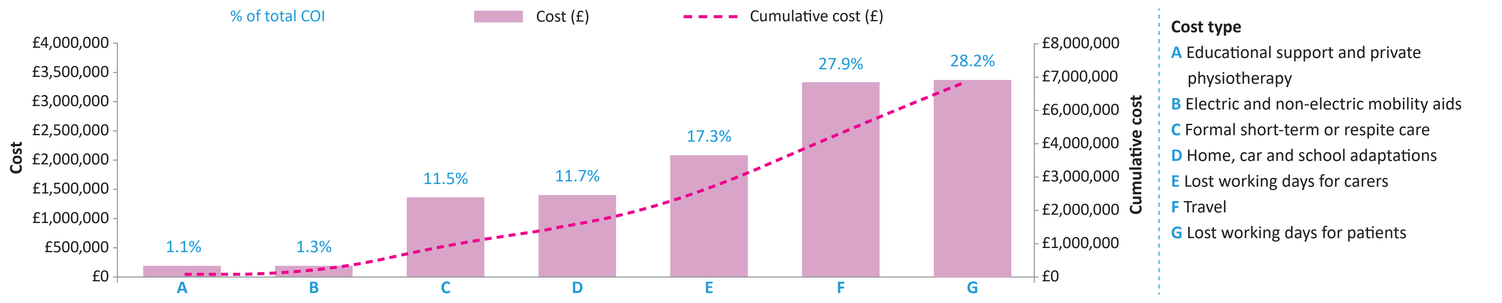
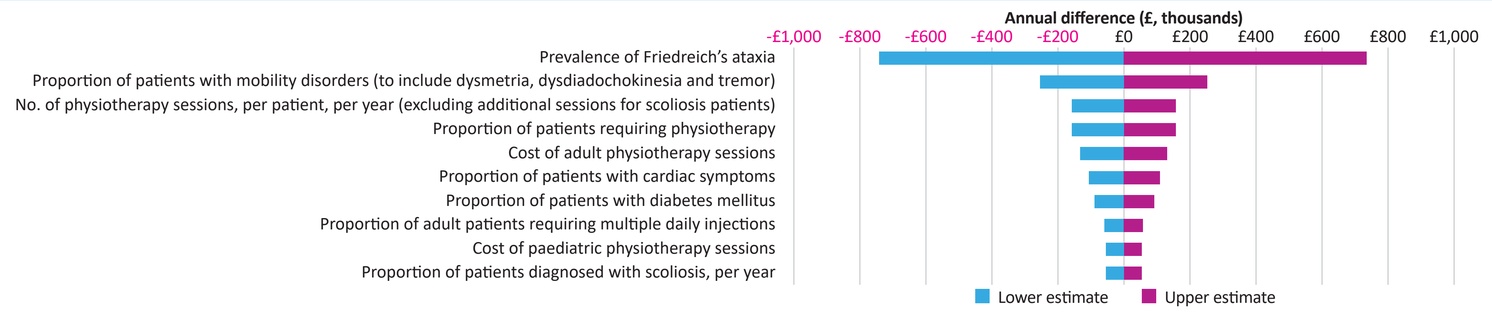


Figure 5. Tornado plot showing the ten greatest cost drivers identified by the DSA



Upper estimate demonstrates the impact on the final COI by increasing the variable by 10%; lower estimate demonstrates the impact on the final COI by decreasing the variable by 10%.

Conclusions

- Despite being a rare disease, direct costs associated with the treatment and management of FRDA to the NHS were found to be substantial.
- The significant indirect costs demonstrate the additional economic burden of FRDA to patients, their families and society as a whole.
- The development and delivery of new, low-cost treatment options, particularly to address the loss of mobility, could substantially reduce the economic burden of this rare and devastating disease.

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Author contributions

Substantial contributions to study conception/design, or acquisition/ analysis/interpretation of data: KH, AG, AB, RF, PG, JG, JV, RST; Drafting of the publication, or revising it critically for important intellectual content: KH, AG, AB, RF, PG, JG, JV, RST; Final approval of the publication: KH, AG, AB, RF, PG, JG, JV, RST.

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