

A Cost of Illness Study Evaluating the Burden of Wolfram Syndrome in the United Kingdom

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PSY52

Objectives

- To evaluate the cost of illness (COI) of all people with Wolfram syndrome in the UK from a service provider perspective (National Health Service, NHS), and to identify major determinants of cost.

Background

- Wolfram syndrome is a rare genetic disorder with an estimated prevalence of 1 in 770,000 people in the UK.¹ It is therefore expected that approximately 80 patients are currently living with the condition.
- Wolfram syndrome, also known as DIDMOAD, is a progressive and neurodegenerative disease characterised by four key symptoms: diabetes insipidus (DI), diabetes mellitus (DM), optic atrophy (OA) and deafness (D) (Figure 1).²
- In addition, patients can manifest a range of other symptoms, including psychiatric problems and neurological abnormalities.^{2,3}

Figure 1. Symptoms of Wolfram syndrome

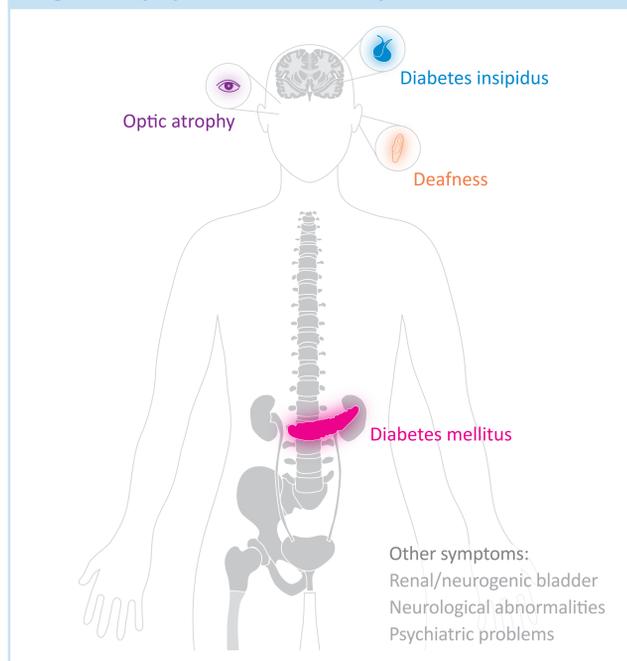
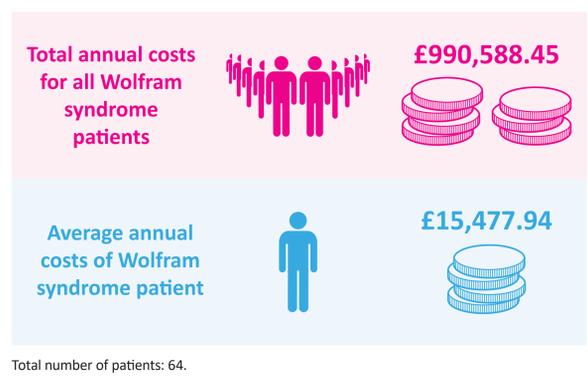


Figure 2. COI of Wolfram syndrome to the NHS in the UK



Results

The total COI to the NHS was £990,588.45 per year

- The final COI of all people with Wolfram syndrome to the NHS and the average cost per patient is shown in Figure 2.

Costs associated with diabetes mellitus had the greatest contribution to total costs

- The costs associated with diabetes mellitus care, late-stage complications of diabetes mellitus, hearing impairment, and visual impairment made the greatest contribution to the final COI (Figure 3).

The greatest driver of the COI was the proportion of patients with sensorineural hearing impairment

- The DSA identified costs associated with hearing impairment and diabetes mellitus as major drivers in the model (Figure 4).

Figure 3. Costs associated with different services and symptom groups

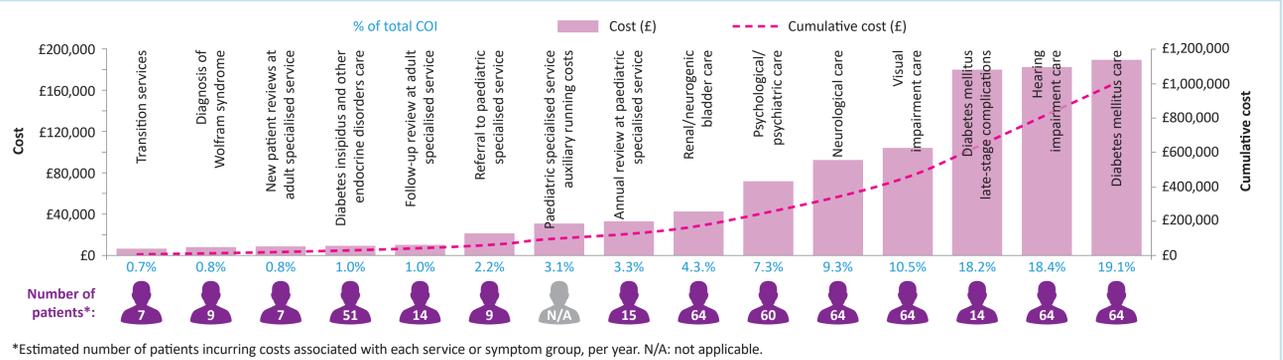
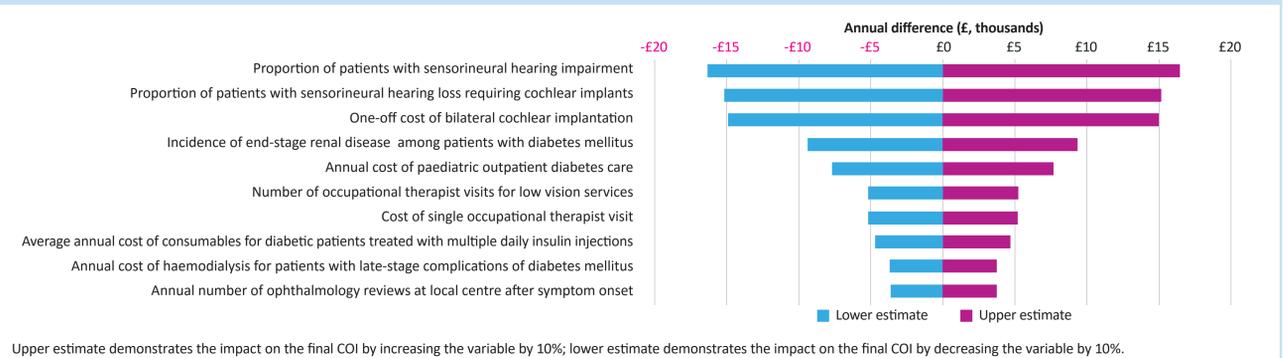


Figure 4. Tornado plot showing ten greatest cost drivers identified by the DSA



Methods

- A COI model was built reflecting the standard practice of the UK Wolfram syndrome specialist service.
- A prevalence-based approach was used and only direct costs to the NHS were considered.
- Model inputs were informed by a pragmatic literature review conducted in MEDLINE via the PubMed platform in April 2016. Additional inputs were sourced from the NHS Reference Costs (2014–15), Personal Social Services Research Unit (PSSRU, 2015) and the British National Formulary (BNF, 2016).^{4–6}

- Information provided by patients with Wolfram syndrome and their families at a focus group in April 2016 was also included.⁷
- All inputs were verified by a clinical expert.⁸
- Key model inputs are presented in Table 1.
- Costs were calculated across symptom groups and auxiliary running costs for the paediatric specialist clinics were included.
- A deterministic sensitivity analysis (DSA) was run at 10% to identify the major cost drivers of the model.

Table 1. Key inputs in the model

Input	Value	Reference
Population inputs		
Number of new patients seen at the paediatric specialist clinic per year	9	NHS Wolfram Syndrome Service
Number of follow-up patients seen at the paediatric specialist clinic per year	15	(Birmingham Children's Hospital, 2016) ⁹
Number of new patients seen at the adult specialist clinic per year	7	NHS Wolfram Syndrome Service
Number of follow-up patients seen at the adult specialist clinic per year	14	(Queen Elizabeth Hospital, 2016) ⁹
Cost inputs		
One-off cost of bilateral cochlear implantation	£33,932.43	NHS Reference Costs (2014–2015) ⁴
Cost of single occupational therapist visit	£134.53	
Annual cost of haemodialysis for patients with late-stage complications of diabetes mellitus	£25,437.00	Roze <i>et al.</i> 2005 ¹⁰
Annual cost of paediatric outpatient diabetes care	£2,943.00	NHS National Tariff (2014–2015) ¹¹
Average annual cost of consumables for diabetic patients treated with multiple daily insulin injections	£890.00	Cummins <i>et al.</i> 2010 ¹²
Clinical inputs		
Proportion of patients with sensorineural hearing impairment	0.66	Barrett <i>et al.</i> 1995 ²
Proportion of patients with sensorineural hearing loss requiring cochlear implants	0.17	Karzon <i>et al.</i> 2013 ¹³
Incidence of end-stage renal disease among patients with diabetes mellitus	0.31	Pratoomsoot <i>et al.</i> 2009 ¹⁴
Number of occupational therapist visits for low vision services	6.00	Warren 1995 ¹⁵
Annual number of ophthalmology reviews at local centre after symptom onset	2.00	Patient experience (Wolfram Syndrome Focus Group, 2016) ⁷

Key inputs shown include population inputs from the NHS Wolfram Syndrome Service and the ten greatest cost drivers as identified by the DSA.

Conclusions

- The annual cost of Wolfram syndrome to the NHS per patient was found to be considerable.
- The results of this study highlight a number of areas of potential cost-savings where the development of an effective treatment to halt progression and provide better control of symptoms is needed.

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Autor Contributions

Substantial contributions to study conception/design, or acquisition/analysis/interpretation of data: SE, SDS, WG, AG, TB, RST; Drafting of the publication, or revising it critically for important intellectual content: SE, SDS, WG, AG, TB, RST; Final approval of the publication: SE, SDS, WG, AG, TB, RST.

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