Demographic characteristics and health resource use of the European participants in the Huntington's Disease Burden of Illness study

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Introduction

- Huntington's Disease (HD) is a rare, inherited, and highly complex neuro-degenerative disorder, affecting all aspects of an individual's life, including physical, cognitive and behavioural processes.[1]
- There is a lack of extensive up-to-date real-world evidence documenting the overall burden of HD by disease stage and from a multinational perspective.
- This study aims to provide an overview of demographic characteristics and HD-related health resource use (HRU) of European participants in the Huntington's Disease Burden of Illness (HDBOI) study.

Methods

- The HDBOI study is a retrospective, cross-sectional dataset that captures demographic, clinical, and HRU of a cohort of HD patients.
- European countries included in the study were Germany, Spain, Italy, France, and the UK.
- The study was governed by an Expert Review Group (ERG), consisting of clinicians, allied health professionals, academics, and patient advocacy representatives, to provide expert input into the study.
- Ethical approval was granted by the Research Ethics Subcommittee at the University of Chester (RESC0420-1038).

Dataset

- Data was collected via two questionnaires:
  — Case Report Form (CRF); completed by physicians, capturing demographic, clinical, direct and indirect costs related to HD over a 12-month period.
  — Patient and Public Involvement and Engagement (PPIE) questionnaires; optional questionnaires completed by patients and caregivers capturing information on demographics, health-related quality of life (HRQoL), non-medical and indirect costs associated with HD.

Outcome variables

  — **Demographic characteristics;** included age, gender, and employment status
  — **HRU;** included consultations with treating physician, HD nurse, and other specialists (physician and patient reported), inpatient and outpatient hospitalizations, length of stay, and variables regarding long term care (LTC)

Data analysis

  — Demographic characteristics were explored descriptively.
  — Differences relating to HRU i.e., consultations with physician, HD nurse and hospitalizations, were explored by disease stage.
  — Statistical significance of differences by disease stage were assessed by analysis of variance (ANOVA) tests.

Results

- The HDBOI European sample for CRFs was comprised of 1,602 HD patients, of which 40% were early stage (ES), 34% were mid stage (MS) and 26% were advanced stage (AS), assessed by the treating physician and based on disease progression.
- Patient representation across countries was similar, with a minimum of 220 patients from France and a maximum of 492 from Italy, displayed in **Table 1.**
- Approximately 59% of the patients were male and the mean age was 47.5 years (SD± 13.7); increasing with advancing disease stage.
- Employment declined in later stages of disease; 72.5% of participants were employed in ES, with 49.4% employed in MS, and 19.6% in AS.
- Across EU countries a total of 445 patients and 465 caregivers completed the PPIE-Patient and PPIE-Caregiver questionnaires, with a similar distribution by disease stage as observed in the CRFs.
- Within the PPIE-Patient sample 65.6% were male, with a mean age of 47.4 years (SD± 12.7), whilst mean age in the PPIE-Caregiver sample was 48.8 years (SD± 13.2).

Table 1. Patients Case Report Form (CRF) by country: total and by disease stage

	All Sample	Early	Mid	Advance/Late
Country	Freq (%)	Freq (%)	Freq (%)	Freq (%)
Germany	264 (16.5)	96 (15.2)	85(15.5)	83 (19.7)
Spain	354 (22.1)	152 (24)	107 (19.6)	95 (22.5)
France	220 (13.7)	104 (16.4)	62 (11.3)	54 (12.8)
UK	272 (17)	91 (14.4)	111 (20.3)	70 (16.6)
Italy	492 (30.7)	190 (30)	182 (33.3)	120 (28.4)
Total	1602	633	547	422
% Total		40%	34%	26%

Note: Early/Mid/Advanced stage is based on an assessment by treating physician

- The most common primary specialty of the treating physician was neurologist (50.9%), general practitioner (GP) (34.1%), or psychiatrist (15%).
- The average number of annual visits to the treating physician was similar across specialties: neurologist 3.3 (SD± 2.3); GP 4.1 (SD± 3.3); and psychiatrist 4.2 (SD± 4.2).
- Most frequent physician reported visits to other specialists were to GPs, physiotherapists, or psychiatrists.
- Similarly, most frequent patient reported visits to other specialists were; GPs, neurologists, psychiatrists, and physiotherapists.
- Regarding HRU for all European participants, the average number of annual visits to the treating physician increased with disease severity (p<0.002): 3.4 (SD± 2.9) for ES; 3.6 (SD± 2.5) for MS and 4.1 (SD± 3.7) for AS. This trend was largely similar on a country level (See **Table 2**).

Results continued

Table 2. Consultations with main physician, HD nurse and other specialists by country: total and by disease stage (Source CRF)

Consult main physician*	All Sample		Early		Mid		Advance/Late	
	Freq	Mean (SD)	Freq	Mean (SD)	Freq	Mean (SD)	Freq	Mean (SD)
Germany	264	4.4 (3.7)	96	4.2 (2.4)	85	3.9 (2.4)	83	5.2 (5.6)
Spain	354	4 (3.4)	152	4.2 (4)	107	3.6 (2.4)	95	4.1 (3.3)
France	220	3.3 (2.3)	104	3 (1.8)	62	3.5 (2.7)	54	3.5 (2.7)
UK	272	2.8 (2.2)	91	2.3 (1.6)	111	2.9 (2.2)	70	3.2 (2.6)
Italy	492	3.7 (2.9)	190	3.2 (2.8)	182	4 (2.8)	120	4.2 (3.1)
Full sample**	1602	3.7 (3)	633	3.4 (2.9)	547	3.6 (2.5)	422	4.1 (3.7)
Consult HD nurse	Freq	Mean (SD)	Freq	Mean (SD)	Freq	Mean (SD)	Freq	Mean (SD)
Germany	264	12.8 (53.3)	96	3.8 (10.8)	85	12.4 (55.3)	83	23.7 (75)
Spain	354	2.9 (3.5)	152	2.8 (3.8)	107	2.6 (2.5)	95	3.4 (3.8)
France	220	3.5 (5.9)	104	2.1 (2.2)	62	3.9 (5.5)	54	5.5 (9.5)
UK	272	2.9 (4.8)	91	2.4 (5)	111	2.6 (2.5)	70	3.9 (7)
Italy	492	5.8 (25.6)	190	2.6 (3.2)	182	3.8 (3.6)	120	14.1 (50.8)
Full sample**	1602	5.5 (26.3)	633	2.7 (5.3)	547	4.7 (22.2)	422	10.8 (43.7)
Consult other Specialist	Freq	Mean (SD)	Freq	Mean (SD)	Freq	Mean (SD)	Freq	Mean (SD)
Germany	118	27.2 (39.1)	44	21.2 (37)	35	26.5 (40.5)	39	34.5 (40.1)
Spain	234	11.2 (12.3)	99	11 (12.8)	73	9.5 (10.8)	62	13.5 (13.1)
France	121	19.3 (24.5)	44	14.6 (24.7)	39	19.5 (21.3)	38	24.4 (26.9)
UK	91	10.3 (22.3)	30	15.7 (36.4)	42	6.5 (6.9)	19	9.9 (12.7)
Italy	188	13 (17.1)	73	12.7 (18.3)	65	12.4 (14.9)	50	14 (18.4)
Full sample**	752	15.3 (23.4)	290	14 (24)	254	13.6 (20.7)	208	19.2 (25.3)

*Consult main physician includes the specialties of neurology, psychiatry, and general practice.
**ANOVA tests were conducted for the full sample for each variable; consult main physician (p<0.002), consult HD nurse (p<0.001), and consult other specialist (p<0.02).

- A similar trend was observed for nurse visits (p<0.001; 2.7 (SD± 5.3) for ES; 4.7 (SD± 22.2) for MS and 10.8 (SD± 43.7) for AS), and for consultations with other specialists (p<0.02).
- Although the number of hospitalizations was smaller than expected they were more frequent in AS patients, for example 10% of AS patients had an inpatient hospitalization vs. 3% in the ES and MS groups (p<0.001).
- There were fewer patients in LTC than expected (9.3%), however, some patients (11.6%) have been recommended LTC.
- Of this 36.1% patients are planning to enter LTC, while others will not take the recommendation (38.5%) (See **Table 4**).

Results continued

Table 3. Hospitalizations (inpatient and outpatient): total and by disease stage (Source CRF)

	All patients		Early		Mid		Advance/Late	
	Freq	Mean (SD)	Freq	Mean (SD)	Freq	Mean (SD)	Freq	Mean (SD)
Inpatient Hosp	101	1.4 (0.9)	25	1.6 (1.4)	19	1.4 (1)	57	1.3 (0.6)
Inpatient days	101	18.9 (38.2)	25	26.5(43.5)	19	27.7 (69.7)	57	12.6 (11.8)
Outpatient Hosp	137	1.6 (2.1)	35	2 (3.8)	31	1.5 (1)	71	1.5 (1.1)

Table 4. LTC recommendation: total and by disease stage

	All patients	Early	Mid	Advance/Late
	Freq (%)	Freq (%)	Freq (%)	Freq (%)
LTC recommended	169 (11.6)	42 (6.9)	40 (7.7)	87 (27)
Will enter LTC	61 (36.1)	14 (33.3)	8 (20)	39 (44.8)
Will not enter LTC	65 (38.5)	21 (50)	17 (42.5)	27 (31)

- Most common reasons for requiring LTC included; requiring full-time assistance with self-care and hygiene, increased frequency/severity of motor symptoms, or loss of mobility.

Conclusion

- The HDBOI study provides new insights into the demographic characteristics and HRU of European HD patients.
- Although, recruiting and sampling of participating physicians and patients reflected real world clinical practice in HD, there was a risk of selection bias, and best-standard care may be over-represented due to the screening criteria. This can be considered as a limitation to this study.
- As evidenced by our findings HRU is higher in later stages of the disease, highlighting the need for intervention at earlier stages of HD.
- The HDBOI study provides novel data to quantify HD related HRU by disease stage, increasing the evidence base for the international HD community.

References

1. Novak MJ, Tabrizi SJ. Huntington's disease. BMJ. 2010 Jun 30;340:c3109. doi: 10.1136/bmj.c3109. PMID: 20591965.