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Economic burden of Duchenne muscular dystrophy patients: A retrospective observational study using Swedish population-based register data

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INTRODUCTION AND AIM

- Duchenne muscular dystrophy (DMD) is a rare X-linked recessive neuromuscular disorder caused by a mutation in the gene for dystrophin protein[1]. At present, the only medical therapy in Europe recommended for all types of DMD, independent of the type of underlying mutation, is corticosteroid medication[2]. Novel disease modifying therapies aimed at increasing expression of dystrophin, reducing inflammation, or acting on other downstream pathways, raise promise for improved therapies for DMD patients.

- Given the changing treatment landscape of DMD, updated evidence on the economic burden of the diseases is needed. The objective of this study was to estimate healthcare resource use, direct medical and indirect costs of DMD in Sweden.

METHODS

- This retrospective, observational study included all patients diagnosed with DMD in the Swedish National Registry for Neuromuscular Disorders (NMiS) between January 1st 2006 and December 31st 2021. Patients were followed from first assessment of health status (index date) until death or end of the study period (2022-12-31), whatever came first.

- During follow-up, patients, with available data-points, were classified into stage 1 (Ambulatory), stage 2 (Transitory/non-ambulatory), stage 3 (partial need for ventilatory support or no hand-to-mouth-function) and stage 4 (full ventilatory support). The health state was updated based on information at every recorded visit in NMiS.

- Data on direct medical costs were retrieved from the Swedish national patient registry and the national Swedish prescribed drug registry and included all out-patient and in-patient visits as well as the cost of all dispensed drugs. Indirect costs were retrieved from the Swedish national social insurance registry and included payments for personal assistance, reimbursement for costs due to car modifications, sick leave/activity compensation, care benefits to parents with disabled children. Both outcomes, direct medical and indirect costs were calculated as per patient-year and stratified by disease stage. All costs were inflated to 2022 price-levels and converted to € (from SEK) using an exchange rate of 10.6.

RESULTS

- A total of 211 patients were included in this study and the average age at index date was 10.77 years. On average, DMD patients had 5.9 (2.9 SD) outpatient visits and 0.5 (0.5 SD) hospital admissions per year of follow-up. The average annualized direct medical cost in DMD patients was €14,590 (36,537 SD). The main driver of the economic burden was the cost of prescribed drugs (€9,658 [36.675 SD]) which represented 66% of total direct medical costs.

- Direct medical costs decreased with increasing disease stage, mainly explained by the cost of treatment with Translarna, a drug which is not prescribed to patients in later, non-ambulatory, stages of the disease.

- The average annualized indirect cost was €136,294 (191,494 SD). The cost of personal assistance and sick leave/activity compensation were the two main drivers of total indirect costs representing 45% and 32%, respectively.

- Indirect costs increased with disease stage and the average annualized cost of patients in stage 4 was more than four times higher compared to patients in stage 1.

Table 1: Patient characteristics at index date and at date of progression to respective disease stage

	DMD cohort	Stage 1	Stage 2	Stage 3	Stage 4
Number of patients (n)	211	44	30	137	48
Age, mean (sd)	10.77 (7.45)	7.52 (2.04)	9.93 (3.1)	12.31 (6.33)	23.85 (6.28)
Comorbidities					
Reduced lungfunction	0 (0%)	0 (0%)	0 (0%)	9 (7%)	15 (31%)
Pneumonia	16 (8%)	0 (0%)	1 (3%)	2 (1%)	7 (15%)
Cardiomyopathy	10 (5%)	0 (0%)	1 (3%)	15 (11%)	9 (19%)
Heart Failure	0.02 (1%)	0 (0%)	0 (0%)	3 (2%)	8 (17%)
Arrhythmia	12 (6%)	0 (0%)	0 (0%)	1 (1%)	2 (4%)
Scolios	6 (3%)	2 (5%)	3 (10%)	38 (28%)	28 (58%)
Kyphosis	26 (12%)	0 (0%)	0 (0%)	2 (1%)	3 (6%)
Lordosis	0 (0%)	16 (36%)	14 (47%)	41 (30%)	5 (10%)
Osteoporosis	0 (0%)	2 (5%)	4 (13%)	18 (13%)	9 (19%)
ADHD	11 (5%)	0 (0%)	2 (7%)	10 (7%)	5 (10%)
Anxiety	11 (5%)	0 (0%)	0 (0%)	0 (0%)	1 (2%)
Autism	2 (1%)	0 (0%)	1 (3%)	5 (4%)	3 (6%)
Depression	3 (1%)	0 (0%)	0 (0%)	0 (0%)	1 (2%)
Obsessive Compulsory Behavior	0 (0%)	0 (0%)	0 (0%)	2 (1%)	1 (2%)
Catarat	2 (1%)	1 (2%)	3 (10%)	15 (11%)	7 (15%)

Figure 1: Direct medical costs

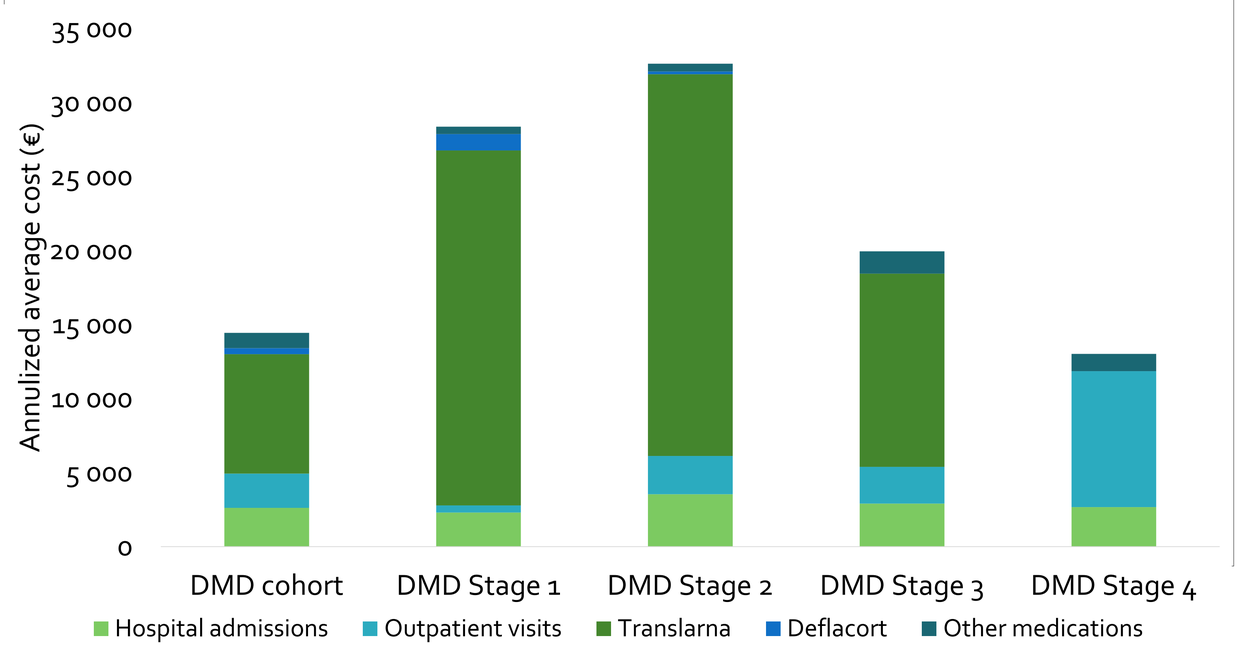
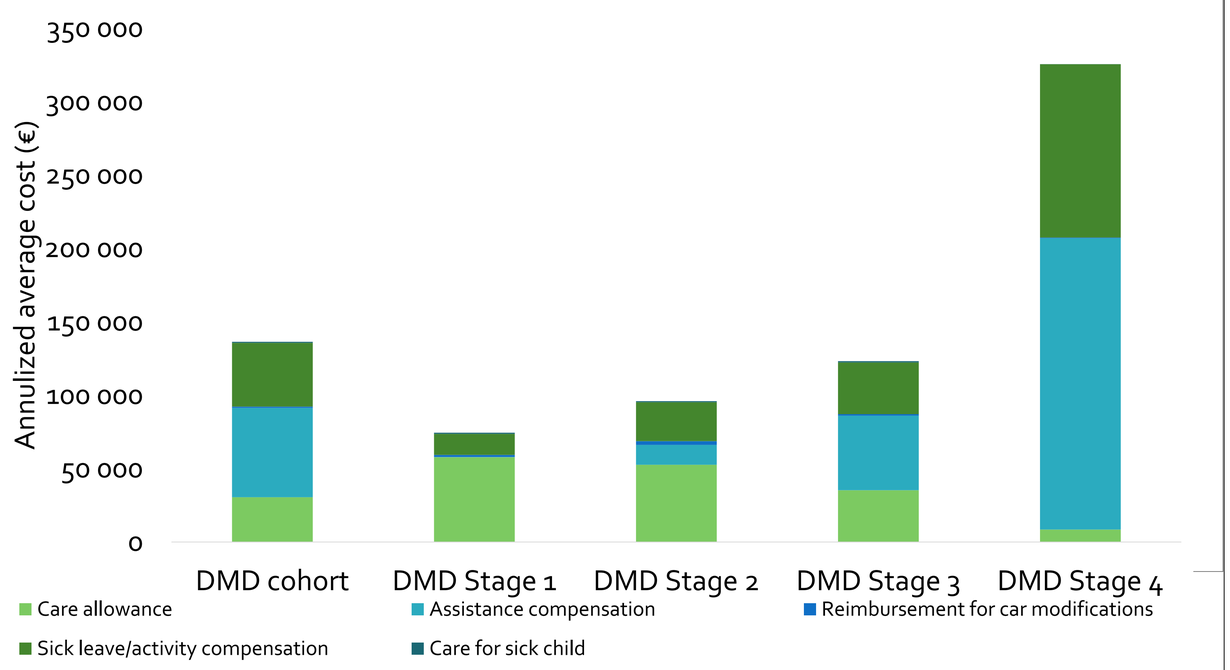


Figure 2: Indirect costs



CONCLUSIONS

- Patients with DMD represent a vulnerable patient population who often suffer from several comorbidities and whose need for support increases as the disease progresses. The total economic burden in this patient group is driven by large indirect costs, such as formal care to meet daily needs in patient's life. Direct medical cost represents a smaller share of the economic burden and is mainly driven by the cost of medication.

- The large indirect costs, in particular in the latter stage of the disease highlights the need and value of novel effective treatments with the potential to reduce disease progression.

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DISCLOSURES

A-BE has received honoraria for lectures or consultancy from Biogen, Novartis, PTC Therapeutics, Italfarmaco and Pfizer. A-KK has received honoraria for lectures or consultancy from Biogen, PTC Therapeutics, and Pfizer. A-K K has further been a Clinical Evaluator in studies performed by Biogen, PTC Therapeutics, Sarepta Therapeutics and Santhera. AT is an employee of Pfizer AB. ST is an employee of Pfizer AB. GO is an employee of Quantify Research AB and owns Quantify Research stocks and stock options. TS has received honoraria for lectures or consultancy from Biogen, Novartis, PTC Therapeutics, Sarepta Therapeutics, Roche, Hansa Biopharma, Pfizer, and Sanofi Genzyme. This study was funded by Pfizer AB, Sweden.