
The burden of Duchenne muscular dystrophy: An international, cross-sectional study.

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The burden of Duchenne muscular dystrophy
An international, cross-sectional study

ABSTRACT

Objective: The objective of this study was to estimate the total cost of illness and economic burden of Duchenne muscular dystrophy (DMD).

Methods: Patients with DMD from Germany, Italy, United Kingdom, and United States were identified through Translational Research in Europe—Assessment & Treatment of Neuromuscular Diseases registries and invited to complete a questionnaire online together with a caregiver. Data on health care use, quality of life, work status, informal care, and household expenses were collected to estimate costs of DMD from the perspective of society and caregiver households.

Results: A total of 770 patients (173 German, 122 Italian, 191 from the United Kingdom, and 284 from the United States) completed the questionnaire. Mean per-patient annual direct cost of illness was estimated at between $23,920 and $54,270 (2012 international dollars), 7 to 16 times higher than the mean per-capita health expenditure in these countries. Indirect and informal care costs were substantial, each constituting between 18% and 43% of total costs. The total societal burden was estimated at between $80,120 and $120,910 per patient and annum, and increased markedly with disease progression. The corresponding household burden was estimated at between $58,440 and $71,900.

Conclusions: We show that DMD is associated with a substantial economic burden. Our results underscore the many different costs accompanying a rare condition such as DMD and the considerable economic burden carried by affected families. Our description of the previously unknown economic context of a rare disease serves as important intelligence input to health policy evaluations of intervention programs and novel therapies, financial support schemes for patients and their families, and the design of future cost studies.

GLOSSARY

CI = confidence interval; DMD = Duchenne muscular dystrophy; RR = relative risk; TREAT-NMD = Translational Research in Europe—Assessment & Treatment of Neuromuscular Diseases.

Rare diseases, although by definition individually uncommon, are associated with a tremendous health burden globally. In the European Union, an estimated 30 million people are affected by diseases with a prevalence of less than 1 in 2,000, and a similar number has been reported for diseases affecting fewer than 200,000 individuals (about 1 in 1,250) in the United States. Many rare diseases are chronic and life-threatening, accompanied by substantial morbidity, extensive health care needs, and considerable psychological and financial stress for affected families. Still, little is known of their economic burden beyond direct medical costs.

The lack of comprehensive cost data for rare conditions is well illustrated in the case of Duchenne muscular dystrophy (DMD), an X-linked neuromuscular disease with a birth incidence of about 1 in 3,800–6,300. Patients with DMD have muscle weakness from early childhood requiring wheelchair use from usually the early teens with fatal complications due

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to cardiac and respiratory involvement in early to mid adult life. The natural history of the disease is relatively well understood, care guidelines are available to assist optimum care,5,6 and several therapeutic modalities are currently in trial. Nonetheless, little is known of the costs associated with DMD.

The objective of this study was to estimate the total economic burden of DMD to society and caregiver households. Our aim was to identify costs of particular importance to rare diseases in general to help policymakers devise appropriate intervention programs, inform financial support schemes for patients and their families, and facilitate evaluations of new treatments.

**METHODS** Participants and procedures. We identified patients with DMD from Germany, Italy, United Kingdom, and United States through national DMD registries, which form part of the global Translational Research in Europe—Assessment & Treatment of Neuromuscular Diseases (TREAT-NMD) network.7 All 4 registries have been in operation for at least 7 years, ensuring good representation across age groups. To be eligible, patients were required to fulfill the following criteria: (1) male, (2) DMD diagnosis, and (3) age 5 years or older. Patients who were from Germany, Italy, United Kingdom, or United States but currently resided in a different country were not eligible for participation.

Eligible patients and one of their caregivers (e.g., parent) were invited to complete a questionnaire online. The questionnaire consisted of questions regarding the patient (demographic information, health status, and DMD-related health care resource use) as well as the caregivers, their households, and DMD-related expenses. Recall periods were specified depending on the frequency of resource use in clinical practice and care guidelines7–9 (1 month, 6 months, or 1 year). Patient and caregiver quality-of-life data were collected using the Health Utilities Index8 and EuroQol EQ-5D9 instrument, respectively. Study materials were presented in the native language of each country and subject to review by the TREAT-NMD coordination team to ensure understandability, accuracy, and completeness. A pilot study was conducted to further establish questionnaire validity. Recruitment started July 2012 and ended July 2013.

**Standard protocol approvals, registrations, and patient consents.** All participants provided informed consent. Study ethical approval was granted from Ludwig-Maximilians-Universität München (Germany), Comitato Etico IRCCS E. Medea—Associazione La Nostra Famiglia (Italy), North East Research Ethics Service, NHS (United Kingdom), and the Western Institutional Review Board (United States). Approval was also obtained from the TREAT-NMD Global Databases Oversight Committee.

**Statistical analysis.** Cost of care was calculated from a societal perspective and annualized by multiplication under the assumption that a similar proportion of subjects would use the same quantity of resources in any given 1-month, 6-month, or 1-year period. We applied local reference prices7–9 to the health care resource data to produce estimates of direct cost of illness. Country-specific costs for aids, devices, and investments were obtained through expert input from TREAT-NMD partners.

Indirect cost (societal loss of production) of DMD was valued according to the Human Capital approach at the cost of employment. Outcomes from the Work Productivity and Activity Impairment Questionnaire20 were used to estimate productivity losses due to absenteeism and impaired productivity while working.

Intangible cost (costs due to pain, anxiety, social handicap, etc.) was estimated by assigning a monetary value to the loss in quality of life for patients and caregivers in relation to the age- and sex-specific mean quality of life in the general population. The societal willingness-to-pay for 1 year of full health, also known as a quality-adjusted life-year, varies by method of assessment and setting, and frequently cited values lie between $50,000 and $100,000.21,22 In this study, a willingness-to-pay for a quality-adjusted life-year of $75,000 was used in the analysis.

We valued each hour of paid informal care according to the Human Capital approach. To estimate unpaid informal care, we first calculated the proportion of the caregivers’ leisure time devoted to informal care, estimated using outcomes from the Work Productivity and Activity Impairment Questionnaire and data from the Organisation for Economic Co-operation and Development on the country-specific mean daily number of hours of leisure time for an adult in the general population.21 Each hour of leisure time was then conservatively valued at 35% of the country-specific mean gross wage, in line with previous research and recently updated estimates of the value of travel time savings.22,23

The economic burden on the household for caring for a boy with DMD was calculated for caregiver households in which the patients with DMD currently lived. We estimated the loss of household income as a result of reducing working hours or stopping work completely because of their son’s DMD by calculating the difference between the country-specific median equalized household disposable income and the self-reported equalized household disposable income.

We related our results to the progression of DMD by classifying patients into 4 groups defined in terms of current ambulatory status and age: (1) early ambulatory (age 5–7 years), (2) late ambulatory (age 8–11 years), (3) early nonambulatory (age 12–15 years), and (4) late nonambulatory (age 16 years or older).

A generalized linear model was fitted to the data to investigate whether the mean per-patient annual cost of illness varied among the 4 ambulatory classes (included as proxies for disease progression) and to predict costs for these groups. To control for confounding effects, the generalized linear model was adjusted for country, household income class, diagnosis of common mental and behavioral disorders, as well as a dummy variable indicating additional household member with DMD. Determinants of patient quality of life and annual household economic burden were investigated analogously.

Results are presented in 2012 international (United States) dollars calculated using purchasing power parity data from Eurostat. The significance level was set to 5%. All analyses were conducted in Stata 11 (StataCorp LP, College Station, TX). We provide descriptions of the proprietary instruments used in the study in appendix e-1 on the Neurology® Web site at Neurology.org. Additional analysis details provided upon request.

**RESULTS** A total of 2,346 patients were invited to participate in the study. Of those, 18 were not eligible (lived in a different country), 996 provided informed consent and started to complete the questionnaire, and 770 patient-caregiver pairs completed all sections of the questionnaire (table 1). The overall study response rate was 42%. In the pooled sample,
patients had a mean age of 14 years (range 5–43) and a median age of 12 years (interquartile range 9–17). The majority of caregivers were mothers to the participating patients with DMD.

Cost of illness. Participating patients indicated the use of many different types of health care resources, including a wide variety of medical devices and aids, medications, tests and assessments, and admissions to hospitals. Patients also made a large number of visits to physicians and other health care practitioners, predominantly physiotherapists. Informal care (caregivers’ nonprofessional paid care and the proportion of caregivers’ leisure time devoted to provide informal care) was extensive in all countries. Labor-force participation among patients was very low (4%), and many caregivers had reduced their working hours, or stopped working completely, because of their son’s DMD. For employed caregivers, the mean overall work impairment (loss in work time and productivity while working) was estimated at 20% (95% confidence interval [CI]: 16%–25%) for the German sample, and 24% (18%–31%), 29% (24%–35%), and 27% (23%–31%) for the Italian, UK, and US sample, respectively.

Costs associated with DMD-related health care resource use, informal care, and production losses (indirect costs) are presented in table 2. The largest cost component was indirect costs in Germany, Italy, and the United States, and nonmedical community services in the United Kingdom (figure 1). Figure 2 presents cost estimates stratified by ambulatory class. The mean per-patient annual direct medical cost of DMD was estimated at $11,240 (95% CI: $9,720–$14,100), $7,300 ($5,770–$9,670), $15,940 ($13,580–$20,610), and $28,590 ($25,030–$35,870), for German, Italian, UK, and US patients, respectively.

Quality of life (intangible costs). Mean proxy-assessed Health Utilities Index–derived utility (0 indicating death, 1 perfect health) was estimated at 0.45 (95% CI: 0.41–0.51), 0.52 (0.45–0.58), 0.43 (0.39–0.47), and 0.45 (0.42–0.49) for patients from Germany, Italy, United Kingdom, and United States, respectively. Corresponding EQ-5D utility estimates for the caregivers were 0.79 (95% CI: 0.76–0.82), 0.84 (0.81–0.86), 0.82 (0.79–0.84), and 0.81 (0.78–0.83). The mean loss of quality of life in relation to the general population was estimated at 0.48 (0.46–0.51) and 0.11 (0.10–0.12) for patients
The aim of this study was to estimate the total cost and economic burden of DMD from the perspective of society and caregiver households. We estimated the mean per-patient annual direct cost of DMD at $42,360, $23,920, $54,160, and $48,260 for patients from Germany, Italy, UK, and US, respectively. This corresponds to a weekly loss of approximately 41, 36, 44, and 33 hours of leisure time. Adjusted regression analysis results showed that patients in the late ambulatory, early nonambulatory, and late nonambulatory classes had 38% (relative risk [RR]: 1.38, 95% CI: 1.20–1.59), 181% (RR: 2.81, 95% CI: 2.41–3.27), and 191% (RR: 2.91, 95% CI: 2.54–3.34) higher annual household economic burden compared with their early ambulatory counterparts.

DISCUSSION The aim of this study was to estimate the total cost and economic burden of DMD from the perspective of society and caregiver households. We estimated the mean per-patient annual direct cost of DMD at $42,360, $23,920, $54,160, and $54,270 for patients from Germany, Italy, UK, and US patients, respectively.

Table 2  Per-patient annual costs of DMD (in 2012 international dollars)

<table>
<thead>
<tr>
<th></th>
<th>Germany</th>
<th>Italy</th>
<th>United Kingdom</th>
<th>United States</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hospital admissions a</td>
<td>2,080 (1,020–4,950)</td>
<td>1,420 (900–2,470)</td>
<td>2,300 (1,500–3,720)</td>
<td>2,220 (900–5,050)</td>
</tr>
<tr>
<td>Visits to physicians and other health care practitioners</td>
<td>3,850 (3,410–4,340)</td>
<td>2,590 (1,970–3,440)</td>
<td>8,230 (6,360–13,150)</td>
<td>18,210 (15,450–22,260)</td>
</tr>
<tr>
<td>Nurse</td>
<td>40 (10–80)</td>
<td>40 (10–220)</td>
<td>550 (300–1,160)</td>
<td>1,270 (650–2,530)</td>
</tr>
<tr>
<td>Specialist physician</td>
<td>330 (280–410)</td>
<td>170 (130–240)</td>
<td>3,290 (2,380–7,100)</td>
<td>3,730 (3,140–4,840)</td>
</tr>
<tr>
<td>Psychologist or therapist</td>
<td>50 (30–110)</td>
<td>50 (30–120)</td>
<td>160 (80–390)</td>
<td>720 (430–1,220)</td>
</tr>
<tr>
<td>Physiotherapist or occupational therapist</td>
<td>2,810 (2,480–3,180)</td>
<td>2,210 (1,610–3,020)</td>
<td>3,290 (2,420–5,820)</td>
<td>9,920 (8,220–12,030)</td>
</tr>
<tr>
<td>Other health care practitioner a</td>
<td>500 (360–700)</td>
<td>70 (50–120)</td>
<td>600 (370–1,400)</td>
<td>2,350 (1,740–3,200)</td>
</tr>
<tr>
<td>Tests and assessments</td>
<td>2,400 (2,180–2,880)</td>
<td>600 (530–690)</td>
<td>1,580 (1,450–1,750)</td>
<td>2,860 (2,660–3,070)</td>
</tr>
<tr>
<td>Medications</td>
<td>1,020 (770–2,000)</td>
<td>1,550 (890–4,650)</td>
<td>930 (820–1,070)</td>
<td>2,070 (1,720–2,710)</td>
</tr>
<tr>
<td>Nonmedical community services a</td>
<td>8,920 (6,890–12,400)</td>
<td>2,740 (1,640–5,380)</td>
<td>19,250 (13,240–28,670)</td>
<td>7,610 (6,030–9,790)</td>
</tr>
<tr>
<td>Aids, devices, and investments a</td>
<td>5,560 (4,160–7,460)</td>
<td>1,850 (970–4,450)</td>
<td>7,520 (5,690–9,790)</td>
<td>7,930 (6,210–10,280)</td>
</tr>
<tr>
<td>Indirect cost of illness (production losses)</td>
<td>20,770 (17,670–24,250)</td>
<td>18,220 (15,430–21,380)</td>
<td>18,700 (16,280–21,150)</td>
<td>21,550 (18,490–24,720)</td>
</tr>
<tr>
<td>Total annual cost of illness</td>
<td>63,140 (57,600–69,710)</td>
<td>42,140 (36,940–47,730)</td>
<td>72,870 (64,350–84,150)</td>
<td>75,820 (69,350–85,270)</td>
</tr>
<tr>
<td>Intangible costs</td>
<td>45,860 (41,830–50,160)</td>
<td>37,980 (32,400–43,550)</td>
<td>46,080 (42,360–50,050)</td>
<td>45,080 (41,100–48,260)</td>
</tr>
<tr>
<td>Total burden of illness</td>
<td>109,000 (100,390–119,510)</td>
<td>80,120 (71,030–89,190)</td>
<td>118,950 (108,280–132,710)</td>
<td>120,910 (111,480–130,770)</td>
</tr>
</tbody>
</table>

Abbreviation: DMD = Duchenne muscular dystrophy.

Data presented as mean (95% confidence interval), rounded to nearest 10.

a Including emergency and respite care.
b Care coordinator/care advisor, dentist, dietitian/nutritionist, and speech/language/swallowing therapist.
c Home help, personal assistants, nannies, and transportation services.
d Include investments to and reconstructions of the home (e.g., adaptations for wheelchair accessibility).

and caregivers, respectively. Associated intangible costs are presented in table 2. Figure 2 presents mean patient utility stratified by ambulatory class.

Total economic burden of illness. Estimates of the total economic burden of DMD, including a monetary value of the loss in patient and caregiver quality of life (intangible costs), are presented in table 2. Using the most recent DMD prevalence estimates, the national burden of DMD in Germany, Italy, United Kingdom, and United States was estimated at $278,058,000, $154,465,000, $200,478,000, and $1,217,373,000, respectively.

Household economic burden of DMD. The mean per-patient annual household economic burden of DMD, calculated for households in which the patients with DMD currently lived, is presented in table 3. Total out-of-pocket payments were notably higher in the US sample. The mean activity impairment (ability to perform regular daily activities) due to the son’s DMD was estimated at 39% (95% CI: 35%–44%), 34% (29%–40%), 42% (38%–46%), and 34% (31%–37%) for German, Italian, UK, and US patients, respectively.

DISCUSSION The aim of this study was to estimate the total cost and economic burden of DMD from the perspective of society and caregiver households. We estimated the mean per-patient annual direct cost of DMD at $42,360, $23,920, $54,160, and $54,270 for patients from Germany, Italy, UK, and US patients, respectively.
16, and 7 times higher than the mean per-capita health expenditure in these countries, respectively. These results suggest that the medical management of the disease in the United Kingdom is considerably more resource intensive relative to that of the general population compared with the other countries, a finding possibly explained by national differences in the general provision of health care or DMD care in particular. We also found differences among countries regarding costs of specific resource types, in particular visits to physicians and other health care practitioners, nonmedical community services, and informal care. Nonetheless, with the exception of Italy (because of relatively lower resource use and tariff prices), direct cost of illness was in absolute numbers remarkably similar across countries.

Comparing our results with previous research into conditions similar to DMD, our estimate of the direct medical cost for the US sample ($28,590) was strikingly similar to the annual medical care expenditures for a mixed population of patients with muscular dystrophy in a privately insured US cohort reported by Ouyang et al. ($24,880), as well as the annual medical cost for a mixed group of patients with muscular dystrophy recently reported by Larkindale et al. ($24,122). However, our study is unique in targeting a single rare disease and taking an international perspective, as well as showing that additional key cost elements not considered in these other studies.
constitute a significant component of the overall burden of disease.

In addition to direct costs, DMD was also associated with large production losses, for both patients and caregivers. Only a trivial proportion of patients was employed, and between 27% and 49% of caregivers had reduced their working hours or stopped working completely because of their son’s DMD. Potential reasons for these intercountry variations include differences in the proportion of caregivers employed, extent of employment (e.g., part-time), but also the divisions of labor within households, where in some countries it may be less common for women to work outside of the home. In addition,

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caregivers who did work reported a mean loss in work time and productivity corresponding to more than 1 day of a 5-day work week. Thus, although our estimates only include production losses for one caregiver, indirect costs of DMD are substantial.

Informal caregiving also constituted a major cost component in all countries (18%–31% of the total cost of illness). We included informal care as a direct cost with the rationale that the care otherwise would have had to be provided by health care professionals. In fact, in the absence of informal care, our results suggest that many patients with DMD would require residence in an institutionalized setting, especially in the later disease stages.

Patient quality of life was remarkably similar across countries and significantly lower compared with general population reference values. The mean utility in the pooled sample was 0.75, 0.65, 0.24, and 0.15 for the early ambulatory, late ambulatory, early nonambulatory, and late nonambulatory class, respectively, which may be compared with the mean utility of a healthy 15- to 19-year-old boy of 0.94. The mean loss of caregiver quality of life in relation to the general population was 0.11. The detrimental impact of DMD on quality of life should be considered in the medical management of the disease to help provide adequate support to both patients and caregivers.

Including all costs, we estimated the total societal burden at between $80,120 and $120,910. It should be emphasized that these estimates do not include mortality costs, which are likely to be a major cost component because of the low life expectancy associated with DMD. Our results may also not fully capture costs associated with end-of-life care. For these reasons, the reported societal burden estimates should be regarded as conservative.

Households caring for a boy with DMD carry a large economic burden that increases markedly with disease progression. We estimated the mean annual out-of-pocket payments at between $3,490 and $14,390, and these variations are primarily driven by differences in the degree of cost-sharing in the national health care systems, but also the type and intensity of health care received. Including income loss, the monetary value of lost leisure time, and reduced quality of life (intangible costs), the mean annual household burden of DMD was estimated at between $58,440 and $71,900. These estimates should be helpful to inform the support programs put in place for families caring for a person with DMD.

It should be noted that we only included the outcomes of the primary caregiver in our cost and burden of illness calculations. Informal care and household burden cost estimates were consequently underestimated for families in which additional individuals, e.g., a second parent, sibling, or other close relative, contributed to the informal DMD care. For this reason, our results should be regarded as conservative.

A limitation of our study concerns external validity. Patients were recruited via TREAT-NMD registries with a mean response rate of 42%. Participation in the registries is voluntary and family-initiated; therefore, we cannot rule out a degree of selection bias. Because the cost of illness for nonresponders remains unknown, it is also not possible to assess the extent of the potential bias. However, given that our sample was fairly evenly distributed across the defined ambulatory classes, in addition to the fact that the collected clinical and epidemiologic data were characteristic for the different patient groups (not reported), suggests that the discrepancy between the sample and study population will be limited.

A second limitation concerns the cross-sectional study design. Our results are based on a single questionnaire administration (at one point in time), and some of the collected resource use data were extrapolated to obtain annual estimates. Thus, there may be recall bias. Some of our estimates may have been more sensitive to this type of bias than others, such as costs related to aids, devices, and investments during the last year, where participants may have been more likely to remember larger expenses. We tried to alleviate this problem by specifying recall periods in accordance with standard DMD care, and also conducted a pilot study with feedback questions to further improve the validity of the responses. Our results underscore the wide variety of costs that accompany a rare disease such as DMD and the substantial economic burden carried by affected families. Our results also demonstrate the utility of global patient registry networks and the need to develop similar infrastructure to facilitate research of other diseases. Consideration to the complete economic context of rare diseases is essential to fully understand the benefits of novel therapies and formulate sound, affordable, evidence-based health policy.

AUTHOR CONTRIBUTIONS
Mr. Landfeldt, Dr. Lindgren, and Mr. Bell designed the study with input from Dr. Lochmüller and Dr. Bushby. Mr. Landfeldt, Dr. Lochmüller, and Dr. Bushby designed the study questionnaire with input from the other authors. Mr. Landfeldt coordinated ethics approval processes and managed the collection of data. Mr. Landfeldt designed, implemented, and executed the statistical analysis. Mr. Landfeldt, Dr. Lochmüller, and Dr. Bushby led the interpretation of findings with input from the other authors. Mr. Landfeldt drafted the manuscript. All authors reviewed the manuscript and approved the decision to submit for publication.

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DISCLOSURE
E. Landfeldt is a consultant to GlaxoSmithKline through his employment at OpumInsight but was not compensated for his contributions as an author of this manuscript. P. Lindgren has acted as a consultant to GlaxoSmithKline. C. Bell is an employee and shareholder of GlaxoSmithKline. C. Schmitt is an employee and shareholder of GlaxoSmithKline. M. Guglinski, V. Straub, H. Luchtmüller, and K. Busbky report no disclosures relevant to the manuscript. Go to Neurology.org for full disclosures.

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REFERENCES


