### INTRODUCTION

Duchenne muscular dystrophy (DMD) is a rare, fatal genetic disorder caused by a lack of dystrophin protein which leads to progressive and irreversible muscle damage from birth. Loss of ambulation occurs at approximately 12 years.1,2 The median age of death with standard care is 26–28 years,1,2 with the major causes of death being respiratory insufficiency and cardiomyopathy1,2. Due to the size and age of the population, determining the humanistic and economic impact of the disease on patients and their families can be challenging.

The objective of this systematic literature review (SLR) was to review the available evidence on health-related quality of life (HRQoL), economic impact, and healthcare resource utilization associated with DMD.

### METHODS

The SLR was conducted on 29th November 2018, and updated on 24th June 2019.

Embase, MEDLINE, the Cochrane Library, and EconLit were searched, and identified citations were screened for inclusion using predefined eligibility criteria.

Additional relevant studies were identified by hand searching of international conference proceedings.

### RESULTS

#### HRQoL

- Most commonly used instrument to assess HRQoL is PedsQL (n=17), followed by SF-36 (n=6), WHOQOL (n=4) and EQ5D (n=4).
- However, PedsQL, SF-36, WHOQOL, and EQ5D had limitations and differences.
- DMD has a substantial impact on the HRQoL of caregivers (n=244), than mental aspects (n=105), than physical aspects (n=116), and than sleep (n=7).
- SF-36 has a substantial impact on the HRQoL of caregivers (n=204), n=583 compared with PedsQL, although it was only used in 2 of the studies identified.

#### Economic evaluations

- Few published economic evaluations were identified in the SLR.
- The only study relevant to a general DMD population was highly theoretical.

### CONCLUSION

DMD is associated with substantial direct and indirect costs; there is considerable variation among countries, with a gap between Eastern and Western Europe.14-16 Direct healthcare costs are high ($239,805 and $269,370 per year, respectively; ref year 2014) and are higher than those related to ventilation, hospitalization and home modification.

The presence of cardiac disease significantly increases the length of hospital stays and cost,18 with indirect costs associated with DMD inconsistently captured. In particular, the costs associated with lost working time for patients and caregivers varied considerably across studies.

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### REFERENCES