Association of Total Annual Costs of Duchenne Muscular Dystrophy with Disease Severity

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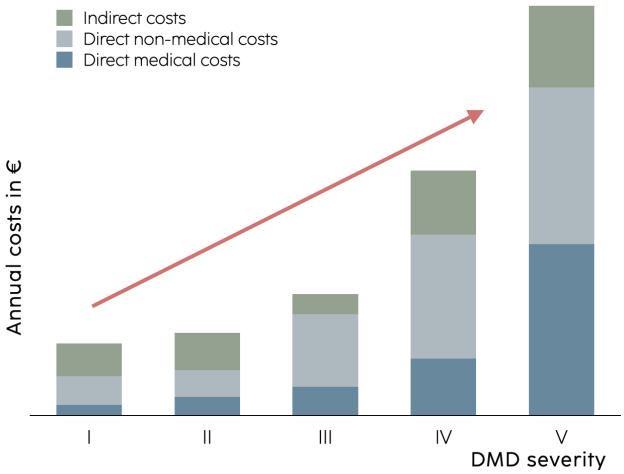
OBJECTIVES: Duchenne muscular dystrophy (DMD) is a genetic disorder which is rare and presents in early childhood with muscle weakness. Current treatments leave much to be desired and there is no causal therapy available. Typically, patients lose the ability to walk by the age of 12. The disease then progresses and life expectancy is severely impaired. To aid future resource allocation decisions, we looked at the cost increase by disease severity.

METHODS: Drawing on a German micro-costing analysis published in 2014 by Schreiber-Katz et al [2], we adjusted the results for Austrian inflation to 2021 prices and calculated the factor increase for direct medical costs, direct non-medical costs, indirect costs and total costs. 5 different states of disease severity were used, from stage 1: diagnosis, 2: early ambulatory, 3: late ambulatory, 4: early non-ambulatory to 5: late non-ambulatory.

RESULTS:
Looking at total costs, there is a factor 5,7 increase between the early ambulatory stage and the late non-ambulatory stage. Considering direct medical costs only, the factor increase between the earliest and latest DMD stages is 16-fold (stage 1 to 2: factor 1,8; 2 to 3: 1,5; 3 to 4: 2,0; 4 to 5: 3,0). For direct non-medical costs, factor increase is 5,4 and for indirect costs 2,5, respectively.



CONCLUSION: Several exciting strategies are on the horizon for people with DMD, such as gene therapy, exon skipping, stop codon read-through and gene repair. If clinical trials show that some of these strategies are causal and in the best case prevent patients from moving through the cascade of DMD stages, these therapies have the potential to not only relieve human suffering, but also to provide good value for money by preventing immense cost increases as patients progress.

2 Schreiber-Katz O et al. Comparative cost of illness analysis and assessment of health care burden of Duchenne and Becker muscular dystrophies in Germany. Orphanet J Rare Dis. 2014 Dec 18;9:210.