The burden of illness in Duchenne Muscular Dystrophy – how much evidence is available?

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Introduction

The burden of illness (BoI) or Burden of Disease (BoD) studies attempt to capture the impact of a disease on a patient, their family, and the healthcare systems being a subset of BoI studies (Greenberg, Ibrahim, & Boncz, 2014).

The aim of this project is to understand the available BoI data in Duchenne Muscular Dystrophy (DMD) with a view to informing what further data will be needed to inform health economic modelling and other governmental appraisals. The focus is to report on the published data. This involves identifying the areas where data is likely to be needed, and then performing a review of existing BoI studies, as well as searches for data available in each specific area. The aim is not to report on the specific BoI studies, but to report on what data would be needed from any data collection exercise.

Methods

In order to determine the areas for inclusion, multiple steps were taken. Firstly common research areas from published burden of illness studies from past experience with HTA were included. Finally consideration was given to the specific issues relevant to Duchenne, a disease of children and young adults. This was then shared with the patient group Duchenne UK and companies developing medicines in the field (as relevant topic experts).

Results

The evidence on each area was searched, and key studies extracted, with quality assessed. The results of this are presented below including assessment of whether there was a sufficient quality of evidence to estimate effects, and whether the quality of evidence was sufficient to have confidence in the results presented.

<table>
<thead>
<tr>
<th>Barrier of illness studies</th>
<th>BoI – Total cost of living</th>
<th>Other governmental costs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Inception &amp; prevalence</td>
<td>Yes</td>
<td>Yes</td>
</tr>
<tr>
<td>Healthcare resource use</td>
<td>Yes</td>
<td>Yes</td>
</tr>
<tr>
<td>Cost of living</td>
<td>No</td>
<td>No</td>
</tr>
<tr>
<td>Productivity losses</td>
<td>No</td>
<td>No</td>
</tr>
<tr>
<td>Impact on family &amp; carers</td>
<td>Yes</td>
<td>Yes</td>
</tr>
</tbody>
</table>

Discussion

Whilst there is a large volume of data on the burden of illness with Duchenne, the quality and reporting is poorly suited to use in economic models. Even where suitable studies are available, these are unlikely to be used in other time periods or jurisdictions without reanalysis.

Duchenne UK is seeking access to the data in studies discussed above and will commission additional work to address the gaps identified in this project. There are plans to develop patient and family surveys that will work along with other initiatives to ensure that the available evidence is used.

Other medical costs

The evidence in this area is lacking in quantity and quality.

Further evidence collection (which preferably would include reanalysis of existing studies) should focus on capturing healthcare resource use (not just total cost) by disease stage, which is seldom reported. Whilst the totality of data with assumptions could be used for modelling, at present no single study presents appropriate data on the resource use by disease stage.

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Impact on families

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Cost of living

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