Hip joint kinetics as clinically meaningful outcomes for therapeutic trials in 4-6 year old boys with Duchenne muscular dystrophy

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Results

Hip kinematics and kinetics are shown in Figure 1. The averages of both limbs were used for the analysis shown in Figure 2. Statistical results for all outcomes are shown in Table 1, and are summarized as follows:

- Significant improvements were found for:
  - Average PHEM (p<0.009)
  - Average PHPG (p<0.003)
  - 10m W/R (p<0.019)

- No significant improvements were found for:
  - 4-step stair climb (p=0.06)
  - Supine to stand (p=0.17)
  - Sit to stand (p=0.26)

- No significant differences were found for velocity, cadence, or step length between the baseline and post visits.

Discussion/Conclusions

The results support the use of gait kinetics as clinically meaningful outcome measures for clinical trials in young boys with DMD. For PHEM, all participants were below age-matched norms at baseline, and three improved to above average after 6 months of steroid intervention. For PHPG, four participants were above average age-matched norms at baseline, and all improved to above average at post.1

For children with typical development, hip kinetics are stable; however, 10m W/R times improve significantly from 7 sec to under 3 sec over this age range. The improvement in hip kinetics found in this study is consistent with increased proximal strength from the steroid intervention; whereas, improvement in the 10m W/R times could be attributed to typical development. As a result, hip kinetics, PHEM in particular, appears to be a sensitive outcome for future clinical trials.

Table 1: Kinetic values and results of timed tests

<table>
<thead>
<tr>
<th>Timed Tests (sec)</th>
<th>10m W/R</th>
<th>4-step stair climb</th>
<th>Supine to stand</th>
<th>Sit to stand</th>
</tr>
</thead>
<tbody>
<tr>
<td>PHEM (Nm/kg)</td>
<td>6.2</td>
<td>4.6</td>
<td>4.6</td>
<td>4.6</td>
</tr>
<tr>
<td>PHPG (Watts/kg)</td>
<td>1.01</td>
<td>0.05</td>
<td>0.05</td>
<td>0.05</td>
</tr>
</tbody>
</table>

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References