Comparison of Clinical Trials for Spinal-Bulbar Muscular Atrophy (SBMA) between the US and EU, 2000-2019

Alice Chang, MS Candidate; Terry Church, DRSc, MA, MS

To examine the similarities and discordances of clinical trials for SBMA in the United States and European Union

**BACKGROUND**

SBMA, also known as Kennedy’s Disease, is a rare X-linked recessive genetic progressive neuromuscular disorder. Globally, 1 in 40,000 individuals are afflicted with this condition. Unfortunately, many are misdiagnosed as amyotrophic lateral sclerosis (ALS) or Huntington’s disease while others remain undiagnosed. Even with supportive care such as feeding tube, walker or cane, and non-invasive ventilation aid, affected individuals experience a decreased quality of life. Currently, no medications are available. Therefore, it is important to assess the current clinical landscape for this condition in the United States (US) and the European Union (EU).

**OBJECTIVE**

To examine the similarities and discordances of clinical trials for SBMA in the United States and European Union

**METHODS**

- ClinicalTrials.gov
- EU Clinical Trials Register
- Key Words: spinal and bulbar muscular atrophy, SBMA, Kennedy’s disease
- Exclude non-SBMA related trials by reading through the study title
- Exclude non-drug trials to have understanding on the developing medications
- Search PubMed for articles about SBMA drug clinical trials to make sure the information from the databases are updated

**RESULTS**

<table>
<thead>
<tr>
<th></th>
<th>Total results: 130</th>
<th>Spinal and bulbar muscular atrophy: 7 results</th>
<th>SBMA: 12 results</th>
<th>Kennedy’s disease: 111 results</th>
</tr>
</thead>
<tbody>
<tr>
<td>Completed: 3</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>(with results: 2)</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Ongoing: 1</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1 trial excluded</td>
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<td></td>
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<td></td>
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<tr>
<td>11 relevant results</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>5 drug trials</td>
<td></td>
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</tbody>
</table>

**FINDINGS**

- From this examination of 206 total results from Clinicaltrials.gov and EU Clinical Trials Register, 13 results (6.31%) were relevant. Further examination revealed:
  - Since 2000, only 6 drug clinical trials for SBMA have been documented in the US and EU, but 1 clinical trial was registered in both databases, illustrating a lack of ongoing studies in those areas (Fig. 2, Fig.3).
  - More studies in the US associate the key term, “Kennedy’s disease,” with SBMA than studies in the EU (Fig. 4).
  - Previously conducted clinical trials in both US and EU were proved to be largely ineffective in the treatment of SBMA.

Overall, this examination illustrates the development of SBMA clinical trials in the US and EU in the last 20 years, emphasizing the scarcity of research for this rare disease. A closer look into the characteristics of these studies may reveal regulatory and biological barriers, clinical trial design challenges, and explanations behind why medications have not been developed for SBMA.

**CONCLUSIONS**

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**REFERENCES**

2. Kennedy’s Disease Association: www.kennedysdisease.org

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