Measuring Burden of Illness in Hemophilia: How to use in Sickle Cell Disease for Advocacy

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The Hemophilia Utilization Group Studies (HUGS)
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- Founded by patient advocate with severe hemophilia
- Largest independent cost of care and burden of illness research in the U.S.A.
- HUGS publications support policies promoting patient access to expert care and new therapeutics.
- Patient Advocate on Steering Committee.
- [http://pharmweb.usc.edu/HUGS/index.html](http://pharmweb.usc.edu/HUGS/index.html)
HUGS model and value

**Model**
- Independent, multiple funders, housed outside Blood Disorders Clinic
- Research team
- Robust scientific methods + publication portfolio = Sustainability and international reputation.

**Value**
- Measures we can defend
- Documents cost of care
- Quantifies illness burden
- Measures changes before/after new therapy is available – documenting impact
- Informs policy – access to new treatments
## Burden of Illness – Underemployment due to Hemophilia

<table>
<thead>
<tr>
<th>Employment Status</th>
<th>Age 18-30 years (N=121)</th>
<th>Age &gt;30 years (N=117)</th>
<th>P value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Full-Time employed</td>
<td>53 (44%)</td>
<td>58 (52%)</td>
<td>0.0477</td>
</tr>
<tr>
<td>Part-Time employed</td>
<td>26 (22%)</td>
<td>11 (10%)</td>
<td></td>
</tr>
<tr>
<td>Part-Time - due to hemophilia*</td>
<td>1 (4%)</td>
<td>7 (64%)</td>
<td>&lt;0.05</td>
</tr>
<tr>
<td>Unemployed</td>
<td>41 (34%)</td>
<td>43 (38%)</td>
<td></td>
</tr>
<tr>
<td>Unemployed due to hemophilia*</td>
<td>15 (37%)</td>
<td>21 (49%)</td>
<td>0.26</td>
</tr>
</tbody>
</table>
### Comorbidities in Hemophilia by age inform health services and policy priorities

<table>
<thead>
<tr>
<th></th>
<th>Age 18-24 years (N=75)</th>
<th>Age 25-34 years (N=66)</th>
<th>Age ≥35 years (N=97)</th>
<th>p value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Have 1 or more comorbidities</td>
<td>39 (52%)</td>
<td>50 (76%)</td>
<td>84 (87%)</td>
<td>&lt;0.0001</td>
</tr>
<tr>
<td>Arthritis</td>
<td>18 (24%)</td>
<td>29 (44%)</td>
<td>63 (65%)</td>
<td>&lt;0.0001</td>
</tr>
<tr>
<td>Liver disease/Hepatitis</td>
<td>22 (29%)</td>
<td>45 (68%)</td>
<td>77 (79%)</td>
<td>&lt;0.0001</td>
</tr>
<tr>
<td>HIV/AIDS</td>
<td>2 (3%)</td>
<td>18 (27%)</td>
<td>30 (31%)</td>
<td>&lt;0.0001</td>
</tr>
<tr>
<td>Cardiovascular conditions a</td>
<td>4 (5%)</td>
<td>10 (15%)</td>
<td>39 (40%)</td>
<td>&lt;0.0001</td>
</tr>
<tr>
<td>Diabetes</td>
<td>0</td>
<td>1 (2%)</td>
<td>4 (4%)</td>
<td>0.1613</td>
</tr>
</tbody>
</table>
Modify Tools for Sickle Cell Specifics

• Physical co-morbidities
• Social determinants & access
  • Races, ethnicities, cultures, languages
  • Insurance
• Healthcare utilization
• Caregiver impact
• Pain management
• Evidence based therapies
Policy Impact

• Document costs and illness burden
• Educate insurance companies about Standards of Care
• Educate government decision makers about financing and workforce
Replicate Success in Sickle Cell Disease

- HUGS publications convince payers that expensive treatments, and care at expert centers are necessary for individuals and families to lead normal lives.
- Now is the time to establish a baseline burden of illness for SCD in advance of new therapies.
- HUGS is proposing to test SCD version of successful methodology at 3 to 4 expert SCD treatment centers.
- Hope to expand to SCD stakeholders nationally to build scientifically robust and uniform burden of illness studies for advocates to use to promote access to expert SC centers and new treatments.
Thank you!

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