Patient experiences and treatment access with pediatric rhabdomyosarcoma

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Pediatric Rhabdomyosarcoma

Background

- Incidence: ~350 cases per year\(^1\)
  - 15-20% arise in the GU
- Treatment: Radiation + chemotherapy with goal of bladder preservation
- Overall 5-year survival is ~84%\(^2\)
- Rare disease that leads to unique challenges faced by patients and their families

Methods

39-question online survey

Members of two online support groups invited to participate
One group for parents of children living with rhabdomyosarcoma
One group for parents of deceased children from rhabdomyosarcoma

Inclusion:
All respondents with RMS age 18 and younger
Excluded multiple responses from same IP address
## Demographics

<table>
<thead>
<tr>
<th>Demographic</th>
<th>Value</th>
<th>Num. Patients</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Sex</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Male</td>
<td>41</td>
<td></td>
<td>56%</td>
</tr>
<tr>
<td>Female</td>
<td>32</td>
<td></td>
<td>44%</td>
</tr>
<tr>
<td><strong>Age</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>0-4 years</td>
<td>30</td>
<td></td>
<td>41%</td>
</tr>
<tr>
<td>4-8 years</td>
<td>18</td>
<td></td>
<td>25%</td>
</tr>
<tr>
<td>12-16 years</td>
<td>8</td>
<td></td>
<td>11%</td>
</tr>
<tr>
<td>16+</td>
<td>10</td>
<td></td>
<td>14%</td>
</tr>
<tr>
<td><strong>Histology</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Embryonal</td>
<td>51</td>
<td></td>
<td>70%</td>
</tr>
<tr>
<td>Alveolar</td>
<td>14</td>
<td></td>
<td>19%</td>
</tr>
<tr>
<td>Undifferentiated</td>
<td>8</td>
<td></td>
<td>11%</td>
</tr>
<tr>
<td><strong>Status</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Living</td>
<td>66</td>
<td></td>
<td>90%</td>
</tr>
<tr>
<td>Deceased</td>
<td>7</td>
<td></td>
<td>10%</td>
</tr>
</tbody>
</table>
Time-to-Treatment

After Initial Diagnosis

Number of Patients

[0, 7]  (7, 14] (14, 21] (21, 28] (28, 35] (35, 42] > 42

Median = 7.5 days

After Recurrence

Number of Patients

[0, 7] (7, 14] (14, 21] (21, 28] (28, 35] (35, 42] > 42

Median = 10.4 days
Treatment Access

- Travelled > 100 miles for care: 48%
- Sought second opinion: 43%
- Fertility preservation offered: 23%

Time to Obtain Second Opinion

- Less than 72 hours: 19%
- Less than one week: 29%
- Less than one month: 38%
- Greater than one month: 14%
Free Response Data

What do you wish you knew back when your child was diagnosed?

- Treatment options
- Realistic expectations
- Alternative therapies
- Side effects
Limitations/Conclusions

• Limitations
  • Patient reported data
  • Users confined to multiple-choice answers

• Treatment delays appear to be common among patients with RMS
• Very few patients are being offered fertility preservation
• Barriers to expert care include insurance coverage, social situation, and travel expenses