2. What is your current role(s) at the Center where you work (You can choose more than one if appropriate)

<table>
<thead>
<tr>
<th>Role</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Attending Physician</td>
<td>28%</td>
</tr>
<tr>
<td>Resident/Fellow/Medical Student</td>
<td>5%</td>
</tr>
<tr>
<td>Researcher</td>
<td>9%</td>
</tr>
<tr>
<td>Nurse (RN, APN, LPN etc)</td>
<td>27%</td>
</tr>
<tr>
<td>Clinic Coordinator</td>
<td>7%</td>
</tr>
<tr>
<td>Research or Trial Coordinator</td>
<td>6%</td>
</tr>
<tr>
<td>Therapist (PT/OT/SLP)</td>
<td>5%</td>
</tr>
<tr>
<td>Genetic Counselor</td>
<td>4%</td>
</tr>
<tr>
<td>Social Worker</td>
<td>8%</td>
</tr>
<tr>
<td>Other Healthcare Professional not listed</td>
<td>8%</td>
</tr>
</tbody>
</table>
3. Please indicate which state your center is in:
4. Is your center a PPMD Certified Duchenne Care Center?

- Yes - CDCC: 84%
- No - Non CDCC: 16%
5. Approximately how many Duchenne patients does your clinic currently serve?

- 1-25: 8%
- 25-50: 12%
- 50-75: 20%
- 75-100: 13%
- 100-125: 9%
- 125-150: 13%
- 150-200: 8%
- 200+: 15%
1. Think about the past 12 months. Around what percentage of your Duchenne patients have inquired at least once about clinical trials?

- Less than 25%: 2%
- 25%: 8%
- 50%: 31%
- 75%: 22%
- 100%: 9%
- My role does not involve conversations with patients about trials: 27%
2. Around what percentage of those inquiries in the last 12 months have been about gene therapy?

- Less than 25%: 8%
- 25%: 11%
- 50%: 16%
- 75%: 23%
- 100%: 8%
- My role does not involve conversations with patients about trials: 34%
3. What percentage of your Duchenne patients do you estimate have no interest whatsoever participating in clinical trials?

- Less than 25%: 42%
- 25%: 26%
- 50%: 3%
- 75%: 1%
- 100%: 28%

My role does not involve conversations with patients about trials: 28%
4. In one word or phrase what do you hope most to get out of attending the Healthcare Professionals Summit?
1. Does your center have a tool used to formally evaluate mental health/psychosocial issues (depression, anxiety, etc.) of Duchenne patients?

- Yes: 30%
- No: 48%
- Unsure: 9%
- My role does not involve this area of care: 13%
2. Does your center have a tool used to formally evaluate caregiver/family mental health?

- Yes: 11%
- No: 78%
- Unsure: 3%
- My role does not involve this area of care: 8%
3. What are the top 3 mental health/psychosocial issues that you see (are aware of) in your Duchenne patients who are up to 12 years old? (Choose 3)

- Obsessive Compulsive Disorder: 2%
- Attention Deficit Hyperactivity Disorder: 24%
- Oppositional/defiant behaviors: 5%
- Depression: 10%
- Anxiety: 16%
- Learning/Educational: 26%
- Autism-like behaviors: 16%
4. What are the top 3 mental health/psychosocial issues that you see (are aware of) in your Duchenne patients who are up to 13-17 years old? (Choose 2)

- Depression: 44%
- Anxiety: 35%
- Learning/Educational: 11%
- Attention Deficit Hyperactivity Disorder: 7%
- Oppositional/defiant behaviors: 1%
- Obsessive Compulsive Disorder: 2%
5. What are the top 3 mental health/psychosocial issues that you see (are aware of) in your Duchenne patients who are 18 and older (Choose 2)

- Obsessive Compulsive Disorder: 2%
- Attention Deficit Hyperactivity Disorder: 1%
- Oppositional/defiant behaviors: 2%
- Depression: 50%
- Anxiety: 42%
- Learning/Educational: 3%
- Autism-like behaviors: 3%
6. Approximately what percentage of your Duchenne patients are on medication for mental health/psychosocial issues?

- Less than 25%: 21%
- 25%: 30%
- 50%: 11%
- 75%: 6%
- 100%: 6%
- Unsure: 19%
- My role does not involve this area of care: 13%
7. Approximately what percentage of your Duchenne patients (that you are aware of) are using some form of Cannabis?

- Less than 25%: 38%
- 25%: 3%
- 50%: 3%
- 75%: 3%
- 100%: 3%
- Unsure: 42%
- My role does not involve this area of care: 14%
1. Thinking about the use of steroids for the treatment of Duchenne, based on your clinical experience, which of the following statements do you believe to be the most true?

- Prednisone is more effective than deflazacort: 38%
- Prednisone and deflazacort are equally effective: 21%
- Deflazacort is more effective than prednisone: 42%
- Unsure: 38%
2. Who at your center manages issues around puberty for patients with Duchenne?

- Primary care provider: 12%
- Neuromuscular provider: 3%
- Endocrinologist: 64%
- Nurse: 22%
- Unsure: 3%
3. What is the primary indication for a referral to endocrinology?

- Weight: 4%
- Height: 3%
- Puberty: 15%
- Bone: 51%
- Unsure: 3%
- My role does not involve this area of care: 24%
4. For patients ages 17 and older who have discontinued use of steroids what are two of the most frequent reasons for stopping? (choose 2)

- Weight gain: 41%
- Not enough benefit: 26%
- Behavior: 15%
- Bone Health: 11%
- Cushingoid: 4%
- Impact of delayed puberty: 2%
- Height: 1%
- Insurance: 1%
2. Who at your center manages issues around puberty for patients with Duchenne?

- Primary care provider: 12%
- Neuromuscular provider: 12%
- Endocrinologist: 64%
- Nurse: 3%
- Unsure: 22%
3. What is the primary indication for a referral to endocrinology?

- Bone: 51%
- Puberty: 15%
- My role does not involve this area of care: 24%
- Weight: 4%
- Height: 3%
- Unsure: 3%
4. For patients ages 17 and older who have discontinued use of steroids what are two of the most frequent reasons for stopping? (choose 2)

- Insurance: 1%
- Weight gain: 41%
- Not enough benefit: 26%
- Behavior: 15%
- Impact of delayed puberty: 2%
- Height: 11%
- Bone Health: 4%
5. For patients ages 4-16 years old who have discontinued use of steroids what are two of the most frequent reasons for stopping? (choose 2)

- Financial burden/Insurance: 1%
- Not enough benefit: 5%
- Weight gain: 41%
- Behavior: 51%
- Bone health: 1%
- Cushingoid: 1%
- Delayed Puberty: 1%
1. In your view, what are the 3 biggest barriers to conducting Duchenne clinical trials at your institution? (Choose 3)

- Staff capacity: 30%
- Sufficient physical space at clinic: 20%
- Funding: 18%
- Interested Patients: 3%
- Institutional support for running trials: 10%
- Patients that meet the inclusion criteria: 17%
- Uncertainty about which trials to run at site: 3%
2. For those centers who care for patients 18 and older, which statement about their healthcare decision making do you believe to be the most accurate? Most adult patients:

- Communicate about their healthcare but defer most decisions to caregiver: 59%
- Communicate about their healthcare and make most decisions: 20%
- Defer to caregiver to communicate and make decisions: 20%
3. For those centers who care for patients 18 and older, which statement about their clinical trial decision making do you believe to be the most accurate? Most adult patients:

- Ask questions about clinical trials but defer most decisions to caregiver: 28%
- Ask questions about clinical trials and make their own decisions: 28%
- Defer to caregiver to ask questions and make decisions: 44%
1. Please tell us the top two areas of national or state policy you believe needs major change or attention

- Navigating Medicaid/Medicaid waiver eligibility process: 13%
- Lowering drug prices: 42%
- Advocating for school needs IEP/504: 5%
- The FDA review process: 1%
- Access to durable medical equipment: 22%
- Access to approved therapies: 17%
2. What are the 3 biggest barriers to facilitating access and reimbursement of approved therapies?

- Assisting families navigating insurance process: 17%
- Staff capacity: 14%
- Time involved with conducting assessments for different insurers: 14%
- Recent influx of newly approved medications for both SMA and DMD: 10%
- Burdensome paperwork (prior authorizations and appeals): 32%
- Ability of institution to provide therapy in-house: 7%
- My role does not involve administering, prescribing, or monitoring patients on newly approved treatments: 7%
3. In your view, who on your team spends the most hours on the process of access to new medicines?

- Clinic Coordinator: 48%
- RN or NP in clinic (non coordinators): 27%
- The prescribing physician: 17%
- Pharmacy staff: 2%
- Unsure: 6%
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- Clinic Coordinator: 48%
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- The prescribing physician: 17%
- Pharmacy staff: 2%
- Unsure: 6%
4. Since the approval of Emflaza, the process for your clinic to administer access has gotten better, worse, or stayed the same?

- Better: 20%
- Worse: 50%
- About the same: 4%
- Unsure: 26%
5. Since the approval of EXONDYS 51, the process for your clinic to administer access has gotten better, worse, or stayed the same?

- Better: 12%
- Worse: 12%
- About the same: 39%
- Unsure: 37%
1. Following this meeting what is one area of care you will consider addressing differently?