

Parent Project Muscular Dystrophy

JOIN THE FIGHT.

END DUCHENNE.

2018 Annual Conference

Polling Report

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2018 Conference Polling Report

The 2018 PPMD Annual Conference provided a unique opportunity to conduct live polling of those joining us, including those joining in person and those joining virtually from home. The following report provides the data collected over the course of the conference.

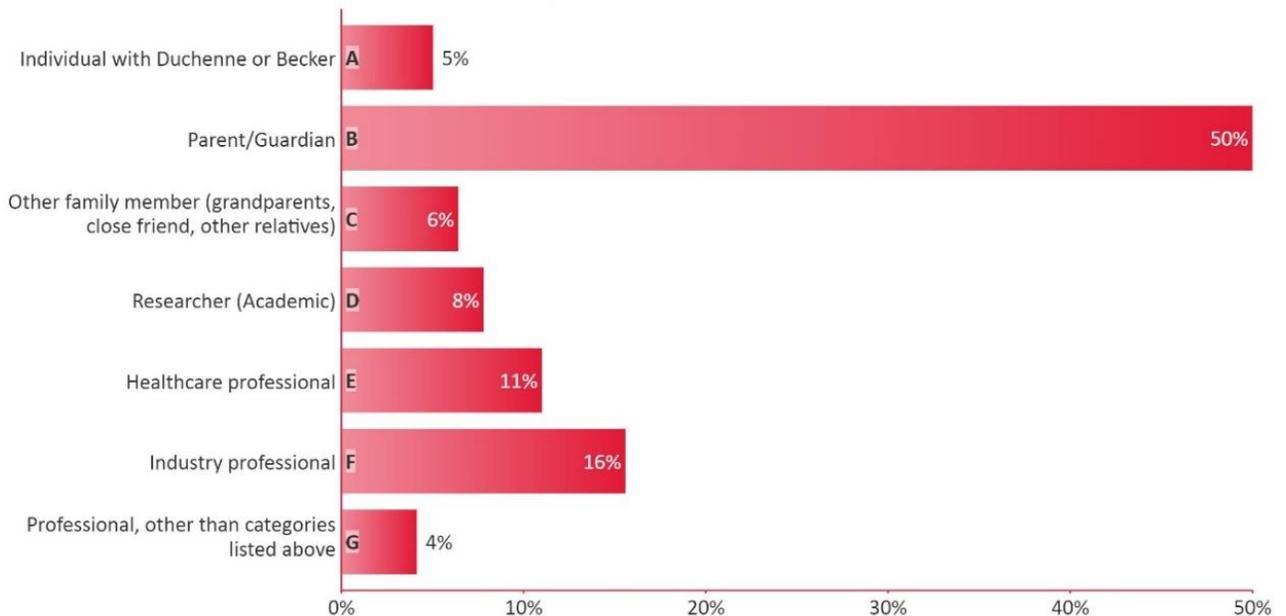
One of the limitations of live polling is that the number of people who answer each question varies depending on who was in the room or watching online at the time the question was asked. Thus numbers (n=) of people who answered each question fluctuate throughout the report.

Overall, the data collected during the conference provides meaningful insight on a range of topics, the purpose of which is to inform all those working in the Duchenne space.

Results of Polling Questions

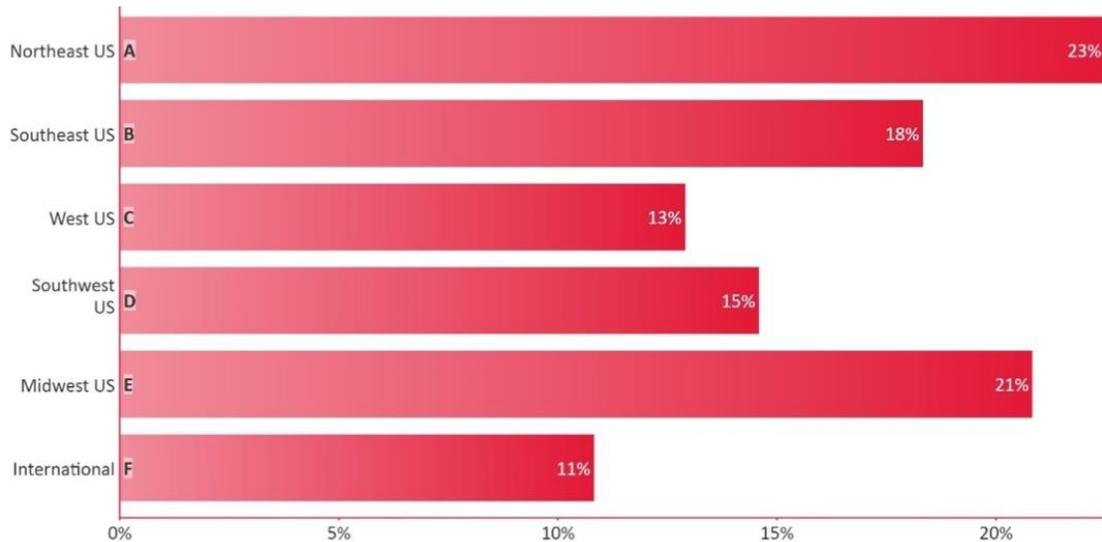
We first asked a few background questions to understand our in-person and virtual audiences. The majority of respondents were **caregivers** (50%) followed by industry professionals (16%) and then healthcare professionals (11%). This number changed depending upon who was answering the polling questions at the time.

Which describes you? (218 responses)



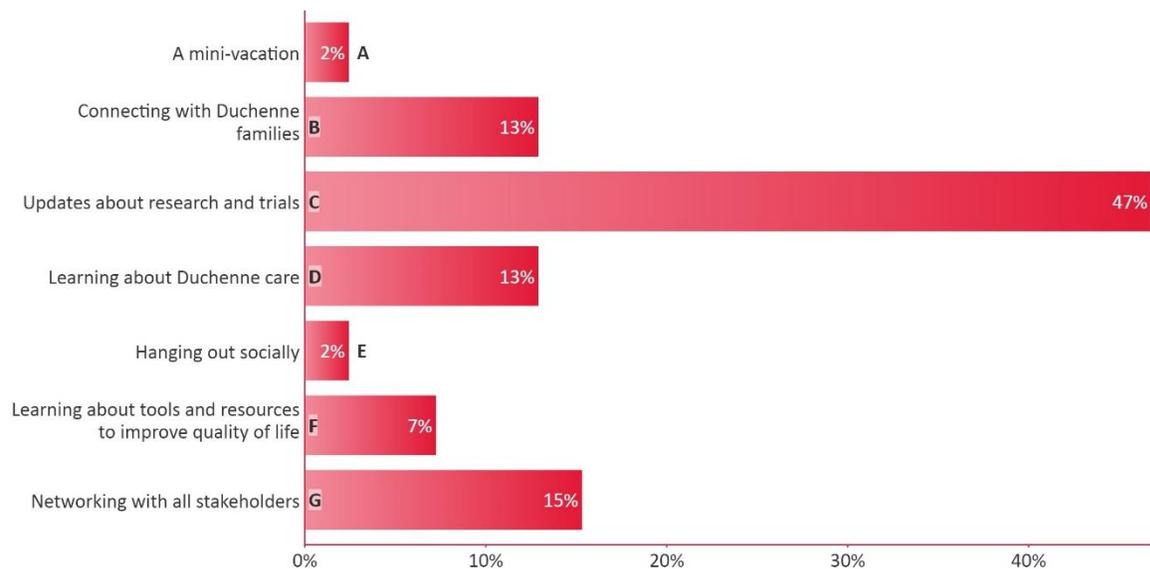
Next we asked where attendees resided. Attendees came from many different areas of the US and a good representation internationally (11%). While the majority reported coming from the **Northeastern US** (23%), there was a considerable balance of geographic representation. The in-person meeting was held in Arizona. Online participation was allowed from anywhere.

? *Where are you from? (241 responses)*



We then asked respondents what they hoped to get most out of attending conference. The majority reported the **desire to obtain updates about research and trials** (47%). This was followed by networking opportunities.

? *What do you hope MOST to get out of attending the conference (choose one)? (248 responses)*

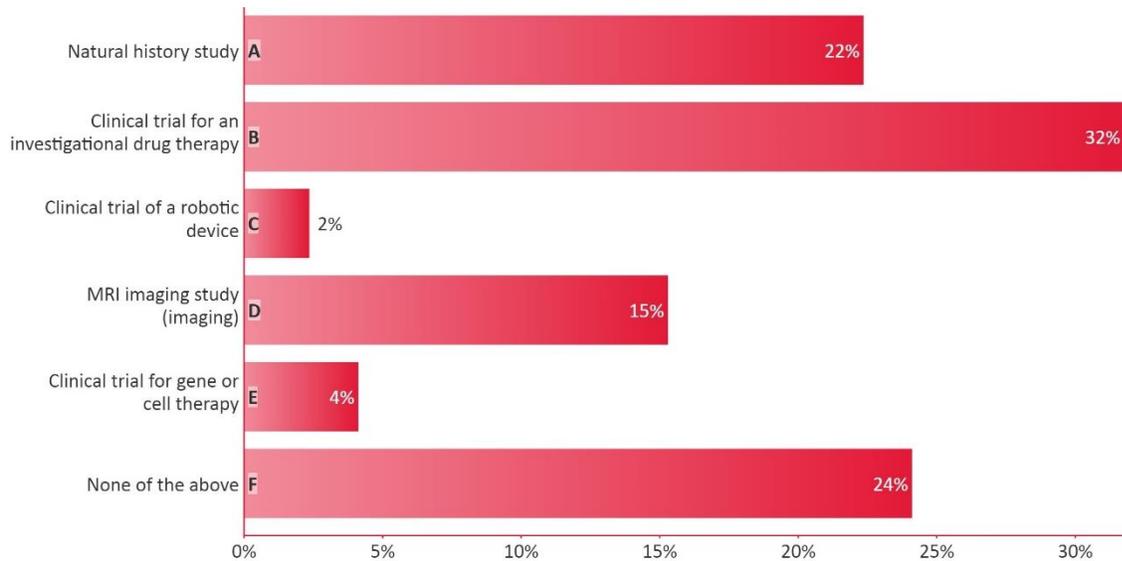


The following set of questions were directed at Duchenne families only.

We asked families to indicate what types of research (interventional and non-interventional) they or their child have participated in. They were allowed to choose all options that applied. We see across all respondents that a **majority have participated in some form of research (76%)**, with about a quarter (24%) indicating they have not. More than one third indicated being in a clinical drug trial.



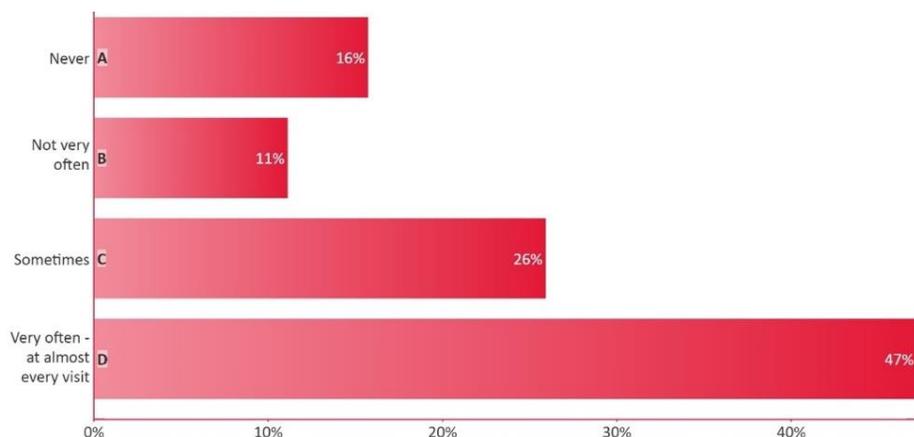
Families only (one per family) – Which of the following types of research have you or your child participated in? (Choose all that apply) (117 responses)



One of the most important interactions related to clinical trial decision making process is that between the patient and their treating physician. We asked families how often their doctor talked with them about research opportunities and advances. Nearly half reported **'very often at almost every visit' (47%)** followed by 'sometimes' (26%). Only 16% reported 'never.'

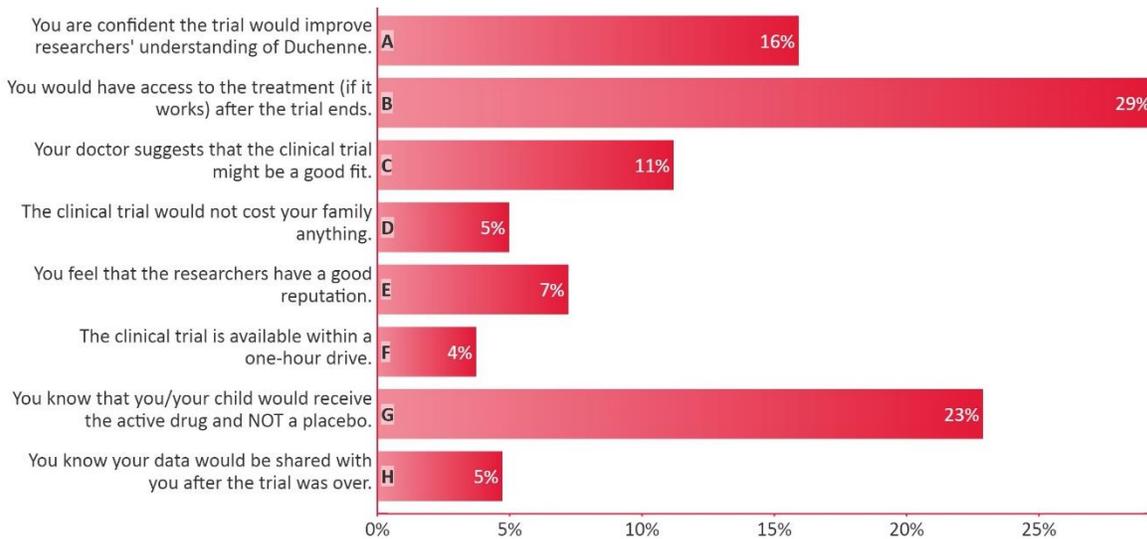


Families only (one per family) - During the past 2-3 years, how often has you/ your child's doctor talked to you about research opportunities and advances? (108 responses)



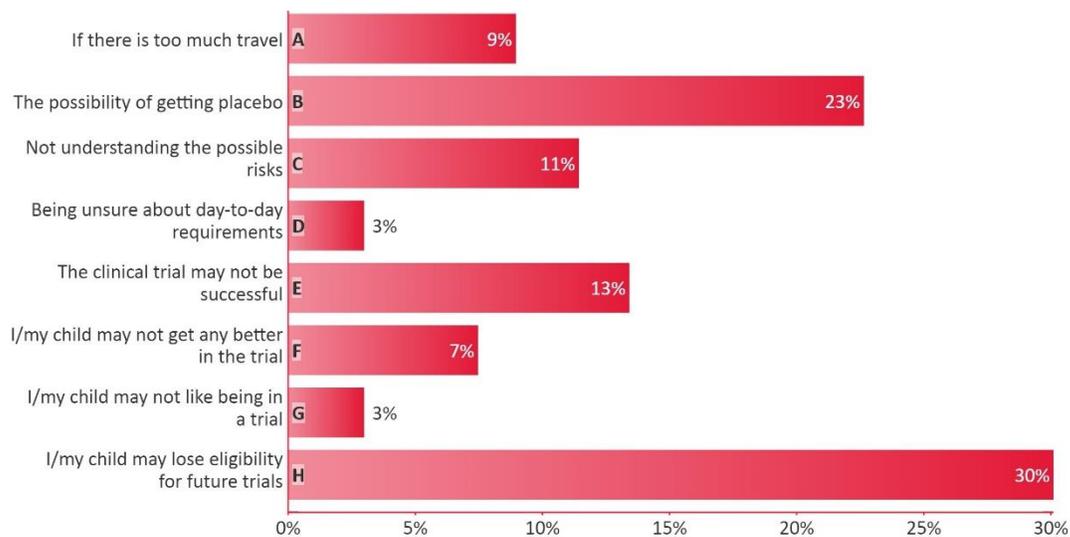
We asked families to indicate things most important in agreeing to a trial. Similar to past studies conducted by PPMD about clinical trial decision making, we find that **access to the potential treatment after the end of the trial** (29%) and **avoiding the potential of a placebo** (23%) among the majority decision making factors. This was followed by an altruistic reason of improving our understanding about Duchenne (16%), another factor indicated as important by families in our past research. Understanding factors influencing clinical trial decision gives PPMD insight about where more education is needed and helps us and trial sponsors make trials as family-friendly as possible.

? Families only – If you were deciding whether to join a new trial in the next few months, which three things would make you MOST likely to join? (Choose 3) (149 responses)



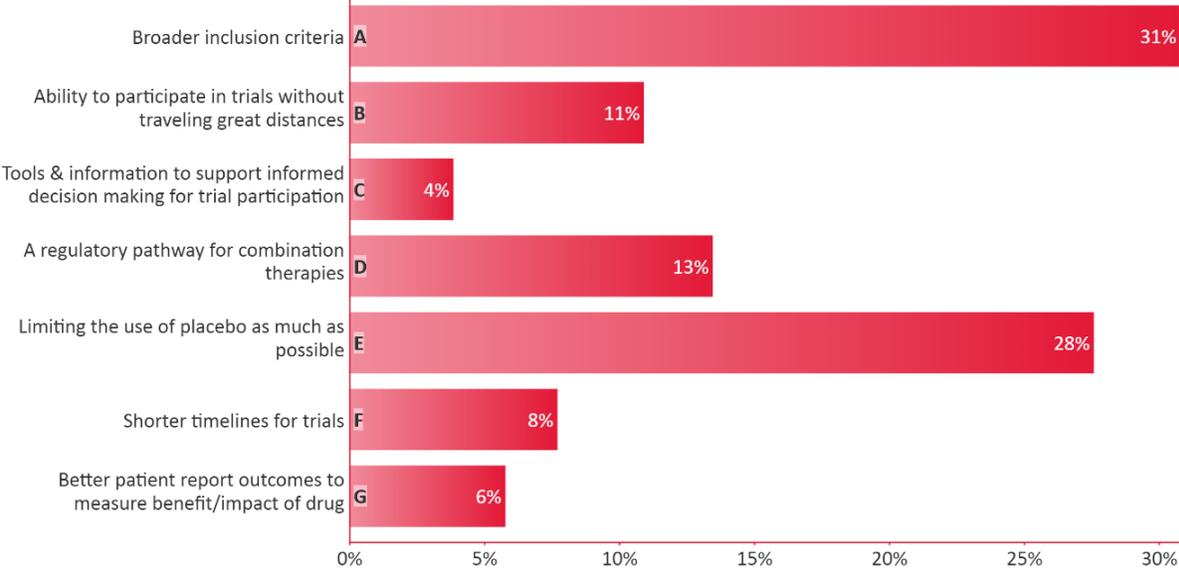
Similar to our question on positive decision-making influences, we sought to understand potential barriers influencing clinical trial decision making. In this next question, we asked about things that would reduce interest in trial participation. **Losing eligibility for future trials** ranked the highest (30%), followed by the possibility of getting placebo (23%). Concern that the trial may not be successful was the next highest ranked item (13%). This may be indicative of the number of Duchenne trial failures, and also, more generally, the fact that a large majority of all clinical trials fail.

? Families only – If you were deciding whether to join a new trial in the next few months, which three things would make you LEAST likely to join? (Choose 3) (147 responses)



We asked about the greatest needs in the current clinical trial landscape. The top items were **broader trial inclusion criteria** (31%) and **limiting the use of placebo** (28%).

? Families only – From the perspective of your family, what are two of the greatest needs in the current clinical trial landscape? (Choose 2) (156 responses)

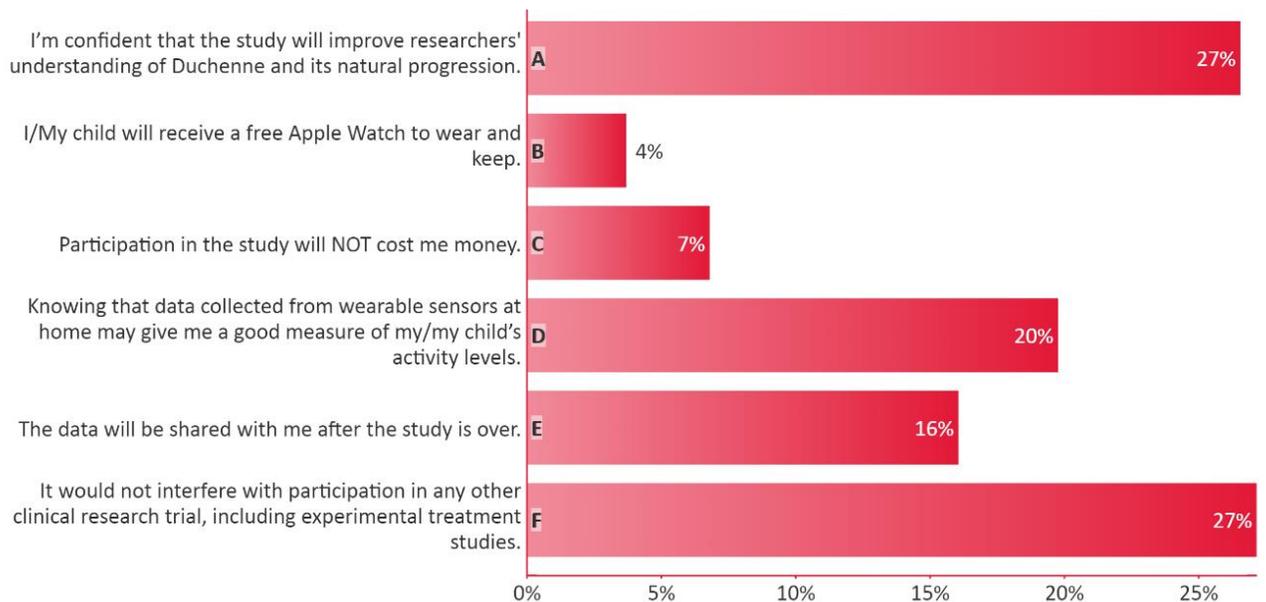


Our next set of questions for families were specific to wearable devices. These new technologies are emerging in their ability to track movement and activity. We were interested in understanding how families felt about participating in a study about wearable devices.

We asked families about things that would make them *most likely* to join a study that involved wearables. **Being able to participate in a treatment trial while doing this study** rated extremely high (27%). Similar to what we have seen in other studies we've conducted on facilitators to participation in trials, **being confident the study would improve researchers understanding about the disease was equally important** (27%). This was followed by knowing the data may give them a measure of their child's activity (20%) and a related item of knowing the data would be shared once the study was over (16%).



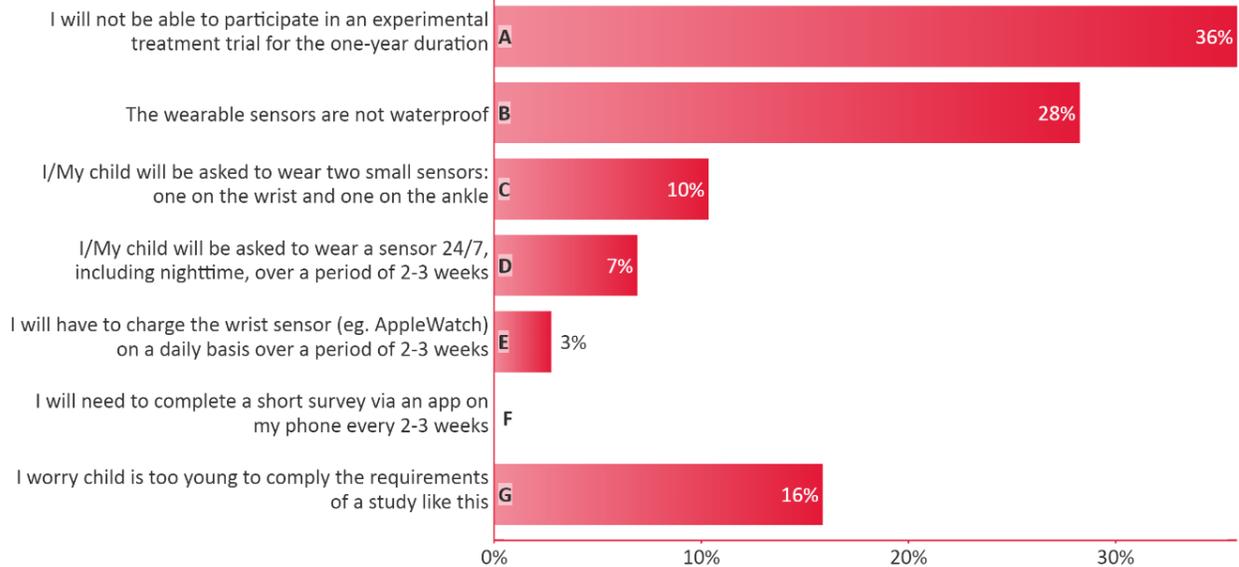
For Families – If you were debating whether or not to join a research study with wearable sensors that track activity over a one-year period, which three things would make you MOST likely to join? (Choose 3) (162 responses)



We then asked families about things that would make them *least likely* to join a study that involved wearables. For the barriers to participation with wearable devices, **the possibility that participation would interfere with participation in a treatment trial** was ranked the highest (36%). This was followed by **non-water proof sensors** (28%). The third most frequently selected item was concern over the child being too young for participation (16%).



For Families – If you were debating whether or not to join a research study with wearable sensors that track activity levels over a one-year period, which three things would be a deal-breaker and make you LEAST to join? (Choose 3) (145 responses)

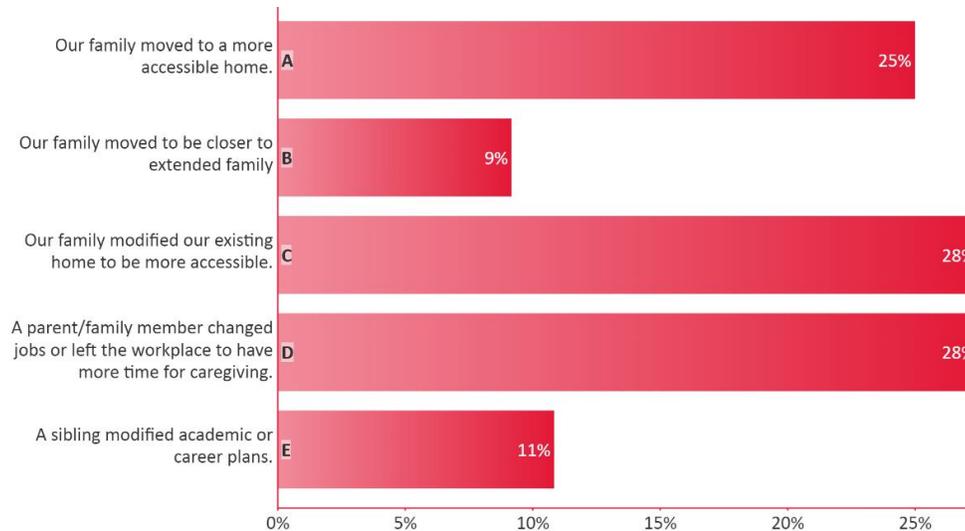


Our next set of questions directed at families pertained to costs associated with caring from someone with Duchenne.

We asked families (one response per family) to tell us about specific burdens associated with housing and employment. We allowed them to choose all that apply. The most frequently chosen items were **modifying homes to be more accessible** (28%) and changing jobs or leaving work for caretaking (28%). This was followed closely by moving to a more accessible home (25%).

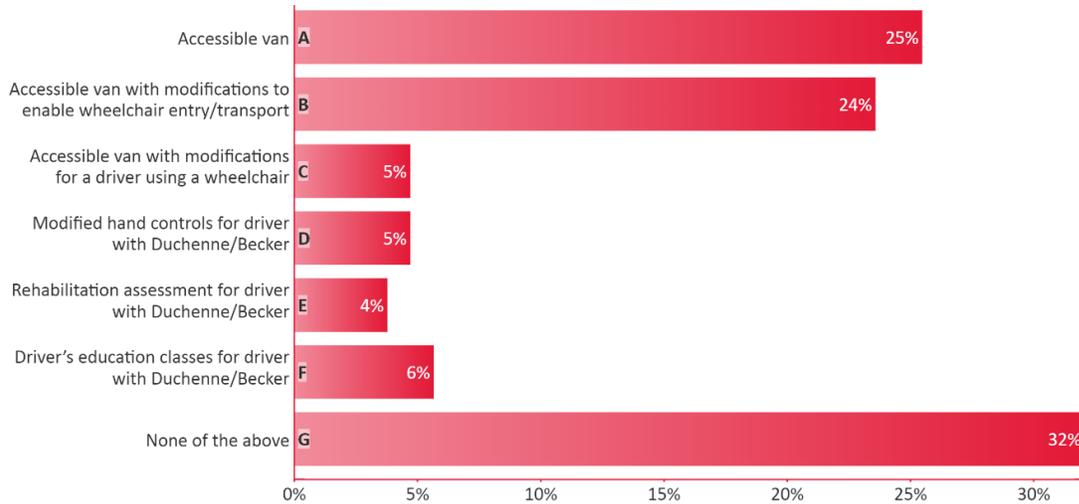


Families only (One per family) – Because of Duchenne/Becker: (Choose all that apply) (120 responses)



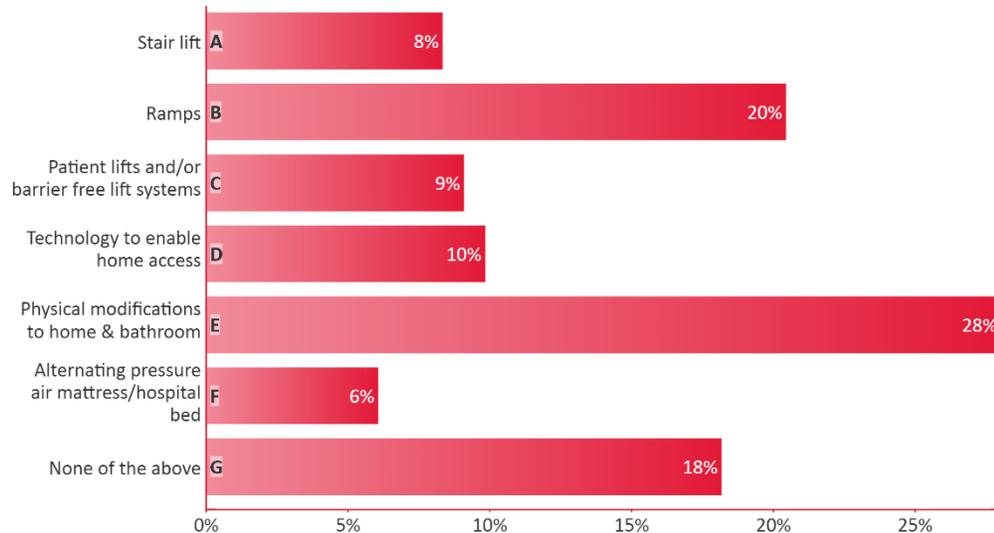
Next, we asked about the burden associated with enabling mobility outside of the house. While about a third (32%) reported not having any of the items as out of pocket expenses, the **majority reported one or more item**. Over 50% reported an accessible van (25%) or a van with wheelchair entry (24%) as their major out-of-pocket mobility expense.

? *For Families only (one per family) – Which of the following items have you paid for yourself (as out-of-pocket expenses) to enable mobility outside of your house? (Choose all that apply) (66 responses)*



We then asked about the burden associated with make homes more accessible. The item chosen most (28%) was **physical modifications to home and bathroom** followed by the addition of ramps (20%). 18% reported having none of the out of pocket expenses, but it is difficult to make any claims related to this group as factors may be attributed to age person affected or level of income.

? *Families Only (one per family)– Which of the following items have you paid for yourself (as an out-of-pocket expenses) to make your home more accessible? (Choose all that apply) (132 responses)*



With the conference including a number of sessions specific to care and management of Duchenne, we wanted to know one area of care families would manage differently after leaving the conference. We did this through a word cloud. We were happy to see a range of care areas indicated, with **Physical Therapy** and **Nutrition** being among the most often chosen areas.

Word Cloud - Based on this meeting what is one area of care you will manage differently in your care? (one word)

🔒 Poll locked. Responses not accepted.

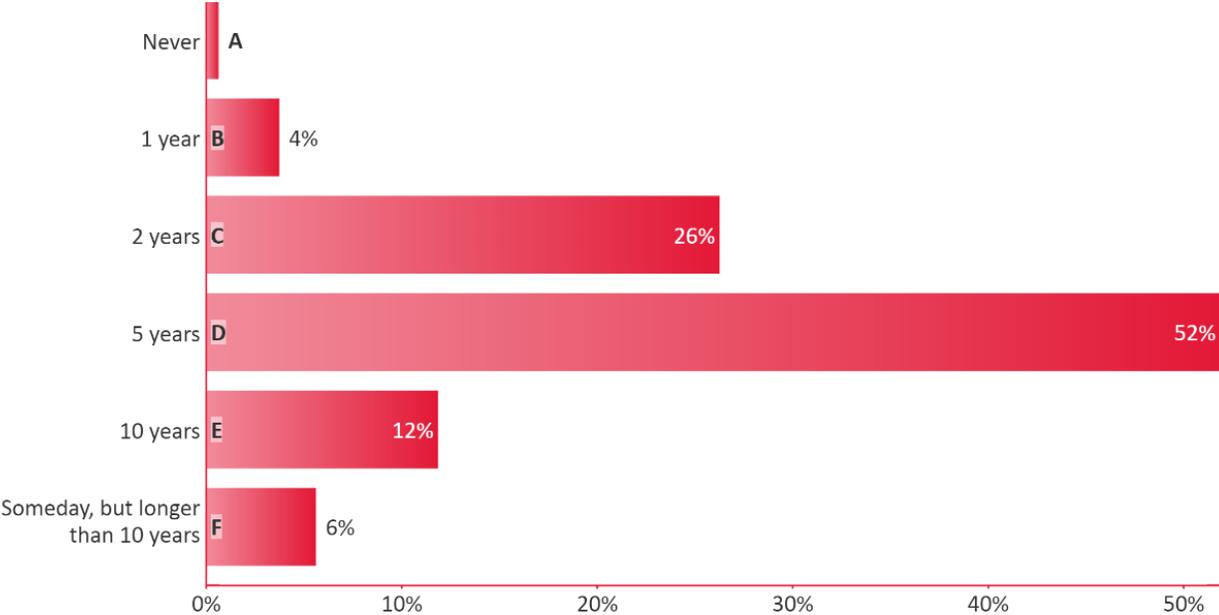


The following questions were asked to ALL members of the audience

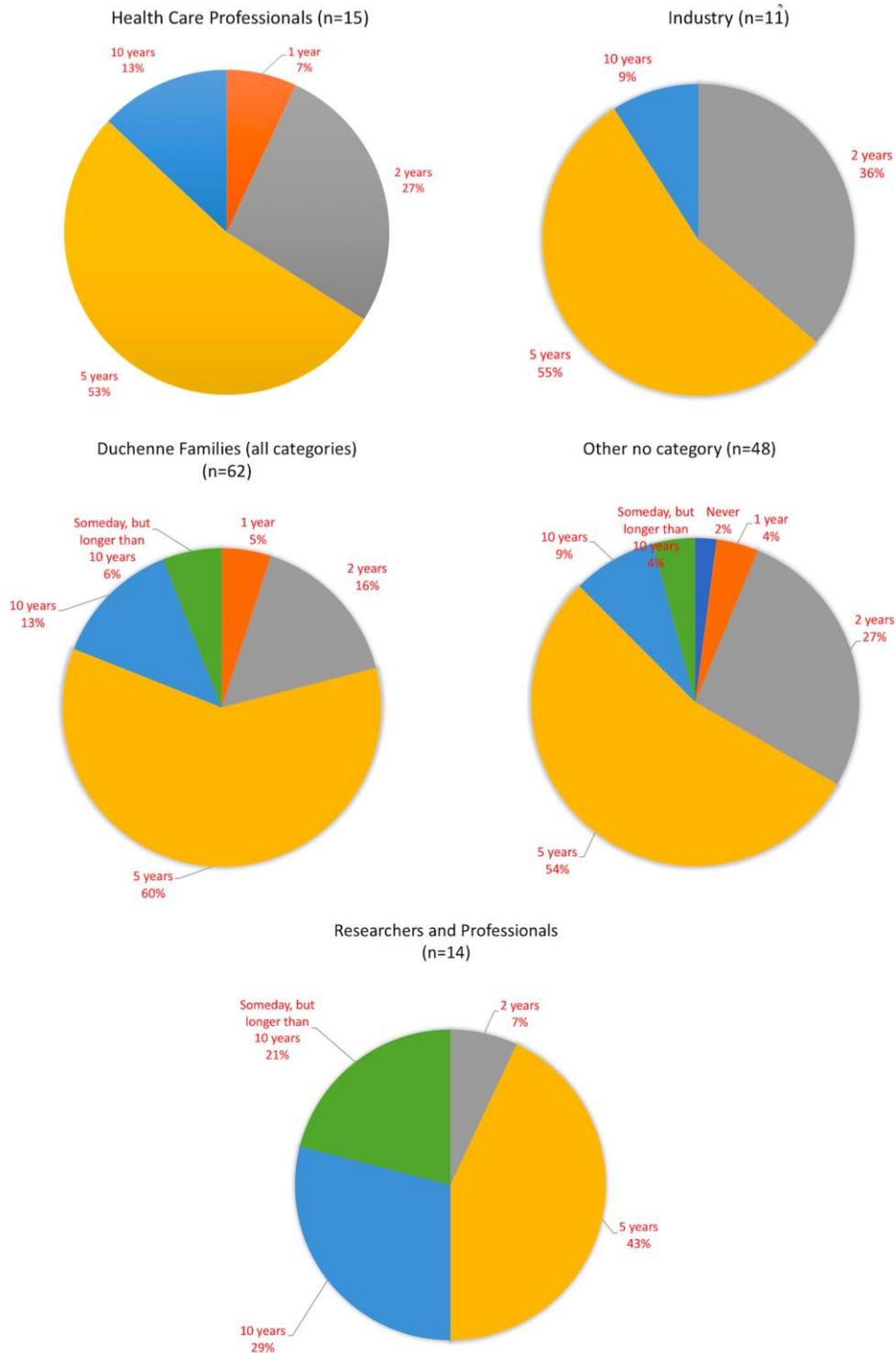
With the current enthusiasm and attention around gene therapy and gene editing in Duchenne, we asked a few questions about expectations around timelines for approval of gene therapy to treat Duchenne. It is important to note we asked this question BEFORE the presentations about these trials to get people's baseline thoughts. All attendees were asked to answer this question, not just families.

First we asked about gene therapy. Here we see the majority (52%) of respondents **indicated five years as the expected timeline** for when gene therapy would be approved, followed by a more optimistic timeline of two years (26%). 12% indicated approval would be in the ten-year range. And a small minority chose never, or chose longer than ten years.

? *Based on what you know, when do you believe gene therapy will be approved by the FDA as a treatment for Duchenne? Answer this based on your own opinion.*
(150 responses)



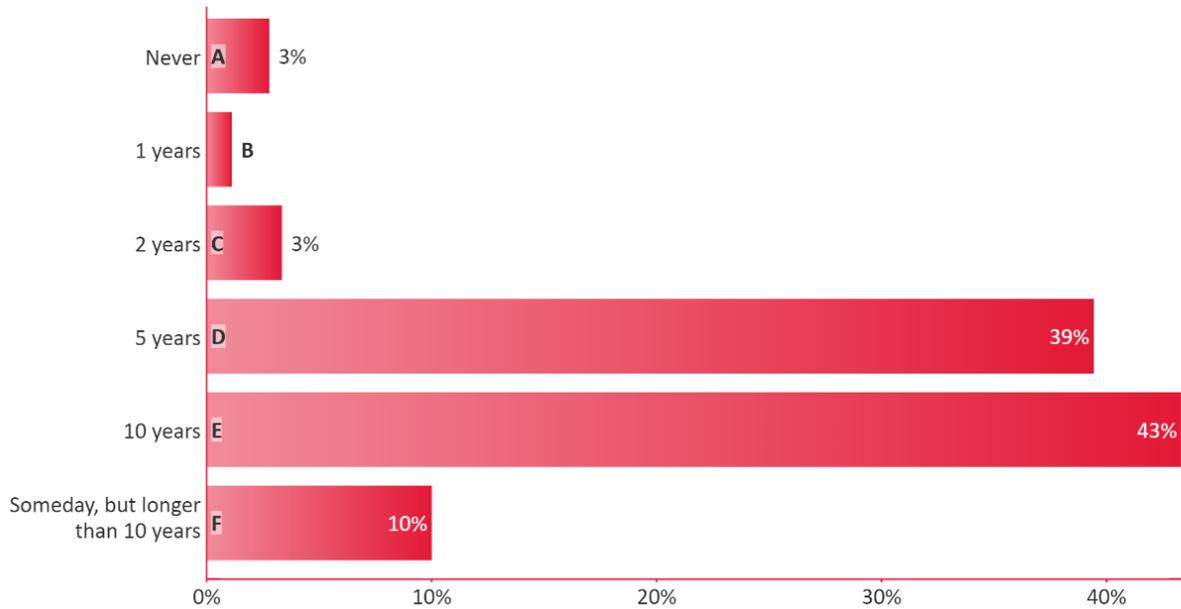
When will Gene Therapy be approved by FDA? – Stratified by stakeholder category



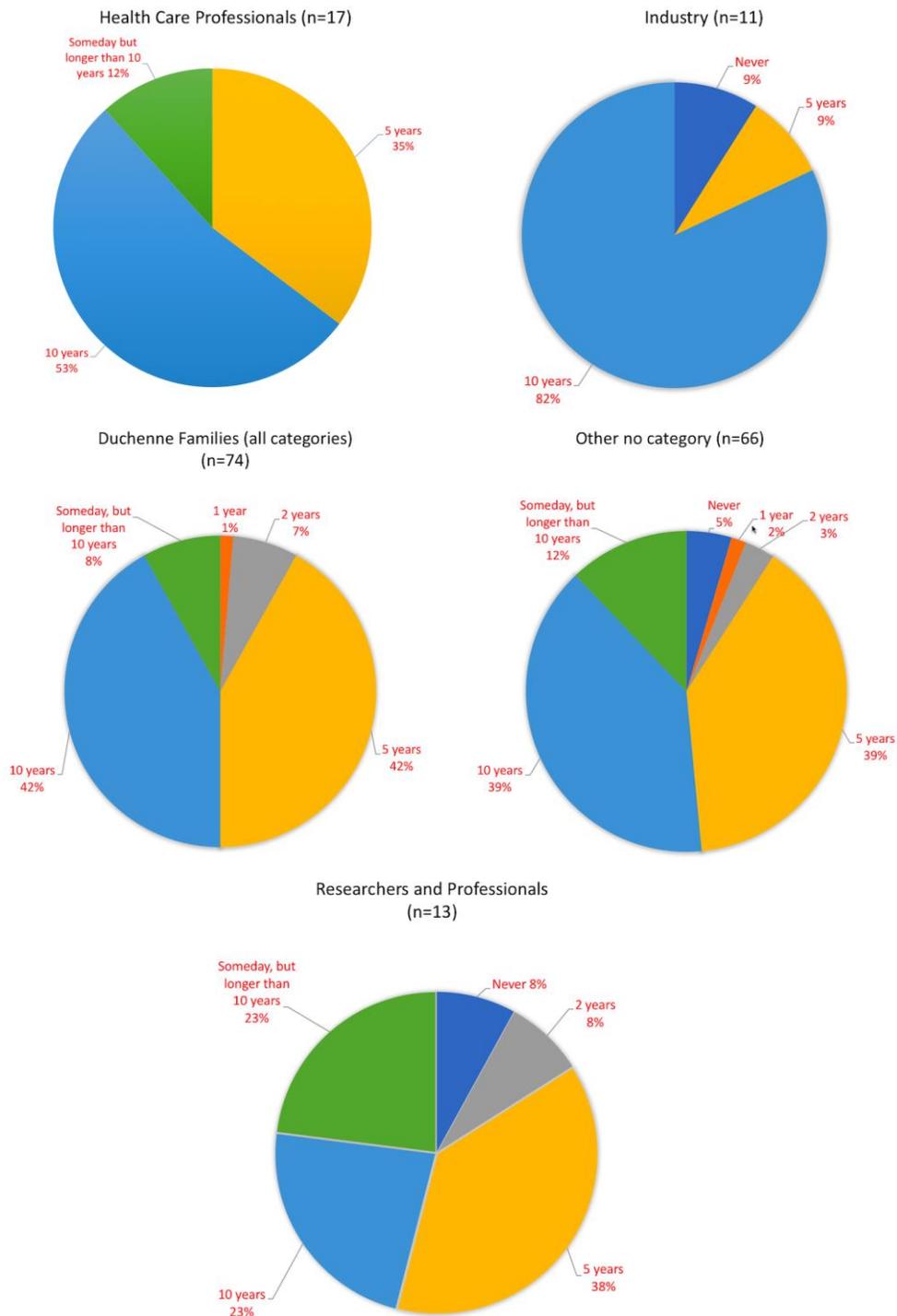
When reviewing the subgroup analysis, we see the majority option (**five years**) contains representation across stakeholder groups, including families. The second most chosen option (**two years**) was made up mainly of industry, healthcare professionals, and those for whom we did not have a category.

We then asked the same question about approval for a more future technology, gene editing or CRISPR Cas9. Here we see the majority (43%) responded with a more conservative estimate of ten **years** compared to five years for gene therapy. However, the second most chosen timeframe was also five years (39%) for gene editing/CRISPR.

? *Based on what you know, when do you believe CRISPR Cas9 (gene editing) will be approved by the FDA as a treatment for Duchenne? Answer this based on your own opinion. (181 responses)*



When will CRISPR Cas9 be approved by FDA? – Stratified by stakeholder category



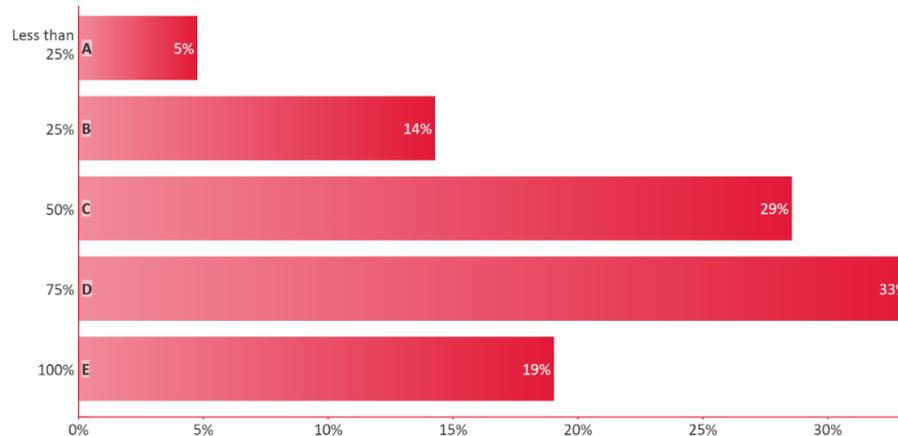
When reviewing the subgroup analysis by stakeholder category, the Duchenne families' number was split between the 5-year and 10-year estimates. The majority of respondents chose the **10 year estimate** followed by 5 years.

Our next set of questions were directed at Duchenne/Becker healthcare professionals.

We asked Healthcare professional what percentage of patients have inquired about clinical trials within the last 12 months. Though limited in terms of number of responses, we see the majority of healthcare professionals indicating **that at least 50 percent of their patients have inquired about clinical trials**. Over half chose items indicating that 75% to 100% of their patients have asked about trials.



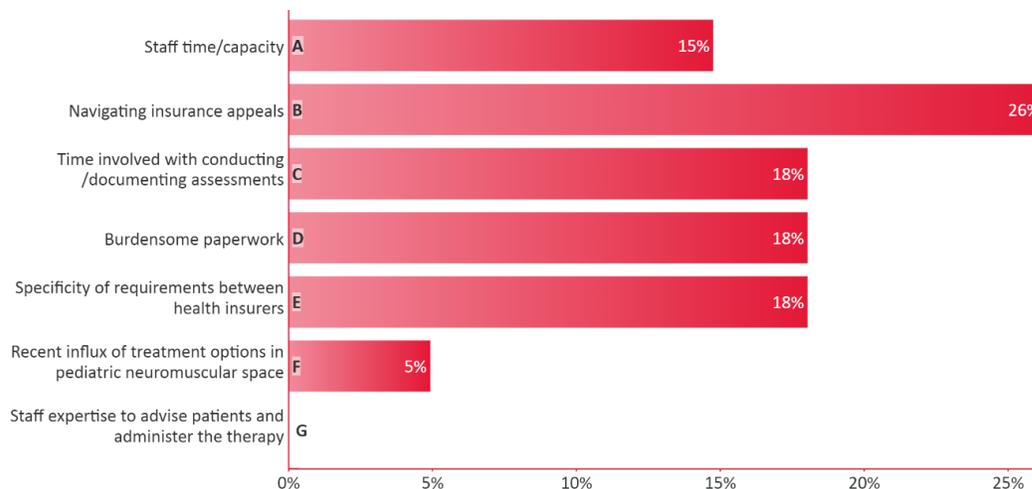
Healthcare Professionals only – Think about the past 12 months. Around what percentage of your patients have inquired at least once about clinical trials? (21 responses)



Next we asked about barriers to access to approved therapies. The highest ranked item (26%) was **‘navigating the insurance appeals process.’** But this was closely followed by a three-way tie between ‘time involved conducting assessments,’ ‘burdensome paperwork,’ and ‘specificity of requirements between health insurers’ (18% each). Staff expertise to advise patient and administer therapy was not considered a barrier by any of the respondents.

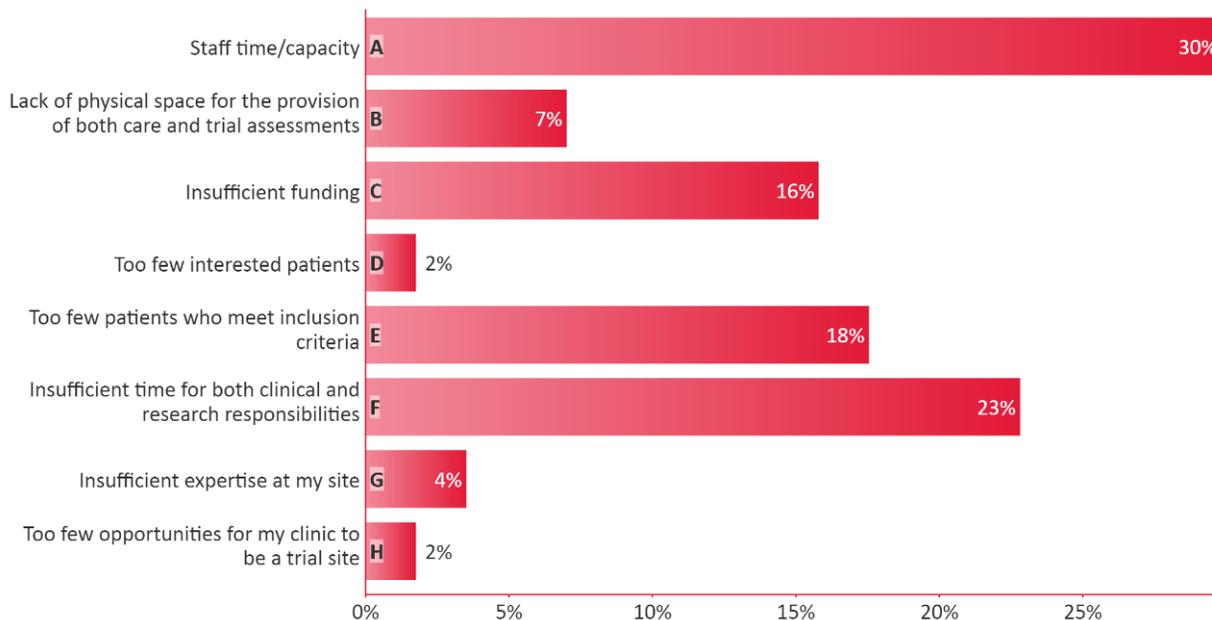


Healthcare Professionals only– What are the three biggest barriers to facilitating access to and reimbursement of approved therapies? (Choose 3) (61 responses)



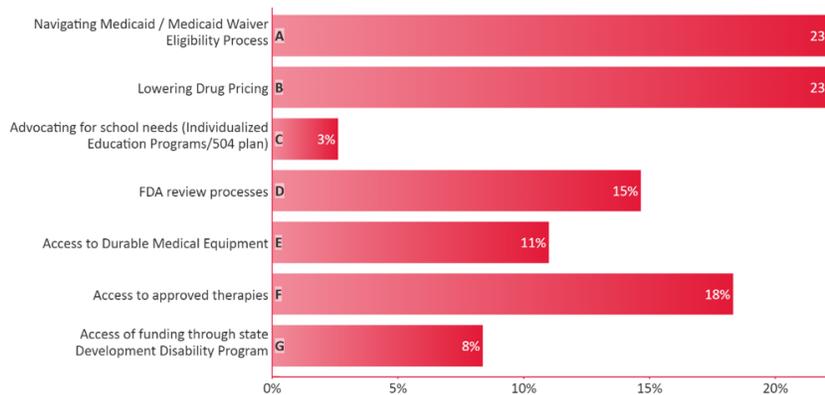
The final question for healthcare professionals asked about *the biggest barriers to conducting clinical trials*. The majority considered ‘staff time/capacity’ as the highest-ranking item (30%), followed by ‘insufficient time for both clinic and research’ (23%), and then ‘too few patients who meet the inclusion criteria’ (18%).

Healthcare Professionals only – What are the three biggest barriers to conducting clinical trials at your institution? (Choose 3) (57 responses)

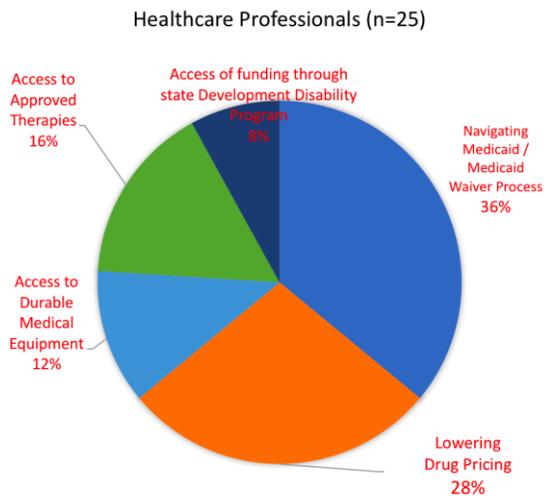
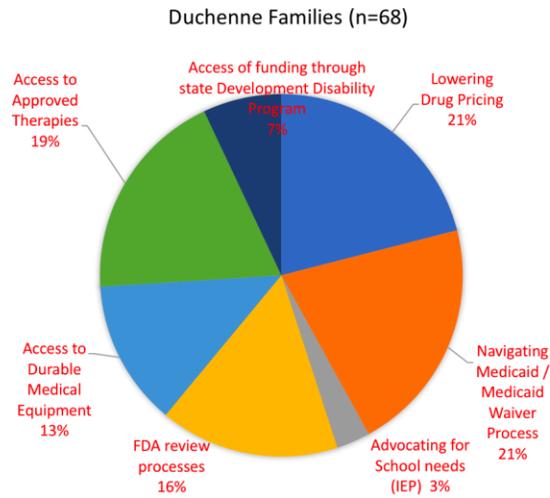


Our next question was open to both *healthcare professional and families*. We wanted to understand their view of the areas of national or state policy that need the most change or attention. **Tied for the most frequently chosen items were navigating the Medicaid/Medicaid Waiver process (23%) and lowering drug prices (23%)**, followed by access to approved therapies (18%). Closely behind was the FDA review process (15%).

Healthcare Professionals and Families – Please tell us two areas of national or state policy that you believe needs major change or attention (Choose which TWO you think needs to the MOST change or attention) (93 responses)



Two areas of national or state policy are that you believe needs major change or attention – Stratified by stakeholder category

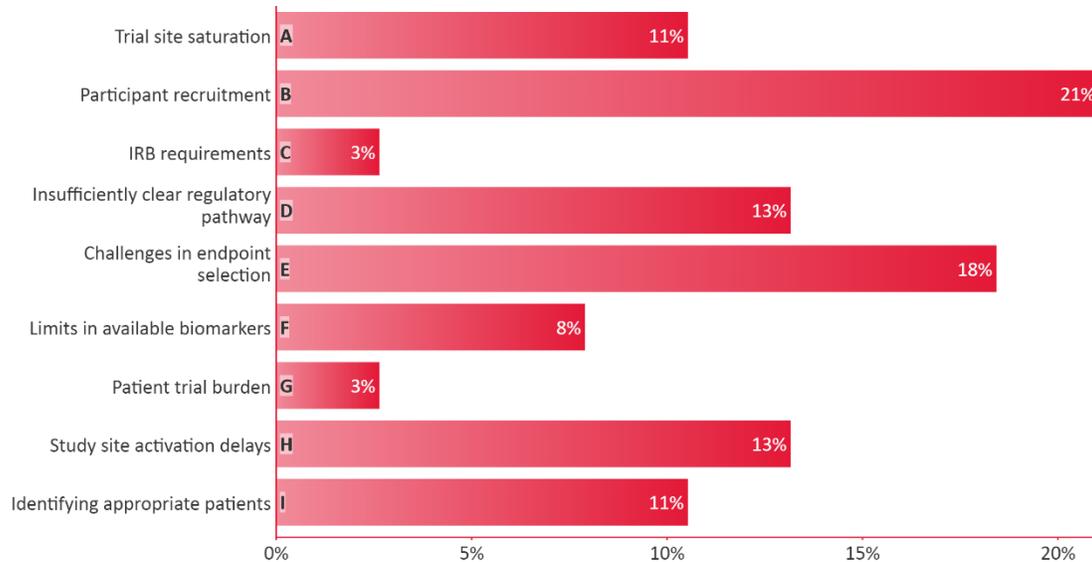


Upon reviewing the sub-analysis, we see families identifying with the full range of issues where as healthcare professionals prioritized all but two (they did not choose 'Advocating for school needs' or 'FDA review process'). We do see strong agreement with the top two issues, Navigating Medicaid/Medicaid waiver, and 'Lowering Drug Pricing.'

The final question in this session was directed toward members of industry, who we asked about the top barriers to conducting clinical trials. The largest barrier was participant recruitment (21%), followed by challenges in endpoint selection (18%). Study site activation delays and insufficiently clear regulatory pathway were each selected by 13%.



For industry only (any member) – Choose the top three barriers to conducting clinical trials in your opinion (Chose 3) (38 responses)



Conclusions

PPMD feels it is vital to engage all stakeholders across the Duchenne therapeutic, research, and care ecosystem. **At the center of this ecosystem is the patient and family.**

With the launch of the Patient Focused Drug Development initiative out of the FDA, PPMD has taken a strategic and focused approach to systematically gather patient and family input to inform all aspects of drug development and care. An important additional layer of this engagement is with key stakeholders developing therapies and administering care for people with Duchenne.

Overall, we feel engaging community members across the ecosystem at our conference through polling added an exciting new tool for gathering input in real time in order to learn from each other about how we think and feel about the current issues in Duchenne. We are eager to hear from all members of the community about what questions we should be asking in future meetings and through other methods of patient focused drug development in Duchenne.