

PPMD Delivers Patient Preference Studies for Improved Engagement

PPMD Applies CTTI's Patient Group Engagement Recommendations

SUMMARY

As part of its mission to fight Duchenne, a fatal genetic disorder that slowly robs people of their muscle strength, Parent Project Muscular Dystrophy (PPMD) wanted to integrate the patient voice across the research ecosystem for Duchenne studies. PPMD used CTTI's Patient Group Engagement (PGE) recommendations, and its "Chevron Diagram" specifically, to better engage across the full drug development continuum through patient preference research studies.

GOAL(S)

PPMD was founded by a small group of parents and grandparents of Duchenne patients who felt there was more they could do to advance Duchenne research. PPMD is very active in advocacy, but wanted to work with drug developers, clinicians and policymakers to ensure the patient voice is at the center of all research efforts.

CHALLENGES

There is a tendency across drug developers to think of the patient voice in research as either 1) something they must do per FDA guidance; 2) something that is really only valuable for recruitment efforts; or 3) something that is not very valuable at all. Those who do see the value often collect market research data privately and do not share results. This competition, says PPMD, is something that can hinder Duchenne research.

SOLUTION(S)

CTTI's [PGE recommendations](#) highlight important roles for patient groups throughout all stages of the research process. These solutions also provide best practices that sponsors, patient groups, and other stakeholders can use to ensure the relationship is mutually beneficial. One tool CTTI offers in its recommendations is its "[Chevron Diagram](#)" that shows the specific value patient groups can bring across all stages of drug development, including discovery, phase 1-3, regulatory review, and post-approval. This diagram was foundational to PPMD's efforts to more deeply embed its patients into Duchenne research.

TAKING ACTION

PPMD decided to use CTTI's chevron diagram to theoretically explore how patient preference data could improve the clinical trials process. Patient preference research reflects what treatment attributes matter to patients, how much these matter to patients and how patients make trade-offs between treatment attributes. Using an adapted version of CTTI's chevron, PPMD saw tremendous value opportunities across the research ecosystem for these insights. "What we came to realize is that patient engagement needs to expand beyond just traditional research and recruitment," said one leader at PPMD. "The next step for patient advocates is to strengthen their portfolios with patient preference studies or other scientific data that can guide decision-making at multiple study phases." For example, a patient preference study of symptom prioritization could help with protocol design, and that same study could also ask about meaningfulness of a particular benefit, like slowing of disease progression, which could bring helpful insights in regard to findings. Then, on the regulatory side, those same insights can be used to make a case for risk/benefit ratio. There are multiple uses across the drug development continuum.

IMPACT

With its adapted chevron diagram as a guide, PPMD has conducted seven patient preference studies to date. PPMD hopes that as patient groups become more sophisticated through this type of research, companies will be more amenable to their input. "Unless you have a very good first-hand knowledge of what is happening in the natural history of disease, you need these insights," said one PPMD leader. "For example, we now know the six-minute walk, while a predictor of Duchenne progression, is not terribly meaningful to patients, but it took time to get there. We can get these insights faster with patients embedded in the process."

ADVICE

From PPMD's perspective, we have the benefit of learning from trials that have failed, so we can evolve into what will work better in terms of outcomes measures. The next step is for sponsors to come together in a precompetitive way early on to tap into these insights, not on a project-by-project basis, but on a therapeutic need basis. Patient preference data should be shared -- at PPMD, all data from these studies are fully public --- to drive faster results to the community. It is also important to understand how to engage with the FDA as a patient community to best support research. FDA tells stakeholders to come early, and come often -- whether you are a sponsor or a patient group, this is good advice to which adherence is recommended. Finally, learn from those who are working in the same space. PPMD learned from the Cystic Fibrosis Foundation, the American Cancer Society, and other robust patient advocacy groups. Patients are waiting for new therapies and treatments, and that means we need to collaborate in every way possible to avoid recreating the wheel and wasting time. The work we do matters, so organizations should make every effort count.

ORGANIZATION

Parent Project Muscular Dystrophy

CONTACT

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ORGANIZATION TYPE

Patient

IMPLEMENTATION DATE

2018

TOPIC

Patient Engagement

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