

# Amplifying Patient Voices to Understand & Better Serve the MPS Community



Mucopolysaccharidoses (MPS) disorders profoundly impact the lives of those who are affected, including family, caregivers, and loved ones, making effective drug development essential. In collaboration, Worldwide Clinical Trials and the National MPS Society surveyed members of this community to learn what considerations impact their decision to participate in a clinical trial and the types of support most helpful during the clinical trial process. We hope these data will better inform how best to support the MPS community and their needs.

"Hope for a cure, less suffering"

The study was not designed with patients and caregivers; thus, it suffered from data confusion and problems with the measures and procedures

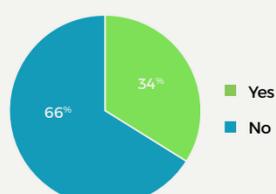
## Methods

During the National MPS Society Annual Conference, we provided an iPad with a 36-question survey to collect perspectives directly from the community regarding clinical trial participation and support. There were 48 respondents. All responses were anonymous, and we received participant consent for each respondent.

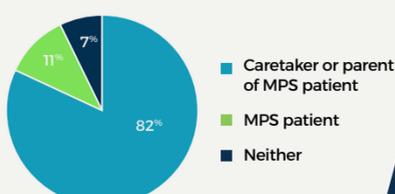
Following the conference, a link to the survey remained active and was available for two weeks following the conference. After that, the data were assembled and assessed.

## Key Takeaways

### Participated in an Interventional Trial

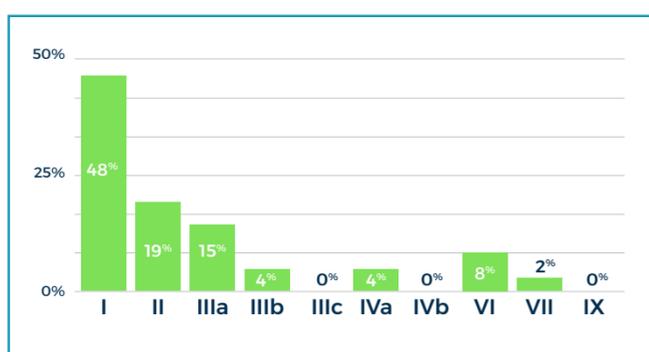


### Respondent's MPS Relationship



**Only**  
15% of those diagnosed received newborn screening

### MPS Type



While the majority of respondents classified as MPS I, a wide array of MPS types contributed.

### Types of Financial Support Needed

- Funded Transportation**
- Per-Diem Out-of-Pocket Expenses per Family Member**
- Reimbursement for Meals & Snacks**

In addition, many indicated that adequate study alerts and reminders (e.g., phone notifications, calls, or emails), as well as ample parking for participants, are important factors for consideration. Surprisingly, compensation for lost wages was not as important.

## Study Participation

### Top Routes to Clinical Trial Discovery

- 63% discovered a clinical trial through a doctor or nurse involved in their circle of care
- 31% discovered a clinical trial through the National MPS Society
- 26% discovered a clinical trial through the National MPS Society

### Top Trial Enrollment Decision-Making Factors

- Treatment complexity** compared with normal standard of care
- Doctor's recommendation** as a possible option for care
- Distance or time** required to travel to study site

### Most Important Study Requirement Factors

- Lifestyle restrictions
- Treatment complexity
- Distance or travel time

Beyond the top routes for trial discovery, respondents reported successfully finding a trial from internet searching, clinicaltrials.gov, and a lesser extent through social media. For deciding to participate in a trial participants expressed concern over participation resulting in exclusion from future investigations and safety of the novel treatment but tended to trust their doctor's recommendation. Finally, beyond the top requirements that impacted study participation, individuals indicated procedure invasiveness, likelihood of receiving an active treatment, and travel time as important considerations.

## Delving Deeper: Trial Expectations & Frustrations

What were your **expectations** from the interventional research study?

- Stabilized or slowed disease progression
- More effective treatment & access to novel therapies
- Hopeful for better quality of life
- Ongoing Monitoring

How did the study **meet or fail to meet your expectations?**

While the study is well executed and accommodating to our needs, it still presented significant challenges logistically due to the many and various visits needed."

Why did you **leave the trial?**

I had participated for several years and got to a point in my life that it was something I began to dread. I had to schedule clinical trial duties during my breaks from school/work and began to burn out. Leaving the trial was a very difficult decision for me."

## Big Picture

- Clinical trials must consider the patients and caregivers at all stages of clinical trial development, and it is impossible to design a study without working with respective patient advocacy groups.
- The survey data provided important insights into critical factors impacting trial awareness and participation, highlighting trial design considerations that create an environment conducive to successful trial participation and study outcomes to develop an effective treatment for those in need.
- Those impacted do not have time to enroll in a study that:
  - May treat participants with a placebo instead of the active therapy
  - Fails to compensate for travel and expenses
  - Uses complex or invasive methods

## National MPS Society & Worldwide Share the Mission to Defeat MPS

Like all rare diseases, MPS is best understood through the patient and caregiver lens. Worldwide Clinical Trials and the National MPS Society know it takes everyone's involvement to create meaningful solutions. We collaborate to work with, try to understand, and best serve those directly and indirectly impacted. For more information, visit the [National MPS Society](#) and [Worldwide Clinical Trials](#).