

**Parent Project Muscular Dystrophy** JOIN THE FIGHT. END DUCHENNE.



# COVID-19

**IMPACT ON CARE, TRIALS, AND  
ACCESS TO APPROVED THERAPIES**

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# INTRODUCTION

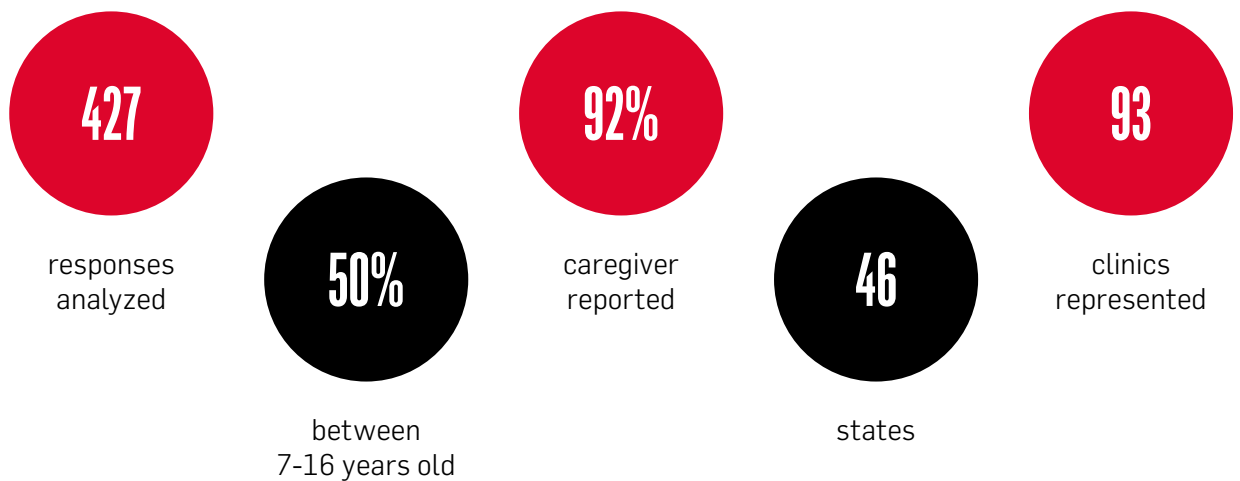
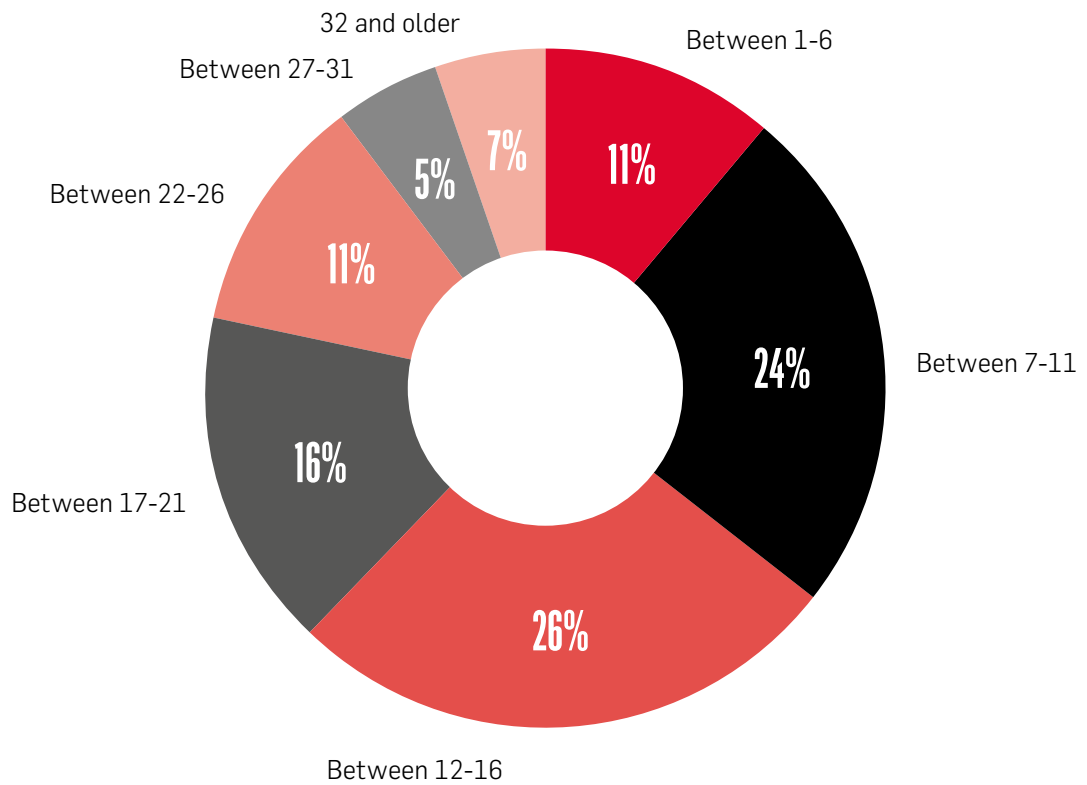
In an effort to learn about the impact of the COVID-19 pandemic on the Duchenne community, Parent Project Muscular Dystrophy (PPMD) released a survey in April 2020 to better understand the patient experience with care, trials, and access to approved therapies during the pandemic. The goals of the survey were to:

- Understand the impact on care, trials, and access
- Inform a range of stakeholders in our community on impact from the patient and caregiver perspective
- Guide PPMD on what resources to develop for the Duchenne community
- Help to plan for future pandemics (Plan B)

Data that we analyzed was collected from **April 2 through June 6, 2020.**

# SURVEY DETAILS

## Age groups

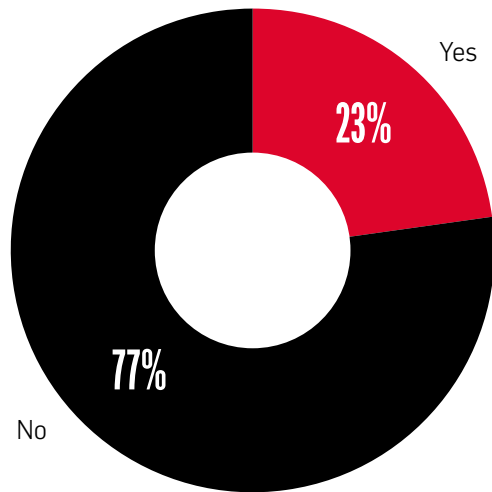


427 responses to the survey were analyzed for this report. Response numbers per questions vary depending on the number of questions answered by respondent. 50% of those who responded had a child between the ages of 7-16. 92% of respondents were caregivers. Families represented people from 46 states and 93 clinics.

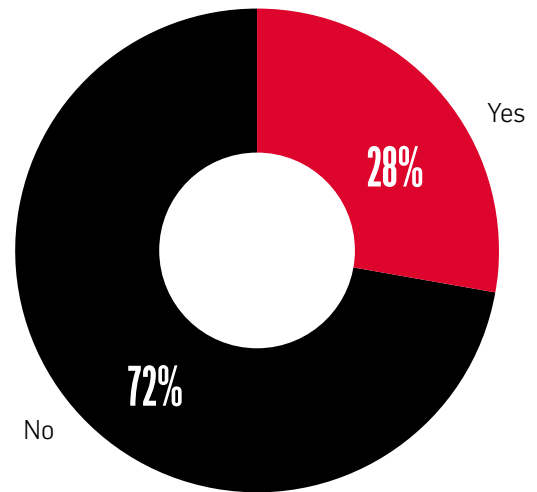
# COVID-19

**IMPACT ON CARE**

Have you cancelled or rescheduled a neuromuscular visit due to the COVID-19 pandemic?



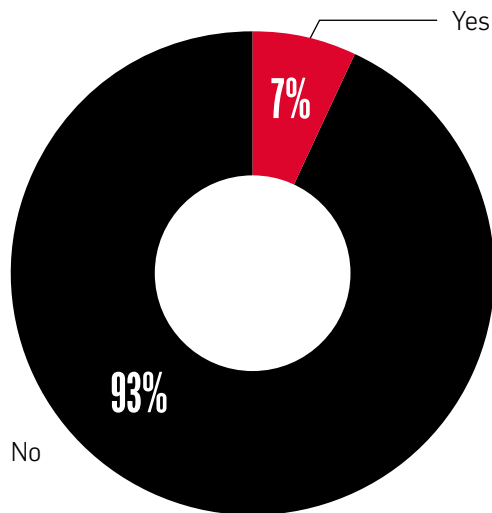
Has your clinic cancelled or rescheduled a neuromuscular visit due to the COVID-19 pandemic?



The majority of people reported that their clinic appointments were **not** cancelled or rescheduled.

**N=413**

Have you had **trouble reaching your neuromuscular clinic team** since the start of the COVID-19 pandemic?



The vast majority had no problems reaching their clinic for communication.

**N=413**

Which areas of care are you **most concerned about accessing** during the COVID-19 pandemic?

Answer	%	Count
Pulmonology care	49%	184
Cardiology	45%	171
Neuromuscular health	38%	142
Physical therapy	32%	120
Primary care	26%	100
Endocrinology	14%	53
Orthopedics	12%	46
Other (type additional area of care concern below)	15%	56

**“MY SON’S LUNG FUNCTION IS ALREADY DECLINING, WE WORRY ABOUT DELAYING APPOINTMENTS FOR TESTING AND TRACKING HOW HE IS DOING.”**

Themes from the 56 people whose chose ‘other’	
Mental Health	32%
Medical Equipment Delay	27%
Aqua Therapy	13%
Speech Therapy	9%
Home Healthcare	7%

In an open ended question about areas of care families were most concerned about accessing during the pandemic, respondents were most concerned about pulmonology care, care related to cardiology, physical therapy, and overall neuromuscular health. COVID-19 is well known to have links to pulmonary complications which may account for some of the percentage of people who chose ‘pulmonary care.’ However, we know from previous preference survey research that the community highly prioritizes treatment of symptoms related to cardiac and pulmonary care even under normal circumstances.

**N=379**

Is telehealth available at your neuromuscular center?

Answer	%	Count
Yes	35.59%	147
No	2.91%	12
Unsure	61.50%	254
Total	100%	<b>413</b>

Have you participated in a telehealth visit?

Answer	%
Yes	43.36%
No	56.64%
Total	100%

62% of respondents were unsure of whether or not telemedicine was available at their neuromuscular clinic. In response to what we learned from this question, PPMI produced a webinar on telehealth to inform community members about the process and how to make the most out of telehealth visits. [parentprojectmd.org/telehealth](https://parentprojectmd.org/telehealth)

Have appointments with your physical therapist (in home or at a facility outside of clinic) been cancelled or paused since the start of the COVID-19 pandemic?

Answer	%	Count
Yes	44.15%	181
No	10.98%	45
Not applicable	44.88%	184
Total	100%	410

**80% CANCELLED OR PAUSED PT**

Physical therapy is an extremely important aspect of rehabilitation care, starting at the time of diagnosis. In Duchenne, the goal of physical therapy is to preserve flexibility, strength, and function of your skeletal muscles. When asked about the impact of the pandemic on physical therapy, 80% of those who had appointments with their physical therapist (in home or at a facility outside of clinic) cancelled or paused appointments since the start of the COVID-19 pandemic. PPMD recently produced a series of home PT videos, we were able to release the videos during the pandemic. [parentprojectmd.org/physicaltherapy](https://parentprojectmd.org/physicaltherapy)

If you had home health services prior to the COVID-19 pandemic, have you continued to utilize home health (i.e. PCA) during the COVID-19 pandemic?

Answer	%	Count
Yes	22.14%	91
No	16.06%	66
Not applicable	61.80%	254
Total	100%	411

**FOR THOSE WHO HAVE HOME HEALTH SERVICES 58% ARE STILL UTILIZING**

What are your reasons for not using home health services?  
(open comment for **66 respondents** who chose no)

Theme	Number
Concern of COVID exposure	47
Aide not available	11
Parent taking over	4
Worried about lack of PPE	2
Insurance not covering	2
Total	66

**“WE ARE HAVING SIGNIFICANT PROBLEMS DUE TO LACK OF OUR HOME HEALTHCARE AIDE AND EXPERIENCING CAREGIVER BURNOUT”**

For those reporting they had in home health services **58%** are still utilizing those services. For who said they were not using services, unsurprisingly, most reported they were concerned about exposure to COVID-19.



Are you experiencing any of the following due to the COVID-19 pandemic?  
(Choose all that apply)

Answer	%	Count
Medical supply shortage or delay in delivery	29%	50
Appointment for wheelchair seating delays	25%	43
Equipment delivery delays	23%	41
Home modification delays	15%	27
Vehicle modification or purchase delays	13%	22
Other	23%	41

**“OUR INSURANCE IS USING COVID AS AN EXCUSE NOT TO COVER OUR ELECTRIC WHEELCHAIR SINCE ACCORDING TO THEM WE DON’T NEED TO LEAVE THE HOUSE”**

Other written in responses	
None/No/NA	28
Night splint	5
Heel chord surgery	3
Delays in GI appt	2
Home Waiver appointment	
Self directed budget preparation	
Unable to access loaner closets	

Medical supply shortage or delay in delivery, appointment for wheelchair seating delays, and equipment delivery delays were reported highest among a list of items related to items related to maintaining or improving quality of life.

**N=175**

Have you or a family member been diagnosed with COVID-19?

Answer	%	Count
Yes	1.73%	7
No	93.56%	378
Tested, but do not have results	0.25%	1
Unsure, yet suspect it is possible	4.46%	18
Total	100%	404

94% reported no one in their family had been diagnosed with COVID-19 at the time of taking the survey.

If the COVID-19 pandemic has caused financial hardship, loss of work, or other impact related to your family, please explain: (open comment)

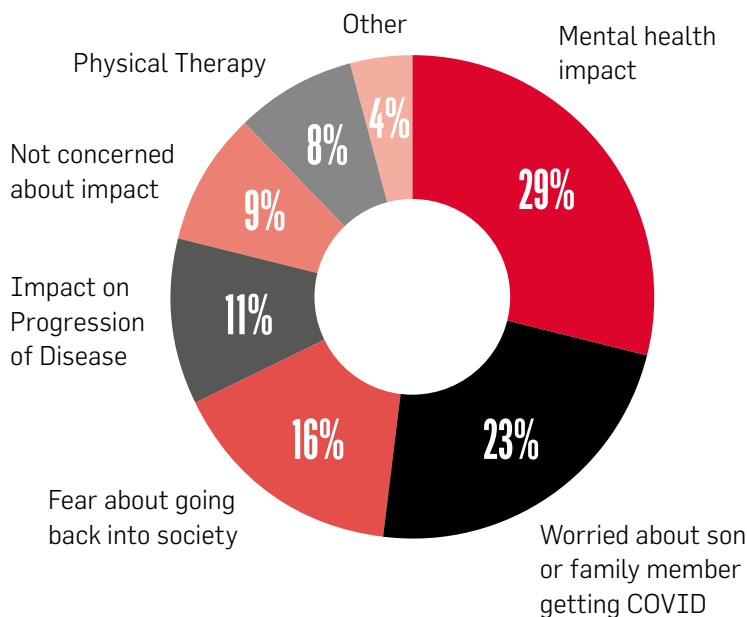
Answer	%
Temporary Furloughed	7%
Loss of Job	33%
Loss of Income/Hours	37%
Family separation	5%
Education on hold	6%
More responsibilities with care	11%
Overall stress	22%

**77% OF THOSE WHO ANSWERED THIS QUESTION REPORTED SOME KIND OF FINANCIAL IMPACT**

**19% SELF-REPORTED NO IMPACT**

**N=312**

**What else would you like us to know** about the impact on you/your child's care due to the COVID-19 pandemic? (open ended)



**'Other' themes written in**

- \_\_\_\_ Clinical Trial/Research
- \_\_\_\_ Managing in home care
- \_\_\_\_ Financial
- \_\_\_\_ Cardiac health
- \_\_\_\_ Worried about Healthcare rationing
- \_\_\_\_ Dental appts
- \_\_\_\_ Emergency Care
- \_\_\_\_ Home Healthcare (PCA)
- \_\_\_\_ Time management issues/routine
- \_\_\_\_ Traveling to appointments
- \_\_\_\_ Infusions
- \_\_\_\_ Sleep
- \_\_\_\_ Drug Shortage

In an open ended question about what else we should know about the impact of the COVID-19 pandemic on their/their child's care, the highest reported item was concern over impact on mental health (29%), followed by worry over a family member of their child getting COVID-19 (23%), and fear of going back into society (16%). In response to this data, PPMD has organized several webinars and Family Friday's about coping with stress, re-entry with school, and developed care related resources specific to COVID-19.

Webinars: [parentprojectmd.org/covid19webinars](https://parentprojectmd.org/covid19webinars)

**N=178**

# COVID-19

**IMPACT ON CLINICAL TRIALS**

The Duchenne community has a large number of clinical trials currently underway. The FDA has recently produced guidance on collecting data and running trials in the midst of a pandemic. We wanted to better understand the impact from the perspective of families participating in trials.

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Are you or your child with Duchenne or Becker currently enrolled in a clinical trial? (These questions apply only to experimental treatment trials, NOT natural history studies)

Answer	%	Count
Yes	21.08%	86
No	78.92%	322
Total	100%	408

Of those who took the survey, 21% reported they or their child were currently enrolled in a clinical trial. The vast majority were not participating in a trial.

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### For those not enrolled in a trial we then asked...

Were you attempting to enroll or in process of screening for a clinical trial in which participation was impacted by the COVID-19 pandemic?

Answer	%	Count
Yes	8.75%	28
No	91.25%	292
Total	100%	320

9% reported they were attempting to enroll but were unable to due to the pandemic.

**“COVID-19 HAS DELAYED OUR ENROLLMENT IN A CLINICAL TRIAL. IT WAS ONE THAT WE HAD TO GO THROUGH A WASHOUT PERIOD TO QUALIFY FOR. HE WAS SUPPOSED TO ENROLL IN EARLY MARCH. THE DELAY IS NOT DUE TO THE SPONSOR OR IRB, IT’S THE CLINIC’S DIRECTIVE TO DELAY UNTIL THE COVID-19 THREAT HAS LESSENER.”**

Have changes occurred to your normal schedule and protocols for your trial due to the COVID-19 pandemic?

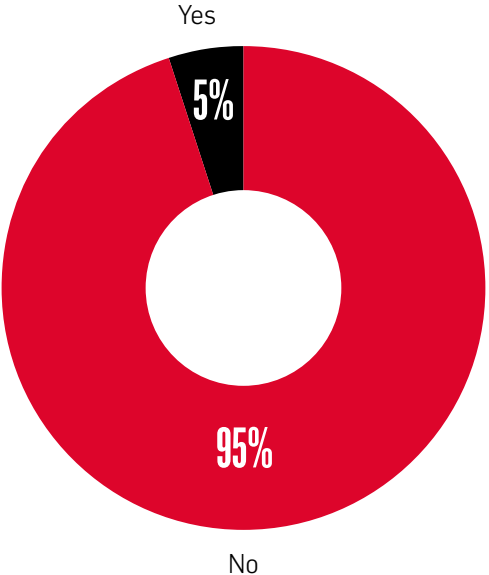
Answer	%	Count
Yes	45.68%	37
No, nothing has changed continuing as we were pre-COVID-19	54.32%	44
Total	100%	81

Additional responses
COVID-19 symptoms in family causing delay
No longer receiving the infusion due to nurses potentially having COVID-19
We are in process of switching to another site

Change Reported	%
Delayed testing by institution	65%
Telehealth	21%
Drug Shipped	16%
Switched to home infusions	16%
Chose to delay testing	16%
Blood work locally	11%
Chose to stop infusions	8%
Delayed Biopsy	8%

Of those reporting they were currently enrolled in a trial, 46% indicated changes had occurred to the normal schedule and protocols. 65% reported they had delayed testing for efficacy and that the delay was attributed to the clinic making the decision.

Have you **missed a scheduled dose** of your trial medication due to the COVID-19 pandemic?



The majority of those who reported about trials did NOT miss a scheduled dose of their trial medication. Those who missed a dose reported that it was voluntary due to concern about COVID-19.

**N=79**

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Has your trial site discussed options for home based video assessments (telehealth) or phone interviews while the COVID-19 pandemic is happening?

Answer	%	Count
Yes	29.11%	23
No	70.89%	56
Total	100%	79

Close to 30% reported they discussed options for telehealth for appointments due to COVID-19.

## What else would you like us to know about participation in a clinical trial and COVID-19?

Quotes from those who reported NO changes to their trial protocol

**“NO MAJOR CHANGES BUT WE ARE FRUSTRATED WE HAVE TO DRIVE 6 HOURS TO APPT NOW AS ADVISED BY CLINIC”**

**“DRUG FOR OUR TRIAL IS MEANT TO HELP THE LUNGS, COVID IMPACTS THE LUNGS SO CONCERNED ABOUT STOPPING IF IT MEANS IT COULD HELP HIM IN THE END (‘CATCH 22’)”**

**“FAMILIES NEED MORE SUPPORT, ANXIOUS THE TRIAL WOULD STOP AND GLAD IT’S STILL GOING”**

**“I AM STRESSED ABOUT USING OUR HOME HEALTH AIDE”**

For those who reported NO major impacts to their trial protocol during the pandemic respondents reported general frustration and stress about participating in trials during the pandemic.

Quotes from those who reported changes to their trial protocol

**“IT MADE MY 6 YEAR OLD VERY CONFUSED TO CHANGE TO THE HOME LAB. WE WERE HOPING XXX WAS GOING TO START A STUDY MY OLDER SON WOULD BE ELIGIBLE FOR IN THE SPRING AND NOW WE HAVE HAD NO NEWS. WHICH CONTRIBUTED TO HIS NEED FOR COUNSELING.”**

**“OUR SITE COORDINATOR HAS BEEN WONDERFUL”**

**“HOSPITALS/SITES NEED TO GET ON BOARD AND LEARN A NEW WAY OF DOING THINGS LIKE WE ALL HAVE HAD TO DO”**

**“IT’S HARD NOT REALLY KNOWING THE IMPACT OF COVID-19 ON THE TRIAL AS A WHOLE. OUR HOPE IS THE DRUG WILL STILL BE ON COURSE FOR APPROVAL AND NOT IMPACTED BY THE DELAY CAUSED BY THIS PANDEMIC”**

**Consistent themes**

Anxious, concerned, stressed over having to be in public for appointments and/or home nurses potentially exposing them to COVID-19

Concern about impact of COVID-19 on trial length/regulatory path

Worried about delay with moving to active drug

**“I AM CONCERNED OVER HOME NURSES COMING TO OUR HOUSE”**

For those who reported changes to their trial protocol during the pandemic respondents reported similar consistent themes related to stress and anxiety over participation during the pandemic.



# COVID-19

**IMPACT ON APPROVED THERAPIES**

Are you currently taking or trying to access one of the following therapies?  
(Choose all that apply)

Answer	%	Count
Emflaza	42.79%	178
EXONDYS-51	8.17%	34
VYONDYS-53	2.88%	12
No, I am not taking or trying to access any of these therapies	46.15%	192
Total	100%	416
Other	23%	41

When asked whether survey participants were taking or trying to get access to approved therapies, 43% reported they were on or trying to get access to Emflaza, 8% they were on or trying to get access to EXONDYS-51, and 3% they were on or trying to get access to VYONDYS-53. 46% reported not taking or trying to access any of these therapies. For those who were trying to access an FDA approved therapy, PPMD provided information on contacting SareptAssist and PTC Cares to work through the issues.

Have you **missed a scheduled dose** of EXONDYS-51 or VYONDYS-53 due to the COVID-19 pandemic?

Answer	%	Count
Yes	17.14%	6
No	82.86%	29
Total	100%	35

**MOST WHO RESPONDED HAVE NOT MISSED THEIR SCHEDULED DOSE**

**The theme reported most often as concern with access to EXONDYS/VYONDYS:**

Ensuring safety for patients and family when interacting with home infusion nurses

For those who reported taking EXONDYS 51 or VYONDYS 53, most (83%) who responded have not missed a scheduled dose. For those who missed a dose, they reported it was voluntary due to concern with interacting with home infusion nurses.

Have you **missed a scheduled dose** of Emflaza due to the COVID-19 pandemic?

Answer	%	Count
Yes	0.58%	1
No	99.42%	171
Total	100%	172

**MOST WHO RESPONDED HAVE NOT MISSED THEIR SCHEDULED DOSE**

**The theme reported most often as concern with access to Emflaza:**

Having enough drug supply on hand during pandemic

For those who reported taking Emflaza, only one person responded that they missed a scheduled dose, they also reported having to fill an emergency prescription of prednisone due to missing a dose.

## Acknowledgments

Parent Project Muscular Dystrophy (PPMD) would like to thank everyone in our community who participated in this survey.

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