

**Parent Project  
Muscular  
Dystrophy**

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END DUCHENNE.



# COVID-19

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

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# INTRODUCTION

Since the start of the COVID-19 pandemic, Parent Project Muscular Dystrophy (PPMD) has surveyed Duchenne and Becker families to better understand how the patient experience with care, clinical trials, and access to approved therapies has been affected.

## Survey aims include:

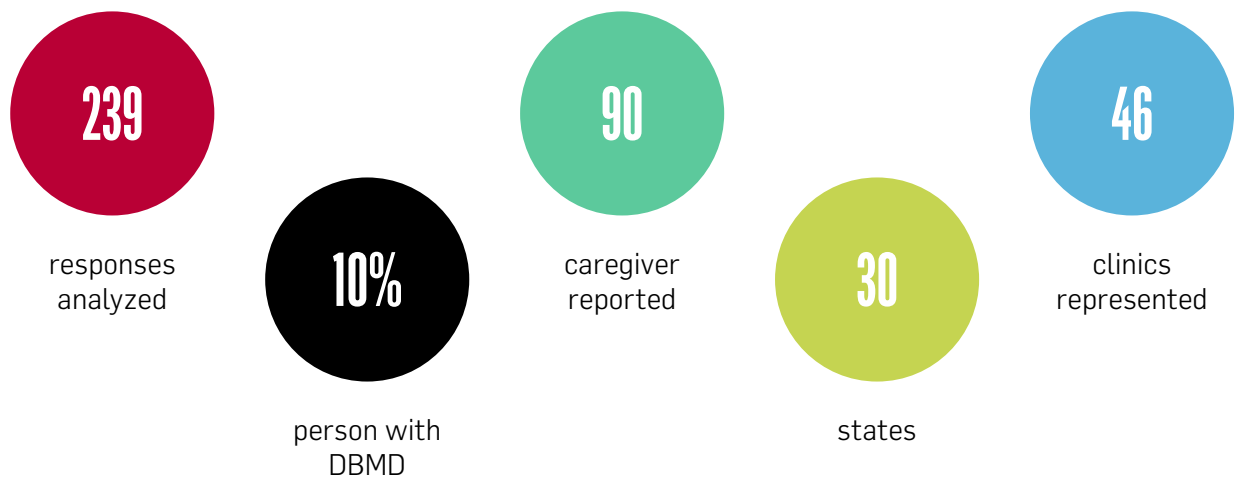
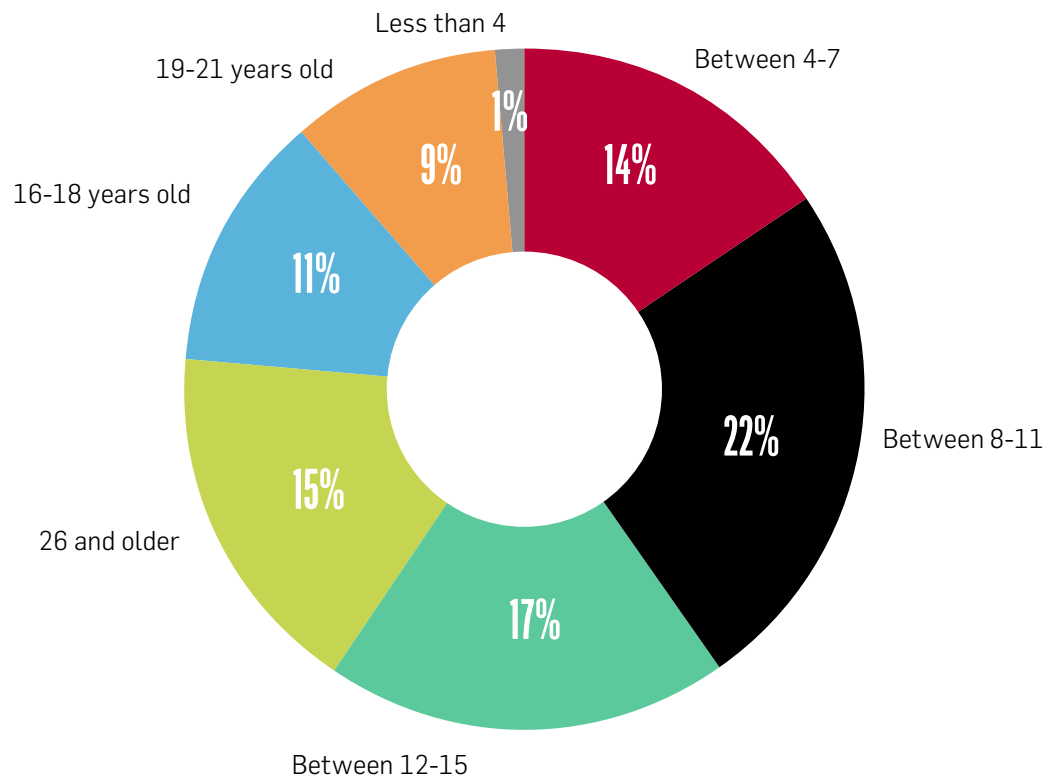
- Understanding the impact on care, trials, and access during the pandemic
- Informing a range of stakeholders in our community on impact from the patient and caregiver perspective
- Guiding PPMD on which resources to develop for the Duchenne community
- Helping to plan our community for future pandemics (Plan B)

The first survey was released in April 2020, results of that survey are reported here: <https://www.parentprojectmd.org/learnings-from-ppmds-covid-19-pandemic-survey/>

This report includes results from our second survey, with data collected from **December 2020 through February 2021**.

# SURVEY DETAILS

## Age groups

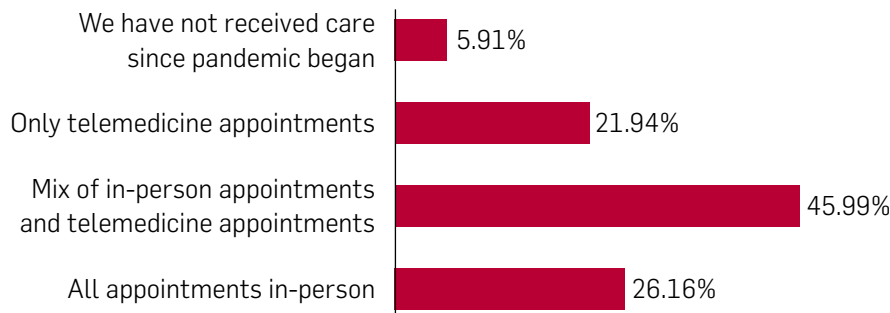


For this report, 239 responses to the survey were analyzed. Response numbers per question vary depending on the number of questions answered by the respondent. The majority of respondents were caregivers. Families represented people from 30 states and 46 clinics. Findings from this report are limited by sample size but provide a framework for future survey research.

# COVID-19

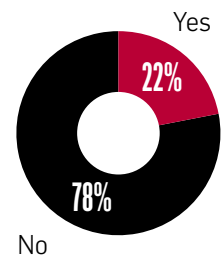
**IMPACT ON CARE**

## What has your care looked like since Spring 2020?



When asked how families were receiving care since the start of the pandemic, nearly half (46%) reported they were doing both in-person clinic visits and telemedicine appointments. 22% indicated they were doing telemedicine appointments only and 26% reported they were only doing in-person clinic visits. 6% of those surveyed indicated they had not received care since the pandemic began. In an open ended follow-on question asking why they have not received care, the majority indicated they did not have an appointment scheduled since the pandemic began.

## Were you required to take a COVID test before coming?



### Biggest challenge reported:

Only one caregiver allowed in visit at a time

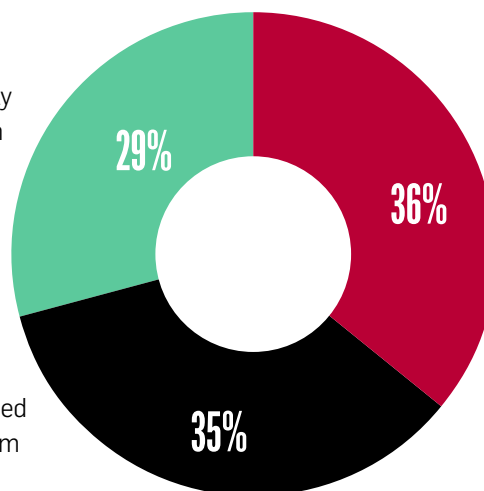
**N=237**

## Please tell us about your experiences during your most recent in-person clinic visit, including changes to clinic procedures and interactions with medical professionals.

### Most common themes reported

Precautions/protection implemented, but mostly full access to care team

Limited or shortened access to care team

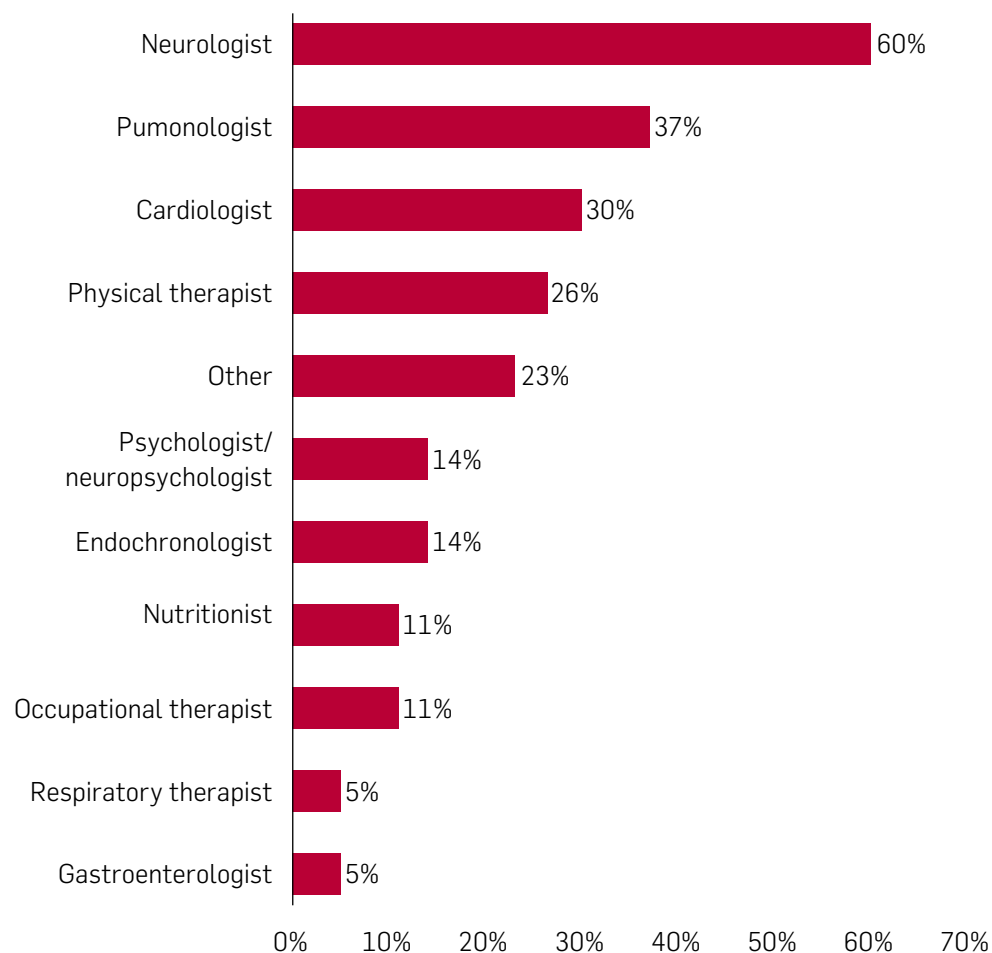


Positive experience, very little change or interruptions

For those who indicated they had gone to an in-person clinic visit, in an open ended question we asked them to tell us about their experiences at clinic, including changes to clinic procedures and interactions with medical professionals. 36% said that precautions or protections were implemented. 35% said that they were limited or that there was shortened access to patient health care. 29% had a positive experience with very little change.

**N=120**

## Types of telemedicine visits



For those who indicated they had participated in a telemedicine visit, we asked respondents to indicate which doctors they participated with through telemedicine. The highest response for for neurologists ( 60%), followed by pulmonologists (37%), cardiologists (30%) and physical therapists (26%). Lower numbers were reported for psychologists/neuropsychologists (14%) Endocrinologists (14%), OT (11%) respiratory therapists (5%), and gastroenterologists (5%).

23% of respondents reported seeing 'other' specialists not listed. The highest reported written in responses included rehabilitation doctor, primary care doctor, social worker, and palliative care doctor.

The majority of respondents (89%) reported they had no issues with insurance covering telemedicine visits.

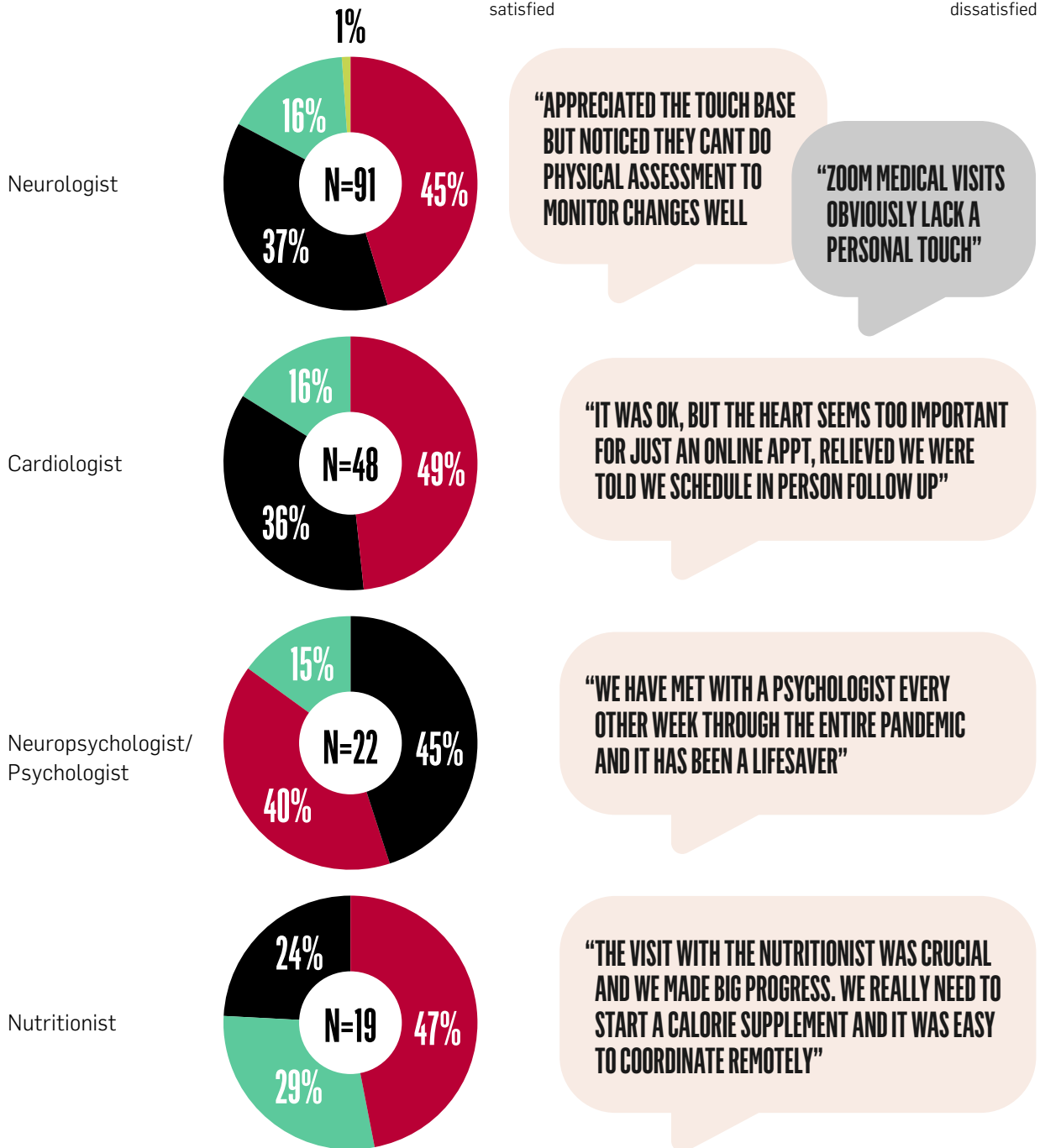
## Have you had any problems with insurance covering telemedicine visits?

Answer	%
No	89%
Yes	1%
Unsure	10%

**N=151**

## Satisfaction with care delivered

● Very satisfied   
 ● Satisfied   
 ● Neutral   
 ● Dissatisfied   
 ● Very dissatisfied



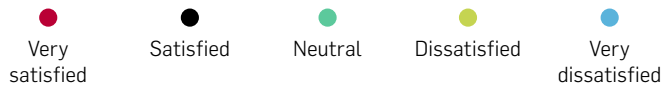
We then asked respondents to rate their satisfaction with the care delivered during the telemedicine using a likert scale of *very satisfied* on the high end to *very dissatisfied* on the low end of satisfaction.

Neurologists, cardiologists, neuropsychologists, and nutritionists received the highest scores for satisfaction. The majority of respondents (over 70% across specialists) were either satisfied or very satisfied with the care they received through their telemedicine visits.

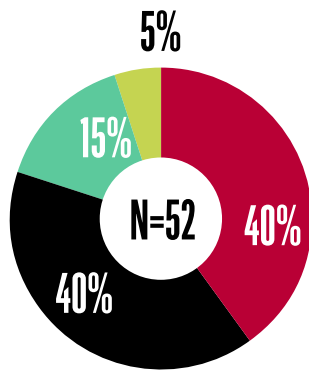
No one reported they were very dissatisfied with their care with these specialists, some reported they were neutral. Overall, most were satisfied or very satisfied with their experience with these providers.



## Satisfaction with care delivered

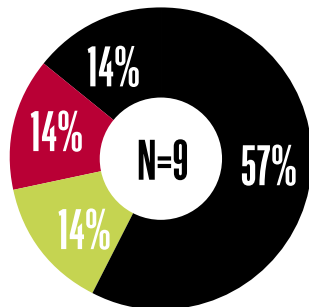


Pulmonologist



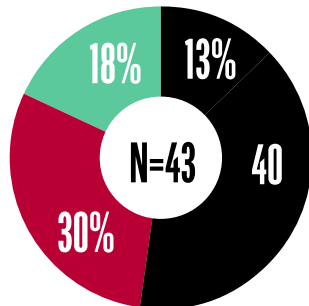
**"GOOD DISCUSSION AND INFORMATION PROVIDED ON MAKING DECISIONS REGARDING SCHOOL."**  
**"NO BREATHING TEST WERE ABLE TO BE DONE TO DETERMINE THE CHANGE IF ANY IN LUNG FUNCTION"**

Respiratory Therapist



**"WE HAVE TROUBLE SOLVING PROBLEMS WITH EQUIPMENT REMOTELY"**

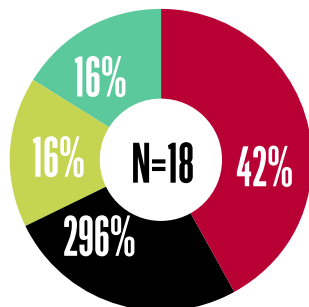
Physical Therapist



**"DIFFICULT TO MEASURE NSAA DURING VIRTUAL VISIT"**

**"DPT ZOOM VISITS DON'T WORK".**

Occupational Therapist

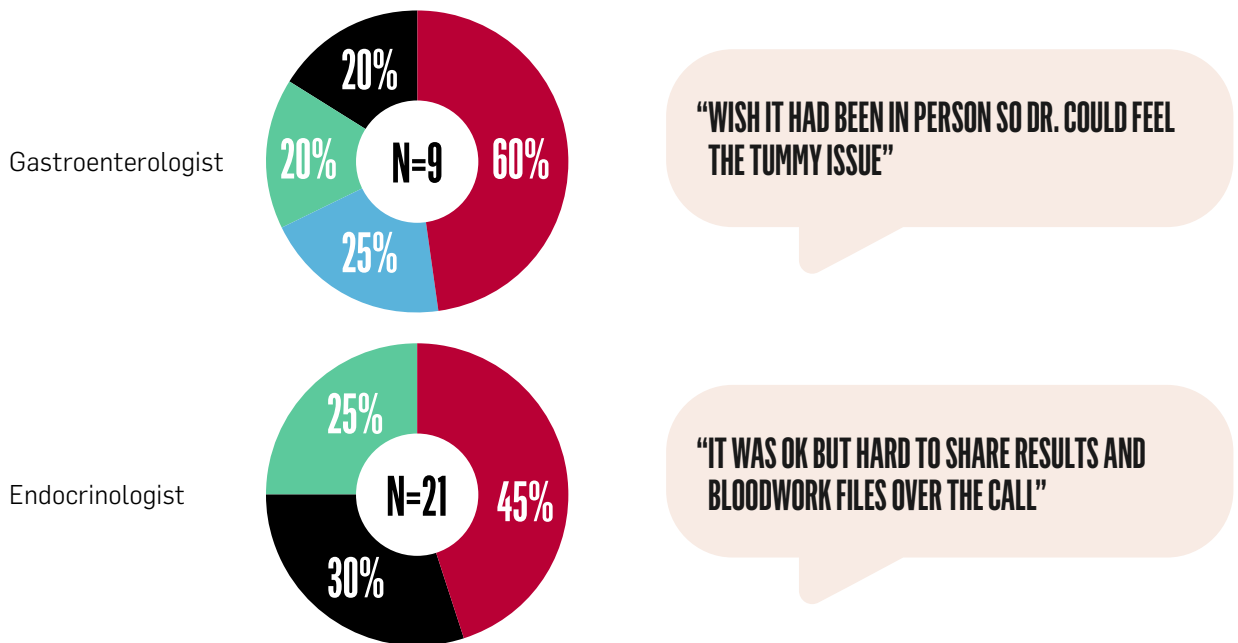
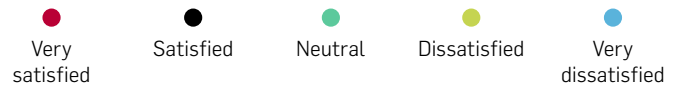


**"WE WERE ABLE TO TALK ABOUT EQUIPMENT NEEDS, BEDS, HAND SPLINTS AND EXERCISES"**

We see some dissatisfaction tick up for pulmonologists, respiratory therapists, physical therapists, and occupational therapists, however the majority still reported they were satisfied or very satisfied with their care from all but respiratory therapists. The survey only captured 9 responses reporting on respiratory therapy satisfaction so there are limitations with interpreting this data compared to other specialists.

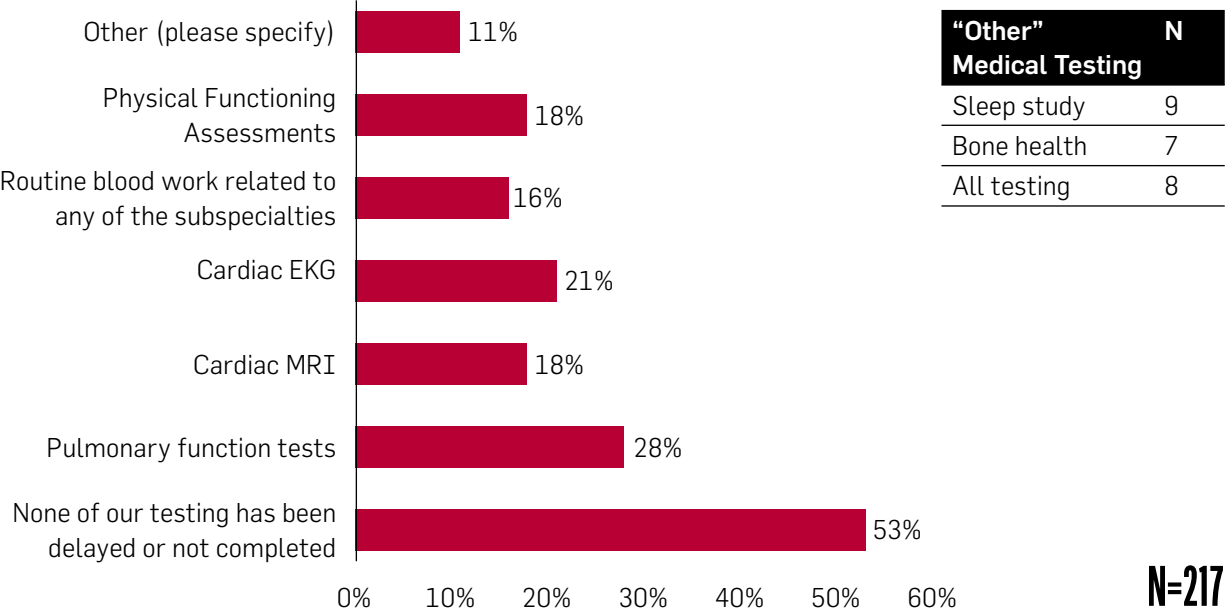
In an open ended question asking respondents "what else would you like us to know about this visit?" many comments indicated an overall positive experience, while some respondents indicated that not having the specialists in person was a negative experience and the need for in person care was important.

## Satisfaction with care delivered



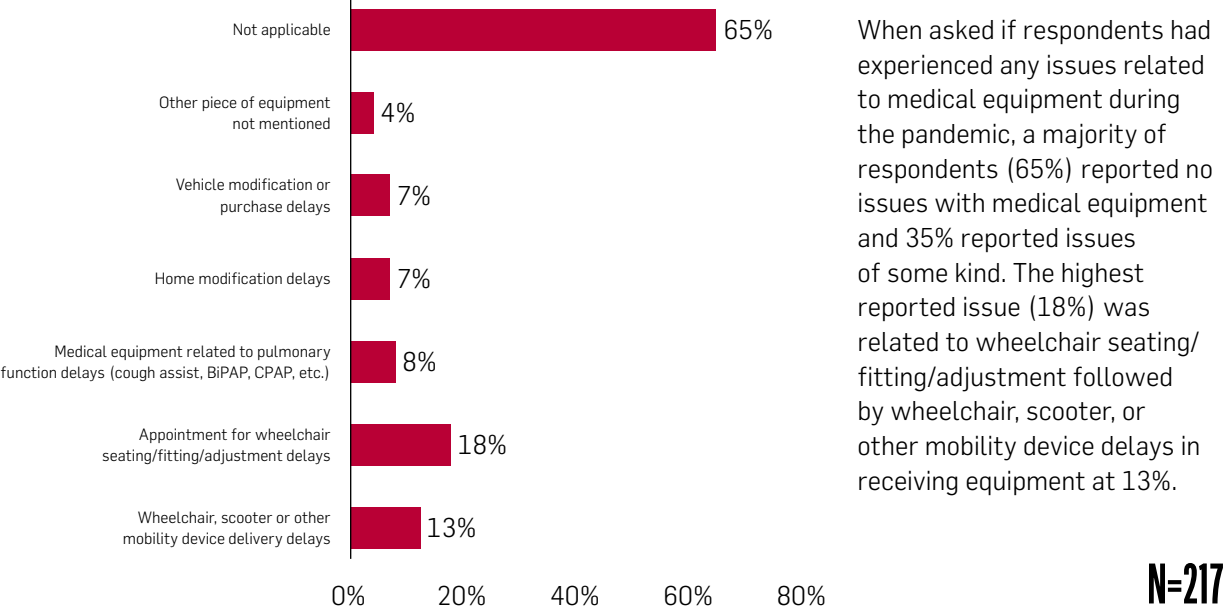
The only specialists who received a very dissatisfied score (25%) was for Gastroenterologists. However, only 9 respondents reported on their visit with their gastroenterologists and more data is needed to understand experiences with this type of care through telemedicine. Endocrinologists followed similar trends exhibited by most of the previous specialties with most very satisfied or satisfied.

Has any medical testing been interrupted since the start of the pandemic?



When asked if any medical testing had been delayed or not completed, over half (53%) reported that none of their testing had been interrupted. 28% reported their pulmonary function testing was delayed, 21% had Cardiac EKG interruptions, 18% had interruptions in cardiac MRI, bloodwork, and physical functioning assessments. 11% reported on 'other' testing not listed, of those the highest reported interruptions included sleep studies and bone health. 8 people indicated all testing was interrupted.

Have you experienced any of the following interruption related to medical equipment due to the COVID-19 pandemic? (Choose all that apply)



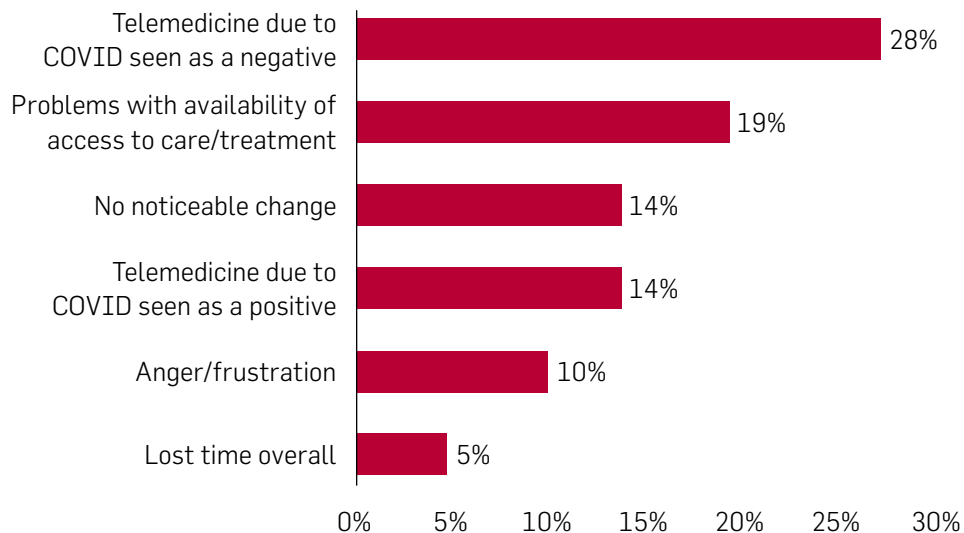
When asked if respondents had experienced any issues related to medical equipment during the pandemic, a majority of respondents (65%) reported no issues with medical equipment and 35% reported issues of some kind. The highest reported issue (18%) was related to wheelchair seating/fitting/adjustment followed by wheelchair, scooter, or other mobility device delays in receiving equipment at 13%.

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

Answer	%	Count
Yes	9%	20
No	86%	184
Tested, but do not have results	2%	4
Unsure, yet suspect it is possible	3%	6
Total	100%	214

Out of 214 respondents, 85% said they had not contracted COVID-19. 9% said they had, and a combined 4% reported uncertainty or unavailable test results

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



In an open ended question, we asked what else the respondents wanted us to know regarding the impact of the pandemic on areas of care, the following themes emerged:

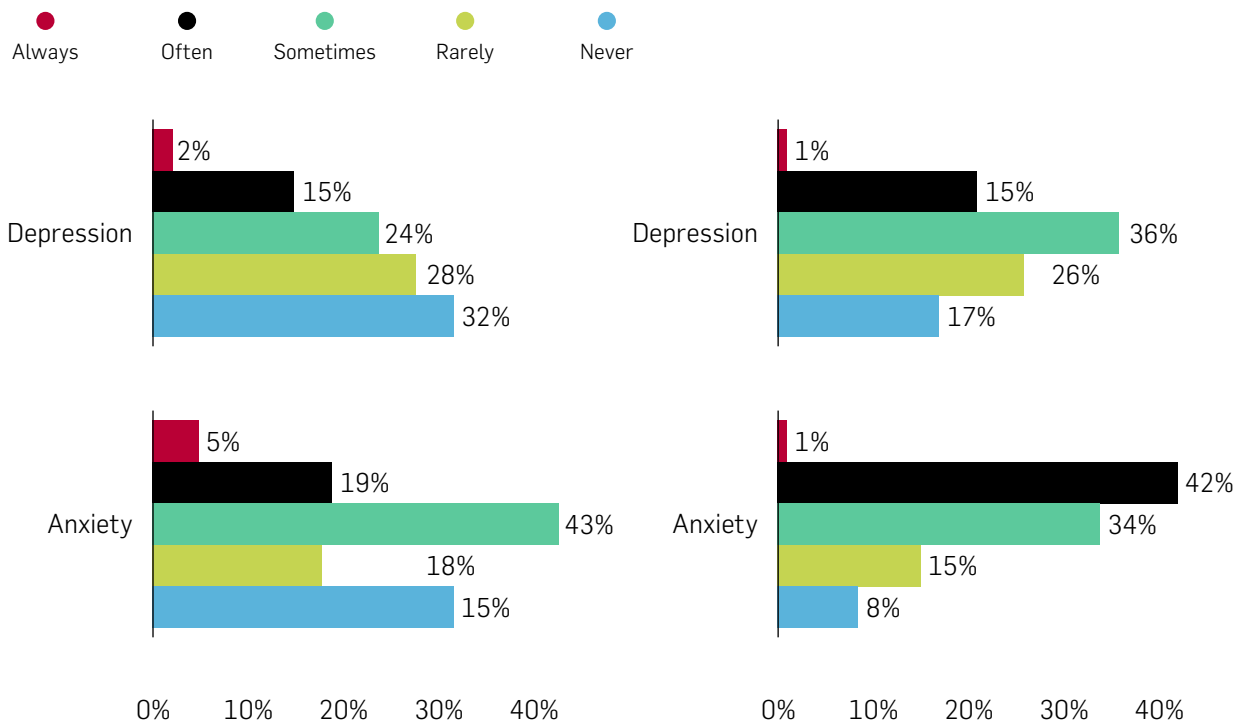
28% wanted us to know receiving care remotely was a negative experience. 19% said they had problems with access to care or treatment. 14% said no noticeable change, and telemedicine services seen as a positive experience. 10% reported anger or frustration, and 5% reported a feeling of lost time in a disease where time is so important.

**N=94**

# COVID-19

**IMPACT ON QUALITY OF LIFE**

**Mental Health** - Please indicate if the person with Duchenne or Becker in your home/care or the caregiver has experienced the following during the pandemic:



The COVID-19 pandemic has impacted everyone across country, and concerns about the impact on mental health are often cited (<https://www.cdc.gov/coronavirus/2019-ncov/daily-life-coping/stress-coping/index.html>).

In this question, we aimed to understand the impact of the pandemic on depression and anxiety for both caregivers and people with Duchenne or Becker.

**Depression:** Over half (60%) of the people with Duchenne or Becker respondents reported they rarely (28%) or never (32%) experienced depression since the pandemic began compared with over half (56%) of caregivers who reported they sometimes (36%) or often (21%) experienced depression.

**Anxiety:** When asked about anxiety, a majority (62%) of people with Duchenne or Becker respondents reported either sometime (43%) or often (19%) experiencing anxiety, with 5% reporting always experiencing anxiety. Caregivers reported even higher levels of anxiety, with 76% reporting experiencing anxiety, either sometimes (34%) or often (42%).

**N=221**

Are there other factors related to COVID-19 that exacerbate your stress? What else would you like to tell about how caring for someone with Duchenne or Becker muscular dystrophy affects your mental health during this pandemic?

Most Common Themes Reported	Count
External stressors exacerbated by pandemic (Money/life/work/school)	35%
Isolation from friends, relatives, impact on relationships	23%
Fear and anxiety of catching/spreading COVID-19	21%
Boredom, monotony	11%
Distrust In others taking proper precautions	5%



In an open ended question, we asked respondents what other factors related to COVID-19 exacerbate your stress? After reviewing the qualitative responses, the most common themes in order of most reported included: External stressors exacerbated by pandemic (Money/life/work/school) (35%), followed by Isolation from friends, relatives, impact on relationships (23%), Fear and anxiety of catching/spreading COVID-19 (21%), Boredom, monotony (11%), and Distrust In others taking proper precautions (5%).

N=132

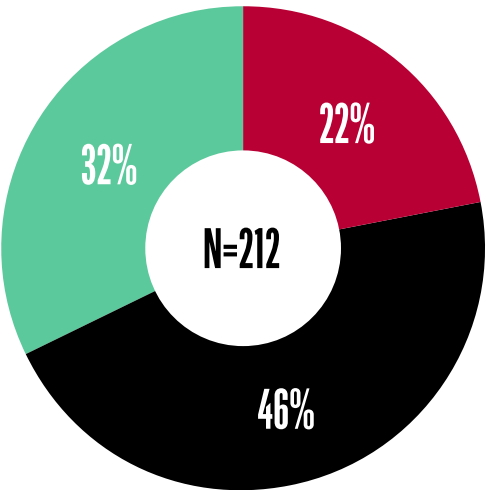
What have you chosen for you/your child's education plan for the beginning of the school year. Please indicate below:

Answer	Count
Homeschool (not associated with a physical school)	12.14%
Remote learning (associated with a physical school)	53.18%
In person learning inside the school	17.34%
Hybrid program: combination of remote learning at home and being in the school	17.34%
Distrust In others taking proper precautions	5%

When asked about their child's education plan going forward 53% responders said remote, 17% said some sort of hybrid plan or in person, and 12% said homeschool.

In your view, has the social isolation and/or inactivity of the pandemic sped up your child's disease progression?

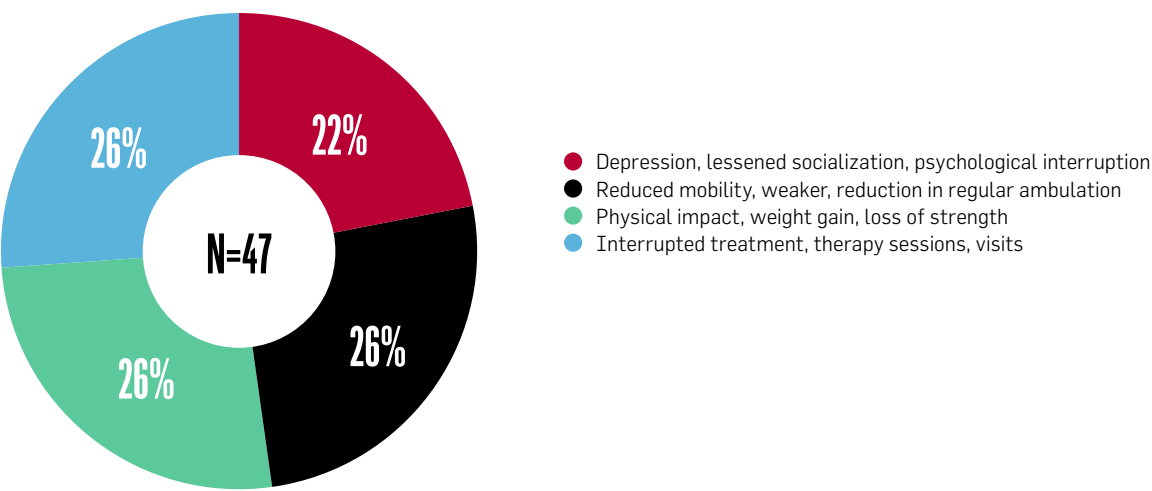
Yes No Unsure



When asked if in their view they felt that social isolation and/or inactivity has sped up their child's disease progression. 46% said no, 22% said yes and 31% were unsure.



For those who felt social isolation and/or inactivity had sped up disease progression, we asked how they felt it did. The responses were nearly equally split into four categories: Depression due to social isolation, reduced ambulation/mobility, overall physical impact such as weigh gain or loss of strength, and finally interrupted treatment.



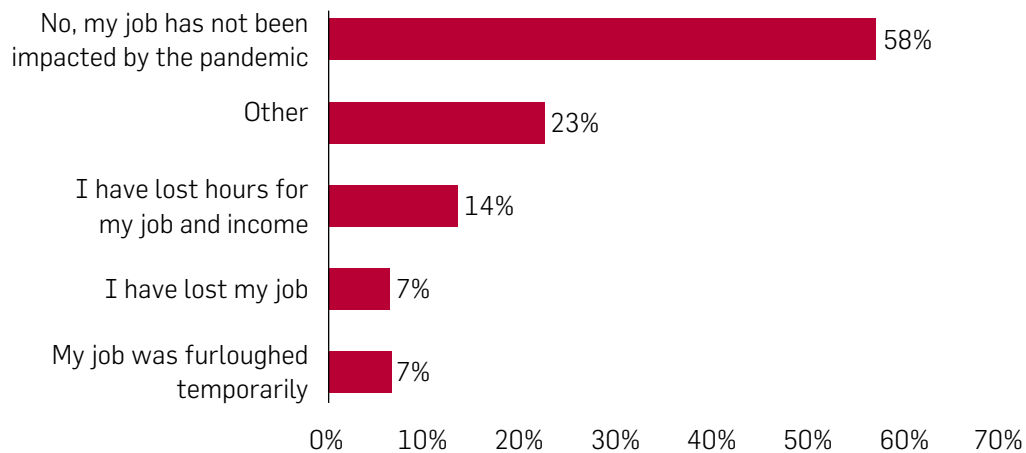
**“NOT GETTING THE ACTIVITY HE WOULD NORMALLY RECEIVE AT SCHOOL AND THE SERVICES HE RECEIVES IN SCHOOL IN TERMS OF OT AND PT HAS DEFINITELY CAUSED PROGRESSION”**

**“LOST A LOT OF RANGE OF MOTION IN ANKLES”**

**“DEPRESSION AND FAILURE TO THRIVE.”**

**“MORE CHALLENGING FOR HIM TO DEAL WITH HIGH LEVELS OF BOREDOM AND MONOTONY BECAUSE HE CAN’T GO TO UNIVERSITY CAMPUS FOR STIMULATION AND SOCIAL EXPOSURE AND HAS GAINED WEIGHT”**

If the COVID-19 pandemic has caused financial hardship related to work please indicate below (Choose all that apply):



**"MY JOB WAS FURLOUGHED FOR AWHILE."**

**"MY HUSBAND WORKS IN A RESTAURANT, HIS HOURS WERE DRASTICALLY CUT..."**

**"I FEEL LIKE WE'VE BEEN INCREDIBLY LUCKY, I WORK IN AN ADMINISTRATIVE CAPACITY AT A HOSPITAL AND THEY FEEL STRONGLY TO MAINTAIN EVERYONE'S HOURS, I AM TREMENDOUSLY GRATEFUL."**

When asked about financial hardships due to the pandemic, a majority (57%) of respondents said their job had not been impacted, 13% said they had lost hours. 7% said they lost their job, and 6.9% said their job was furloughed. 23% reported on other ways the pandemic had caused financial hardship like unexpected out of pocket expenses.

**N=?**

# COVID-19

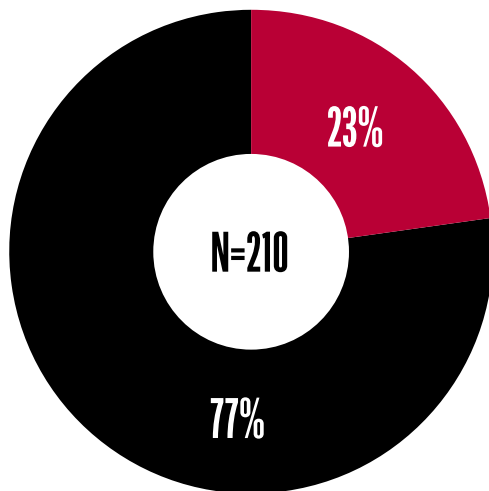
## IMPACT ON TRIALS

## Trial Participation

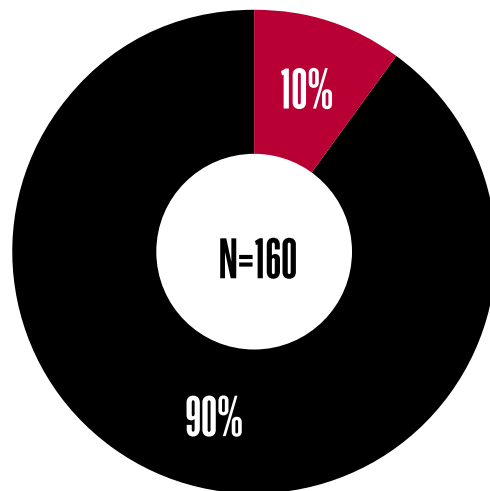
●  
Yes

●  
No

Are you or your child with Duchenne or Becker currently enrolled in a clinical trial?



Were you attempting to enroll in a trial?



When asked if them or their child was enrolled in a clinical trial, 77% said no, and 22% said yes.

Of those who reported not participating in a clinical trial, 10% reported that their trial enrollment was interrupted due to the pandemic.

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For those who reported participating in trials

**8% REPORTED MISSING A TRIAL  
MEDICATION DUE TO THE PANDEMIC**

**31% REPORTED MISSING SAFETY AND  
EFFICACY TESTING DUE TO THE PANDEMIC**

# COVID-19

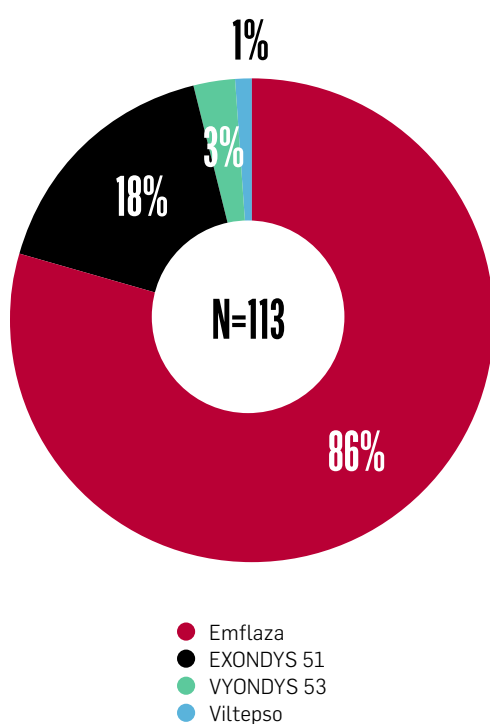
**IMPACT ON ACCESS TO FDA APPROVED MEDICATIONS**

## Are you currently taking any of the following FDA therapies?

Answer	%	Count
Emflaza	42%	90
EXONDYS 51	9%	19
VYONDYS 53	1%	3
Viltepso	.5%	1
Not on any FDA approved therapies	47%	101
Total	100%	214

We asked whether survey participants were taking any FDA approved Duchenne therapies, 42% reported they were on Emflaza, 9% were on EXONDYS-51, 1% reported they were on VYONDYS-53 and .5% reported they were on Viltepso. 47% reported they were not taking any FDA approved Duchenne therapies.

## Duchenne FDA approved therapies



Approved Therapy	Yes	No	Count
Emflaza	1%	99%	90
EXONDYS 51	0%	100%	19
VYONDYS 53	0%	100%	3
Viltepso	0%	100%	1

**THE VAST MAJORITY OF RESPONDENTS DID **NOT** MISS THEIR DOSES OF FDA APPROVED MEDICATIONS.**

When asked which FDA approved therapies they were taking, 86% of respondent's said Emflaza, 18% said EXONDYS 51 2% said VYONDYS 53 and 1% said Viltepso.

The overwhelming majority of respondents did not miss their doses of FDA approved medications.

# OVERALL ASSESSMENT

## CARE

- Most families reported attending in-person care visits during the pandemic (either only in-person visits (22%) or a mix of in-person and telemedicine (46%).
- Just over half (53%) of those surveyed reported not missing any medical testing, however 47% reported some or all medical testing missed due to the pandemic which is a cause for concern due to the need for consistent, regular monitoring of people with Duchenne.
- Overall, families reported positive experiences with telemedicine though some areas of care (physical therapy, gastrointestinal care, respiratory therapy) were not viewed as ideal for telemedicine by those reporting in this survey.

## IMPACT ON QUALITY OF LIFE

- Unsurprisingly, findings showed that the pandemic has continued to have a negative impact on overall mental health and quality of life (QOL). Strategies to mitigate overall negative impacts should be explored.
- Respondents reported that delayed medical testing and monitoring with standard of care caused high levels of anxiety for families.
- External stressors related to work, money, school, were further exacerbated by the pandemic for families already dealing with the complexities of managing life.
- 22% of respondents reported that they felt social isolation and/or inactivity during the pandemic sped up their child's disease progression in their view.
- The number of people reporting financial impact due to the pandemic in our previous survey (77%) decreased in this survey, however 42% still reported some impact related to their job or income overall which is not insignificant.

## CLINICAL TRIALS AND ACCESS TO FDA APPROVED MEDICATIONS

- There were no major issues reported with access to Duchenne FDA approved therapies.
- For those participating in clinical trials, 31% reported missing safety and efficacy assessments during the pandemic. The impact of potential loss of clinical trial data on the overall trial remains to be seen and will need to be evaluated.
- 10% reported that the pandemic had interrupted enrolling in a clinical trial.



## Acknowledgments

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

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