

Duchenne Patient-Focused Compass Meeting *Polling Data Results*

Parent JOIN THE FIGHT.
Project END DUCHENNE.
Muscular
Dystrophy

Diagnosis and Category

Describes you	Total (n=128)	Percentage
Parent/Guardian	113	88.28%
Individual with Duchenne or Becker	7	5.47%
Family member (grandparents, other relatives)	8	6.25%

Total number of responses for each polling question fluctuates (but not significantly) based on who filled the question out at the time it was asked during the meeting.

Diagnosis	10 or younger	11 through 17 years old	Adult 18 or older	Age not included	Total (n=124)	Percentage
Male with Duchenne	35	51	32	2	120	96.77%
Male with Becker	1	2	1	0	4	3.23%

How do you usually get around

How you get around	10 or younger	11 through 17 years old	Adult 18 or older	Age not included	Total (n=141)	Percentage
Use a wheelchair or other mobility device and rarely or never walk.	3	28	37	6	74	52.48%
Can get around on own but sometimes need help from a mobility device	11	19	0	6	36	25.53%
Can usually or always walk without help or mobility devices.	26	4	0	1	31	21.99%
My child is an infant/toddler and has not yet taken his/her first steps.	0	0	0	0	0	0

Research Participation

Research	Young (n=40)	Teen (n=52)	Adult (n=37)	No age indicated (n=15)	Total (n=144)	Percentage
Clinical trial for an investigational drug	14	31	20	7	72	50%
Natural history study	18	19	19	9	65	45%
MRI imaging study	5	21	7	5	38	26.39%
Have never participated in any of the above	13	10	9	4	36	25%
Clinical trial for a robotic device	0	4	2	2	8	5.56%
Clinical trial for gene or cell therapy	2	5	0	0	7	4.86%

75 percent of respondents have or are participating in some form of research, representing a very active and engaged sample set of our overall population

Polling results for those 10 years and younger:

- *Symptoms*
- *Activities of Daily Living*
- *Global health measures*

Which 3 symptoms have the biggest impact on your child's day-to-day life? (10 and younger)

Symptom	Total (n=42)	Percentage (n=42)
Fatigue/Feeling Tired	32	76.19%
Muscle Cramps/Tightness	21	50%
Behavior issues	19	45.24%
Difficulty Walking	12	28.57%
Frequent Falls	12	28.57%
Learning or speech issues	8	19.05%
Toe Walking	6	14.29%
Pain	4	9.52%
Breathing problems	2	4.76%
Heart Problems	2	4.76%

Fatigue/feeling tired reported as *most significant* symptom, and is associated with the second most chosen item of muscle cramp and tightness. We currently lack good ways to measure fatigue in Duchenne

Caregivers are frequently reporting issues about behavior. This has implications for trial enrollment but also represents an area of unmet medical need (along with anxiety which is sometimes associated with the behaviors). Furthermore, when doing a sub analysis on this cohort for the question about level of concern with behavior (slide 10), 71% reported they were either *somewhat* or *very* concerned with behavior issues

If a new treatment could help your child, which **3 activities** would be **most important** to preserve or maintain right now?
(10 and younger)

ADL	Total	Percentages
Walk for a longer time	32	74.42%
Walk up stairs	28	65.12%
Get up off the floor	26	60.47%
Dress themselves	12	27.91%
Stand for longer time	7	16.28%
Carry a backpack	6	13.95%
Stand up from sitting in chair	5	11.63%
Carry things on own	5	11.63%
Feed themselves	3	6.98%
Get in and out of bed	0	0%

The ADL's chosen (significantly) *most important to preserve or maintain* match up with outcome measures commonly used in ambulatory trials:

- 6MWT
- 4 Stair Climb
- North Star

If a new treatment could help your child, which would be most important right now? Please choose 3 of the most important things from the following list of overall health measures.
(10 and younger)

Global measure	Total (n=42)	Percentage
More Energy	32	76.19%
Stronger Heart	17	40.48%
Stronger bones	15	35.71%
Better Behavior	13	30.95%
Better mood	11	26.19%
Stronger Breathing	11	26.19%
Less Pain	10	23.81%
Keeping Healthy weight	8	19.05%
Better School work	3	7.14%
Better Sleep	1	2.38%

More energy, matching up with symptom of fatigue as *most valued*

While not making the Top 3, the items circled represent things that are still valued and all came within close percentages

Polling results for teens 11-17 years old:

- *Symptoms*
- *Activities of Daily Living*
- *Global health measures*

Which 3 symptoms have the biggest impact on your child's day-to-day life? (Teens -11-17 years old)

Symptom	Total (n=60)	Percent
Fatigue/Feeling Tired	42	70%
Difficulty Walking	31	51.67%
Learning or behavior issues	24	40%
Arms above head	20	33.33%
Unhealthy weight	19	31.67%
Pain	18	30%
Fractures	13	21.67%
Frequent Falls	6	10%
Scoliosis	3	5%
Breathing problems	2	3.33%

Similar to the younger cohort, fatigue reported as most significant (by 20 points)

While not making the Top 3, these are still significant and valued, all came within close percentages

If a new treatment could help your child, which 3 activities would be most important to preserve or maintain right now?
(Teens -11-17 years old)

ADL	Total (n=60)	Percentage
Feed himself	34	56.67%
Walk around your home	25	41.67%
Stand up from sitting in a chair	22	36.67%
Use a cell phone or controller	19	31.67%
Pick up object from the floor and stand back up	16	26.67%
Use a portable urinal	16	26.67%
Stabilize self on toilet/shower chair	15	25%
Ability to transfer himself	15	25%
Get in and out of bed	9	15%
Open a fridge door on his own	6	10%

The 11-17 age range is when a great deal of change in a patient's function occurs, loss of key milestone and transition into wheelchairs.

Many of the ADL's fall into a similar range indicating all are valued but the value may depend on where they are in progression.

If a new treatment could help your child, which would be most important right now? Please choose 3 of the most important things from the following list of overall health measures. (Teens 11-17)

Global Measures	Total (n=60)	Percent
Stronger heart	32	53.33%
Stronger breathing	29	48.33%
More energy	28	46.67%
Stronger bones	27	45%
Keeping a healthy weight	24	40%
Less pain	12	20%
Better mood	8	3.33%
Better sleep	8	3.33%
Better behavior	6	10%
Better school work	4	6.67%

Cardiac and pulmonary rate highest, coinciding with results from PPMD preference studies

Energy again near top of list.

Items in the 40''s close enough to other leading global health measures to be significant

Polling results for adults 18 and older:

- *Symptoms*
- *Activities of Daily Living*
- *Global health measures*

Which 3 symptoms have the biggest impact on you/your child's day-to-day life? (Adults 18+)

Symptom	Total (n=45)	Percentage
Problems lifting arms above head	30	66.67%
Unable to walk alone	25	55.56%
Problems using hands	19	42.22%
Breathing problems	14	31.11%
Fatigue/feeling tired	13	28.89%
Digestive Issues	10	22.22%
Fractures	6	13.33%
Unhealthy Weight	6	13.33%
Pain	5	11.11%
Scoliosis	4	8.89%

Only group where Fatigue is not ranked Highest. Otherwise, results are consistent with that we know about adult progression

Emphasizing importance of grip strength testing measure in non ambulatory trials

If a new treatment could help you/your child, which 3 activities would be most important to preserve or maintain right now?
(Adults 18+)

ADL	Total (n=45)	Percentages
Bring your hands to mouth	29	64.44%
Reposition yourself at night	24	53.33%
Use a cell phone or controller	14	31.11%
Lean forward then sit back up	12	26.67%
Bear own weight during transfer	12	26.67%
Shift position while seated	9	20%
Stabilize self on toilet/shower chair	9	20%
Use a portable urinal	8	17.78%
Lift object to read (book, tablet)	7	15.56%
Pour from a soda can	0	0

Top two significantly higher than the next 3 ADL's which are close in numbers.

ADL's associated with eating and sleeping greatly impact overall quality of life.

If a new treatment could help your child/you, which would be most important right now?
Please choose 3 of the most important things from the following list of overall health measures.
(Adults 18+)

Global measure	Total (n=45)	Percentage
Stronger heart	35	77.78%
Stronger breathing	35	77.78%
Stronger bones	24	53.33%
More energy	16	35.56%
Better sleep	10	22.22%
Improved mental health	9	20%
Keeping a healthy weight	6	13.33%
Better attention/focus	3	6.67%
Less pain	2	4.44%
Better mood	1	2.22%

Cardiac and pulmonary rate highest, coinciding with results from PPMD preference studies

Energy once again making top half of list

Behavior, Learning, Depression

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Learning

Do you have concerns about learning at school for the person with Duchenne/Becker that are more than typical than someone your/their age?

Level of concern	Young (n=35)	Teen (N=50)	Adult (n=30)	No Age Indicated (n=21)	Total (n=136)	Percentage
Somewhat concerned	25	17	8	10	60	44.12%
Not at all concerned	5	20	18	4	47	34.56%
Very Concerned	5	13	4	7	29	21.32%

65 percent either *somewhat* or *very* concerned

Behavior

Do you have concerns about behavior problems for the person with Duchenne/Becker that are more than typical than someone your/their age?

Level of Concern	young (n=42)	Teen (n=50)	Adult (n=31)	No age indicated (n=20)	Total (n=143)	Percentage
Somewhat concerned	22	18	10	13	63	44.06%%
Not at all concerned	5	17	17	1	40	27.97%%
Very Concerned	15	15	4	6	40	27.97%%

Over 70% percent either *somewhat* or *very* concerned

Depression or Anxiety

Do you have concerns about depression or anxiety for the person with Duchenne/Becker that are more than typical than someone your/their age?

Level of Concern	Young (n=36)	Teen (n=50)	Adult (n=31)	No age indicated (n=22)	Total (n=139)	Percentage
Very Concerned	11	20	14	16	61	43.88%
Somewhat concerned	16	25	11	5	57	41.01%%
Not at all concerned	9	5	6	1	21	15.11%

Almost 85% percent reported either *somewhat* or *very* concerned

Economic Burden

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Which is true for your family?

True for family	Young (n=33)	Teen n=49)	Adult (n=34)	No age indicated (n=17)	Total (n=133)	Percentage
Our family modified our existing home to be more accessible	12	36	31	13	92	69.17%
A parent or family member changed jobs to have more time for caregiving	7	20	14	10	51	38.35%
Our family moved to a more accessible home	12	22	9	7	50	37.59%
A parent or other family member stopped working to have more time for caregiving	11	20	14	2	47	35.34%
Our family moved to be closer to extended family	3	8	1	3	15	11.28%

How do you pay for your PCA?

How you pay for PCA	Young (n=33)	Teen (n=47)	Adult (n=32)	Age not indicated (n=15)	Total (n=127)	Percentage
Don't use	31	26	13	10	80	62.99%
Medicaid	1	13	11	2	27	21.26%
Self Pay	0	3	5	2	10	7.87%
State Funded	0	5	3	1	9	7.09%
Private	1	0	0	0	1	0.79%

Yearly out of pocket expenses: Caregiver Expense

Caregiver	Young (n=19)	Teen (n=37)	Adult (n=18)	Total (n=74)	Percentage
Less than \$500	12	22	10	44	59.46%
\$500-\$1000	1	3	3	7	9.46%
\$3000 - \$5000	2	3	1	6	8.11%
\$5,000 - \$7000	2	2	1	5	6.76%
\$1000 - \$3000	0	3	1	4	5.41%
\$7000 -\$10,000	1	2	0	3	4.05%
\$12,000 - \$15,000	1	1	1	3	4.05%
\$10,000 - \$12,000	0	1	1	2	2.70%
\$15,000 - \$20,000	0	0	0	0	0.00%
More than \$20,000	0	0	0	0	0.00%

Direct medical expenses
(out-patient appointment costs, out of pocket prescription payments,
supplements, hospitalizations):

Med Costs	Young (n=19)	Teen (n=37)	Adult (n=18)	Total (n=74)	Percentage
Less than \$500	6	4	5	15	20.27%
\$1000 - \$3000	3	6	4	13	17.57%
\$5,000 - \$7000	2	7	4	13	17.57%
\$500-\$1000	3	7	2	12	16.22%
\$3000 - \$5000	1	7	2	10	13.51%
\$7000 - \$10,000	1	3	1	5	6.76%
\$10,000 - \$12,000	2	3	0	5	6.76%
\$12,000 - \$15,000	1	0	0	1	1.35%
\$15,000 - \$20,000	0	0	0	0	0.00%
More than \$20,000	0	0	0	0	0.00%

Equipment, home modifications, vehicle modifications, and physical accommodations (durable medical equipment not funded by insurance, robotics, assistive technology, school & workplace accommodations not funded through other sources, out-of-pocket costs for home modifications or moving expenses to be in an accessible home, vehicle modifications for accessibility):

Ranges	Young (n=19)	Teen (n=37)	Adult (n=18)	Total (n=74)	Percentage
Less than \$500	10	5	3	18	24.32%
More than \$20,000	2	10	2	14	18.92%
\$500-\$1000	2	4	4	10	13.51%
\$1000 - \$3000	4	2	3	9	12.16%
\$5,000 - \$7000	0	6	3	9	12.16%
\$3000 - \$5000	0	4	1	5	6.76%
\$7000 -\$10,000	1	2	0	3	4.05%
\$15,000 - \$20,000	0	3	0	3	4.05%
\$12,000 - \$15,000	0	1	1	2	2.70%
\$10,000 - \$12,000	0	0	1	1	1.35%