

DuchenneConnect Clinic Care Survey and PPMD's Transforming Duchenne Care Initiative

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**Parent Project
Muscular Dystrophy**

LEADING THE FIGHT TO END DUCHENNE

Examples of PPMD's Care-Related Programs

- Expert consensus groups to identify gaps and standardize care
- Outcome data (predictors of ambulation) from DuchenneConnect Registry
- Webinar series on care topics (cardiac and bone health)
- Early identification of motor weakness/delay: childmuscleweakness.org
- Fact sheets (available) and revised care pages (coming soon)
- Mothers' needs and adaptation
- Treatment preferences and risk tolerance
- Transforming Duchenne Care Initiative

DuchenneConnect Registry

- Patient-report registry
- As of April 2013: over 2,200 completed profiles
- Recent **new** registrant numbers
 - 37 in February 2013
 - 92 in March 2013

Registry Profile Topics

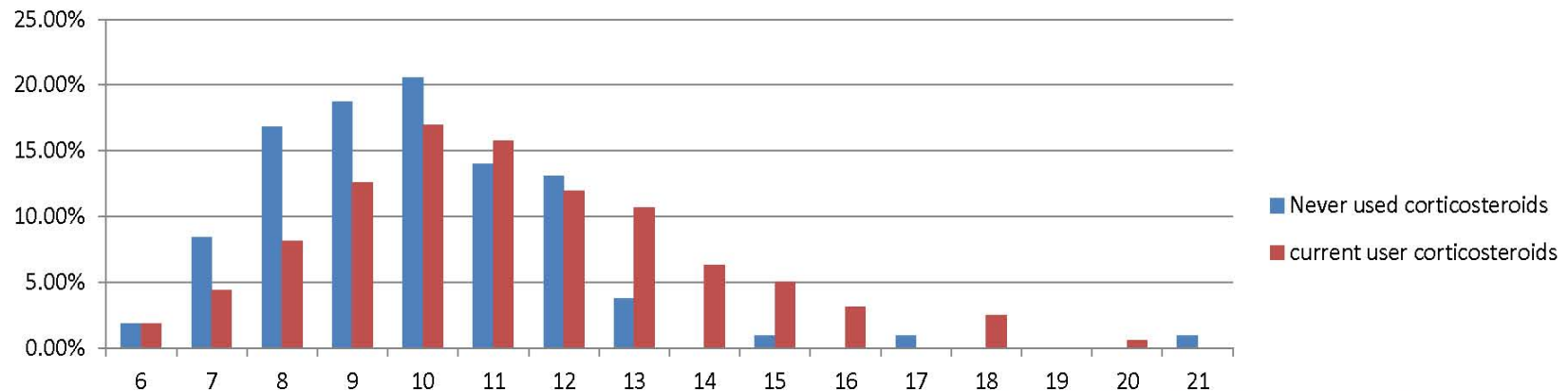
- Diagnosis and Family History
- Mobility, Walking and Sitting
- Steroids & Procedures
- Breathing
- Heart
- Back, Bone & Tendon
- Behavior & Learning
- Therapies
- Genetic Testing and Insurance
- Clinical Trials, Research & Registry Participation

DC data observe steroids effect (Stan Nelson data)

	Number Observations	Mean Wheelchair Age (years)	Sd (years)
Never Used Steroids	107	9.88	2.18
Current Steroid User	159	11.18	2.65

Never Used Steroids verses Current Steroid User
Wicoxon test P-value = **2.1e-05**

Wheelchair Bound Age with Corticosteroid Usage



DuchenneConnect Clinical Care Survey Goal

- Pilot program using Care Considerations
 - determine feasibility and uptake
 - Do benefits of care survey connected to registry (comparison to **registrant outcomes**; ability to **follow clinic data over time**) outweigh more complex platform and difficult recruitment?
- Resource to understand the services offered by clinics and empower families to improve neuromuscular care
- Provide clinics the data that they can use to improve their clinical care and trial readiness

Clinical Care Survey Data

- N=273
- 68 clinical settings reviewed in the United States and Canada
- # respondents for each clinic range from 1 (21 clinics) to 63 (largest clinic in U.S.)

Number of Years Attending Clinic

	Response Percent	Response Total
Less than one year	20%	37
1-3 years	34%	64
4-6 years	22%	41
7-9 years	12%	23
10-12 years	5%	10
13-15 years	3%	6
More than 15 years	4%	7
Total Respondents :		188

Physical Status

	Response Percent	Response Total
Presymptomatic	9%	24
Early-ambulatory	40%	108
Late-ambulatory	18%	45
Early non-ambulatory	4 %	12
Non-ambulatory I	16%	43
Non-ambulatory II	11%	29
Non-ambulatory III	3%	8
Total Respondents :		269

Dichotomized to ambulatory vs non-ambulatory

Learn about Provider

(Check all that apply)

	Response Percent	Response Total
Closest clinic	27%	79
Referral by pediatrician	18%	53
Referral by peer	16%	47
Sought expertise/reputation	20%	60
Covered by insurance	4%	13
I'm not sure	1%	3
Other	14%	42
Total Respondents :		268

Reasons for Change of Clinic

(Check all that apply)

	Response Percent	Response Total
Too far	5%	14
No insurance coverage	1 %	4
Wanted more experienced provider	16%	49
Wanted provider get along with better	4%	11
Wanted better quality of care	20%	61
Wanted easier access to the clinic	3%	8
Moved away	5%	16
Clinic or office closed	2%	7
Have not changed clinics	36%	107
Other	7%	21

Clinic that specializes in D/BMD

	Response Percent	Response Total
Yes	90%	242
No	6%	16
I don't know	4%	11

How well do providers answer your questions?

	Response Percent	Response Total
Poor	3%	9
Fair	7%	18
Good	15%	41
Very Good	25%	67
Excellent	49%	132
Total Respondents :		267

Higher satisfaction in largest clinic vs others (p<.005)
No difference based on ambulation status

Rate the medical skills

	Response Percent	Response Total
Poor	3%	9
Fair	7%	18
Good	10%	27
Very Good	23%	62
Excellent	56%	150
Total Respondents :		266

Higher satisfaction in largest clinic vs others (p<.005)
No difference based on ambulation status

Ability of clinic to meet child's long-term needs

	Response Percent	Response Total
Poor	10%	28
Fair	5%	14
Good	13%	36
Very Good	26%	69
Excellent	45%	121
Total Respondents :		268

Higher satisfaction in largest clinic vs others ($p < .005$)
Higher satisfaction for ambulatory vs non ($p = .03$)

Rate providers' knowledge of clinical trials

	Response Percent	Response Total
Poor	6%	17
Fair	9%	25
Good	14%	37
Very Good	18%	48
Excellent	42%	114
Have never asked questions about clinical trials	11%	29
Total Respondents :		270

Specialists Seen

	Response Total		Response Total
Cardiology	240	Orthopedics	131
Developmental pediatrics	25	PM&R	55
Endocrine	85	Physical therapy	222
Gastroenterology	48	Psychology/psychiatry	30
Genetic counseling	135	Pulmonology	184
Neurocognitive	36	Speech/language therapy	41
Neurology	215	Social work	79
Nutrition	146	Surgery	62
Occupational therapy	131	No other medical specialists	4
		Other	15
Total Respondents: 269			

Specialists Desired

	Response Total		Response Total
Cardiology	9	Orthopedics	6
Developmental pediatrics	13	PM&R	7
Endocrine	10	Physical therapy	6
Gastroenterology	11	Psychology/psychiatry	24
Genetic counseling	17	Pulmonology	9
Neurocognitive	2	Speech/language therapy	9
Neurology	14	Social work	8
Nutrition	8	Surgery	6
Occupational therapy	9	None	135
		Other	9
Total Respondents: 193			

Genetic Testing

	Response Percent	Response Total
My child had genetic testing	90%	240
Provider has not talked about genetic testing for my child	2%	5
I'm not sure	6%	15
Other	2%	6
Total Respondents :		266

Use of Corticosteroids

	Response Percent	Response Total
Yes, currently takes steroids (prednisone or deflazacort)	72%	196
No, does not take steroids now, but used steroids in the past	8%	22
No, has never used steroids	20%	54
I'm not sure	0.5%	1
Total Respondents :		273

Reason for No Steroid Use

	Response Percent	Response Total
Too expensive	--	0
Not available	--	0
Provider has not talked about steroids	20%	13
Provider said should not use steroids	6%	4
We didn't want to	31%	20
Other reasons	42%	27
Total Respondents :		64

At what age did your child start using steroids?

	Response Percent	Response Total
Less than 2 years old	--	0
2	1%	2
3	11%	21
4	16%	29
5	20%	37
6	19 %	36
7	12%	23
8	9%	16
9	6%	12
10	3%	5
11 or older	3%	5
Total Respondents :		186

Immunization Recommendations (steroid users)

	Response Percent	Response Total
Recommended regular immunizations	74%	144
Did not recommend regular immunizations	7%	13
I'm not sure	19%	38
Total Respondents :		195

Evaluate bone problems (steroid users)

	Response Percent	Response Total
Regularly checks bone health	63%	121
Does not regularly check bone health, but has in the past	11%	22
Chose not to have bone health checked, though offered	2%	3
Never checked for bone problems	18%	35
I'm not sure	6%	11
Total Respondents :		192

Use of Physical Therapists

	Response Percent	Response Total
Currently sees a PT	63%	170
Does not currently see a PT but has seen a PT in the past	30%	82
Chose not to see a PT, though it was offered	1%	3
Provider has not talked about child seeing a PT	3%	7
Provider said child does not need to see a PT	2%	6
I'm not sure	0.74%	2
Total Respondents :		270

Cardiac screening (echo)

	Response Percent	Response Total
At least once every 6 months	22%	59
At least once every year	56%	149
Less than once per year	16%	44
Never had an echocardiogram	4%	10
I'm not sure	2%	5
	Total Respondents :	267

No significant difference at largest clinic
Higher in non-ambulatory ($p < .005$)

Lung function test (spirometry or FVC)

	Response Percent	Response Total
At least once every 6 months	26%	70
At least once every year	39%	104
Less than once per year	8%	22
Done regularly in the past; child uses ventilator	2%	6
Never had	22%	60
I'm not sure	2%	6
Total Respondents :		268

No difference in largest clinic
 Significantly more often in non-ambulatory
 (p<.005)

Psychological care, emotional support available to family

	Response Percent	Response Total
Whole family	24%	63
Child and siblings	1%	2
Child only	6%	15
Chose not, though offered	10%	26
Provider did not talk about psychological care/support	32%	86
Provider said child does not need	2%	4
I'm not sure	27%	71

Rate care against minimum level of recommended care

	Response Percent	Response Total	
Way below		6%	15
A little below		12%	32
Exactly		19%	51
A little better		18%	49
Way better		41%	109
I don't know what is recommended		4%	12
Total Respondents :			268

Higher rating at largest clinic ($p < .005$)

Higher rating in ambulatory vs non ($p < .005$)

Overall quality rating

	Response Percent	Response Total
Poor	4%	11
Fair	7%	18
Good	17%	45
Very Good	24%	64
Excellent	48%	129
Total Respondents :		267

Higher rating at largest clinic ($p < .005$)

No difference ambulatory vs non

Limitations

- How do you measure “good care?”
 - Care Considerations guidelines are not easily evaluated
 - Survey is long and burdensome for parents
 - Have to incentivize clinics
 - Limited evidence-based data on relationship to clinical outcomes
- Is a patient registry an appropriate home?
- Practical and financial limitations
- Requires clinician “buy-in” for ultimate success
- Addressing many of these limitations through the TDCI

Connecting to Care in the US

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Vice President, Clinical Care

Parent Project
Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE

Muscular Dystrophy Community Assistance Research and Education Act MD CARE Act

- Through the efforts of PPMD (and others)
- Authorized in 2001 (up for reauthorization 2013)
- Allocated \$\$ allowing the CDC to develop the Care Considerations (published in 2009)
- Priorities that would standardize health care and improve health outcomes in DMD
- Sub-specialty specific

Care Considerations (CC)

- Best available expert opinions of the time
- Issues:
 - No methodology for evaluating whether providers were providing care in agreement with the CC
 - Now slightly dated
 - New advances in cardiac management
 - New advances in pulmonary management
 - Novel therapies on the horizon
 - Gaps in knowledge
 - Did not address GI, GU, oral health, bone health, endocrine, adult care, etc

Transforming Duchenne Care

- First meeting June 2012
- 57 attendees from 17 institution, parents and others
- To address discrepancies in clinical care across the United States for individuals living with Duchenne

Findings from TDC Meeting

- Parents and providers are dissatisfied with disparities in the care provided to DBMD patients across North America
- There is a lack of transparency regarding the clinical services and clinical care available to DBMD patients in clinics across North America
- Industry is having difficulty conducting clinical trials/research due to discrepancies in care provided

Resulted in the TDC Initiative

Result: Transforming Duchenne Care Initiative (TDCI)

- Objectives:
 - We will work to **develop methodology of external evaluation** of care that will assure families that neuromuscular providers are providing healthcare in adherence with the best and most current guidelines available
 - **Patient outcome data will be collected** in an effort to inform quality improvement efforts

Objectives (cont'd)

- Clinics will be encouraged to participate in **continuing quality improvement efforts** in order to continue to improve clinical care
- **Provide data to patients and families** informing them as to whether neuromuscular providers are providing healthcare in adherence with the CDC Care Considerations and/or other more current guidelines.

TDCI – 2 phases

- Phase I: Transparency of Clinical Services
- Phase II: Transparency of Clinical Care

Phase I: Transparency of Clinical Services

- Enhanced MDA Clinical Pages
- How and where to communicate information to the community re: non-MDA supported clinics/providers

Phase II: Transparency of Clinical Care

- 6 sub-specialties identified as important for optimal care
 - cardiology
 - pulmonary
 - endocrine/bone health
 - neurology/steroids
 - PT/PM&R
 - coordination/communication

Phase II: Transparency of Clinical Care

- TDCI Core Team meeting
 - identify 2-3 attributes for each sub-specialty that would indicate optimal care and can be used as metrics by which to evaluate care
- Methodology for evaluating clinical care

Many Challenges

- Identify and address issues as they emerge
- Keep moving forward