



OMS *life* Foundation

OMS Patient Registry & Natural History Study

OMSLife Foundation Caregivers Conference

September 29, 2018

The OMSLife Foundation - Who we are

Founded in 2012

Our mission is

- Raise awareness of OMS
- Provide a support network for patients and caregivers
- Fund OMS research

We now work with a network of 1,025 OMS patients in 56 countries

We have hosted seven OMS caregivers conferences since 2014

We co-host the OMS Medical conference in Abingdon England

And now, we are building the first OMS Patient Reported Natural History Study

Before we discuss our Registry – A special thank you...

Our doctors / presenters:

Dr. Mark Gorman

Kitty Petty

Dr. Angela Waanders

Dr. Wendy Mitchell

Dr. Yasmin Khakoo

Sue Rossov

Brian Cisneros

Our Board of Directors:

Christy Dennis

Hank Holley

Becky Jennings

Bill Bradley

Shandi Riccioti

Billy Enochs

Anja Norman

Methodology – The OMS Patient Registry

- The OMS Patient Reported Registry is a [natural history study over time](#) chronicling changes in treatments, therapies, behaviors, relapses, education, and overall quality of life.
- Registry funding is supported by a [5 year](#) cooperative agreement between [National Organization of Rare Disorders \(NORD\)](#) and the [U.S. Food and Drug Administration \(FDA\)](#).
- Data input is through an online portal [housed and maintained by NORD](#). The portal allows customized data prompts and questions.
- Data are stored in a manner that meet all [US government mandated standards for data security and PHI protection](#).
- The data collection forms, processes, and outputs were [reviewed and approved by Hummingbird IRB](#).
- Data collection initiated Feb 2017. Data views provided in this presentation were as of [April 2018](#).

Our Survey Process - The OMS Patient Registry

- We had 2 teams assist in the survey generation:
 - Development team – 4 OMS caregivers/stakeholders
 - Medical team – Mitchell, Lotze, Gorman, Tardieu, Berg
- Surveys were created by development team / modified by medical team
- Surveys and reports are sent for IRB approval
- Programmed by NORD
- Then launched
- Analytics for this presentation were done with assistance from Trio Health

What the Registry means to you

OMS Specialists

- Provides view on larger sample sets than they can otherwise access
- Allows opportunities to have surveys developed for trends they are seeing
- Allows quick turnaround on patient data

Clinicians

- Provides reference materials on a variety of OMS topics
- Give alternative options when typical protocols are not working

Researchers

- Provide secondary mechanism to validate findings
 - Research
 - Other OMS registries
- Provide a base of potential patients for clinical studies

Patients and Caregivers

- Provide resource information for discussions with clinicians
- Provide data for fighting insurance claims
- Give realistic view into patient outcomes

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Patients and Caregivers

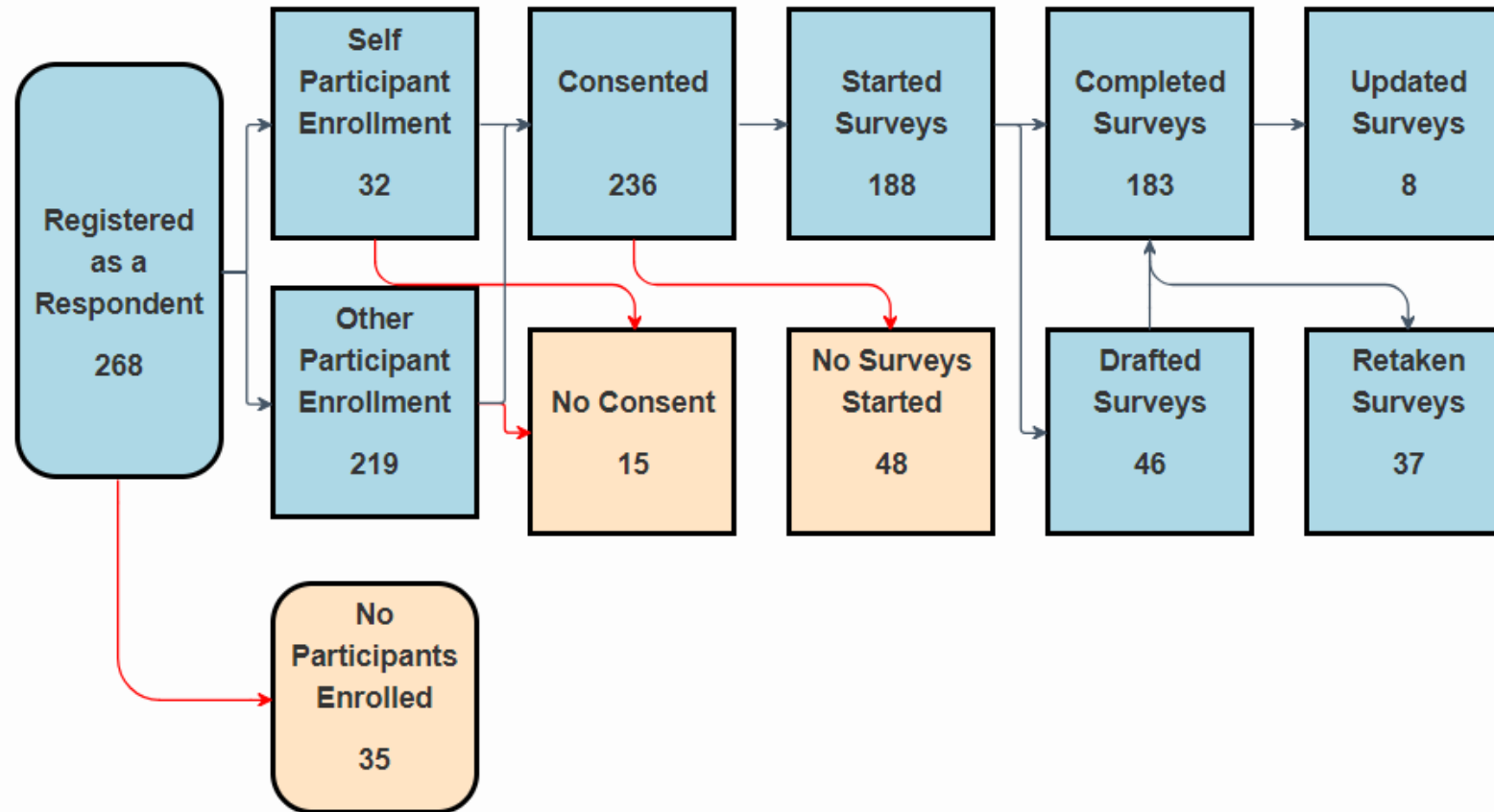
- Provide resource information for discussions with clinicians
- Provide data for fighting insurance claims
- Give realistic view into patient outcomes

Numbers have increased since February 2017 launch

User Workflow

Note: All values refer to the number of respondents (rounded boxes) and participants (square boxes). For example, the "Completed Surveys" box shows the number of participants who have completed surveys.

Click on each box for further details.



OMS Natural History Study Forms and Fields

148 respondents across 4 primary forms as of April 2018..

Demographics (n=139)

- Age
- Race
- Ethnicity
- Nationality
- Gender
- Sexual Preference
- Occupation
- Family
 - Income
 - Size
 - Education
- Insurance Type

Onset & Diagnosis (n=123)

- Age at time
- Dates
- Symptoms
- Diagnosis
 - How
 - Who
 - Where
- Severity

Drug Treatments (n=104)

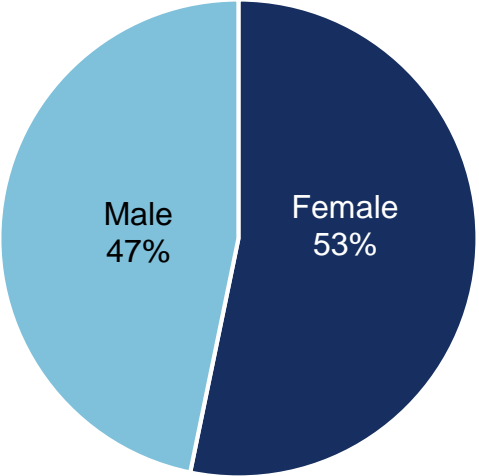
- Type
 - IVIG
 - Chemo/Immuno
 - Steroids
 - Other
- Route
- Frequency
- Duration
- Side effects

Therapies (n=97)

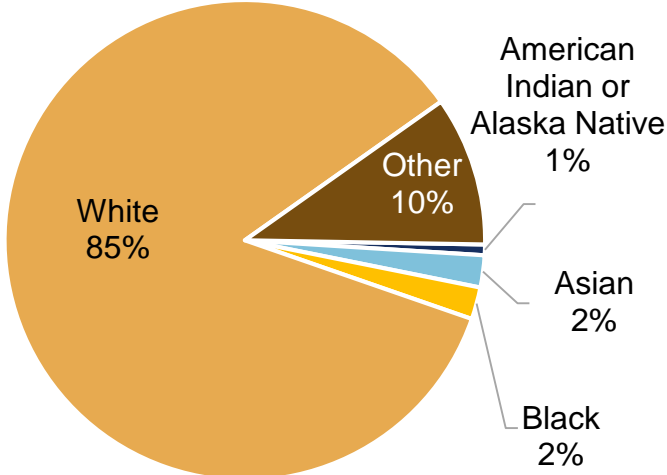
- Type
 - Speech
 - Physical
 - Occupational
 - Behavioral
- Reason for therapy
- Start/Stop Dates
- Frequency
- Effectiveness
- Care Agencies

Patient Demographics (n=139)

Gender



Race



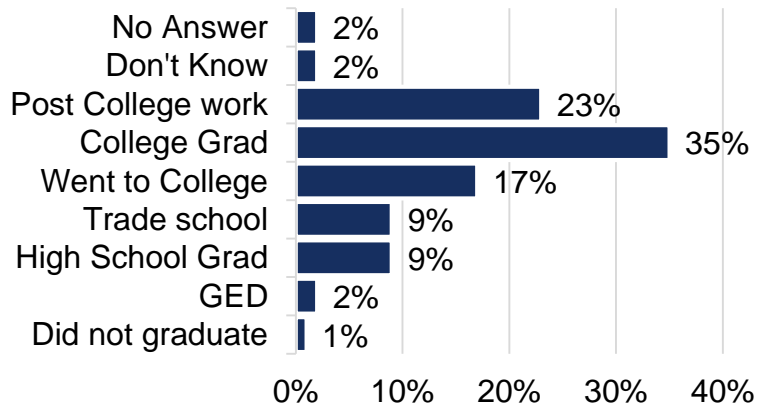
Birth Country

No. (%)

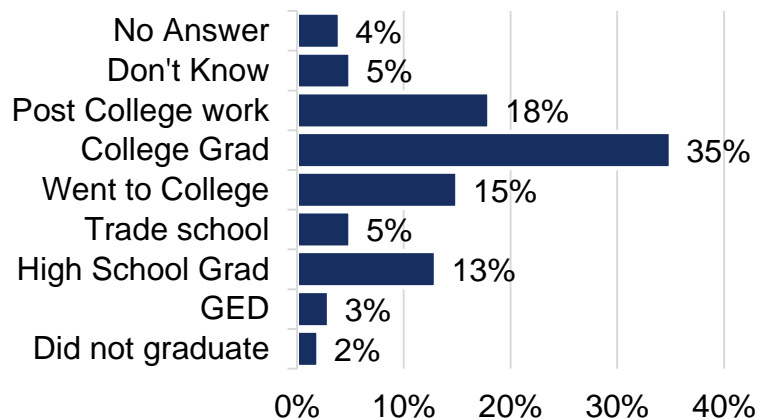
Australia	2/139 (2%)
Brazil	1/139 (1%)
Canada	5/139 (3%)
Germany	5/139 (3%)
Ireland	1/139 (1%)
Netherlands	1/139 (1%)
Paraguay	1/139 (1%)
Spain	1/139 (1%)
Sweden	1/139 (1%)
United Kingdom	3/139 (2%)
United States	118/139 (84%)

Patient Demographics (n=139)

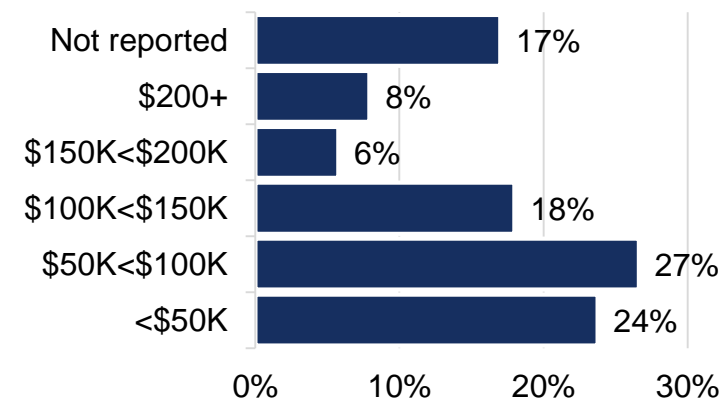
Mother's Education



Father's Education



Household Annual Income

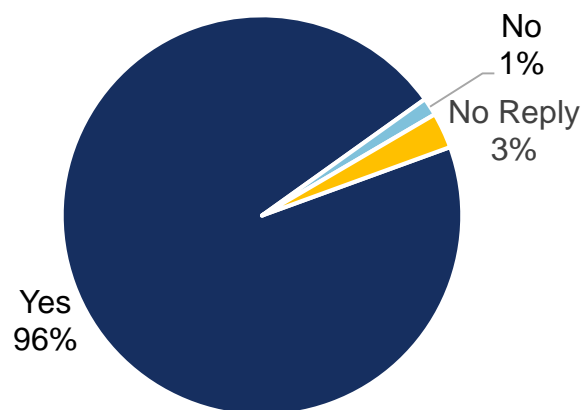


Family Size

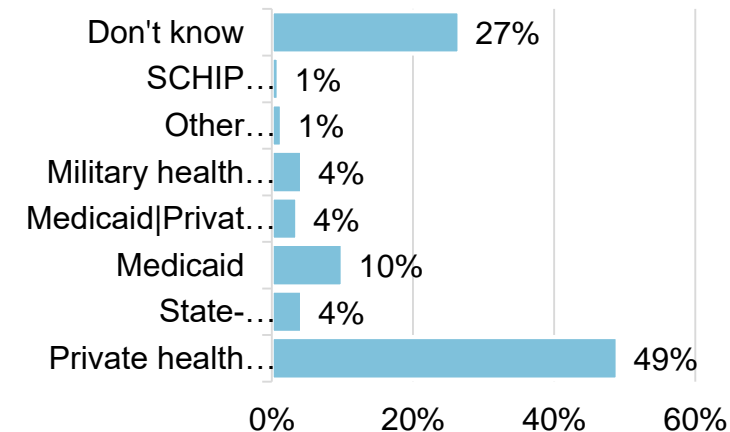
No. (%)

2 family members	4/139 (3%)
3 family members	31/139 (22%)
4 family members	48/139 (36%)
5 family members	35/139 (25%)
6 family members	7/119 (5%)
7 or more family members	8/119 (5%)
Not Reported	6/139 (4%)

Health Insurance

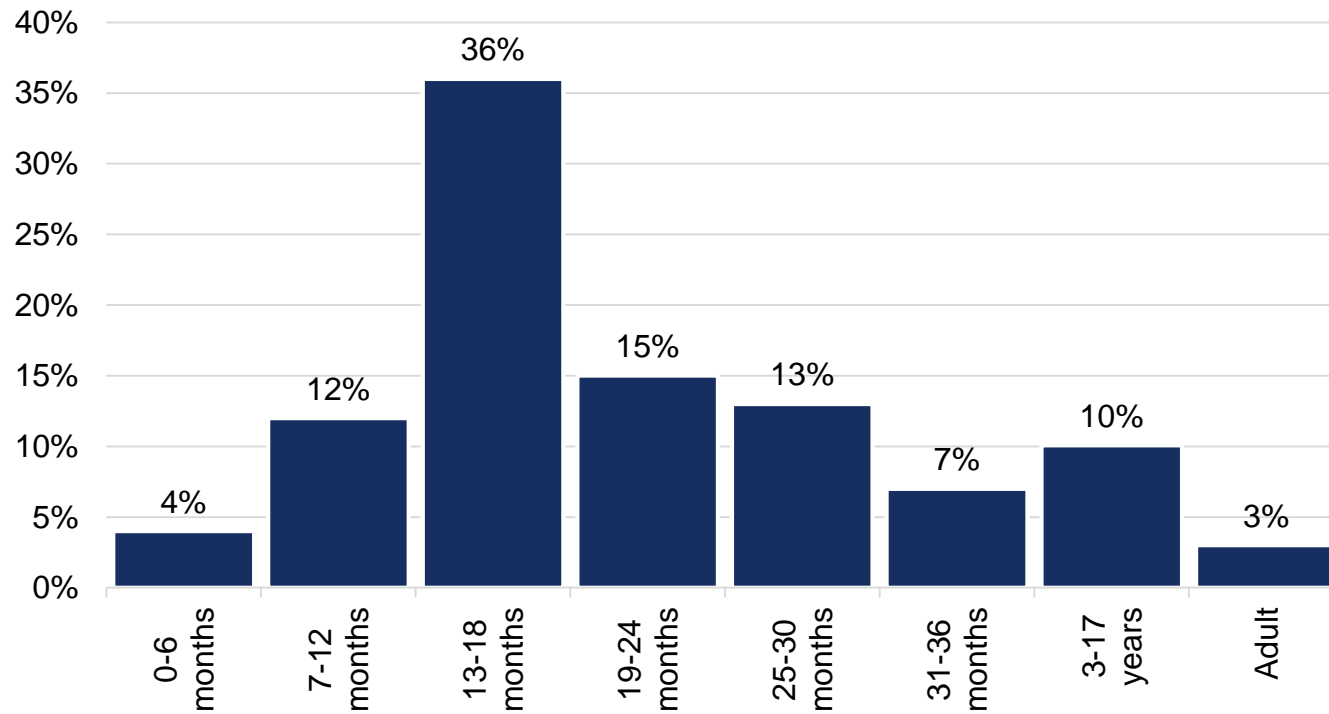


Primary Insurance



87% of patients were ≤ 36 months at symptoms onset; median age of pediatric onset was 18 months

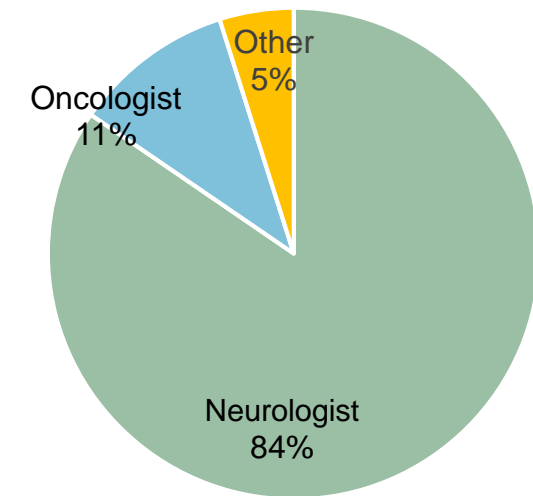
Age at Onset
N=123



Age at Onset (months)

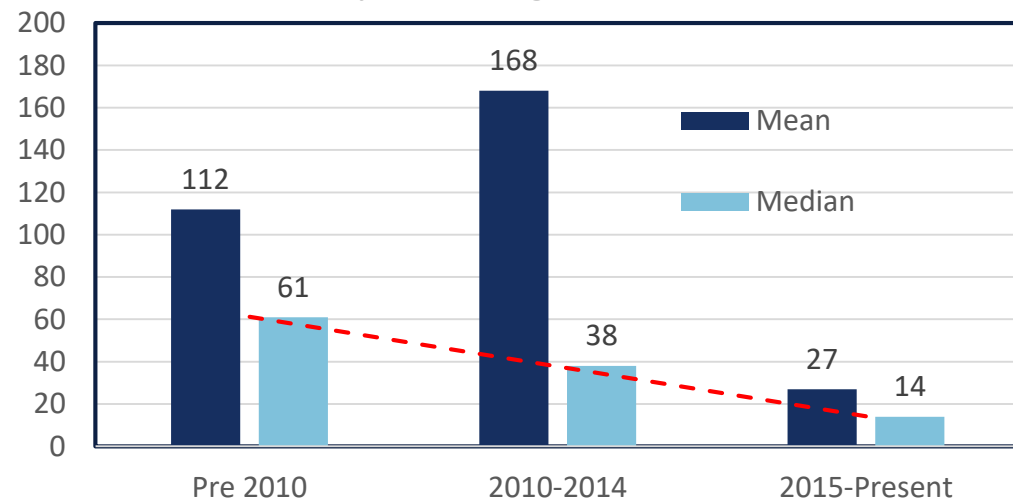
Number Summary	Overall	Pediatric
min	2.0	2.0
25th percentile	14.0	14.0
median	18.0	18.0
mean	32.3	24.2
75th percentile	29.8	29.0
max	420.0	120.0
n	123	120

Who diagnosed patient n=123

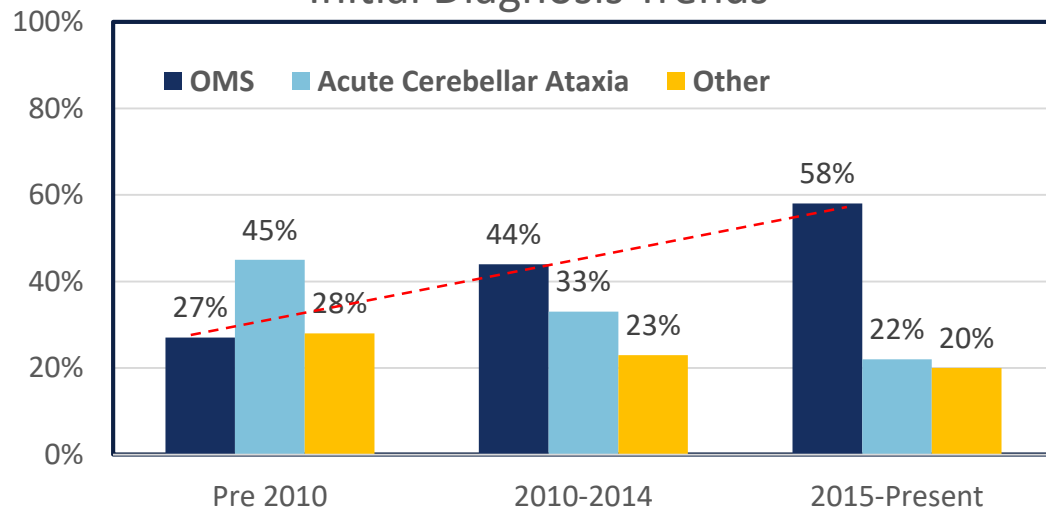


Days to Diagnose has decreased; Initial Diagnosis has become more Accurate

Days to Diagnosis OMS



Initial Diagnosis Trends



Days to Diagnose OMS (n=118)

Onset Date	Before 2010	2010-2014	2015 – Present
Min	1	1	1
25 th Per	19	16	5
Median	61	38	14
Mean	112.2	167.6	26.8
75 th Per	157	127.5	31
Max	922	2038	280
n	33	40	45

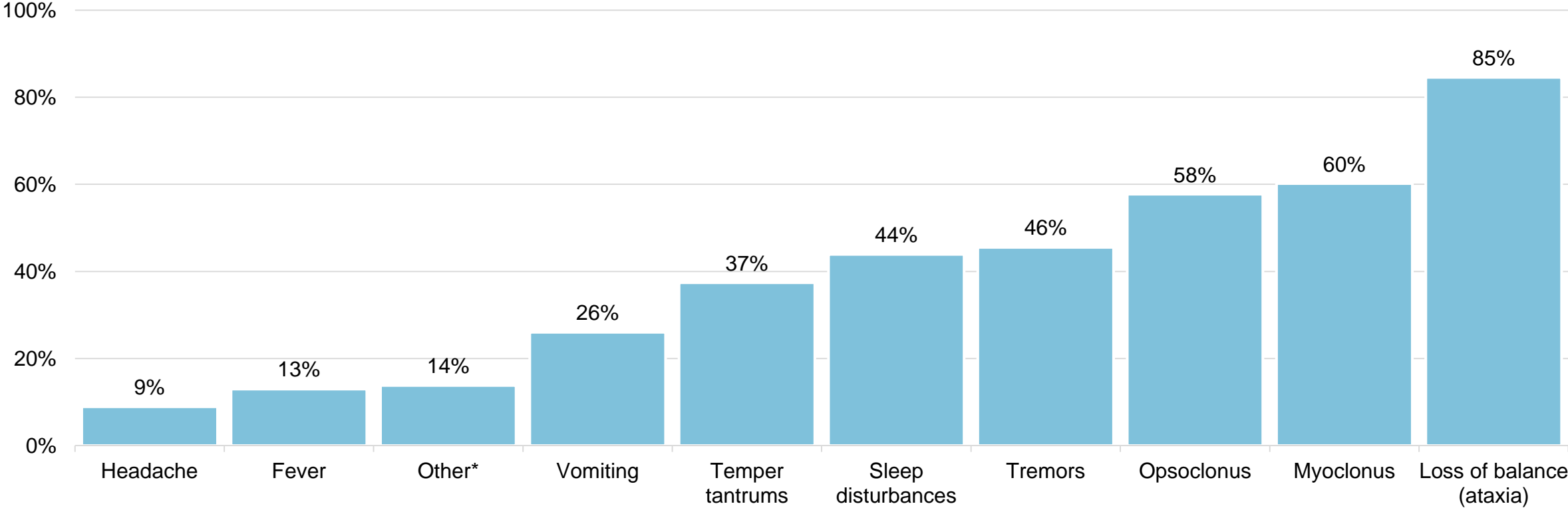
Initial Diagnosis (n=118*)

Onset Date	Before 2010	2010-2014	2015 – Present
OMS	9	17	26
Misdiagnosed	24	22	19
- Acute Cerebellar Ataxia	15	13	10
- Other	9	9	9
n	33	39	45

*Note – five people did not respond

Ataxia was the most common symptom at onset, indicated in 85% of patients

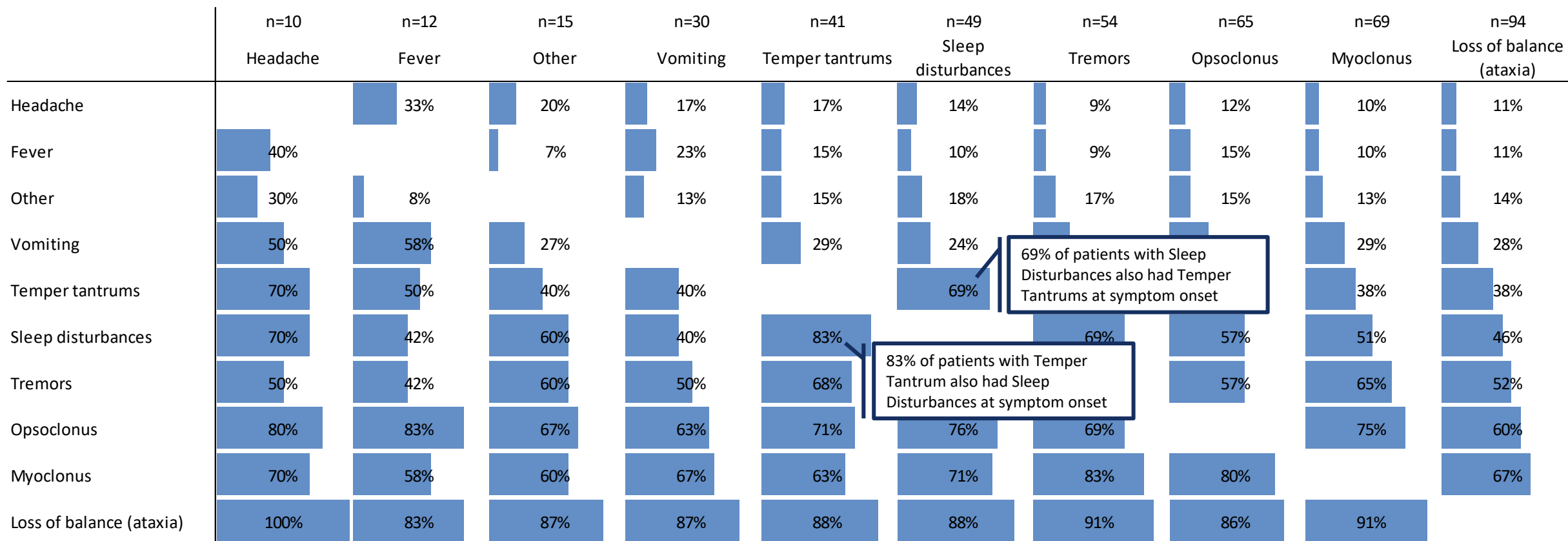
% Patients with a given symptom at onset (n=123)



*Other includes Clinginess (2), Depression (2), Diarrhea, Excessive Drooling (2), Head Tilt, Hypotonia, Loss of Appetite, Muscle Spams, Nystagmus, Seizures, and Spaced Out.

Association of Symptoms at Onset

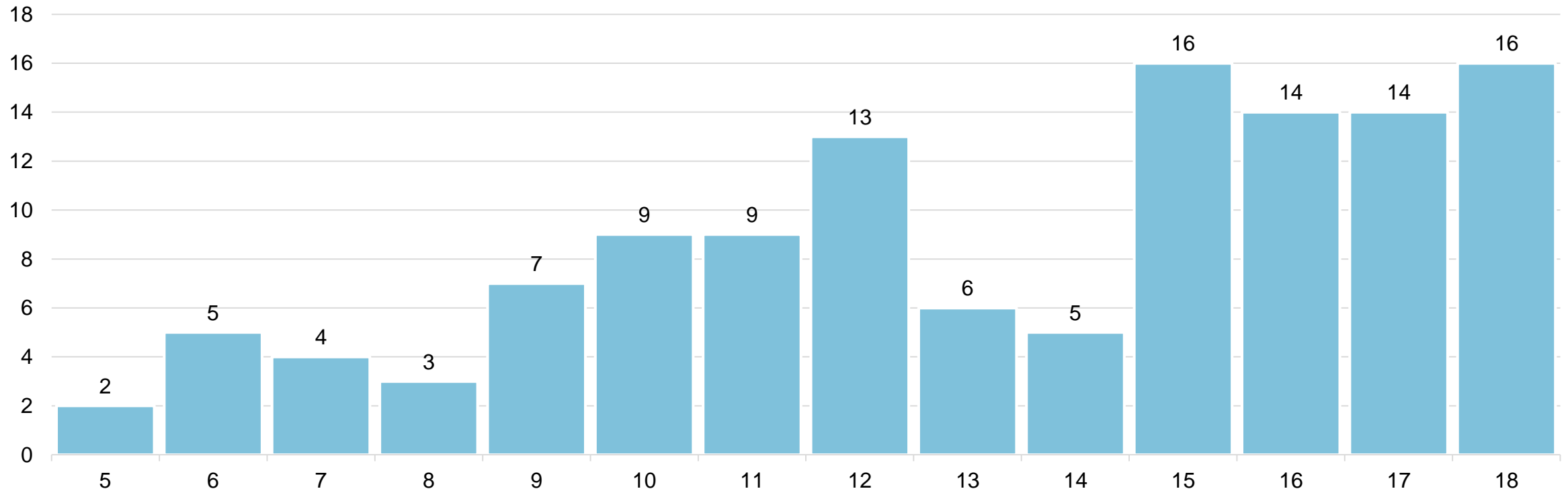
Frequency of Symptoms at Onset (columns)



OMS Severity

MITCHELL-PIKE OMS SEVERITY SCALE

No. Patients by aggregate OMS Severity Score (n=123, max score = 18)



OMS Severity by Individual Measures

Stance Score	DESCRIPTION	NO.	%
0	Standing and sitting balance normal for age	1	1%
1	Mildly unstable standing for age, slightly wide based	19	17%
2	Unable to stand without support but can sit without support	19	17%
3	Unable to sit without using hands to prop or other support	71	65%

Opsoclonus Score	DESCRIPTION	NO.	%
0	None	4	4%
1	Rare or only when elicited by change in fixation or "squeeze test"	16	15%
2	Frequent, interferes intermittently with fixation or tracking	50	45%
3	Persistent, interfering continuously with function and tracking	40	36%

Gait Score	DESCRIPTION	NO.	%
0	Walking normal for age	1	1%
1	Mildly wide-based gait for age, but able to walk indoors and outdoors independently	14	13%
2	Walks only or predominantly with support from person or equipment	34	31%
3	Unable to walk even with support from person or equipment	61	55%

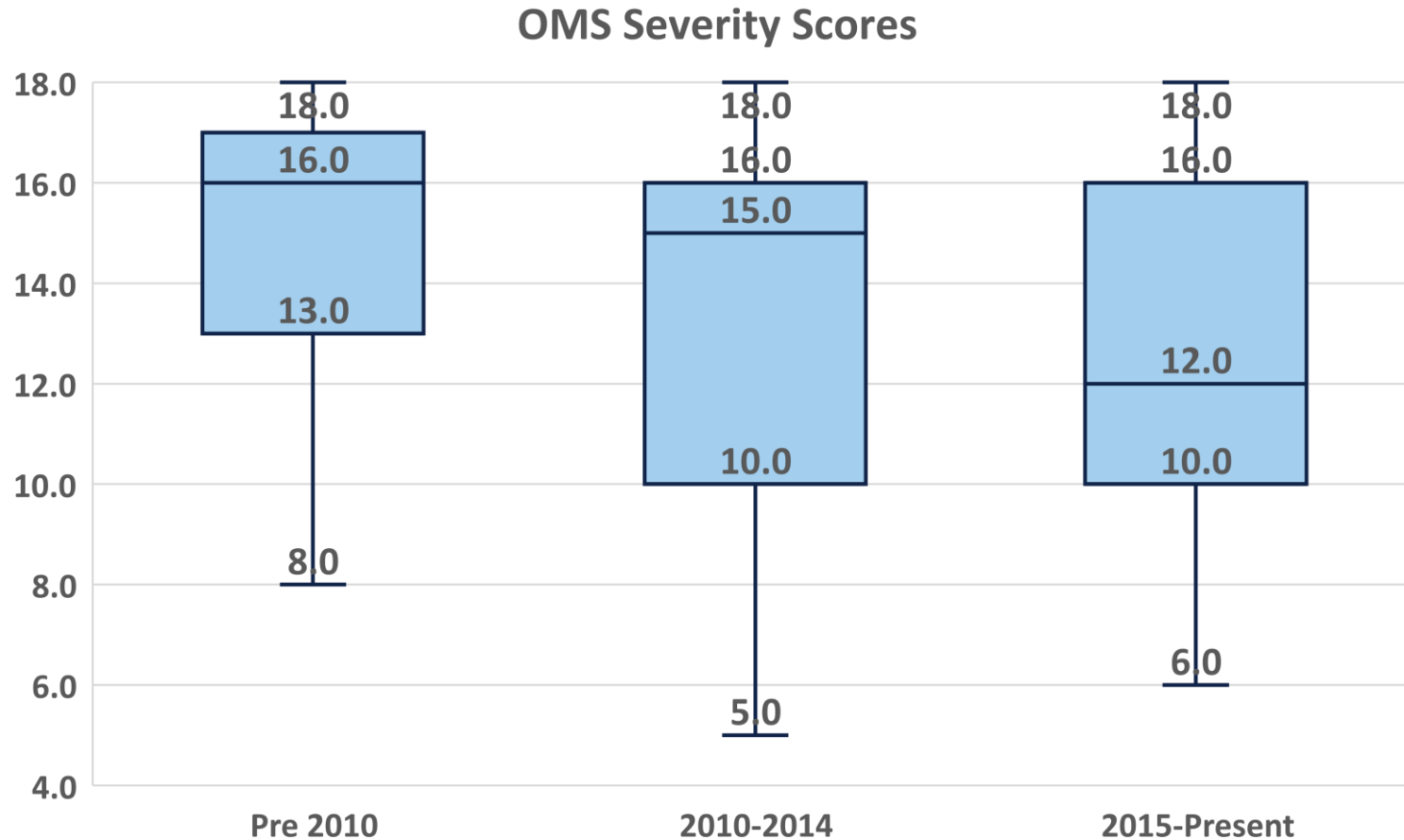
Mood/Behavior Score	DESCRIPTION	NO.	%
0	Normal	6	5%
1	Mild increase in irritability but consolable; and/or mild sleep disturbances	18	16%
2	Irritability and sleep disturbances interfering with child and family life	49	45%
3	Persistent severe distress	37	34%

Arm/Hand Function Score	DESCRIPTION	NO.	%
0	Normal for age	4	4%
1	Mild, infrequent tremor or jerkiness without functional impairment	13	12%
2	Fine motor function persistently impaired for age, but less precise manipulative tasks normal or almost normal	22	20%
3	Major difficulties in all age-appropriate fine motor and manipulative tasks	71	65%

Speech Score	DESCRIPTION	NO.	%
0	Normal for age, no loss	23	21%
1	Mildly unclear, plateaued in development	14	13%
2	Loss of some words or some grammatical constructs (i.e. from sentences to phrases) but still communicates verbally	22	20%
3	Severe loss of verbal communication and speech	51	46%

OMS SEVERITY SCORES OVER TIME

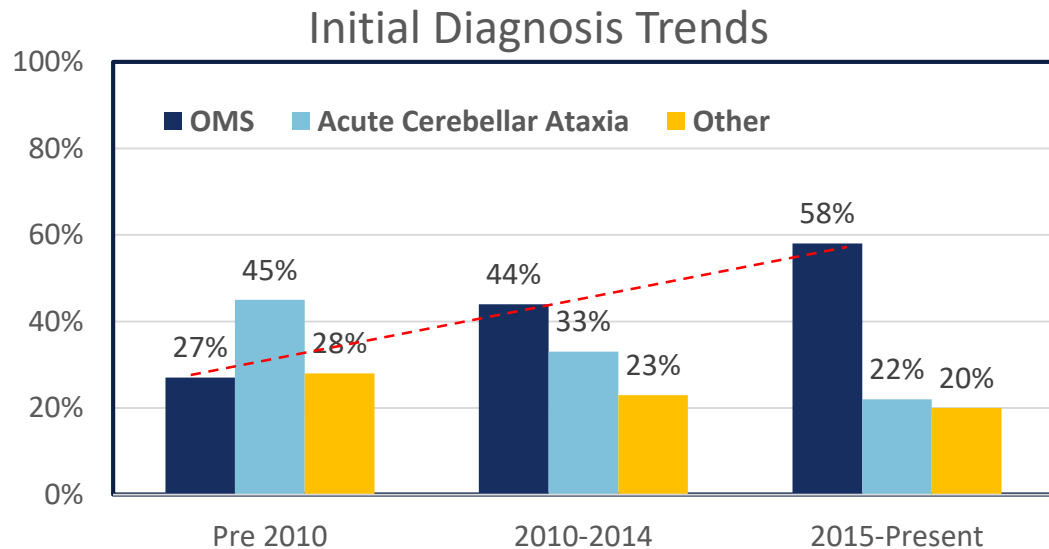
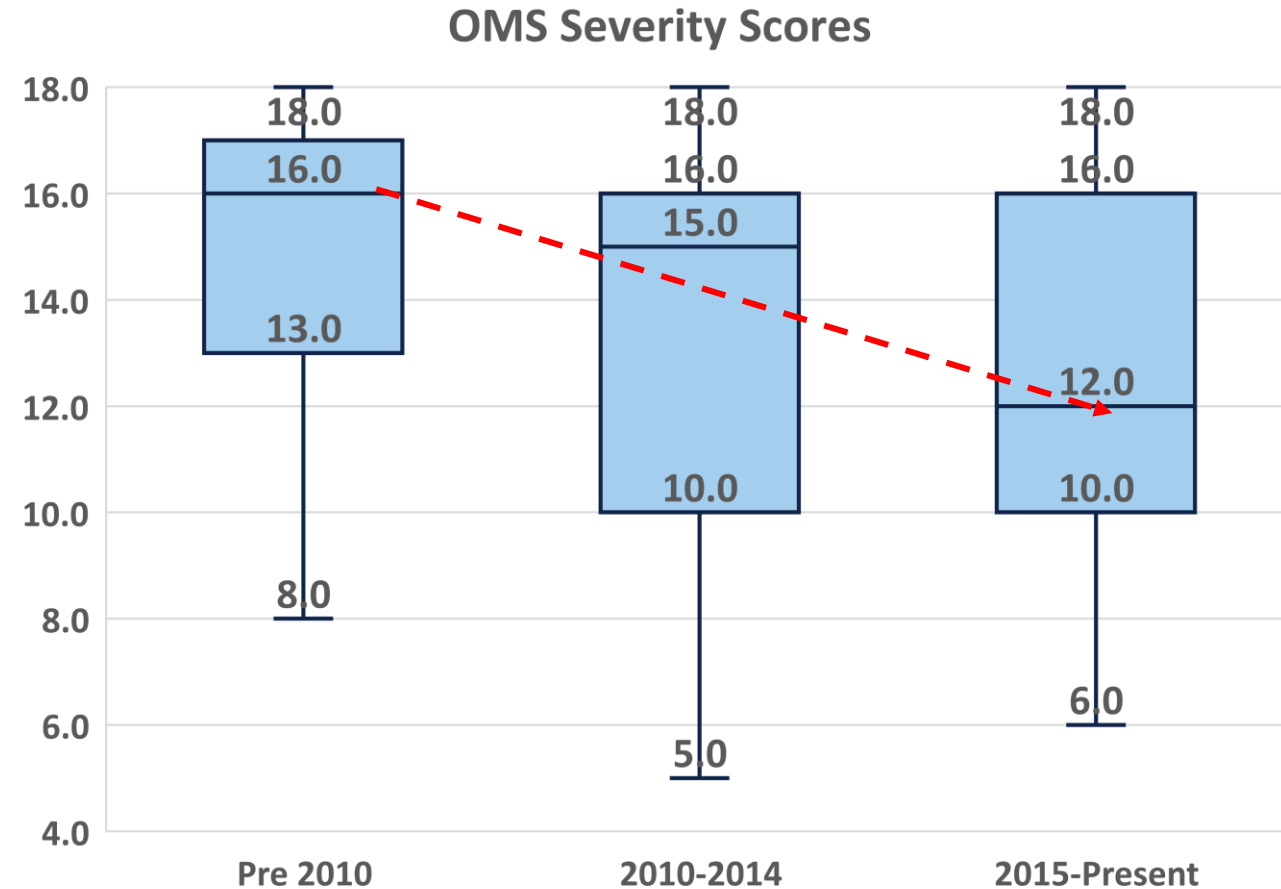
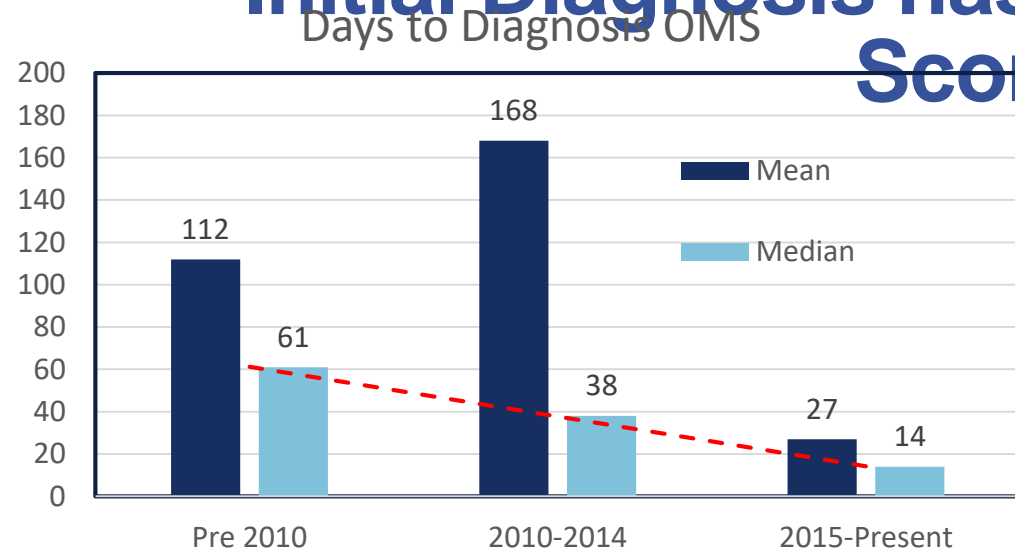
MITCHELL-PIKE OMS SEVERITY SCALE (N=123)



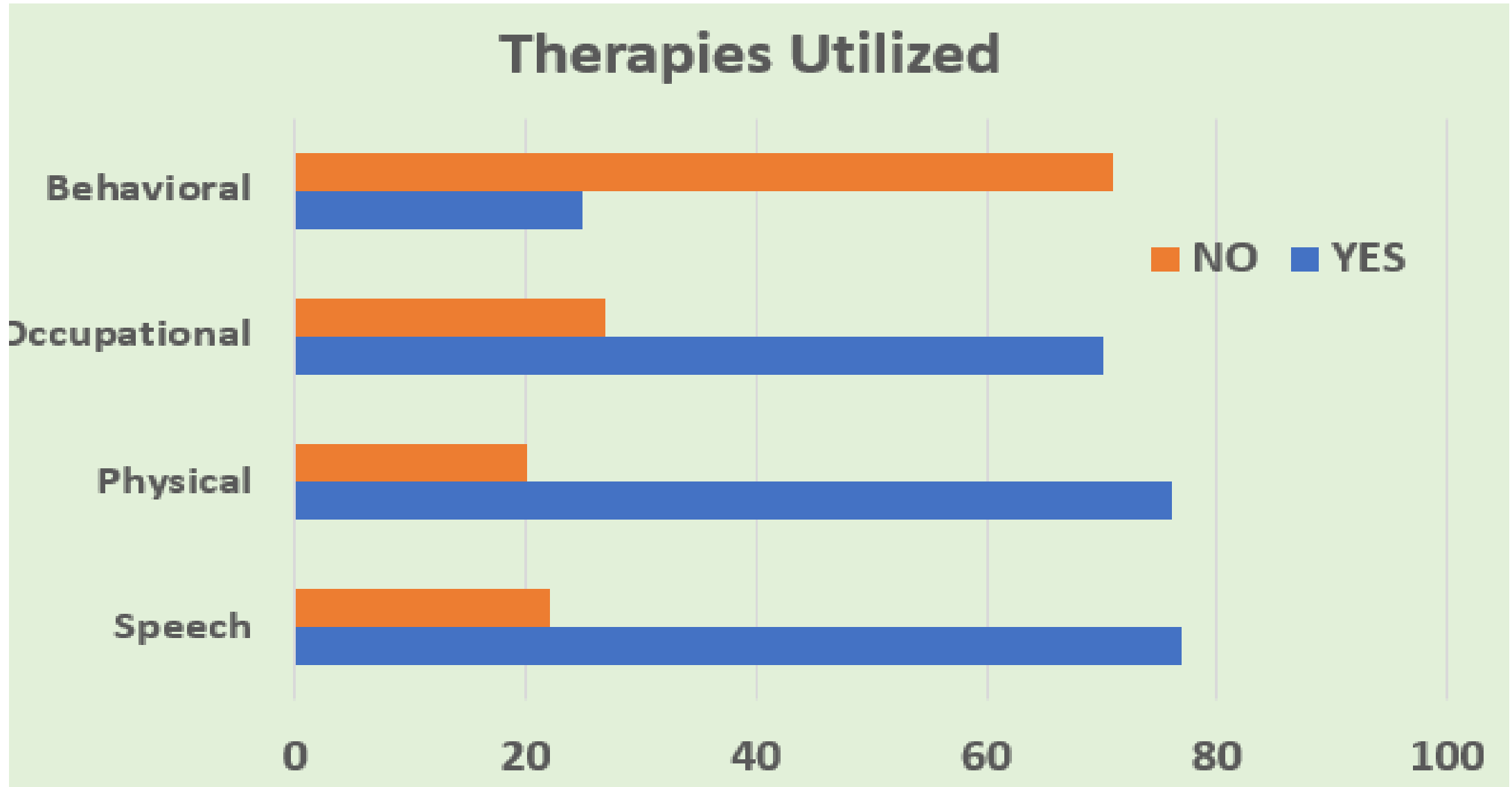
OMS Severity Scores			
Onset Date	Before 2010	2010-2014	2015 – Present
Min	8	5	6
25 th Per	13	10.3	10
Median	16	15	12
Mean	14.9	13.3	12.3
75 th Per	17	16	15
Max	18	18	18
n	37	38	46

* Four (4) participants did not have onset date validated

Days to Diagnose has decreased; Initial Diagnosis has become more Accurate; Scores are Down

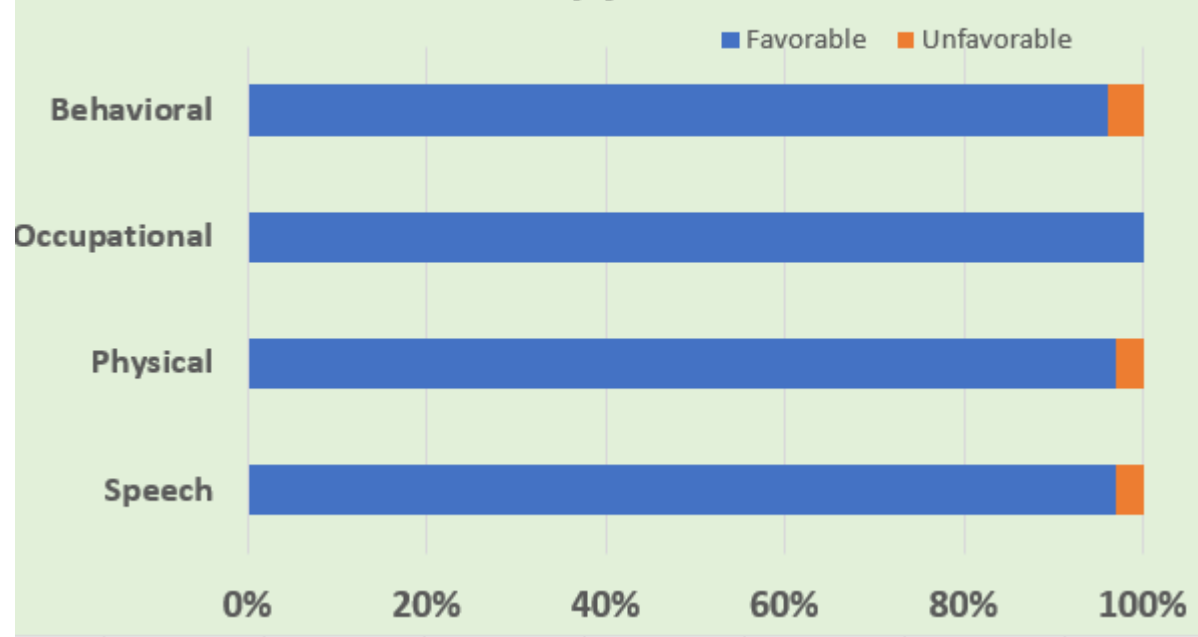


Therapies Consistently Showed Favorable Results (n=97)

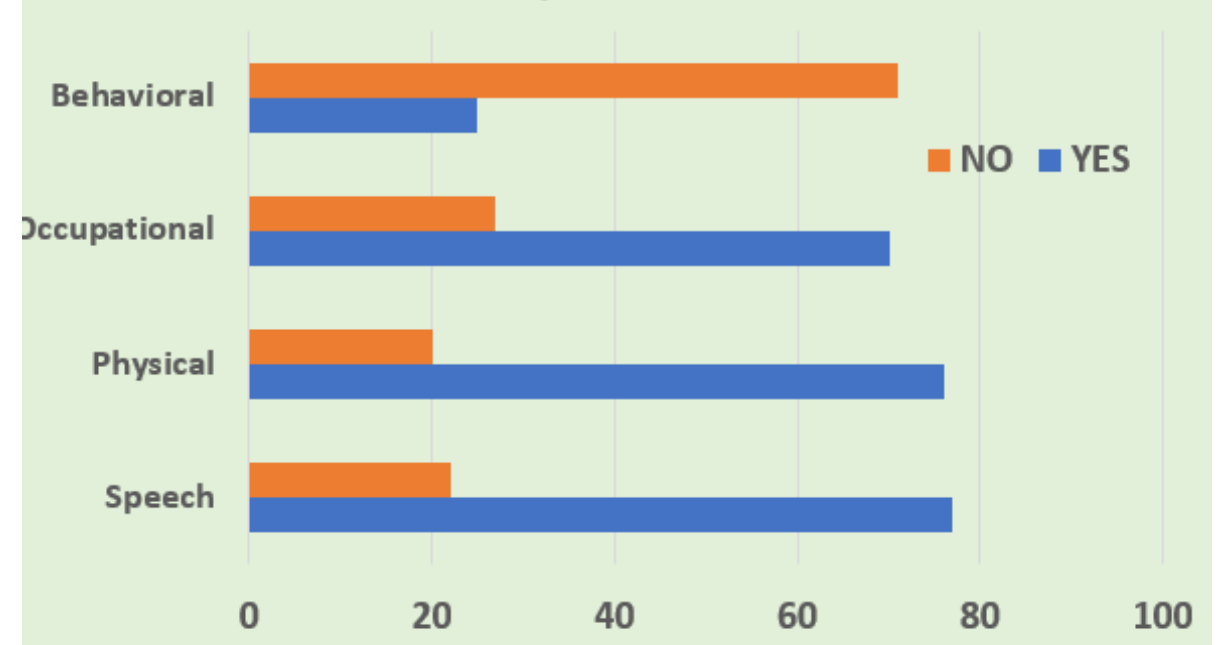


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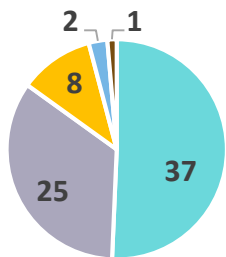
Was Therapy Beneficial?



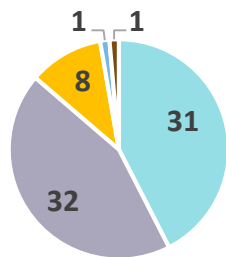
Therapies Utilized



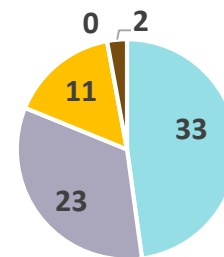
Speech Therapy



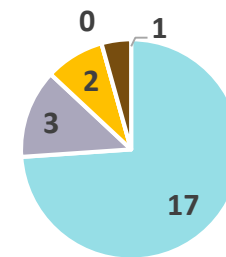
Physical Therapy



Occupational Therapy



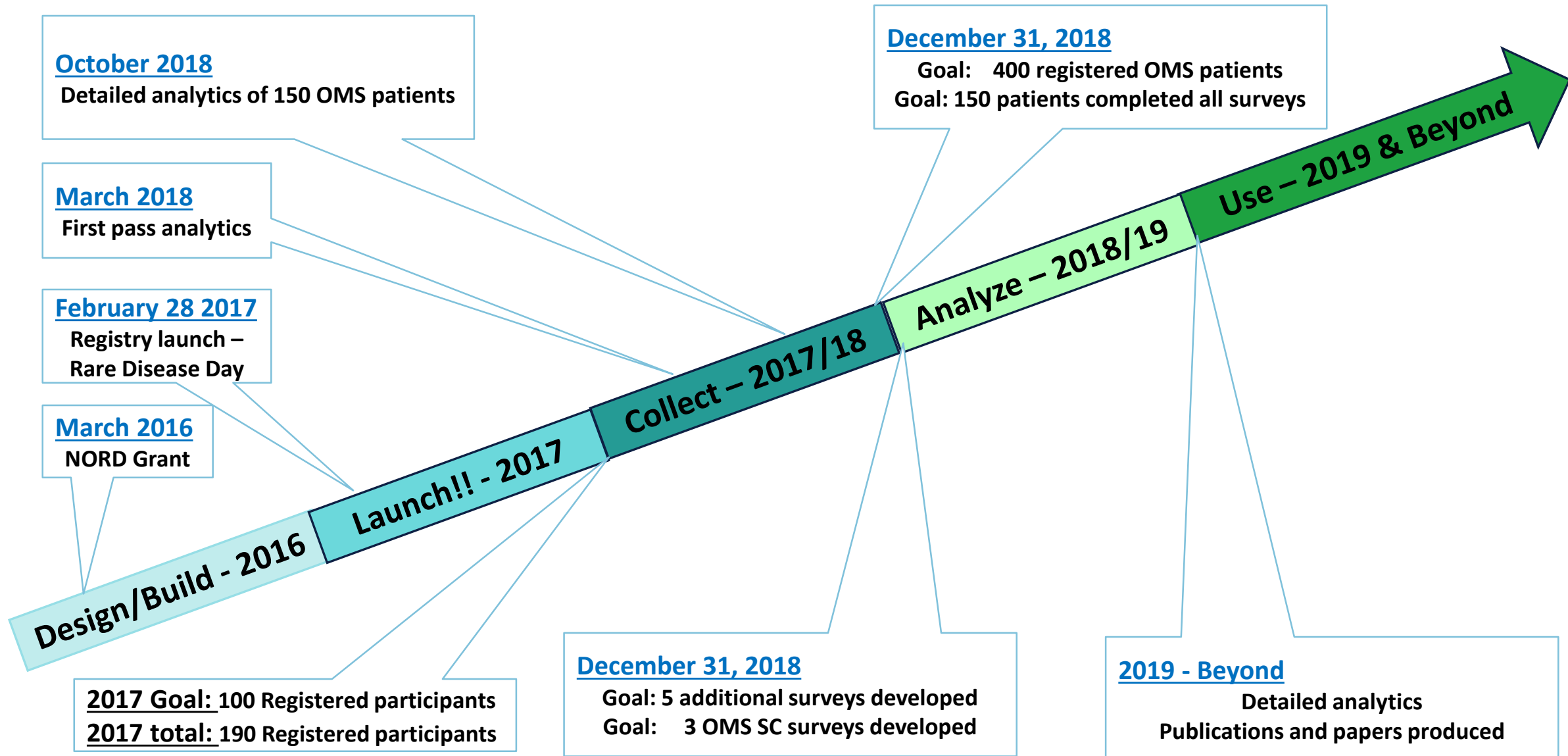
Behavioral Therapy



Weekly Therapy Sessions

■ One
 ■ Two
 ■ Three
 ■ Four
 ■ Five

Here are the Next Steps on our 5 Year Roadmap



Our next steps

- Working on surveys for doctors:
 - Adolescent and young adult trends (Wendy Mitchell)
 - Vaccine schedules post remission (Lotze, Mitchell)
 - Rituximab trends in treatment (frequency, dosage)
 - Relapse Trends
- Patient Roadmap
- Health Tracker

Would you consider participating in the Registry???

- Work on your surveys
- Keep your surveys updated as life events change
- Suggest topics and/or questions for the registry

Ways you can help OMSLife

- Amazon Smile – sign up, tell your friends and family
- Social media help – YouTube, Instagram, Twitter, OMSLife en Espanol
- Wiki – help us build the Wiki
- Donate
 - Fun runs, walks, Facebook events, write a check, buy a t-shirt, etc...
- Other help
 - Web site development, help with conferences, etc...

Special thanks to

The OMS Registry Project Team

Brooke D'Souza

Christy Dennis

Kellee Miller

Trio Health

Scott Milligan

Our Medical Advisory Team

Dr. Wendy Mitchell

Dr. Mark Gorman

Dr. Tim Lotze

Dr. Marc Tardieu

Dr. Anne Berg

The OMSLife Foundation Board of Directors

The patients and caregivers who contributed to the registry

...And **NORD** who provided the 5 year grant to The OMSLife Foundation



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