OMS Patient Registry & Natural History Study

OMS Workshop – Abingdon, England
October 6, 2022

A recap of some questions from 2014 workshop*

Plan of action for quicker diagnosis

- Most patients **DO NOT** see an OMS specialist
- Median time to diagnose in 2014 was > 30 days

- Contributing factors include collaborative efforts, publications, Board Exams, social media, websites, and caregivers' persistence

Consensus on treatment & Treating physician playbook

- A consensus on treatment
- Consensus statement testimonies
  - Treatments changed
  - Specialists consulted
- Collaboration among medical professionals

* From January 2014 OMS Workshop

A recap of some questions from 2014 workshop*

Caregiver playbook

- NORD information
- NORD collaboration video
- **Back to School documents**
- Consensus statement
- Insurance – insurance letter
- OMS Caregiver Conferences (7 to date)
- **Publication Repository**
  - OMS Box Notes - OMS Publications & Research | Powered by Box
    - Bhavna Diaz

Plan of action for Latin America & Guide on Adult OMS

OMSLife Registry

History

- Grant received from NORD via FDA in 2016
  - One of 10 rare diseases to receive the grant
- Patient self-report registry involving fifteen unique surveys, with 370 patients enrolled
- Self-report data provides valuable complement to clinically-obtained metrics

Overview & Registry Aims

- Parents able to provide rich, detailed insight into their child's OMS characteristics, course of care, and day-to-day life
- Doctors are able to see trends over time and beyond disease onset

OMSLife Registry

Potential

- **Find patients outside of the "specialists" areas**
- **Large untapped group of patients remains**
- **Able to ramp up surveys for specific questions**

Boston Children's Collaboration, Data Analysis & Publication

- Ongoing collaboration between OMSLife and Boston Children's Hospital
- Primary areas of interest: demographics, treatment, and time-to-diagnosis
- Goal will be to examine ongoing disease characteristics, treatment trends over time
- Initial manuscript submission expected fall 2022

The OMS patient network has grown over 12 years*
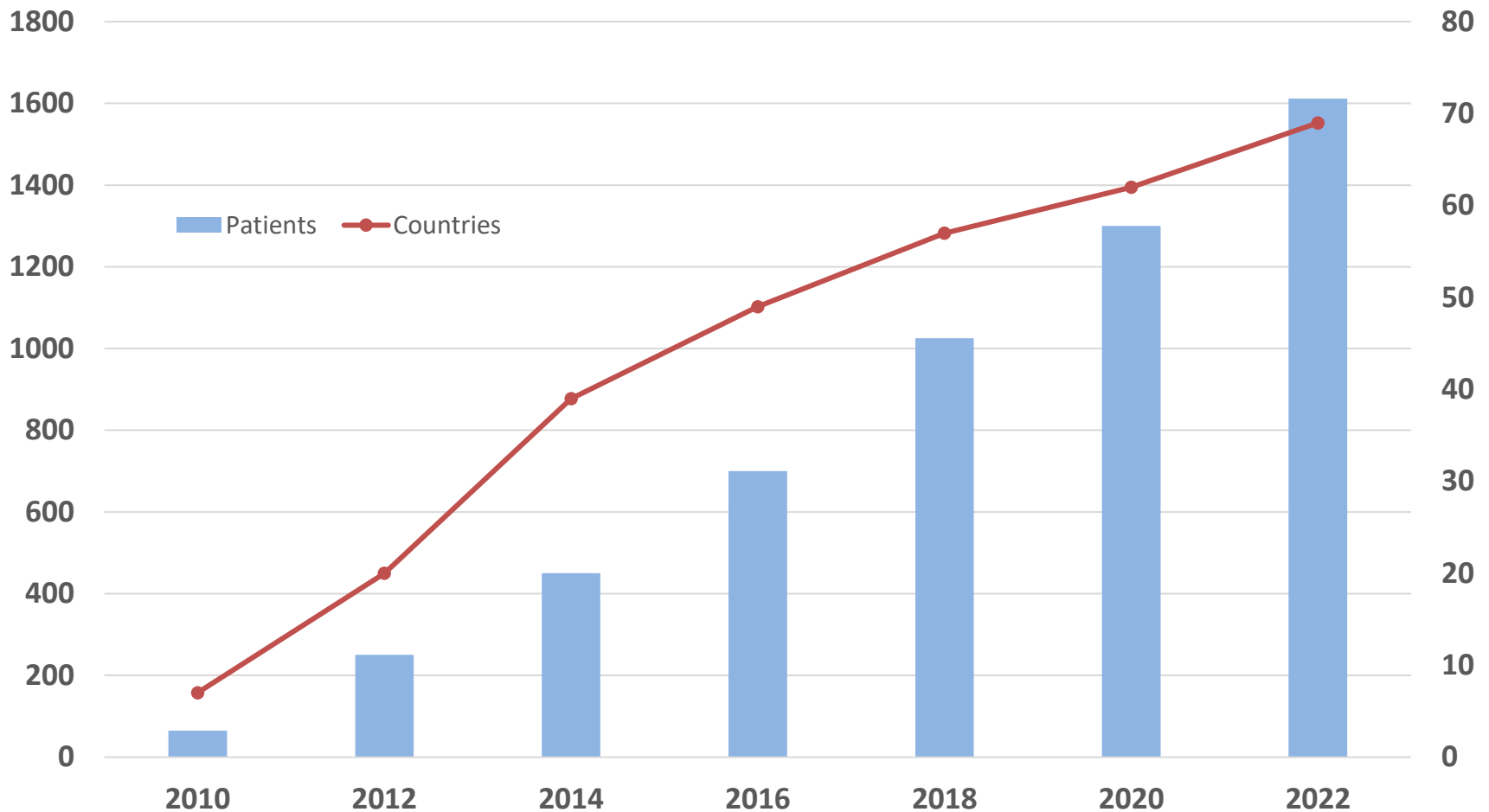
* Sources includes social media, OMS caregiver conferences, doctor referrals, webs
NORD, Dr. P's MCDonald House, OMSLife register, family referrals, etc…

OMSLife Registry Studies

Trio Health, NORD, OMSLife Collaboration 2019

- General demographic data previously published
- Shows that the data can be trusted
- Data updated annually
- Capturing patients from many years

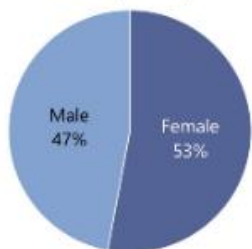## Boston Children's Hospital Collaboration 2022

- General demographic data from 2020 data set of patients
- Plans are being developed for continued collaborative efforts on trends of OMS patients over time
- Opportunities to grow the BCH registry with new patients out side the participating group of hospitals
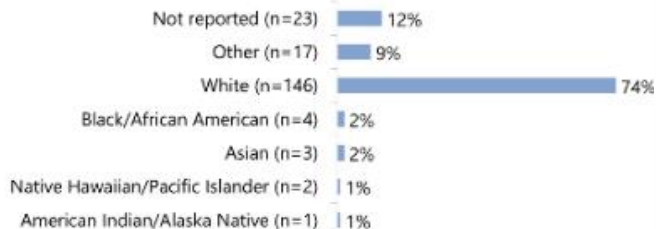
3. PATIENT DEMOGRAPHICS

As of Sep 2018, 275 patients or caregivers registered and 196 contributed data to the registry; 74 patients filled out all available surveys.

Gender (n=170)

- Male 47%
- Female 53%

Race (n=196)

Category	Percent
Not reported (n=23)	12%
Other (n=17)	9%
White (n=146)	74%
Black/African American (n=4)	2%
Asian (n=3)	2%
Native Hawaiian/Pacific Islander (n=2)	1%
American Indian/Alaska Native (n=1)	1%

Country of Residence (n=169)

(Map values: 6, 144, 1, 1, 1, 1, 1, 15, 1, 1, 2)
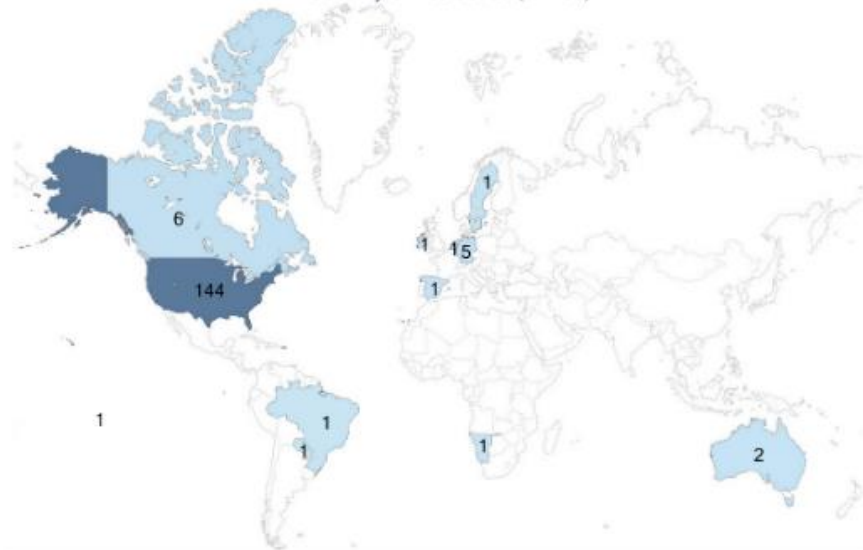
Age at Diagnosis (n=166)

Age group	Percent
0-6 months	3%
7-12 months	26%
13-18 months	18%
19-24 months	30%
25-30 months	4%
31-36 months	8%
3-17 years	7%
Adult	4%
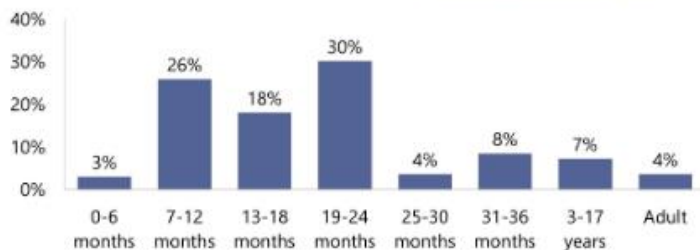
Age (months)	Overall	Pediatric
min	2.0	2.0
25th percentile	12.0	12.0
median	20.0	19.5
mean	33.3	22.5
75th percentile	24.0	24.0
max	420.0	120.0
n	166	160

4. PATIENT DEMOGRAPHICS

Insurance Type (n=159)

Type	Percent
Commercial (n=98)	62%
Medicaid (n=31)	19%
Military (n=7)	4%
Other Govt (n=23)	14%
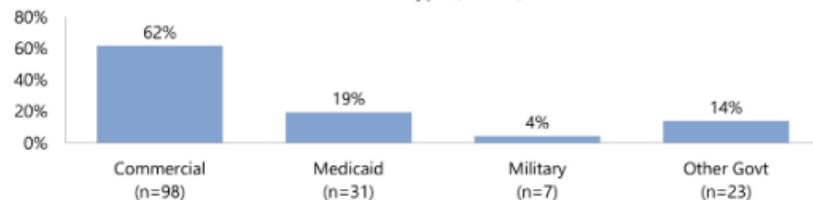
Family Income Level (n=137)

Level	Percent
<$10K (n=6)	4%
$10K<$20K (n=9)	7%
$20K<$50K (n=23)	17%
$50K<$100K (n=44)	32%
$100K<$250K (n=45)	33%
$250K+ (n=10)	7%

Highest Education Level in Household (n=164)

Level	Percent
Not Specified (n=2)	1%
Did not finish HS (n=0)	0%
Technical or HS Graduate (n=17)	10%
Post-Secondary Education (n=19)	12%
Post-Secondary Graduate (n=75)	45%
Post-Graduate Education (n=52)	32%
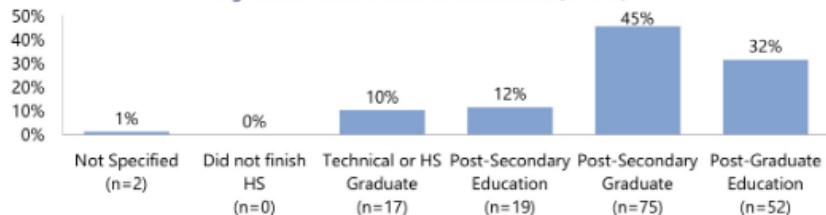
4. ASSOCIATIONS OF SYMPTOMS AT ONSET

Frequency of other symptoms at onset for each individual symptom. Arrows in
higher or lower likelihood than expected from the population distribution base
standardized residuals.

■ Not Significant ■ Significant (p<0.05)

Ataxia (n=131)
Symptom	%
Headache	10%
Fever	13%
Vomiting	27%
Temper tantrums	39%
Sleep disturbances	46%
Tremors	48%
Opsoclonus	59%
Myoclonus	64%
Ataxia	100%

Myoclonus (n=91)
Symptom	%
Headache	11%
Fever	12%
Vomiting	33%
Temper tantrums	42%
Sleep disturbances	53%
Tremors	59%
Opsoclonus	77% ↑
Myoclonus	100%
Ataxia	92%

Opsoclonus (n=
Symptom	%
Headache	11%
Fever	17%
Vomiting	32%
Temper tantrums	45%
Sleep disturbances	56%
Tremors	50%
Opsoclonus	
Myoclonus	80% ↑
Ataxia	88%

Tremors (n=69)
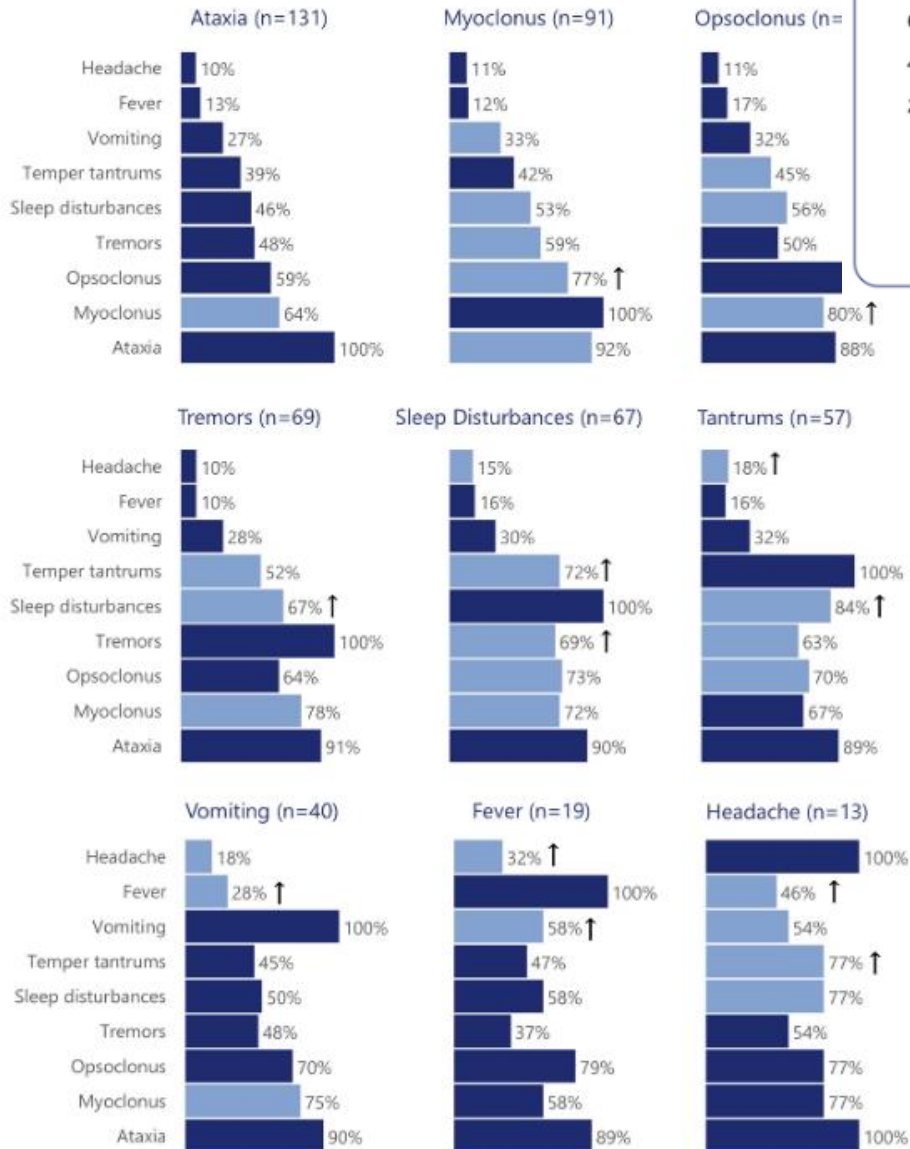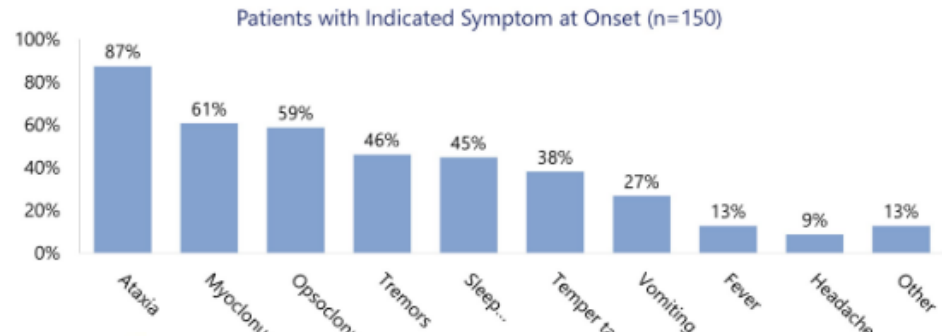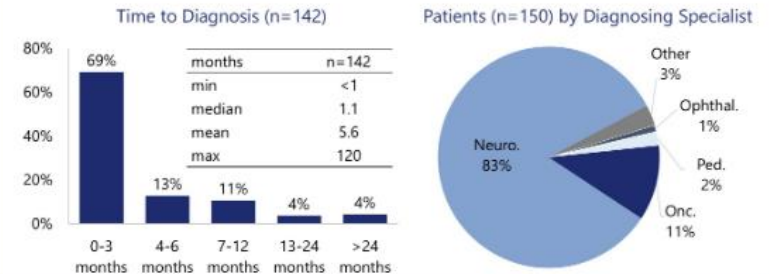| Symptom	%
Headache	10%
Fever	10%
Vomiting	28%
Temper tantrums	52%
Sleep disturbances	67% ↑
Tremors	100%
Opsoclonus	64%
Myoclonus	78%
Ataxia	91%

Sleep Disturbances (n=67)
Symptom	%
Headache	15%
Fever	16%
Vomiting	30%
Temper tantrums	72% ↑
Sleep disturbances	100%
Tremors	69% ↑
Opsoclonus	73%
Myoclonus	72%
Ataxia	90%

Tantrums (n=57)
Symptom	%
Headache	18% ↑
Fever	16%
Vomiting	32%
Temper tantrums	100%
Sleep disturbances	84% ↑
Tremors	63%
Opsoclonus	70%
Myoclonus	67%
Ataxia	89%

Vomiting (n=40)
Symptom	%
Headache	18%
Fever	28% ↑
Vomiting	100%
Temper tantrums	45%
Sleep disturbances	50%
Tremors	48%
Opsoclonus	70%
Myoclonus	75%
Ataxia	90%

Fever (n=19)
Symptom	%
Headache	32% ↑
Fever	100%
Vomiting	58% ↑
Temper tantrums	47%
Sleep disturbances	58%
Tremors	37%
Opsoclonus	79%
Myoclonus	58%
Ataxia	89%

Headache (n=13)
Symptom	%
Headache	100%
Fever	46% ↑
Vomiting	54%
Temper tantrums	77% ↑
Sleep disturbances	77%
Tremors	54%
Opsoclonus	77%
Myoclonus	77%
Ataxia	100%

3. SYMPTOMS AT ONSET

Ataxia (87%, 131/150) was the most common symptom at onset.

Patients with Indicated Symptom at Onset (n=150)

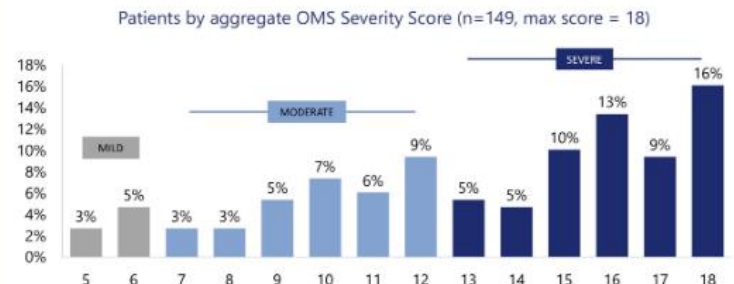| Symptom	%
Ataxia	87%
Myoclonus	61%
Opsoclon.	59%
Tremors	46%
Sleep...	45%
Temper t.	38%
Vomiting	27%
Fever	13%
Headache	9%
Other	13%

5. TIME TO DIAGNOSIS AND DIAGNOSING PROVIDER

Most (69%) patients were diagnosed by 3 months following onset of symptoms.

Time to Diagnosis (n=142)

	0-3 months	4-6 months	7-12 months	13-24 months	>24 months
%	69%	13%	11%	4%	4%

months	n=142
min	<1
median	1.1
mean	5.6
max	120

Patients (n=150) by Diagnosing Specialist

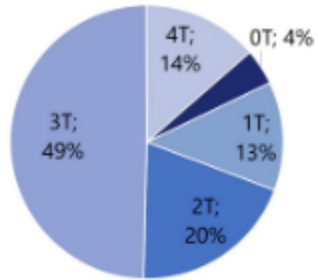| Specialist	%
Neuro.	83%
Onc.	11%
Other	3%
Ped.	2%
Ophthal.	1%

6. DISEASE SEVERITY

The Mitchell-Pike OMS Severity Scale classifies OMS cases as mild (0–6),
moderate (7-12), or severe (13–18). The aggregate score is based on 6 individual
assessments, with a score of 0 (normal) to 3 (severe impairment or state)
assigned individually for stance, gait, arm/hand function, opsoclonus,
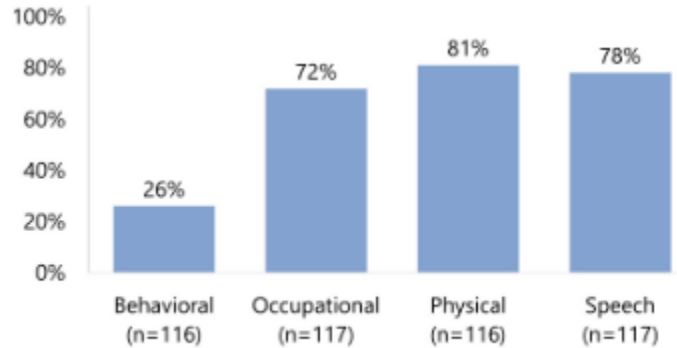mood/behavior, and speech.

Patients by aggregate OMS Severity Score (n=149, max score = 18)

Score	%	Category
5	3%	MILD
6	5%	MILD
7	3%	MODERATE
8	3%	MODERATE
9	5%	MODERATE
10	7%	MODERATE
11	6%	MODERATE
12	9%	MODERATE
13	5%	SEVERE
14	5%	SEVERE
15	10%	SEVERE
16	13%	SEVERE
17	9%	SEVERE
18	16%	SEVERE

3. TYPES OF THERAPIES

Of the 117 respondents, 83% received more than 1 type of therapy.
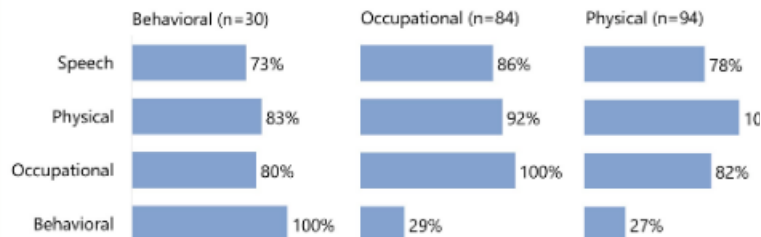
% of Patients by # of Therapy Types Received

- 4T; 14%
- 0T; 4%
- 1T; 13%
- 2T; 20%
- 3T; 49%

% of Patients receiving each Therapy Type

Therapy Type	%
Behavioral (n=116)	26%
Occupational (n=117)	72%
Physical (n=116)	81%
Speech (n=117)	78%

- Frequency of sessions per week varied by therapy type
- Physical therapy had the greatest fraction of patients receiving >1 session per week (56%)

Behavioral (n=29) — Sessions per Week
- <1: 7%
- 1: 66%
- 2: 14%
- 3: 7%
- 5: 3%
- 7: 3%

Occupational (n=84) — Sessions per Week
- <1: 2%
- 1: 46%
- 2: 33%
- 3: 14%
- 6: 2%
- 7: 1%

Physical (n=93) — Sessions per Week
- <1: 3%
- 1: 41%
- 2: 40%
- 3: 12%
- 4: 1%
- 5: 1%
- 6: 1%
- 7: 1%

Speech (n=90) — Sessions per Week
- <1: 3%
- 1: 46%
- 2: 30%
- 3: 14%
- 4: 2%
- 5: 3%
- 6: 0%
- 7: 1%

Respondent perception of therapy

- ≥90% of respondents indicated that specified therapy was beneficial

- Behavioral (n=29): 90%
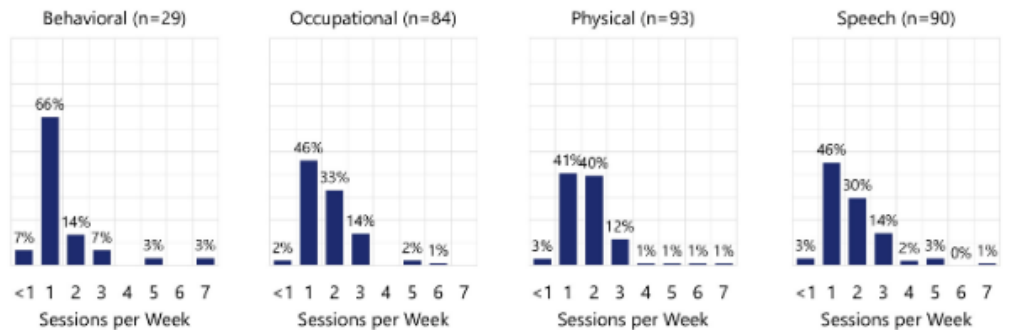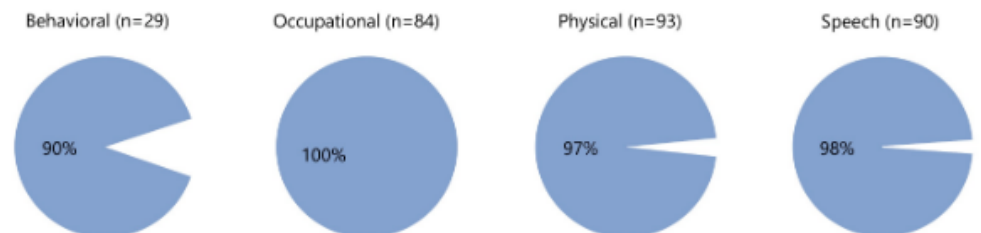- Occupational (n=84): 100%
- Physical (n=93): 97%
- Speech (n=90): 98%

4. ASSOCIATION OF THERAPY TYPES

Past or present use of other therapy types for patients with specified typ
- Most respondents received occupational, physical, or speech therapy
- Multiple therapeutic approaches were common

Behavioral (n=30)
- Speech: 73%
- Physical: 83%
- Occupational: 80%
- Behavioral: 100%

Occupational (n=84)
- Speech: 86%
- Physical: 92%
- Occupational: 100%
- Behavioral: 29%

Physical (n=94)
- Speech: 78%
- Physical: 10
- Occupational: 82%
- Behavioral: 27%

And new opportunities for the 2022 workshop

Behavioral issues

- Can the registry help gather behavioral data?
- Are Sensory Processing Disorders (SPD) being misdiagnosed as behavioral issues?
- Are there Occupational Therapy solutions?

Plan of action for Latin America & Guide on Adult OMS

- Collaboration with Latin American doctors
- Development of Spanish publications

- Adult onset of OMS
- Transition of pediatric patients to adult care

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