EP. 322 - Relevance of maximal performance measurement during real-life ambulation in neuromuscular diseases: patients' and caregivers' perspectives

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Introduction

Development of a new **real-world endpoint: Stride Velocity 95th centile (SV95C)**

- Digital clinical outcome assessment (COA) measured at the ankle with a suitable wearable device during daily life
- Qualified by EMA as secondary endpoint in DMD to assess new drug efficacy
- Reflects the maximal ambulatory performance during normal day living

Clinical relevance of passively measuring the maximal ambulation speed of a patient in an uncontrolled environment to assess the efficacy of new drugs ?

Methods

International cross-sectional survey

Neuromuscular diseases	Patients Families / Caregivers
English, French, Dutch, German, Italian,	From October 2020 to January 2021

4 domains:

- Mobility meaning
- Participation to clinical trial
- ActiMyo® as a wearable device in CT
- **Outcome measure meaningfulness**

Results

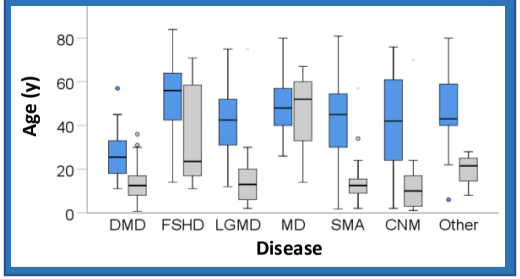
Population

6415 email addresses

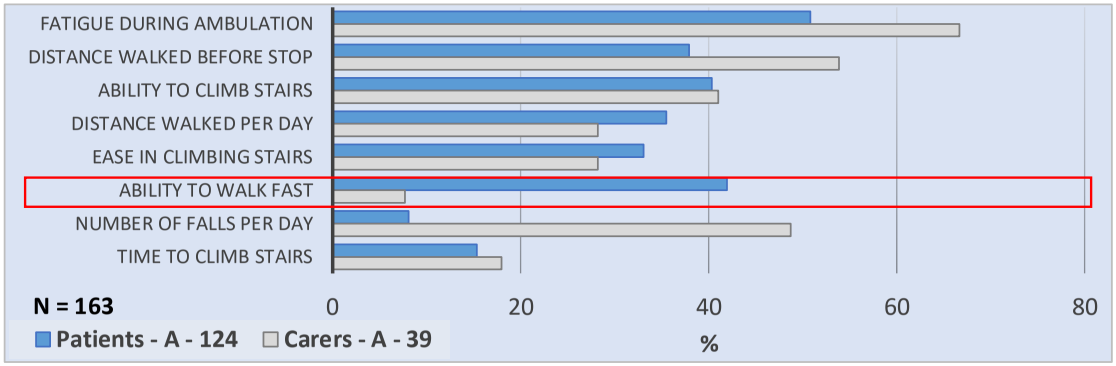
549 respondants

	Patients = 403		Carers = 146	
	A 221	NA 174	A 69	NA 77
DMD	3	11	46	32
FSHD*	73	46	5	3
LGMD*	53	48	9	5
MD***	70	23	3	6
SMA*	4	26	2	14
CNM**	9	15	2	15
Other	9	5	2	2

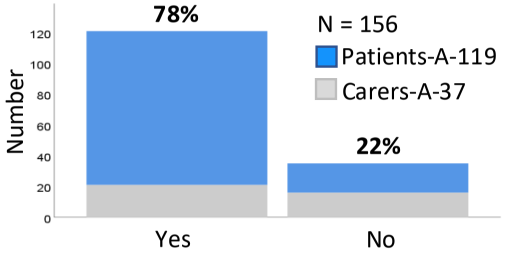
** Number of patients with unknown ambulatory status*



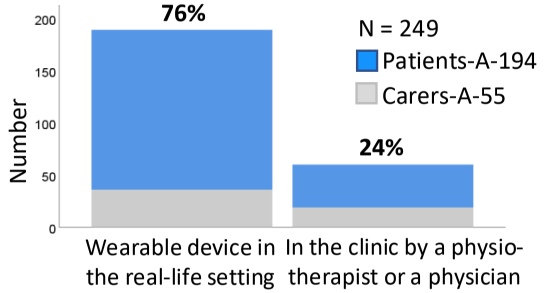
Among the followings, which best represents an ambulation improvement (max 3 items)?



Do you think that measuring a change in the top speed while walking is representative of an ambulation improvement?



If you have the choice, how would you prefer that your mobility be assessed during a trial?



Take Home messages

- Perspectives are different between patients and their families or caregivers (carers).
- For ambulant patients, ability to walk fast is the 2nd most representative aspect of an ambulation improvement.
- 79% of ambulant patients would prefer that their mobility be assessed in real-life setting using a wearable device.
- 84% of ambulant patients agree that measuring a change in the top speed while walking is representative of an ambulation improvement.

Acknowledgement: We would like to sincerely thank all patients and their families/caregivers for their participation.

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*A: Ambulant, **CNM**: Centronuclear myopathy, **COA**: Clinical outcome Assessment, **CT**: Clinical trial, **DMD**: Duchenne muscular dystrophy, **EMA**: European medicine agency,, **FSHD**: Facio-scapulohumeral muscular dystrophy, **LGMD**: Limb girdle muscular dystrophy, **MD**: Myotonic Dystrophy **SMA**: Spinal muscular atrophy, **NA**: Non-Ambulant,.*