Stand for Duchenne Canada

Jesse's JOURNEY
DEFEAT DUCHENNE. CHANGE LIVES.

LAFORCE

Time For Change

October 2, 2019

Agenda

- Who We Are and What We Do
- Why We Are Here
- About Duchenne: Key Facts with Short Video
- Canadian Duchenne Patient and Caregiver Experience
- What We Need- Access to Current Medications
- Future Considerations for Emerging Treatments
- Discussion/Next Steps

Who We Are

- **Jesse's Journey,** founded in 1995, is Canada's only charity dedicated solely to funding Duchenne research. To date Jesse's Journey has granted over $11.5M to DMD research worldwide.
- <u>Statement of Purpose</u>

  Jesse's Journey is a partnership of science and families dedicated to the discovery of treatment and cure of Duchenne muscular dystrophy – because parents should not outlive their children.

Jesse's JOURNEY
DEFEAT DUCHENNE. CHANGE LIVES.

Who We Are

- **La Force DMD's** who are you?
- Our mission is to unite the DMD community to raise awareness, provide access to new treatments as fast as possible, and participate in the funding of promising research projects.

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Who We Are

- **Stand for Duchenne Canada** is a national not-for-profit organization founded by a Duchenne family and their close friends.
- Our mission is to unite families living with Duchenne to strengthen their lives through advocacy and support.

Stand for Duchenne Canada

What We Do

- **Stand for Duchenne Canada, La Force DMD** and **Jesse's Journey** have a collective mandate to ensure that Duchenne patients, families and caregivers are able to receive the best available treatment for their disease **as quickly as possible**.

- For those living with Duchenne, time is not on our side when it comes to this progressive, debilitating and ultimately fatal disease.

Why We Are Here

There are currently no treatments for Duchenne approved in Canada while there are treatments available in other countries.

- To discuss the expedited approval of current and emerging Duchenne treatments.
- To share the Duchenne patient and caregiver experience.
- To propose pathways to have the Duchenne patient and caregiver perspective included in the review of current and future new drug submissions.

What is Duchenne

- Duchenne muscular dystrophy is a rare, progressive and ultimately fatal genetic disorder that affects 1 in 7000 boys and sometimes girls.

- Muscle weakness can begin as early as 1 year of age and leads to a loss of their independence as they progress to a wheelchair by their early teens.

- As the disease continues to progress, boys will develop heart and respiratory problems which will eventually become life-threating and lead to early mortality in their 20s to 30s.

In Their Own Voice

Canadian Duchenne Patient/Caregiver Experience Preliminary Results for 2019 Survey

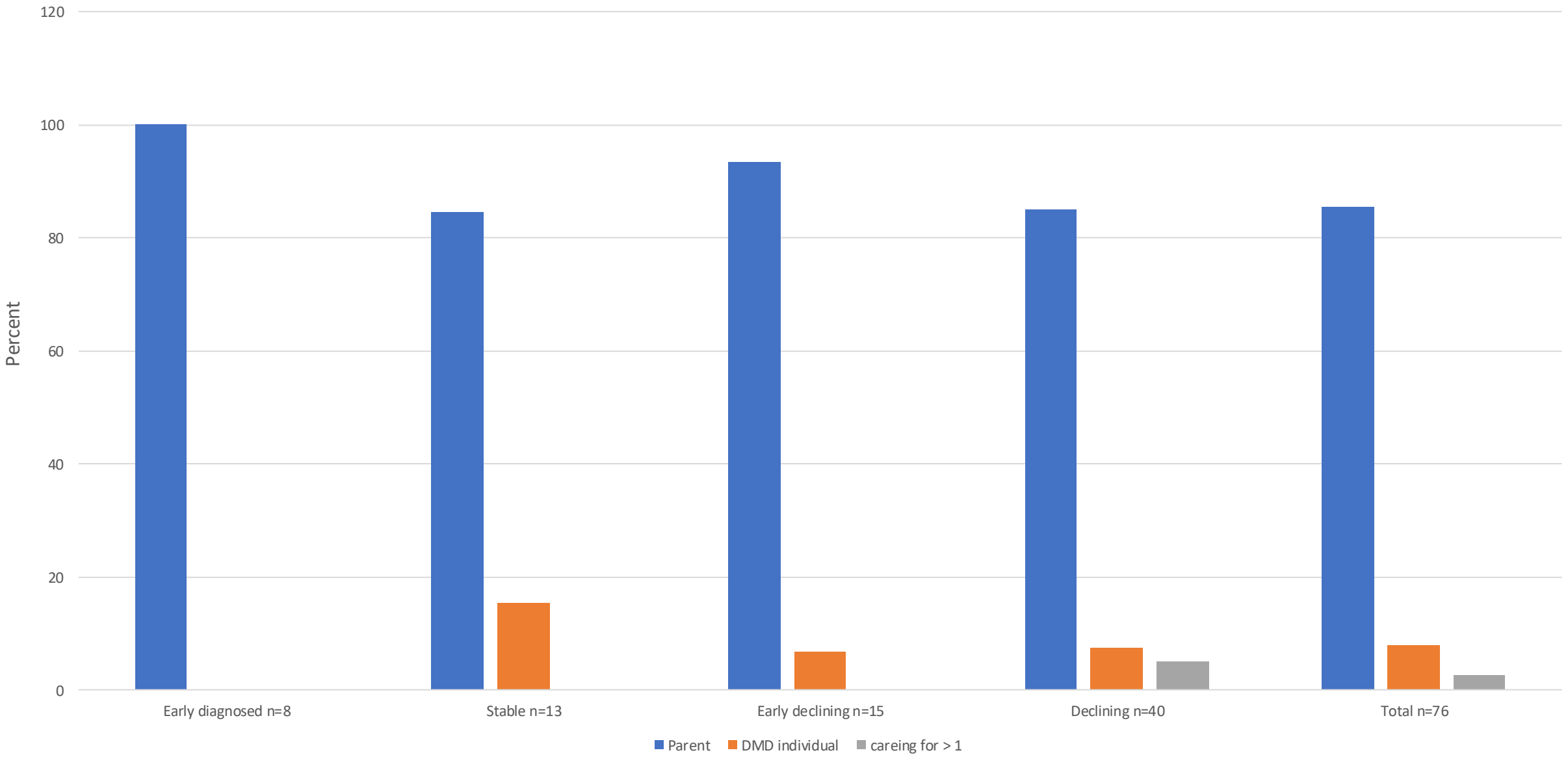
Methodology

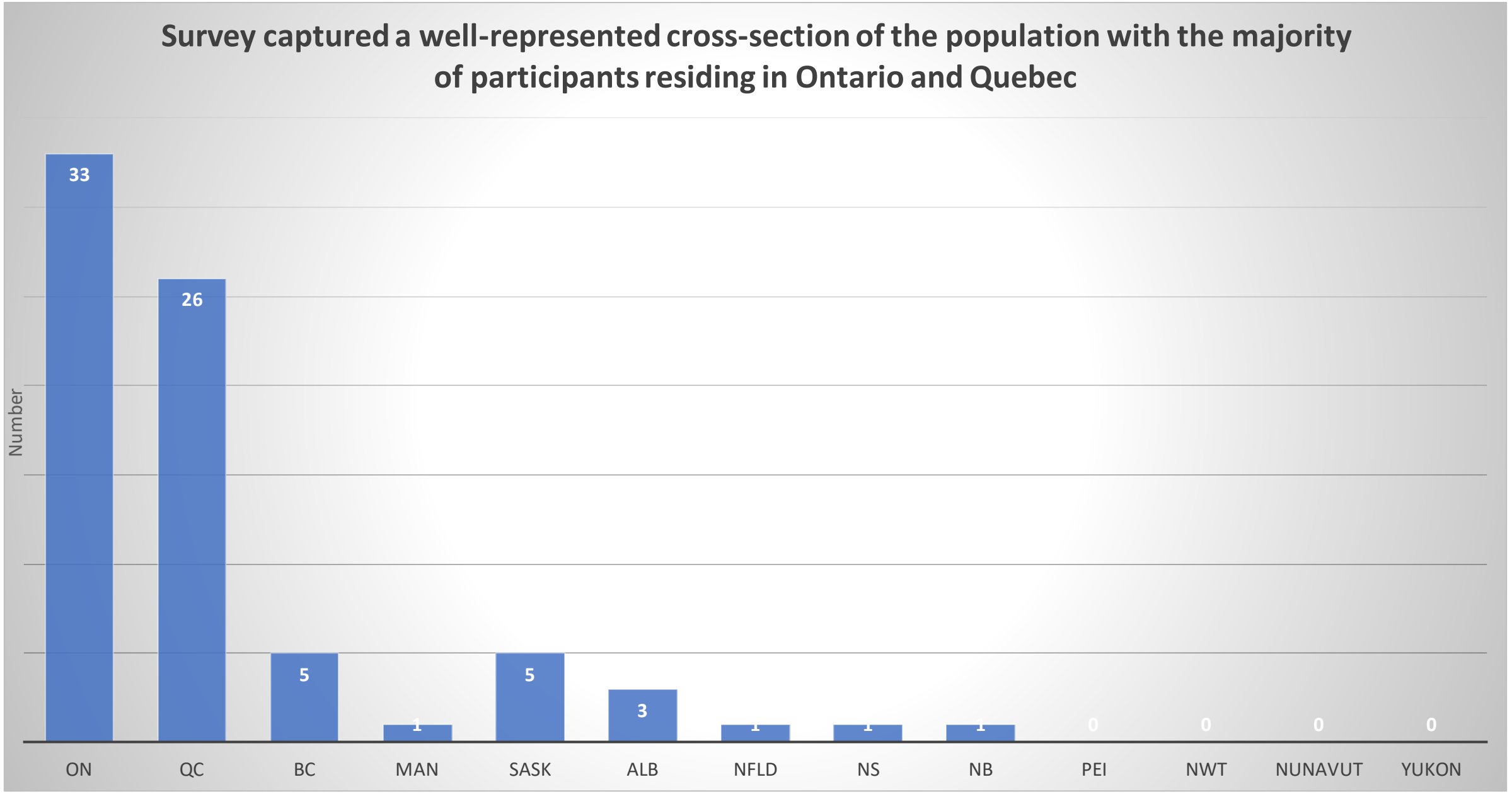
- Stand for Duchenne Canada and Jesse's Journey developed and conducted an online survey in partnership with La Force DMD to assess the Canadian patient and caregiver experience of living with Duchenne muscular dystrophy (Duchenne).

- The survey was distributed through our Canadian Duchenne network of families between July 26 and August 7, 2019.

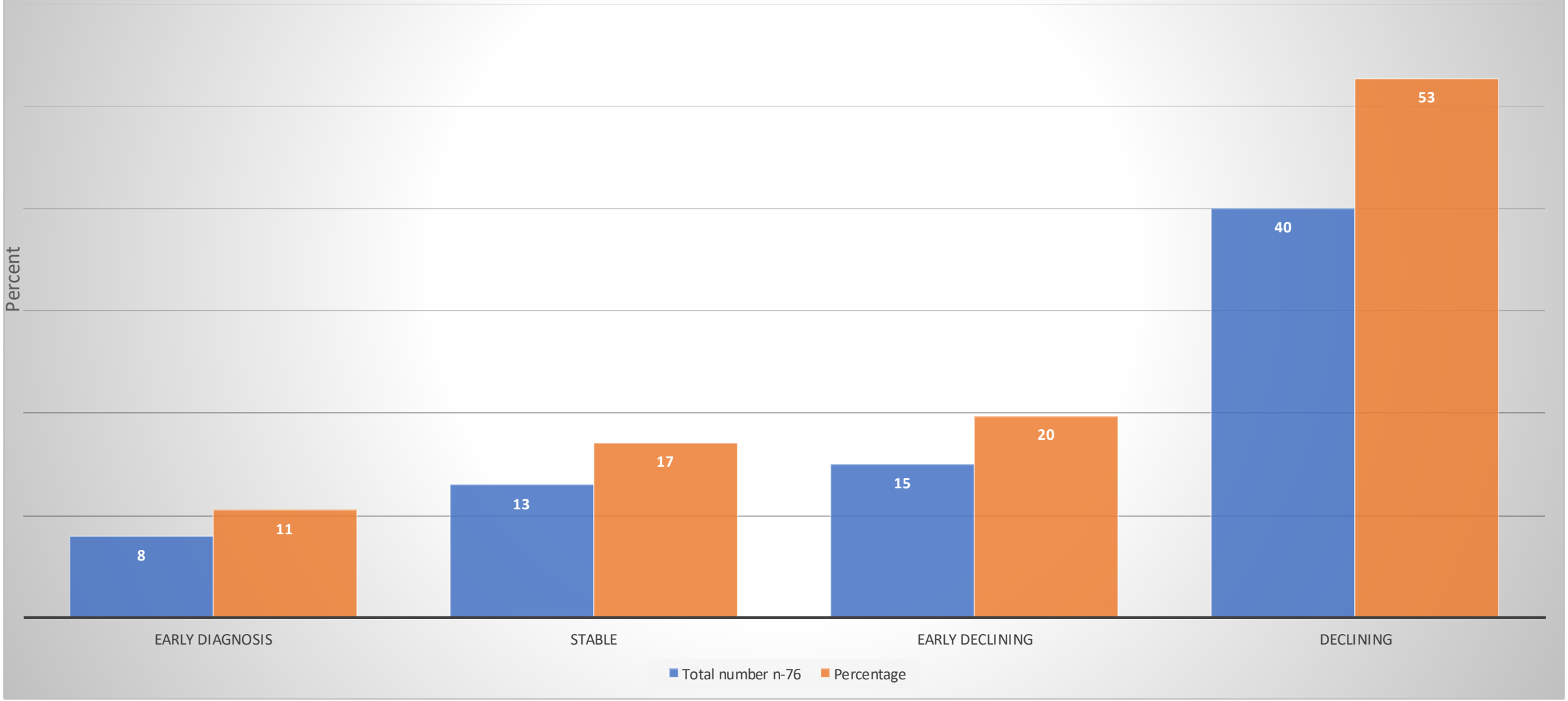
- Impetus Digital built the online platform, anonymize and manage the responses, and provide us with the completed data.

- All question development and data analyses were conducted independently by Stand for Duchenne and Jesse's Journey.

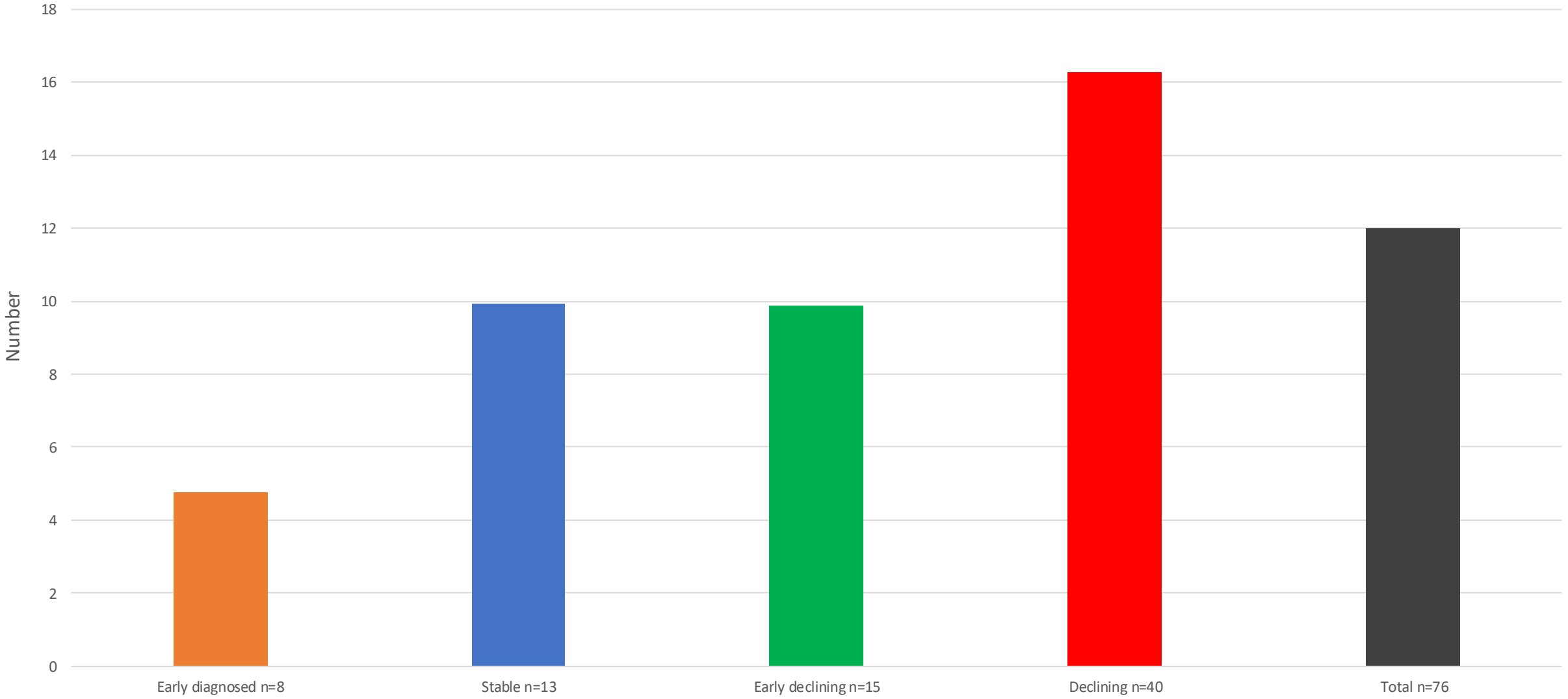

Out of a total of 77 participants, 76 were included, most of whom were caregivers; 6 were individuals with Duchenne
120
100
80
60
40
20
0
Percent
Early diagnosed n=8
Stable n=13
Early declining n=15
Declining n=40
Total n=76
Parent
DMD individual
careing for > 1


Survey captured a well-represented cross-section of the population with the majority of participants residing in Ontario and Quebec
Number
33
26
5
1
5
3
1
1
1
0
0
0
0
ON
QC
BC
MAN
SASK
ALB
NFLD
NS
NB
PEI
NWT
NUNAVUT
YUKON

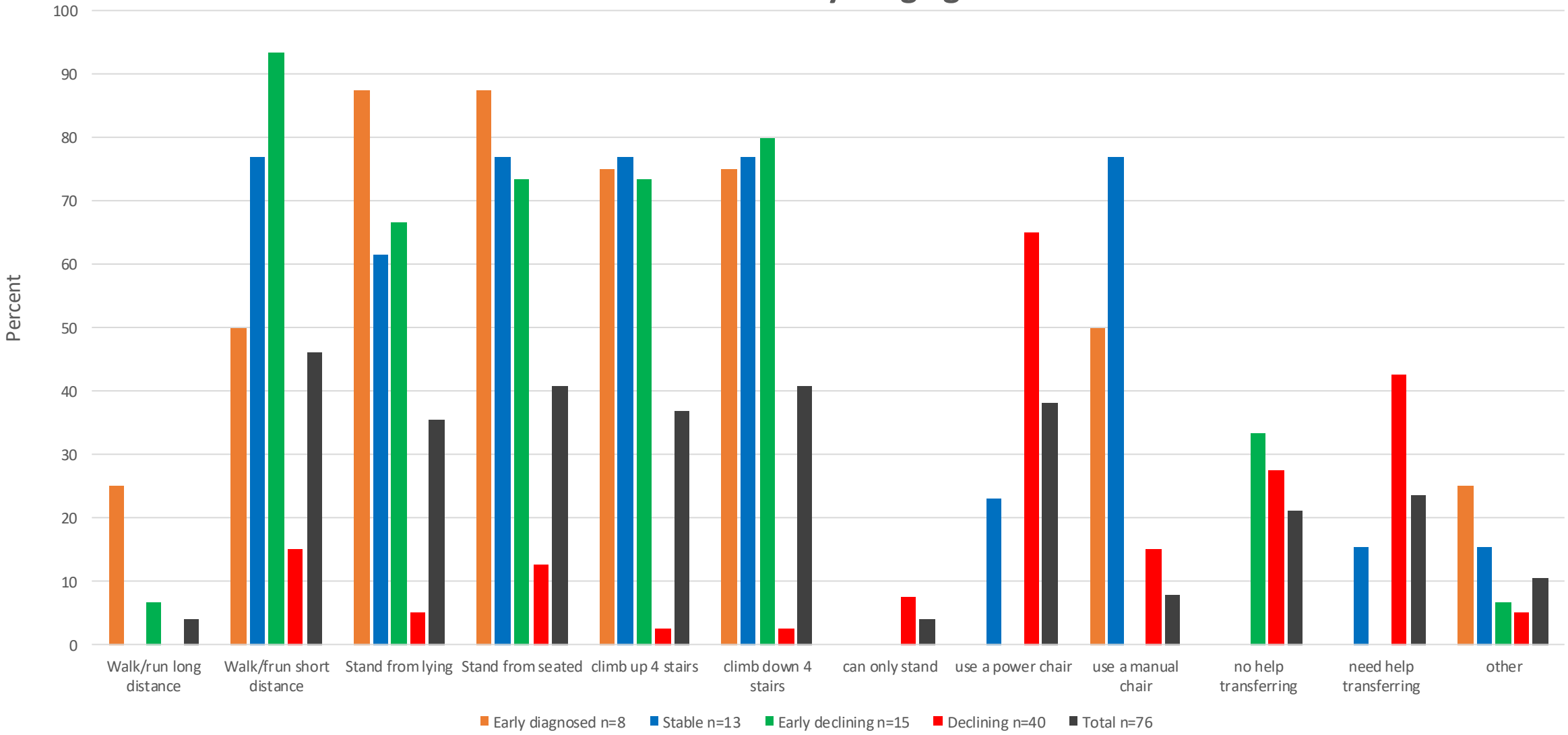
Subgroup analyses was conducted in order to capture the different needs and priorities by stage of disease, half of the participants surveyed represented the declining phase



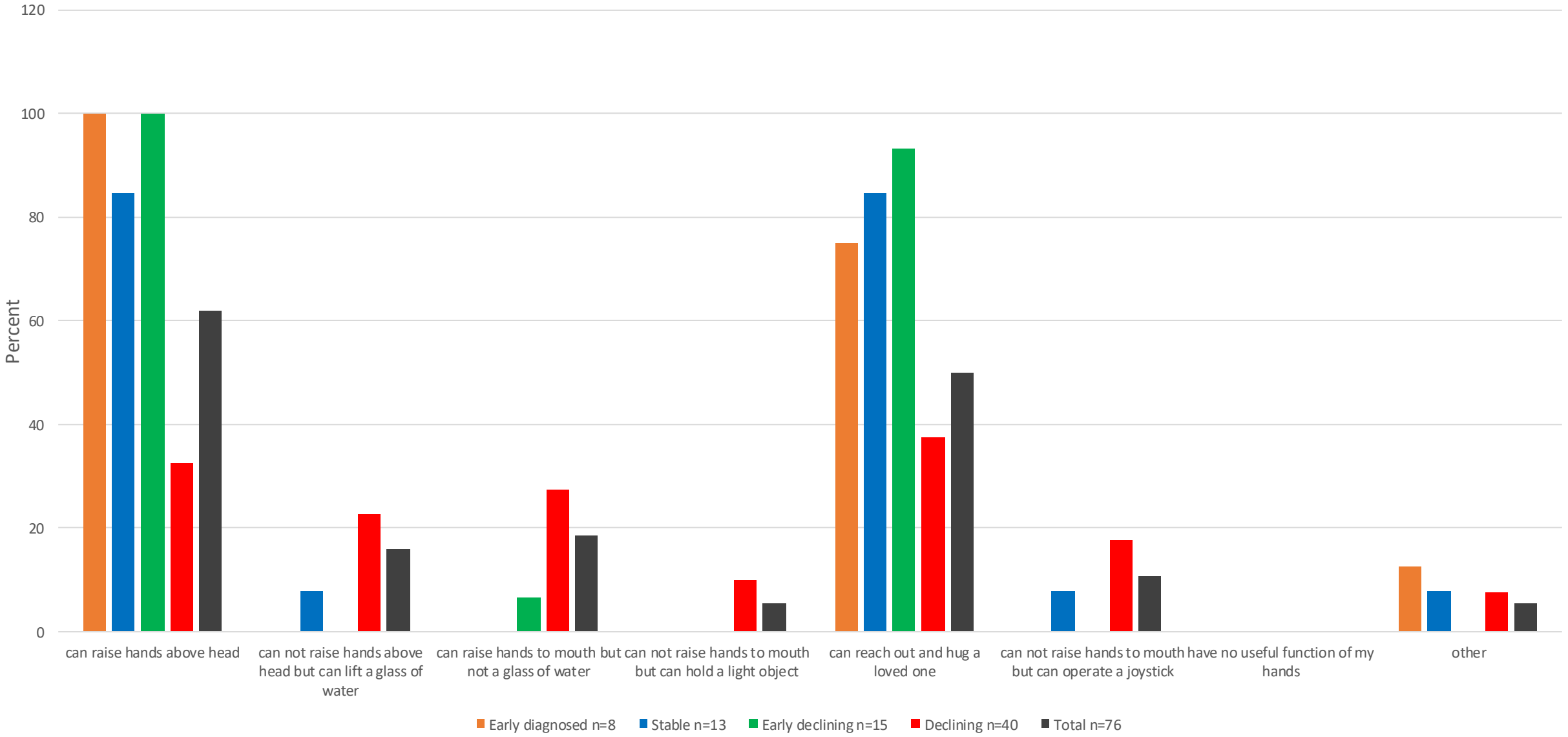
Age ranged from 2 to 25+ years. The median age was 12 years and including 10 individuals over 25



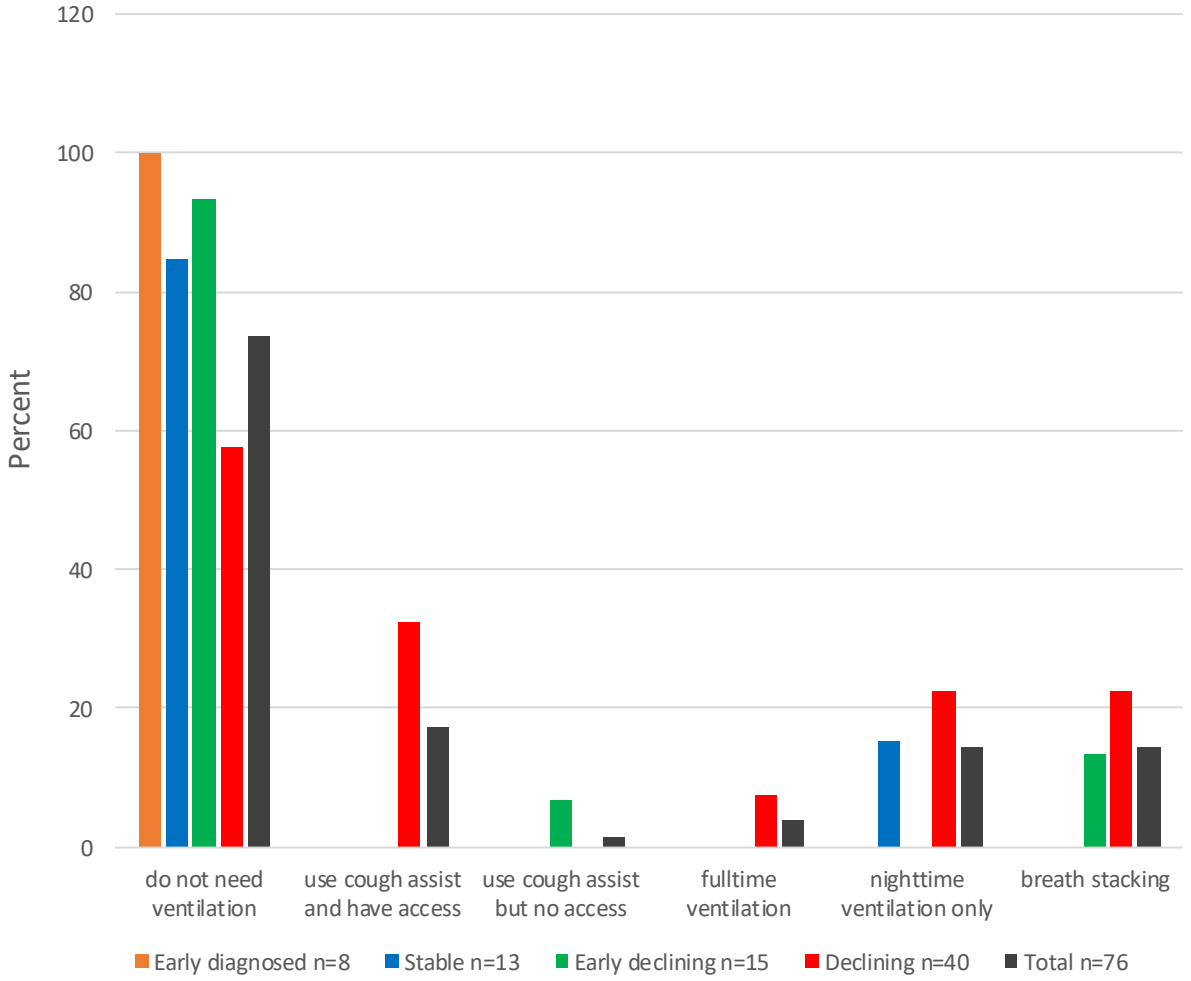
The progressive nature of muscle weakness in Duchenne leads to loss of lower limb function indicated by the need for a wheelchair, inability to stand or climb stairs at a young age

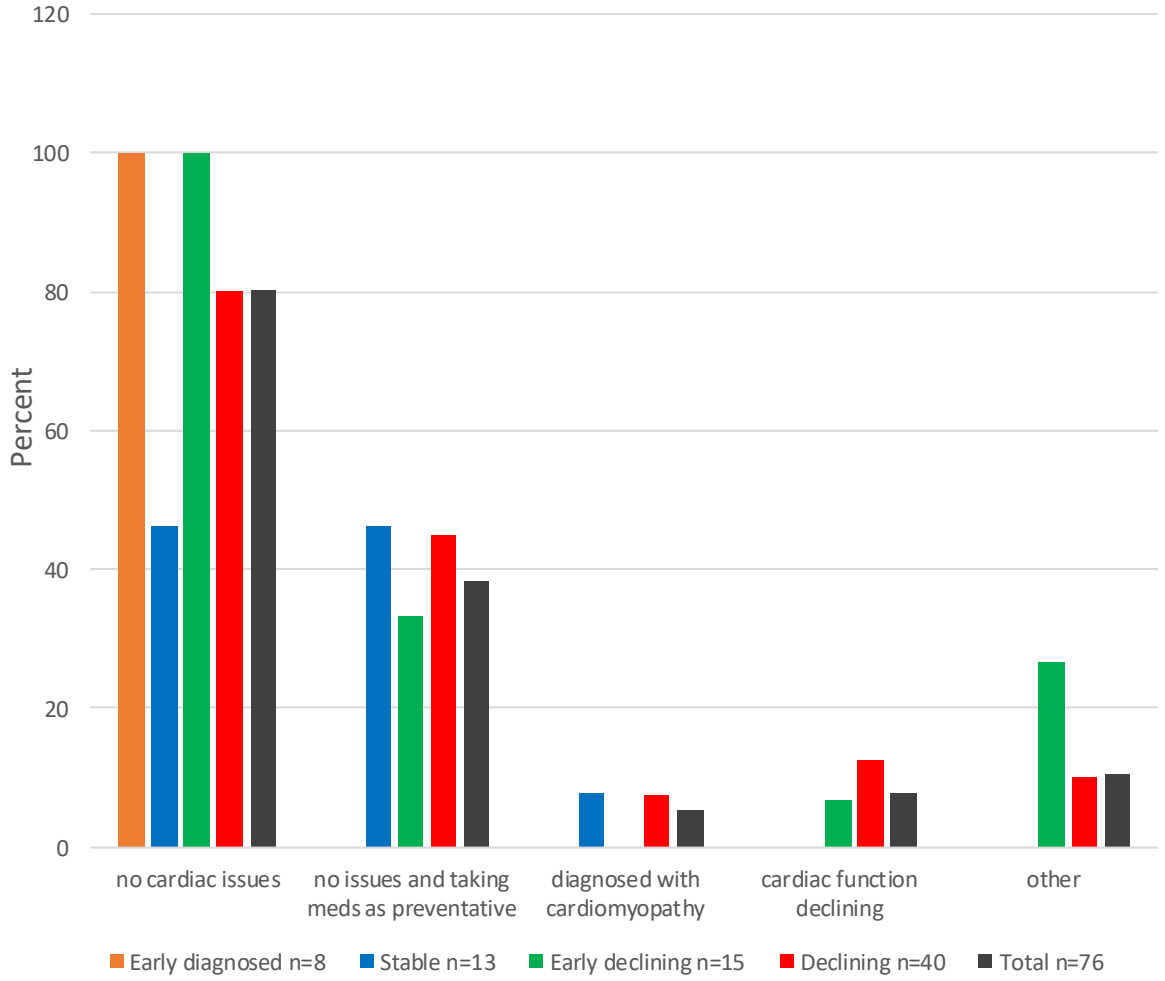


Upper limb function in non-ambulant children/young adults is vital for performing activities of daily living and declines as the disease progresses

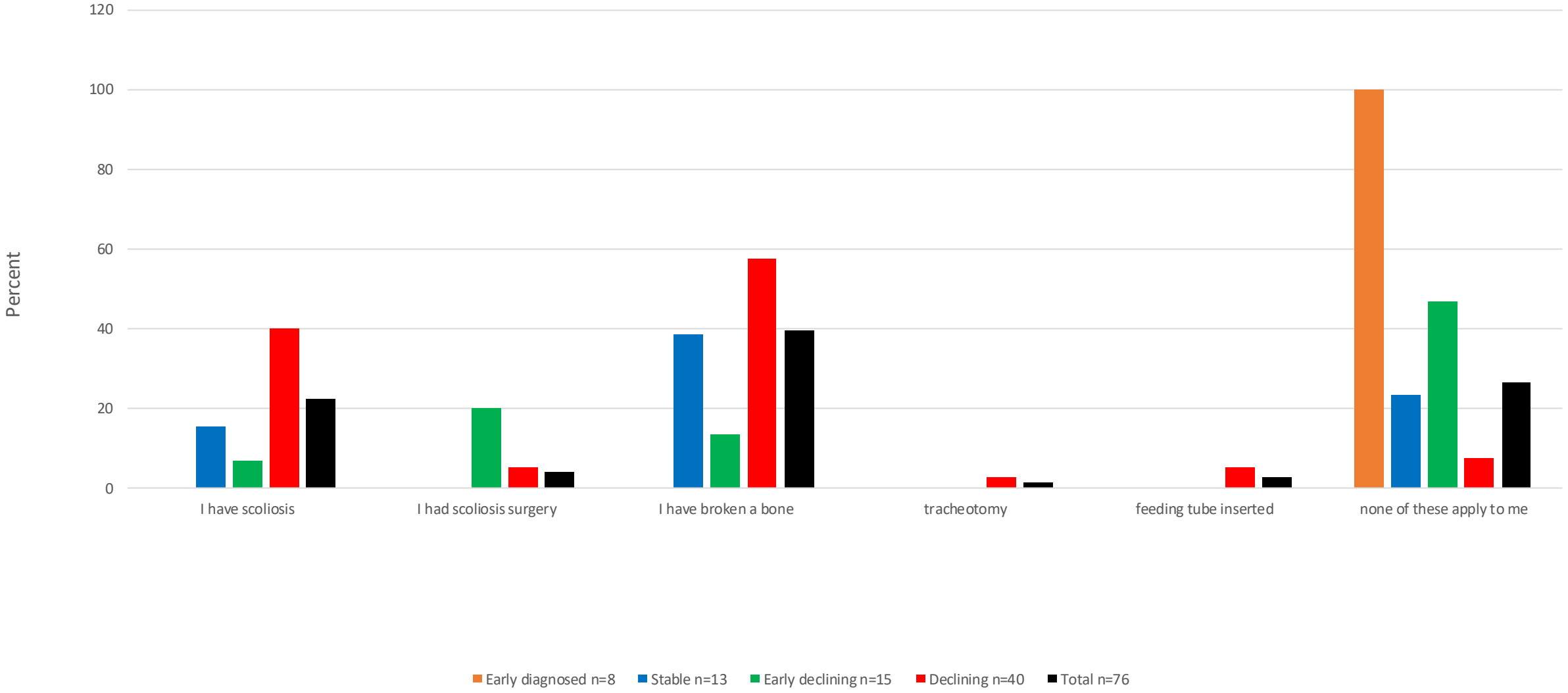


Declining lung and cardiac function, left untreated, is an inevitable milestone no parent looks forward too

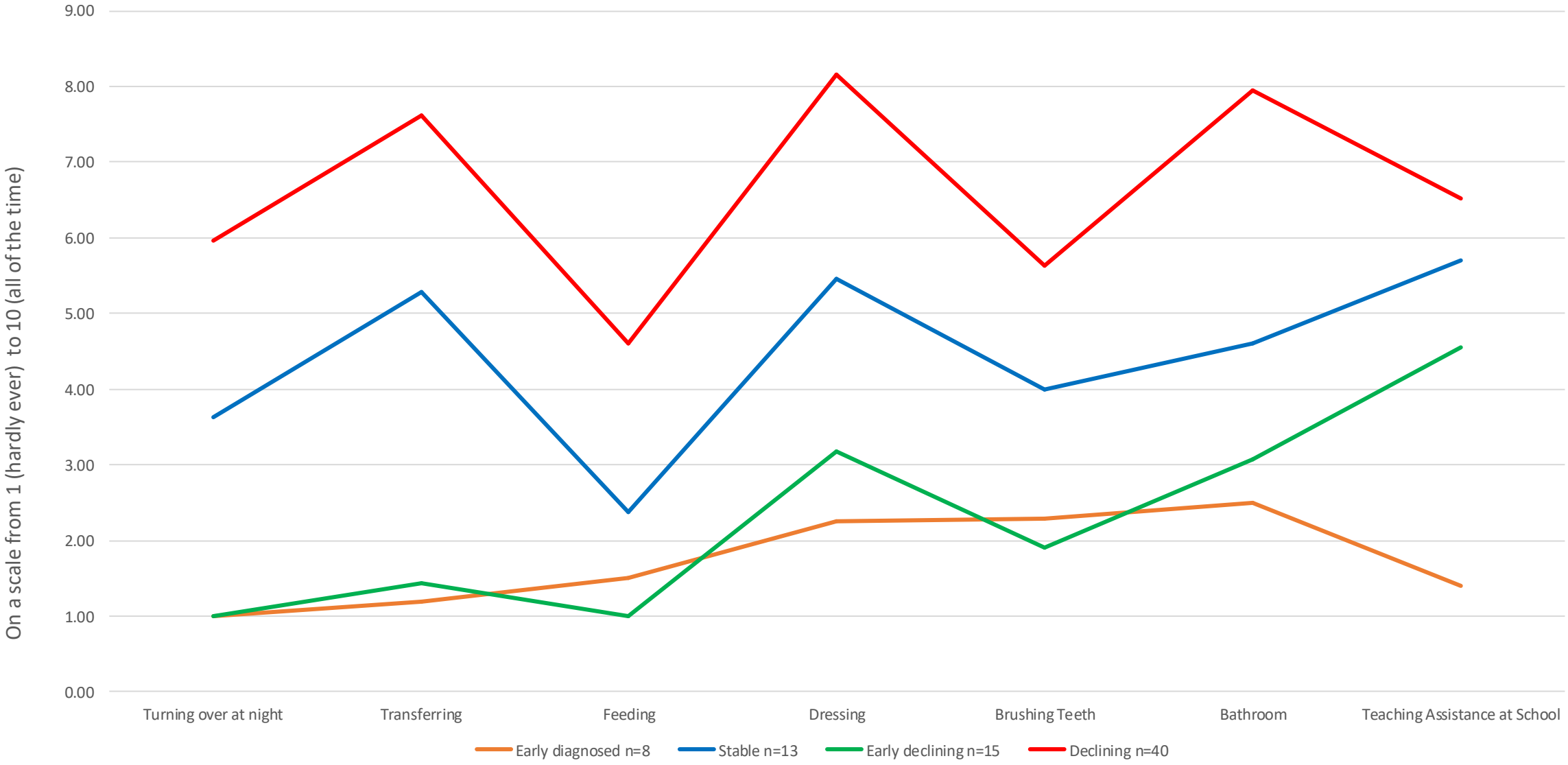




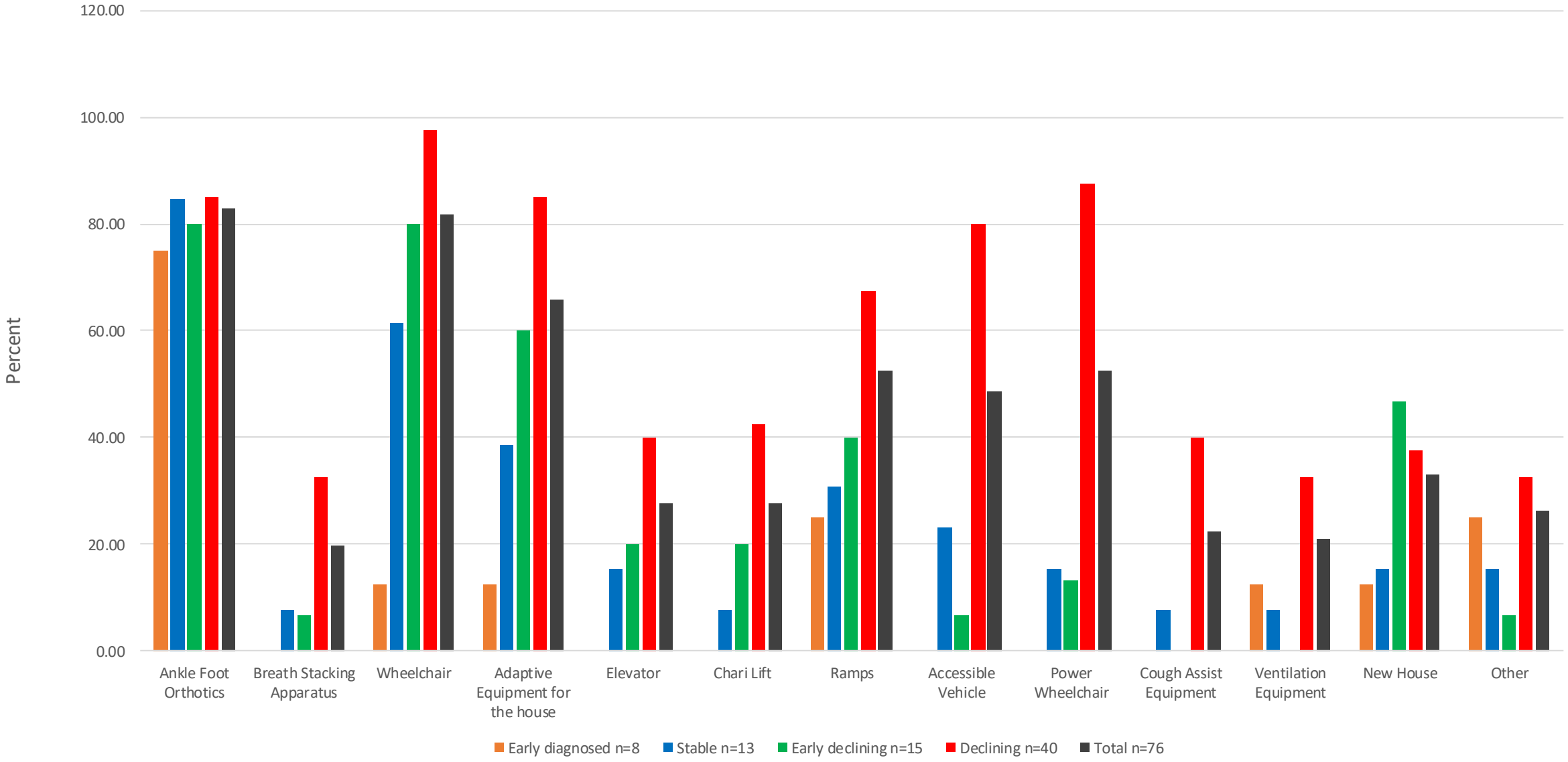
Early diagnosis and treatment of Duchenne is important as disease related complications such as scoliosis, broken bones and need for feeding tubes increase as the disease progresses



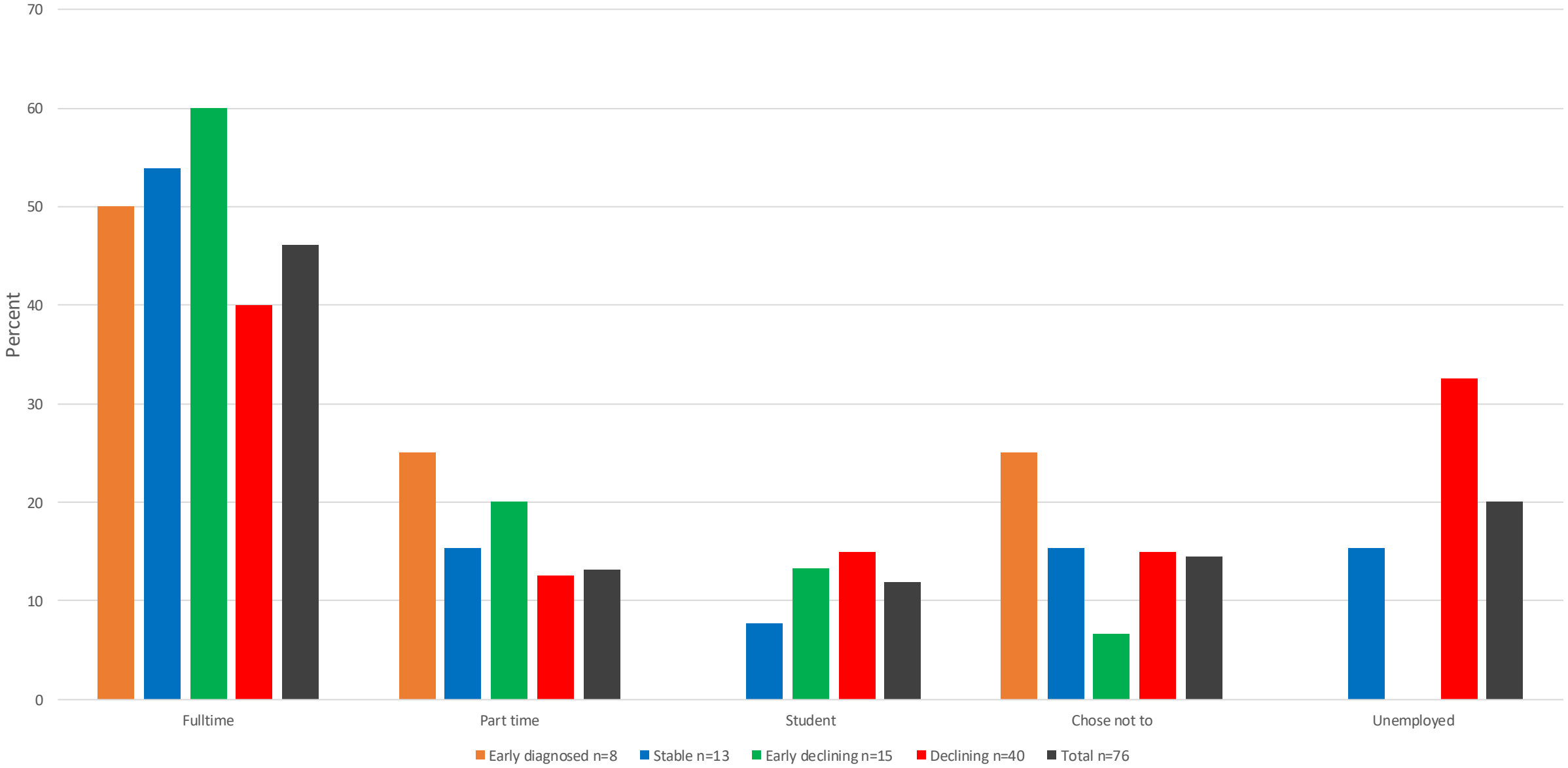
Duchenne robs teenagers and young adults of their independence as they require more help to perform daily tasks

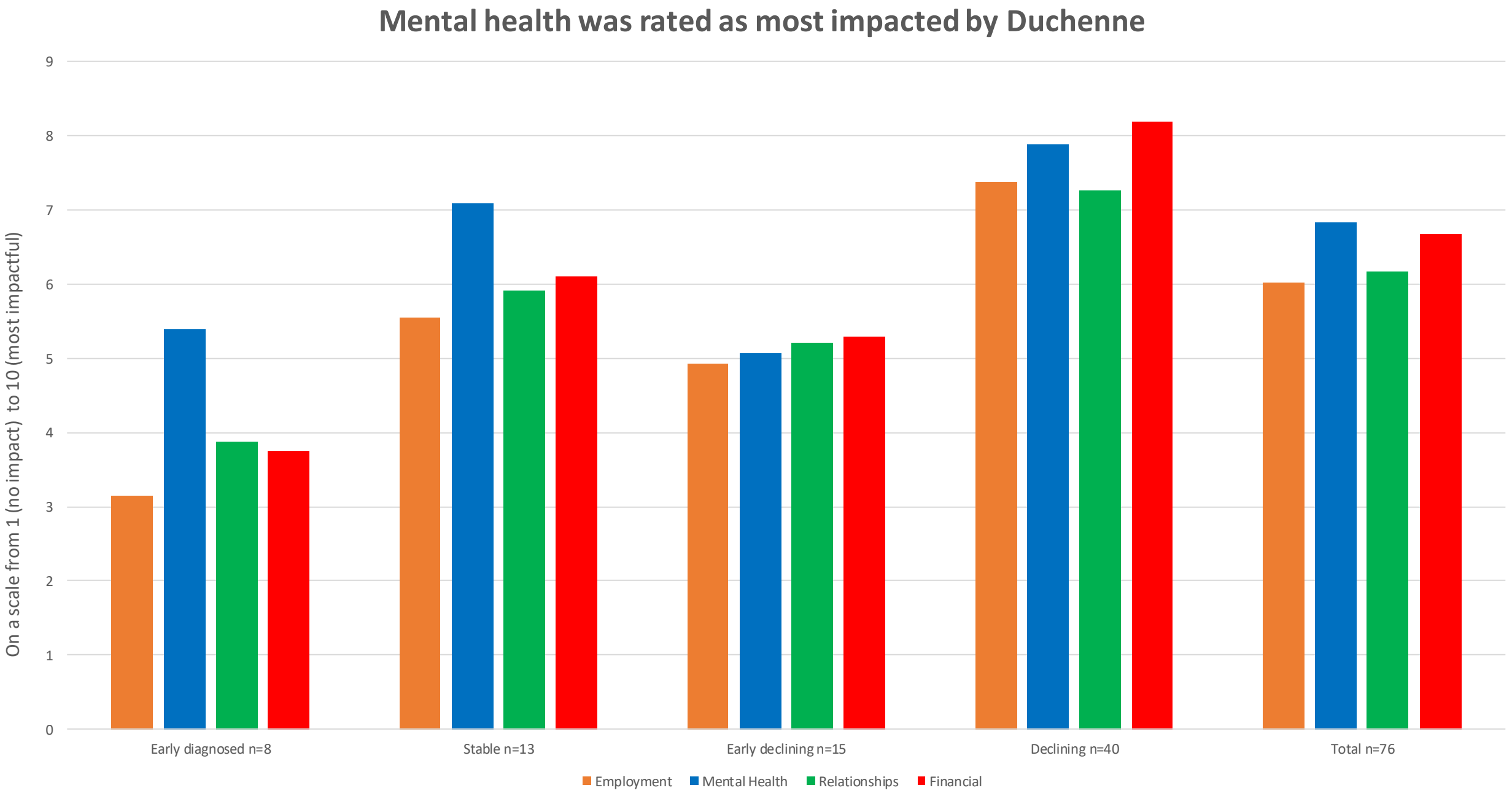


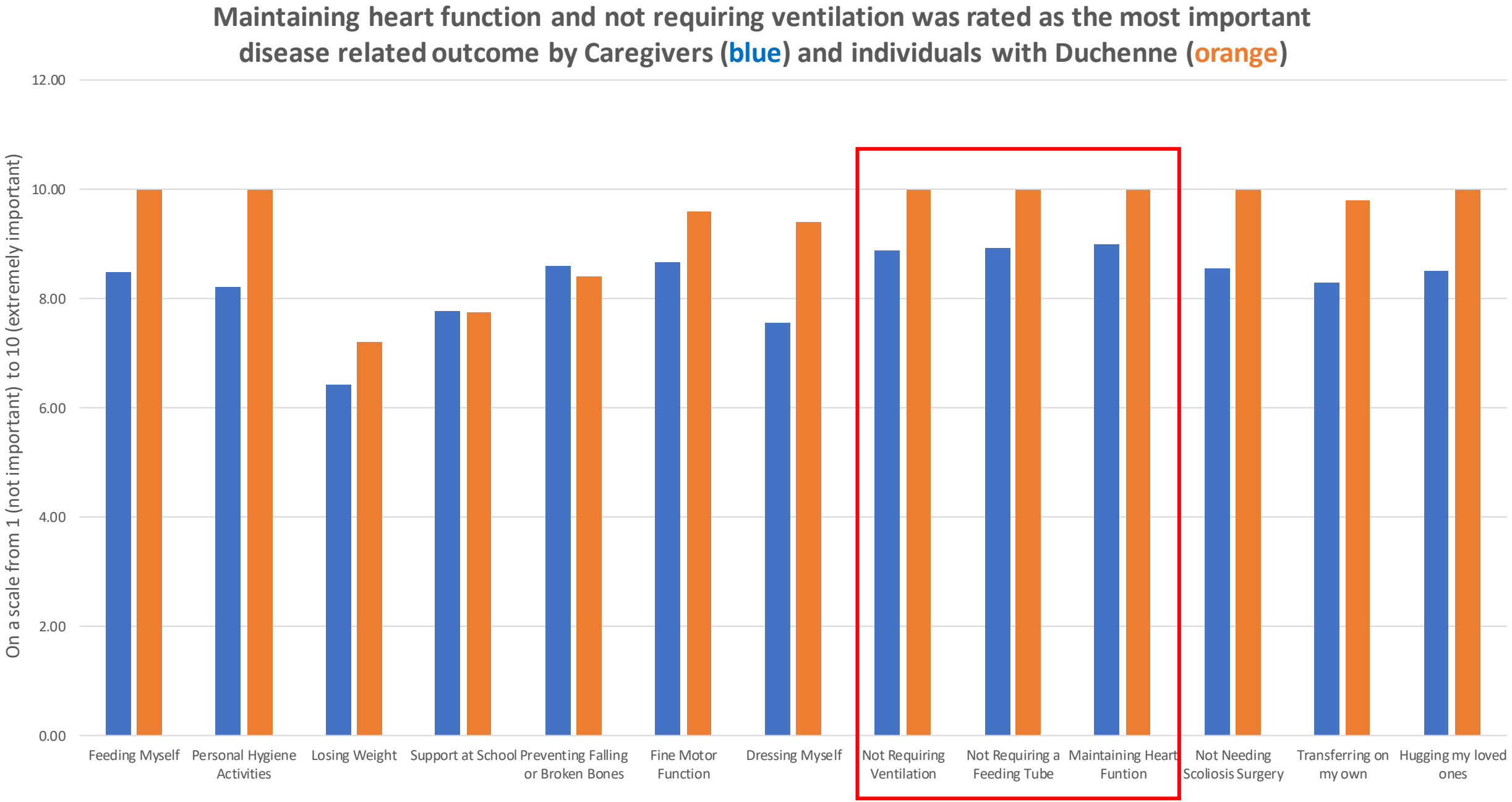
The financial burden increases on families as the need for more supportive equipment and their accommodation increases as the disease progresses



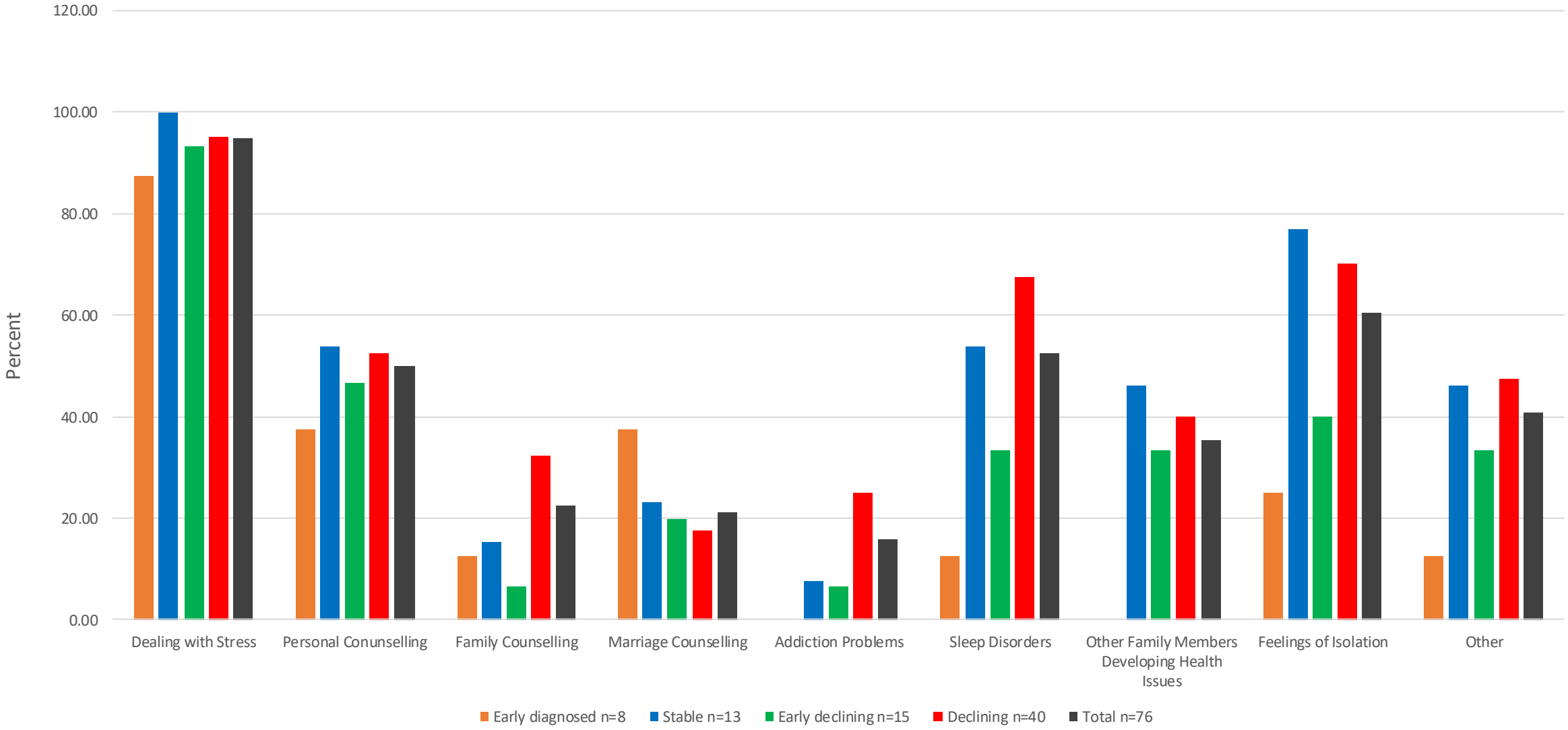
Employment becomes harder for Duchenne parents as their caregiving responsibilities increase with the progression of the disease




Mental health was rated as most impacted by Duchenne
On a scale from 1 (no impact) to 10 (most impactful)
0
1
2
3
4
5
6
7
8
9
Early diagnosed n=8
Stable n=13
Early declining n=15
Declining n=40
Total n=76
Employment
Mental Health
Relationships
Financial

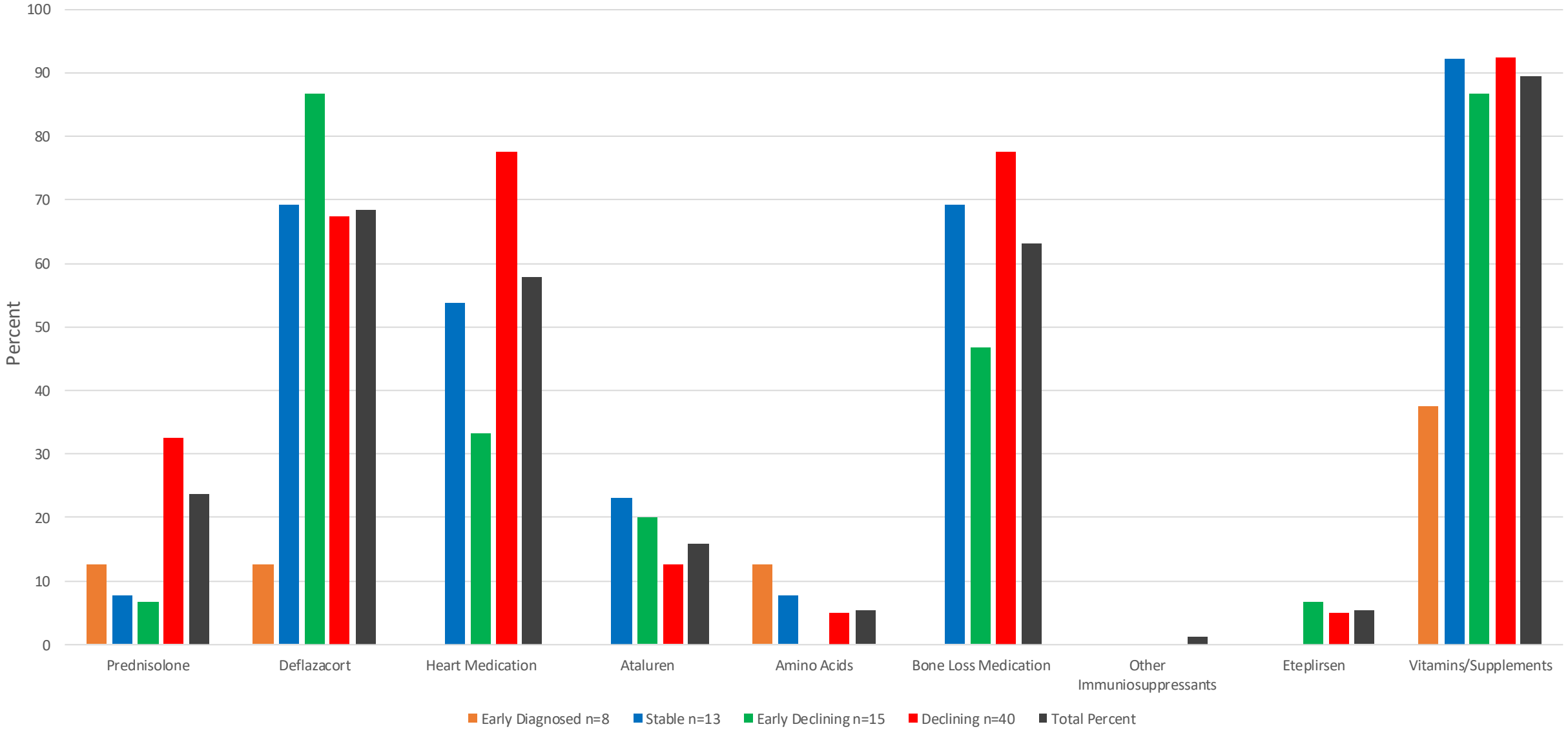

Maintaining heart function and not requiring ventilation was rated as the most important disease related outcome by Caregivers (blue) and individuals with Duchenne (orange)
On a scale from 1 (not important) to 10 (extremely important)
12.00
10.00
8.00
6.00
4.00
2.00
0.00
Feeding Myself
Personal Hygiene Activities
Losing Weight
Support at School
Preventing Falling or Broken Bones
Fine Motor Function
Dressing Myself
Not Requiring Ventilation
Not Requiring a Feeding Tube
Maintaining Heart Funtion
Not Needing Scoliosis Surgery
Transferring on my own
Hugging my loved ones

Living with Duchenne has severe impact on the life and wellbeing of individuals and their families, dealing with stress was rated as having the highest impact

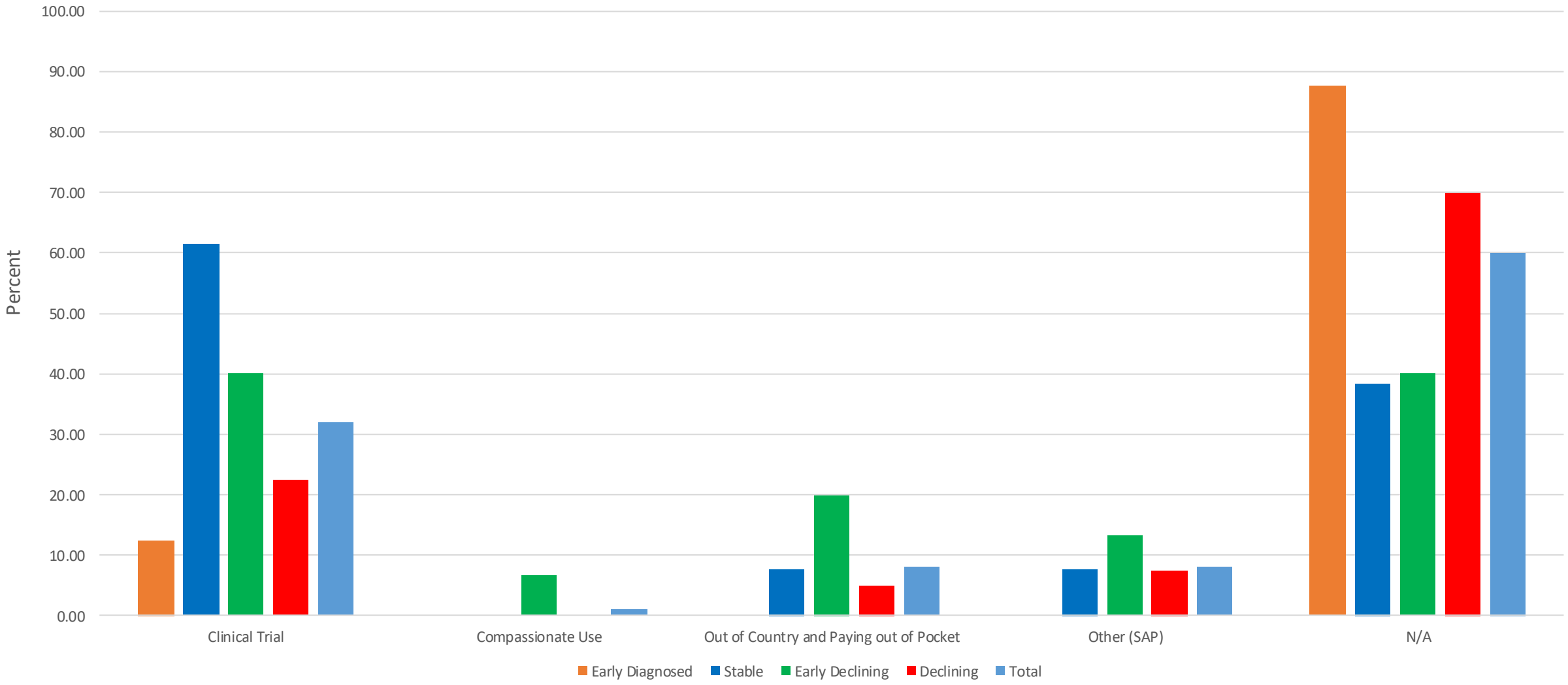


Treatment, outcomes and Risk Perception

Majority of individuals (91%) are taking corticosteroids (68% deflazacort, 24% prednisolone) followed by heart medications and bone loss medication



Clinical trial participation is highest in the stable group, with very few individuals participating in a special access program (SAP); why the Duchenne community is desperate for treatment

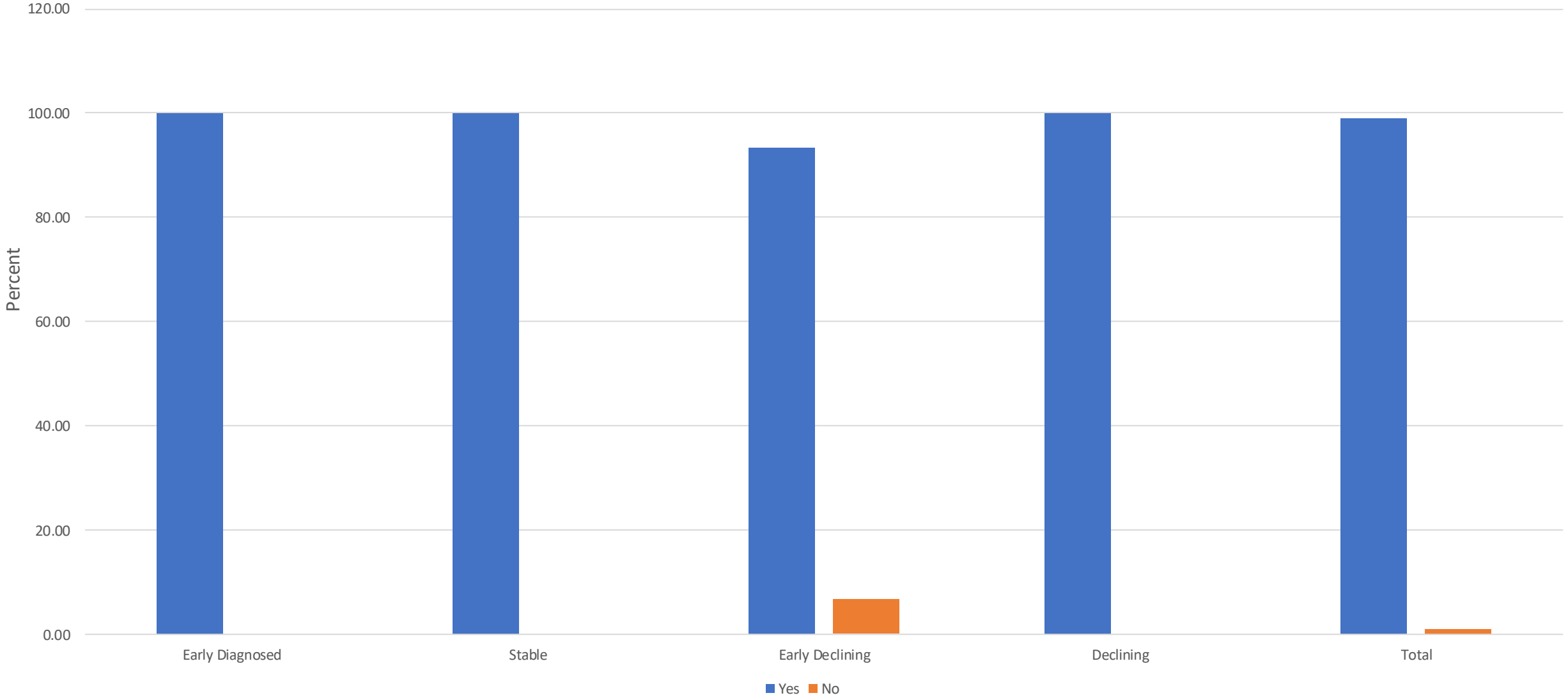


- Eleven individuals are currently taking ataluren, 9 indicated they are experiencing improvement in their QoL and 2 responded no change.

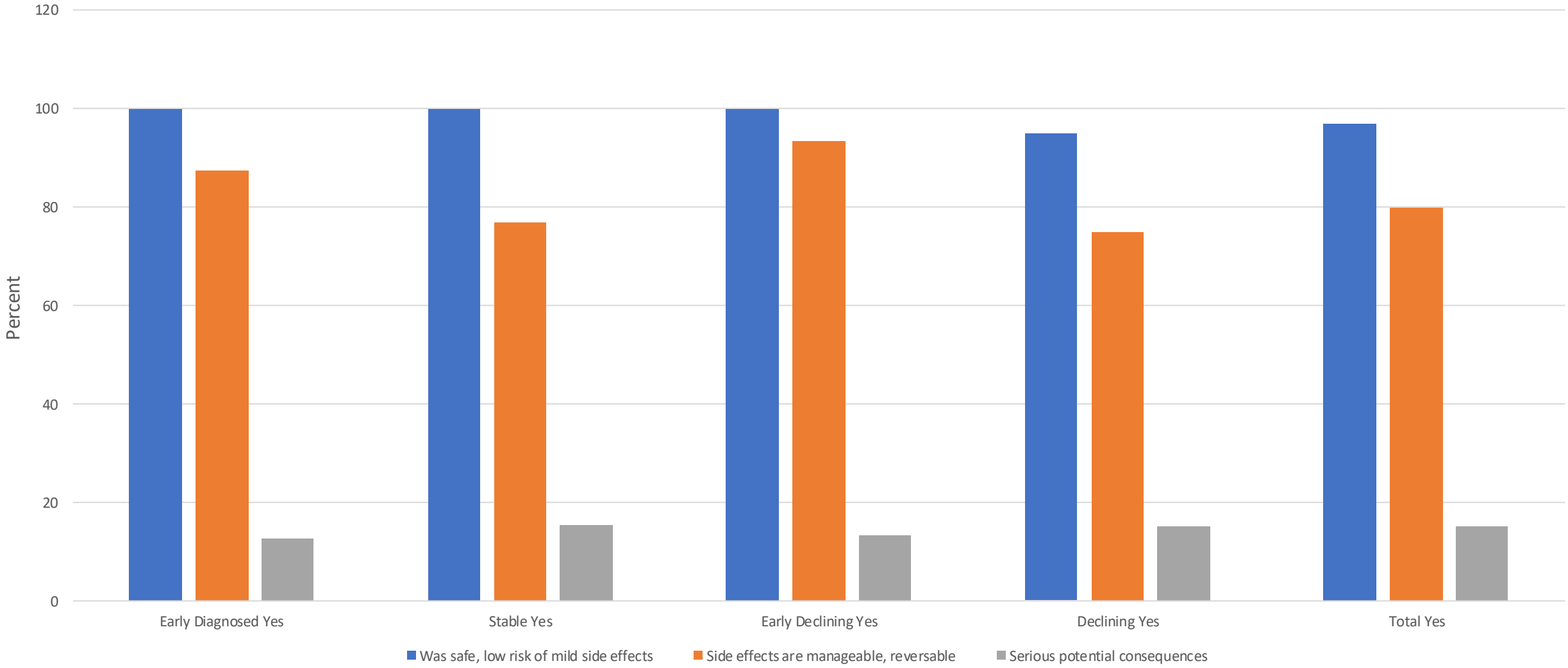
- Four individuals are currently taking Eteplirsen, 1 indicated they are experiencing improvement in their QoL and 2 responded no change. One participant did not answer this question.

- All participants regardless of therapy indicated no change was still considered a benefit.

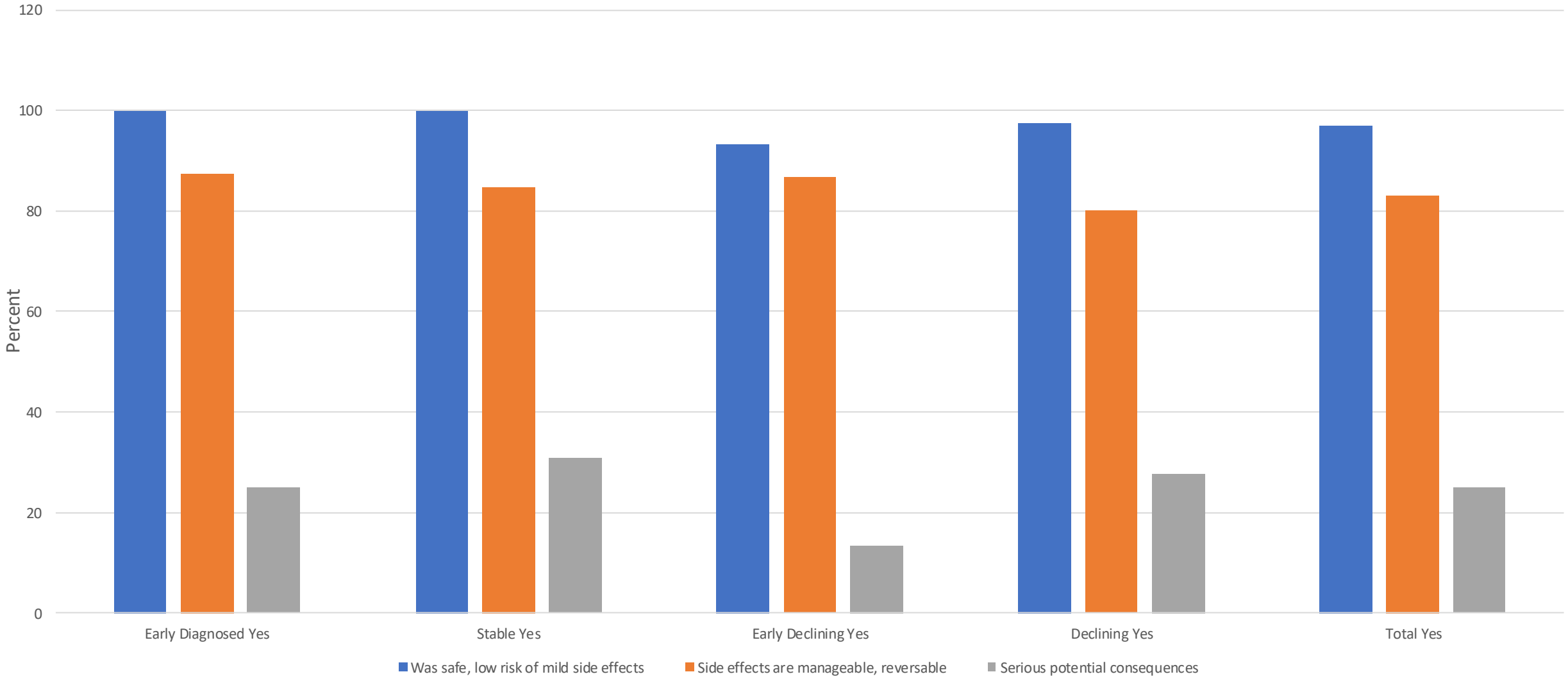
All but one participant agreed that all Canadians living with Duchenne should have immediate access to any treatment that has been proven to be safe, and has the potential to be effective, regardless of their current stage of disease



Most participants were willing to take on a low or moderate risk of treatment if the benefit was uncertain



Most participants were willing to take on a low or moderated risk of a treatment when the benefit was demonstrated in a different stage of the disease



What We Need- Access to Current Medications

- **Act on the sense of urgency and accept treatments for Duchenne considered to be safe and effective in other jurisdictions, beginning with ataluren, by making them available in this country as quickly as possible.**

  The harsh reality is that any child diagnosed with Duchenne around the time that treatments such as ataluren were available in other jurisdictions, is most likely no longer able to walk – and may be starting to experience life-threatening respiratory or cardiovascular issues.

- Given the urgency, the Duchenne community made a submission to Health Canada, which included data from the patient and caregiver survey, to support drugs under review.

Future Considerations For Emerging Treatments

- As part of Health Canada's regulatory renewal (R2D2), ensure Canadian approval of rare disease treatments can be based on reviews from other jurisdictions
- Include meaningful patient input in current and future new drug reviews, for example:
  - Routinely solicit patient input during (or prior to) NDS review
  - HC review reports, including publicly available documents (e.g. SBDs) should include a summary and assessment of patient input; and
  - Voting patient members should always be included on expert panels and advisory boards used for review of specific submissions and treatment classes

Discussion/Next Steps

- Can Health Canada ensure our submitted patient and caregiver data will be considered in the current review?
- What is Health Canada's feedback to our survey findings and how can we improve the value of the information provided for current and future reviews?
- Are there other ways we can assist with Health Canada's review of ataluren?
- Can we schedule a date for a follow up meeting now to discuss the pathway to include patient and caregiver input in future Duchenne drug reviews?