What two recent surveys told us about how SMA impacts patients' and caregivers' lives

Key findings – 05/03/2019

Disclaimers

- *This report presents unpublished findings from two surveys (survey A and survey B) that have not been peer-reviewed and should be used with due caution commensurate with the exploratory nature of the research conducted*

- *Any statements contained herein should not be used in isolation*

- *No healthcare decisions should be made based on the findings of this research solely; the aim of this research is purely to provide additional context to the impact SMA places on patient and caregivers lives in the UK*

- *Survey A was designed and administered by SMA UK in January-February 2018*

- *Survey B was developed in collaboration with SMA UK and Wickenstones, an independent strategy consultancy, sponsored through an arms-length agreement* by Biogen in December 2018-January 2019*

*Biogen has received the report but has not seen individual responses from either surveys

What the surveys asked

KEY QUESTIONS ASKED

SURVEY A

- What is the health impact of SMA on children, young people and adults with the condition who have not been treated with nusinersen?

- How is SMA managed?

SURVEY B

- What is the time, financial and work-related impact for unpaid caregivers of children, young people and adults with SMA?

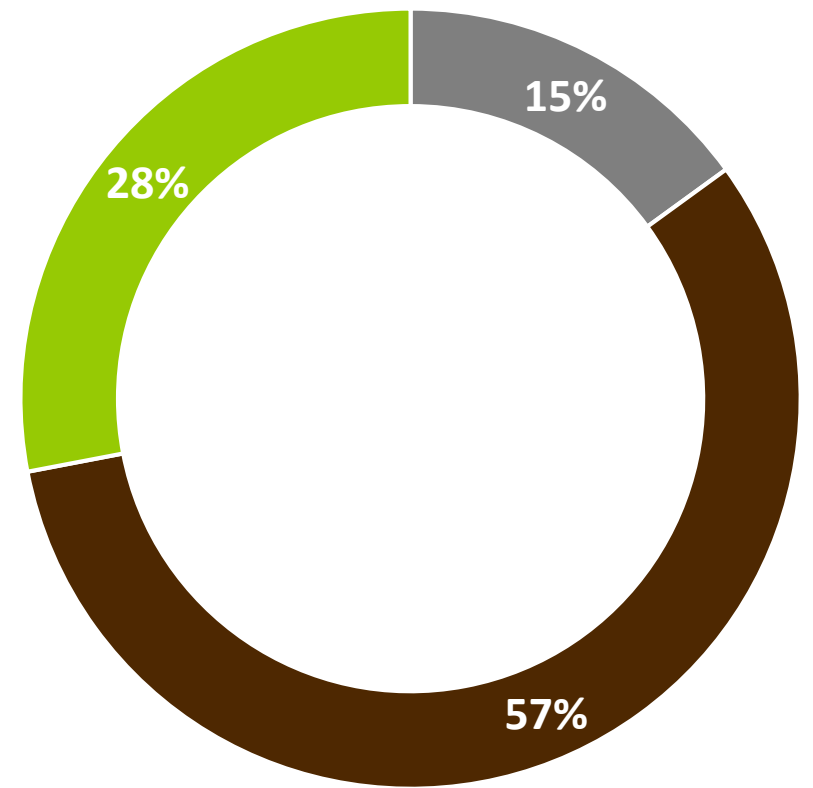# 10-15% of the UK SMA population responded to the surveys

- Approximately 1,000-1,500 people in the UK have SMA

- Survey A had too few responses from caregivers whose child has SMA type 1 to be able to include them in the results

WICKENSTONES

Survey A (n=125)

Survey B (n=188)

39%
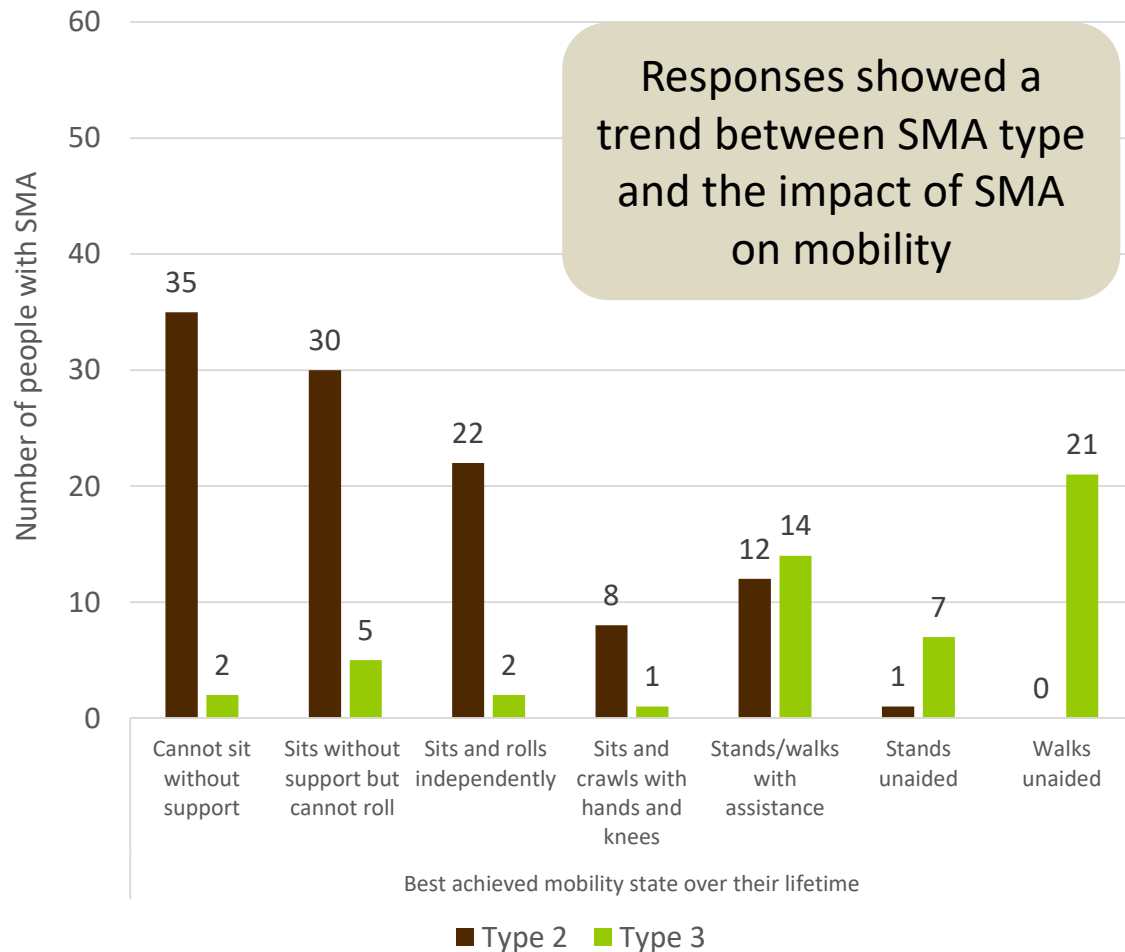
61%

28%

15%

57%
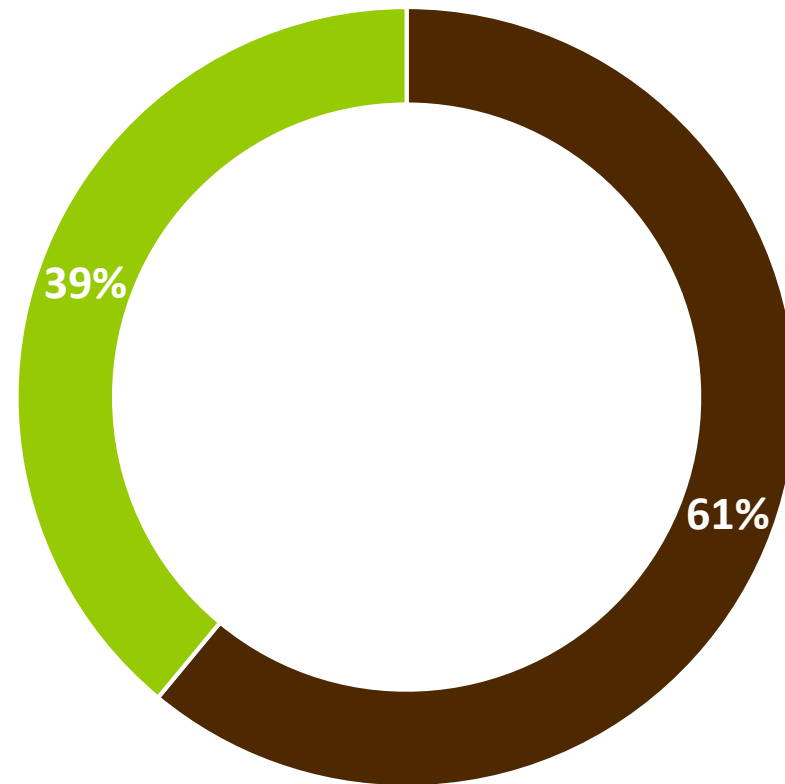
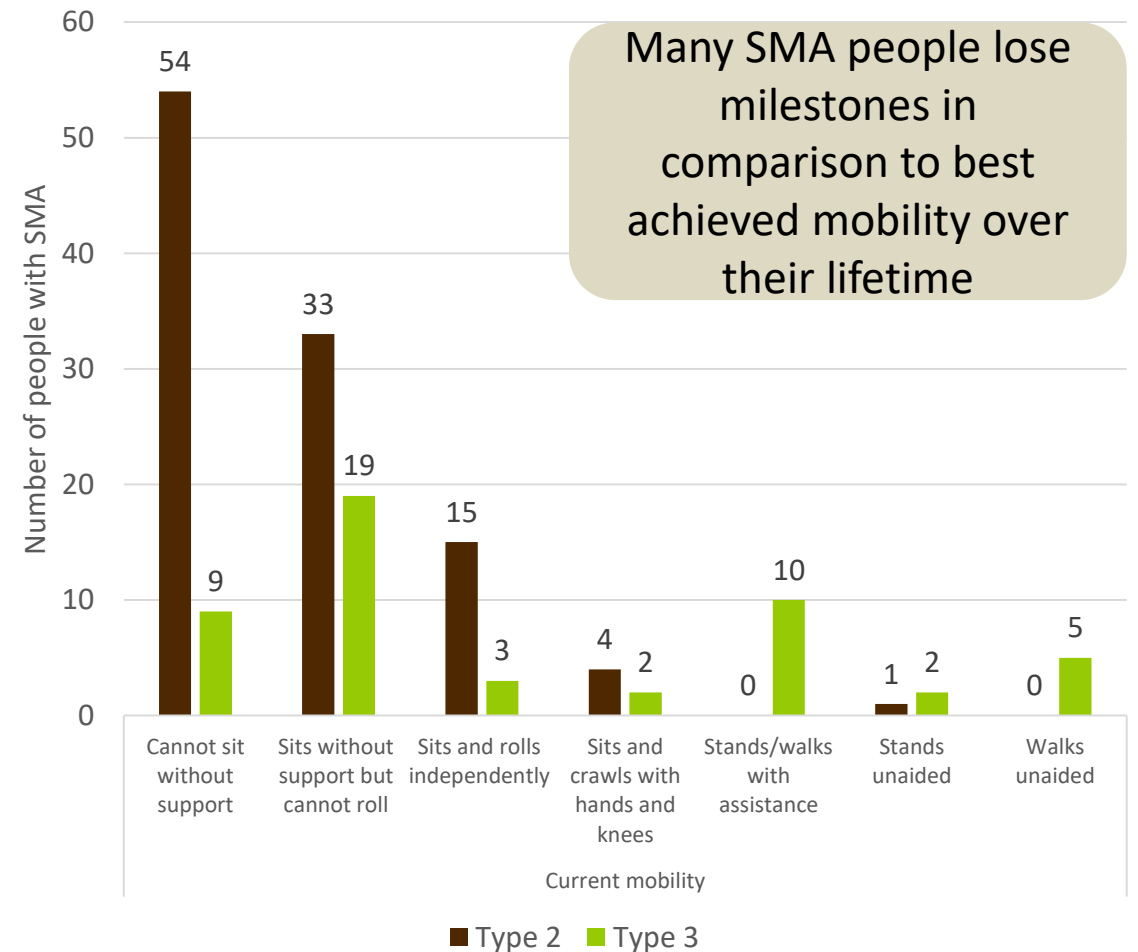■ Type 2 ■ Type 3

■ Type 1 ■ Type 2 ■ Type 3

Mobility of people with SMA deteriorates over time

WICKENSTONES

What is the best achieved lifetime mobility state achieved by the person with SMA?

Number of people with SMA

Responses showed a trend between SMA type and the impact of SMA on mobility

Best achieved mobility state over their lifetime	Type 2	Type 3
Cannot sit without support	35	2
Sits without support but cannot roll	30	5
Sits and rolls independently	22	2
Sits and crawls with hands and knees	8	1
Stands/walks with assistance	12	14
Stands unaided	1	7
Walks unaided	0	21

■ Type 2 ■ Type 3

What is the current mobility state achieved by the person with SMA?

Number of people with SMA

Many SMA people lose milestones in comparison to best achieved mobility over their lifetime

Current mobility	Type 2	Type 3
Cannot sit without support	54	9
Sits without support but cannot roll	33	19
Sits and rolls independently	15	3
Sits and crawls with hands and knees	4	2
Stands/walks with assistance	0	10
Stands unaided	1	2
Walks unaided	0	5

■ Type 2 ■ Type 3

Data is from Survey B in which 28 patients were treated with nusinersen, but no information was gathered on disease duration and duration of treatment, therefore type 1 patients have been excluded from this overview on deterioration of mobility

Over time, the number of symptoms experienced by people with SMA appears to increase

WICKENSTONES

How many additional symptoms per person with SMA?

Average number of symptoms

There is an increase in the number of symptoms on average per person over time

3.38

1.67

6.17

5.2

Per person: past

Per person: now

Type 2

Type 3

Examples of additional symptoms include: contractures, pain, chest infections/pneumonia
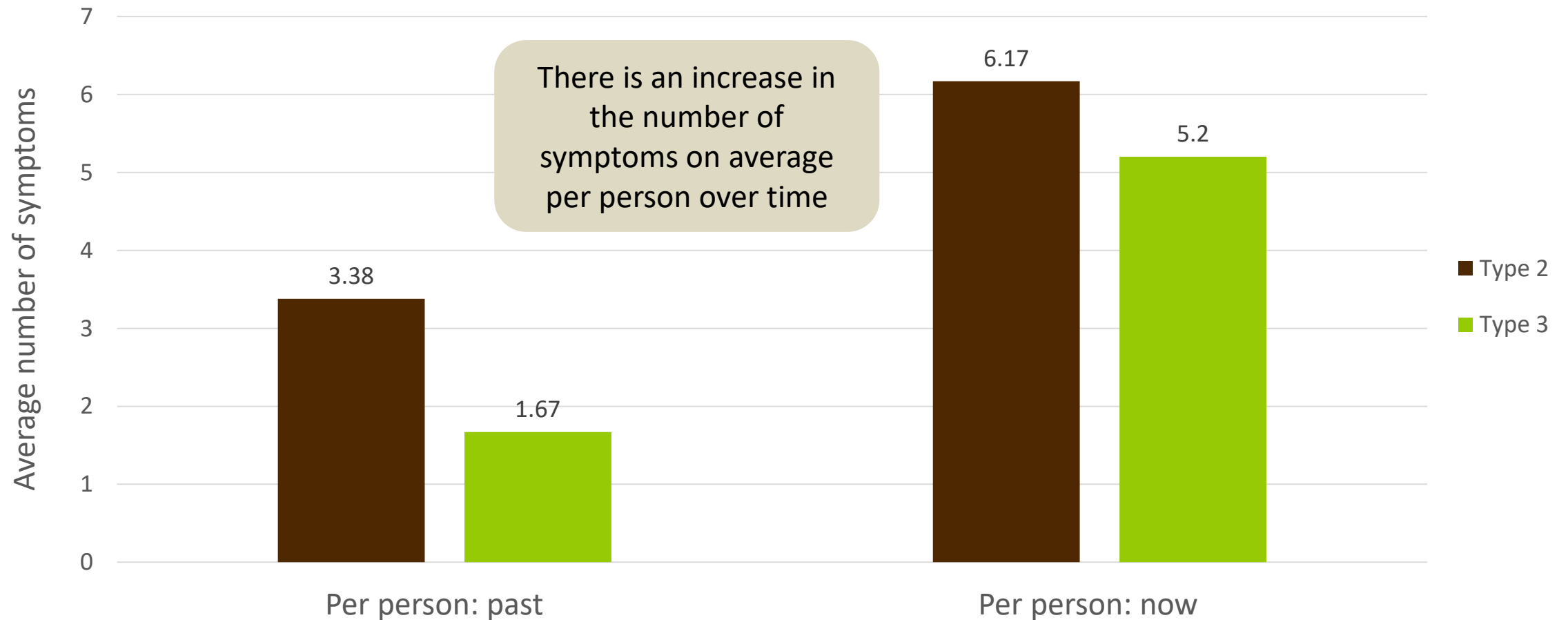
People with SMA have high levels of need for specialist equipment and health interventions

WICKENSTONES

How many interventions and what amount of equipment does the person with SMA have currently?

There are considerable levels of interventions that are not NHS funded

Average number of interventions and equipment per person with SMA

- Type 2
- Type 3

NHS funded: 8.7, 4.27
Not NHS funded: 4.29, 2.98
Wanted but not in place: 2.61, 0.76

Examples of interventions and equipment include: standing frame, specialist seating, nebuliser
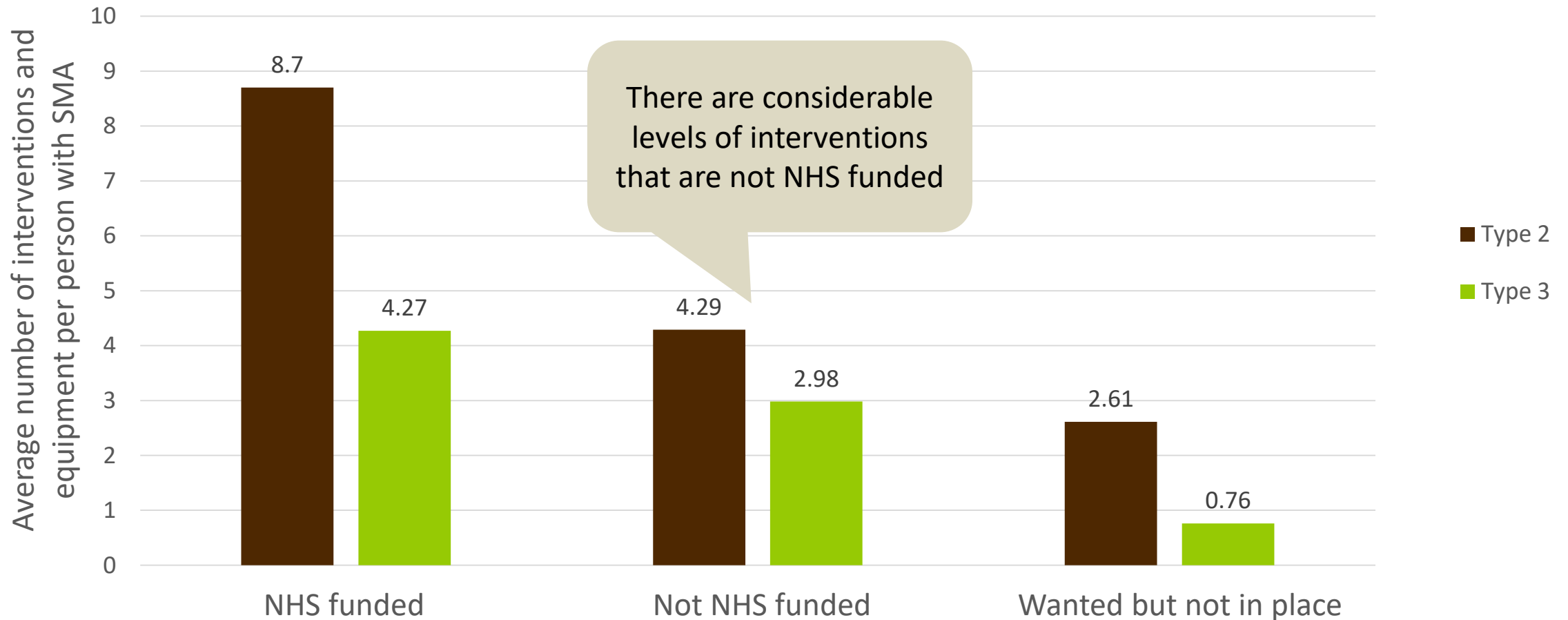
Many people (with SMA types 1, 2 & 3) see a high number of Health Care Professionals (HCPs) each year

How many HCPs are seen each year by people with SMA (types 1, 2 & 3)?

General population would be the same

But **almost one third** of people with SMA saw **more than 10 HCPs** per year

Percentage of people with SMA

- 37% — 0-5 HCPs
- 32% — 6-10 HCPs
- 16% — 11-15 HCPs
- 10% — 16-20 HCPs
- 6% — >21 HCPs

Examples of HCPs include: consultants, physiotherapists, orthotists
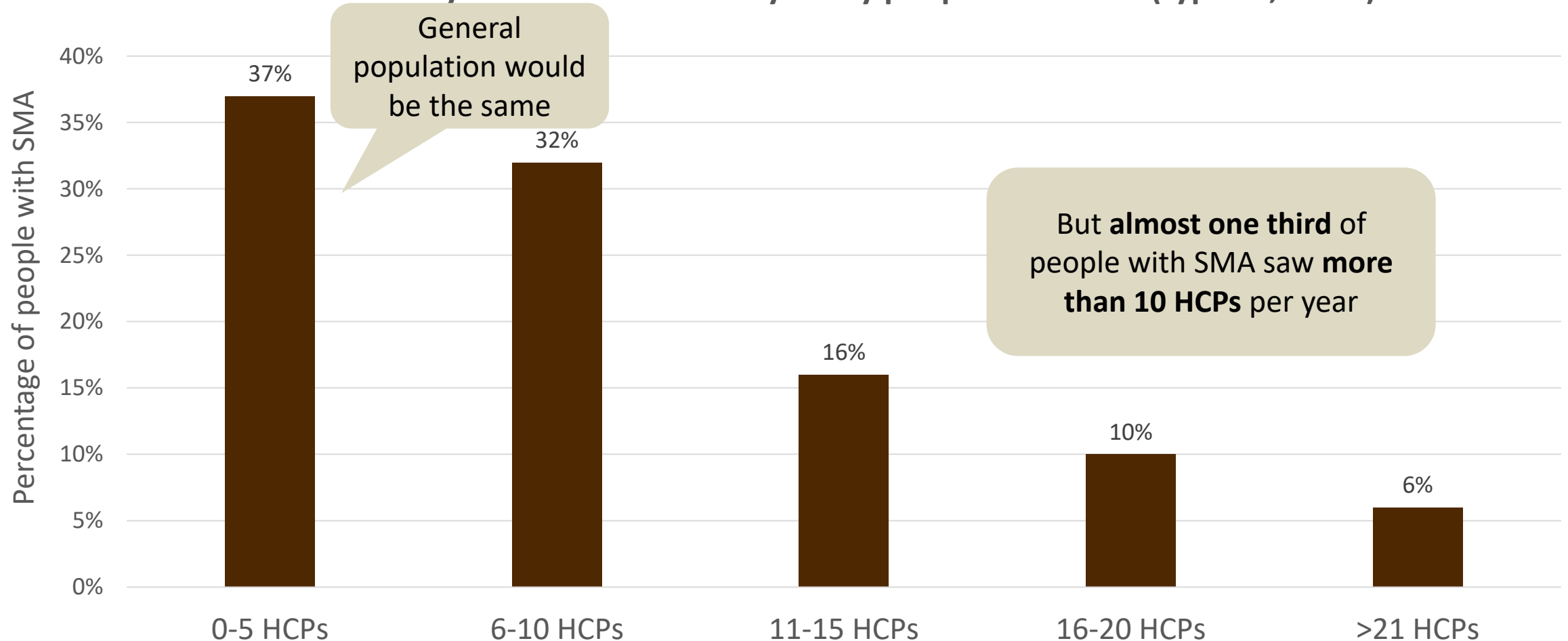
People with SMA often require "full support" with daily living activities, such as washing, dressing and preparing meals

WICKENSTONES

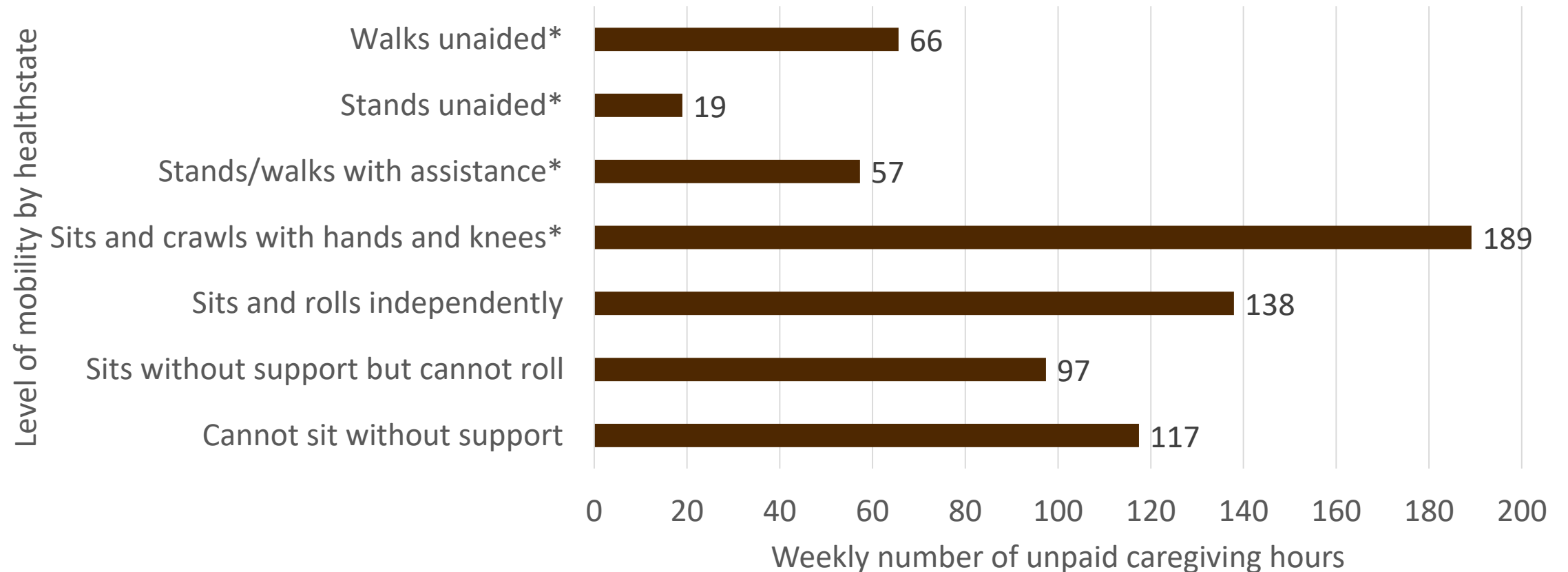## What level of support do daily living activities require?

Persons with type 2 SMA reported needing "full support" for more activities of daily living compared with persons with type 3 SMA

- Type 2
- Type 3

Average number of daily living activities per person with SMA (y-axis)

	Full support needed	Some support needed	No support needed
Type 2	5.49	0.64	0.07
Type 3	2.71	1.8	1.43

Daily living activities include: washing, dressing, toileting, transfers, eating and drinking, preparing meals and other

The total number of caregiver hours per SMA person per week can be considerable

What is the average number of unpaid caregiving hours per person with SMA?

Level of mobility by healthstate

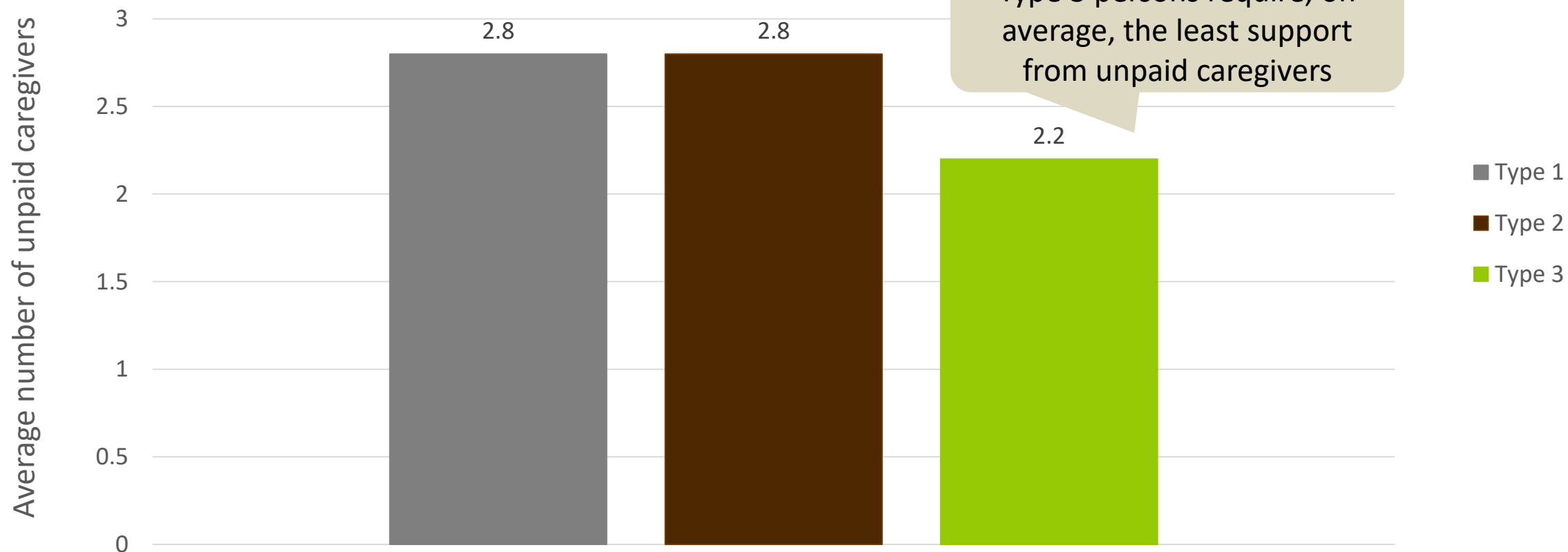|	
Walks unaided*	66
Stands unaided*	19
Stands/walks with assistance*	57
Sits and crawls with hands and knees*	189
Sits and rolls independently	138
Sits without support but cannot roll	97
Cannot sit without support	117

Weekly number of unpaid caregiving hours

*Please interpret these results with caution due to low sample size (≤10)

On average, almost three unpaid caregivers provide some support to each person who has SMA
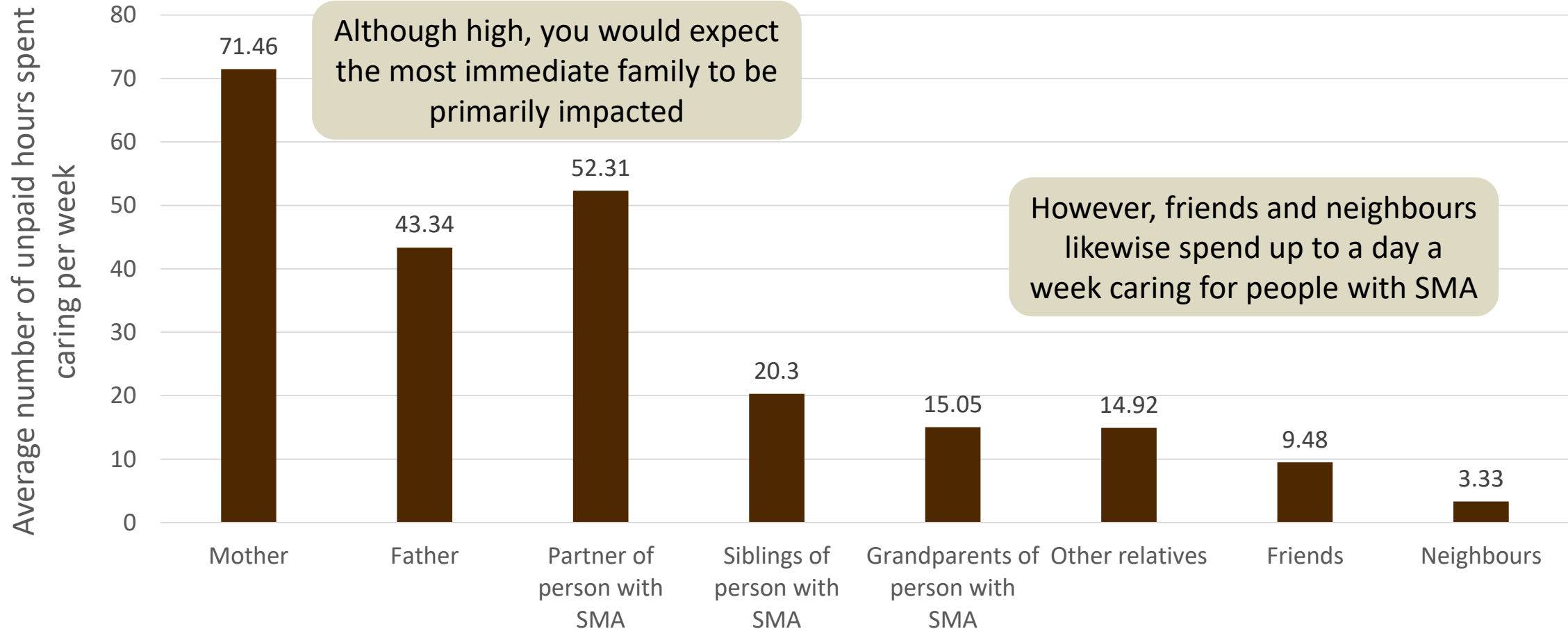
WICKENSTONES

On average how many unpaid caregivers provide support to each person with SMA?

Type 3 persons require, on average, the least support from unpaid caregivers

Average number of unpaid caregivers

- Type 1
- Type 2
- Type 3

Nusinersen treated patients were included in the overview, however due to absence of information around disease and treatment duration, it is unknown how much effect nusinersen had on these patients and their caregivers

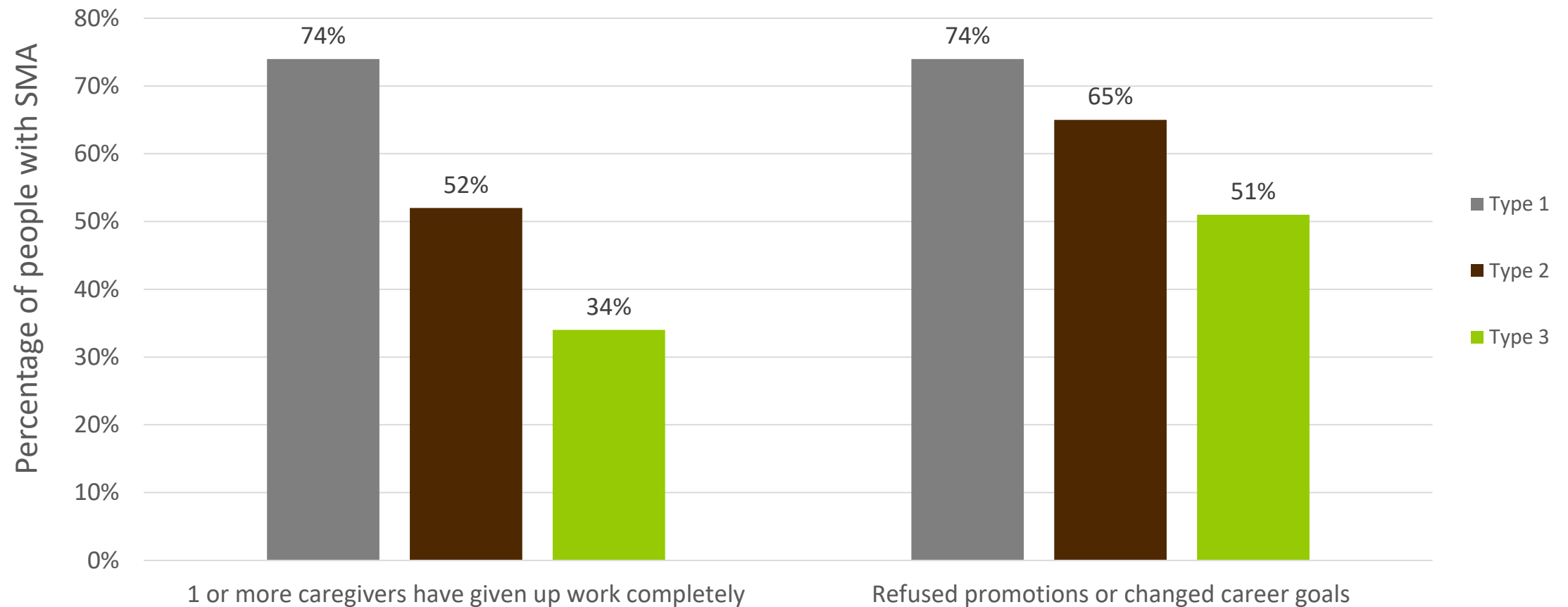# A wide range of unpaid caregivers give support to people with SMA each week

WICKENSTONES

On average, how many hours does each group of unpaid caregivers spend per week caring for the person with SMA?

Average number of unpaid hours spent caring per week

- Mother: 71.46
- Father: 43.34
- Partner of person with SMA: 52.31
- Siblings of person with SMA: 20.3
- Grandparents of person with SMA: 15.05
- Other relatives: 14.92
- Friends: 9.48
- Neighbours: 3.33

Although high, you would expect the most immediate family to be primarily impacted

However, friends and neighbours likewise spend up to a day a week caring for people with SMA

Caring for people with SMA has a major impact on caregivers' jobs and careers

What impact does caring for a person with SMA have on unpaid caregiver's jobs and career progression?

Percentage of people with SMA

- 74% / 52% / 34% — 1 or more caregivers have given up work completely
- 74% / 65% / 51% — Refused promotions or changed career goals

Type 1
Type 2
Type 3

WICKENSTONES

Estimates indicate that SMA places a substantial financial burden on society

- Survey B estimated the average annual cost for loss of productivity per unpaid caregiver at £14,350 based on reducing their hours by 25 hours per week.

- Survey B found the mean annual out of pocket (OOP) costs incurred for health materials and travel and accommodation (associated appointment costs and hospital stays) per SMA person were on average £8,025.
  - This did not include any cost of care that may be paid for by caregivers or SMA persons as this information was not collected.

- From Survey A we conclude that, on average, an SMA person impacts the NHS £49,723 in direct costs annually. Direct costs include interventions, drugs, equipment and formal care.
  - Costs were calculated using the human capital approach linked back to interventions and HCP engagement
  - Admissions and high-dependency units were not included in this