

## EUPESMA-2019 – European Patient Experience Survey in SMA to understand patient expectations towards therapeutic development in spinal muscular atrophy

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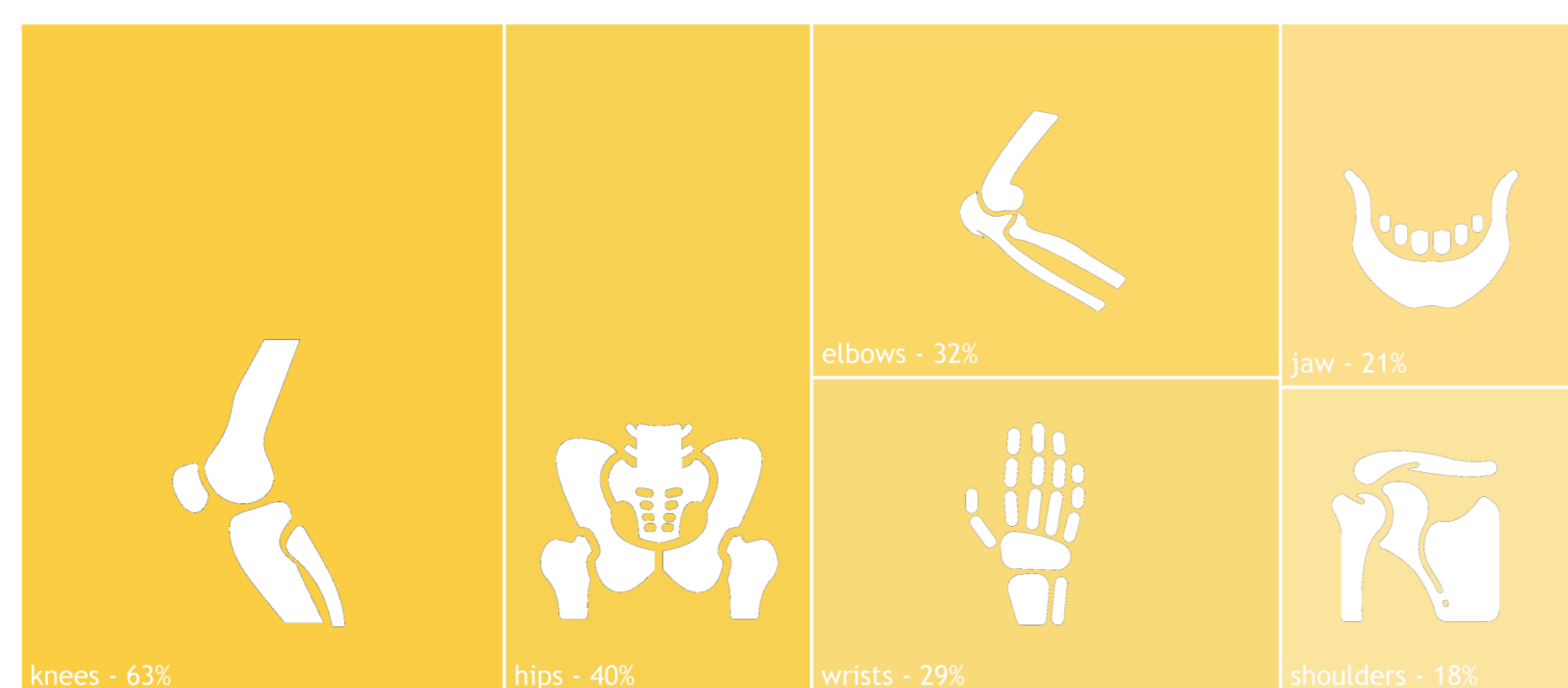
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### What is EUPESMA-2019?

EUPESMA-2019 survey for people living with SMA and their caregivers, designed to understand your treatment expectations, the reality of daily life with SMA and your experience with clinical trials and treatments.

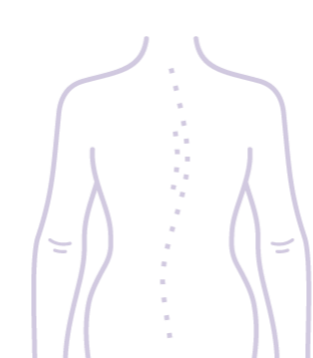
### Who took part?

All kinds of people affected by SMA, with both unique and shared experiences, with all SMA types and mobility levels across 26 European countries.

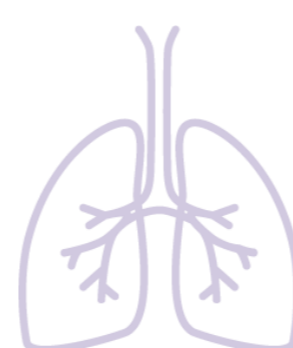


Most participants had **contractures** which were most common in the knees hips and elbows.

**1474** responses patients, parents, caregivers 0-81 years old



half of the participants had **scoliosis**

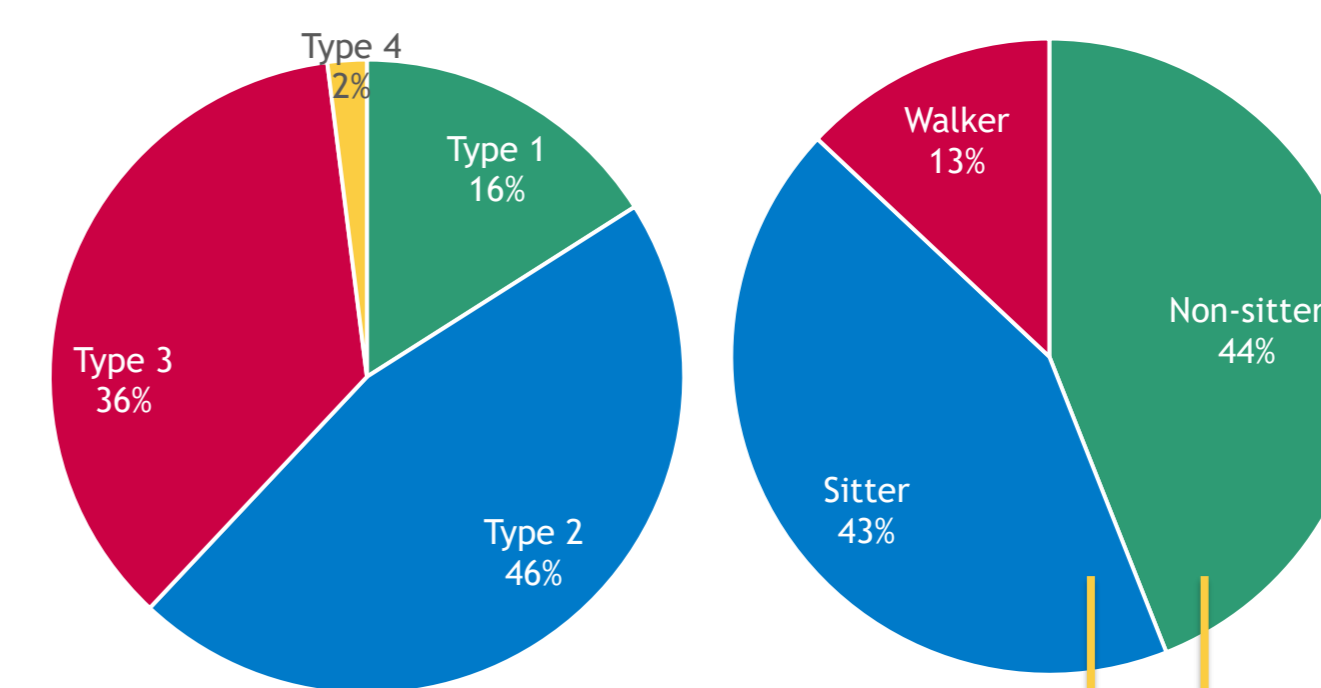


7 out of 10 did not need **breathing assistance...**

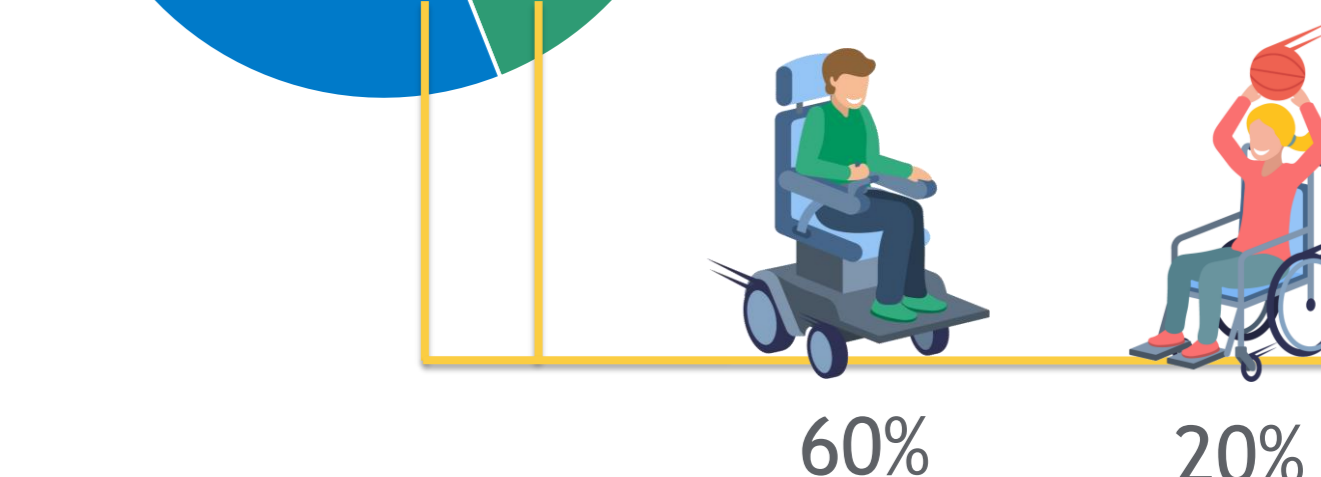
...of those who did, 2 out of 10 had a **tracheostomy**

### Why was it needed?

Understanding expectations and experiences of people affected by SMA is vital to the successful development of treatments.



Among those who could not walk, most were using motorised wheelchairs.



60% 20%

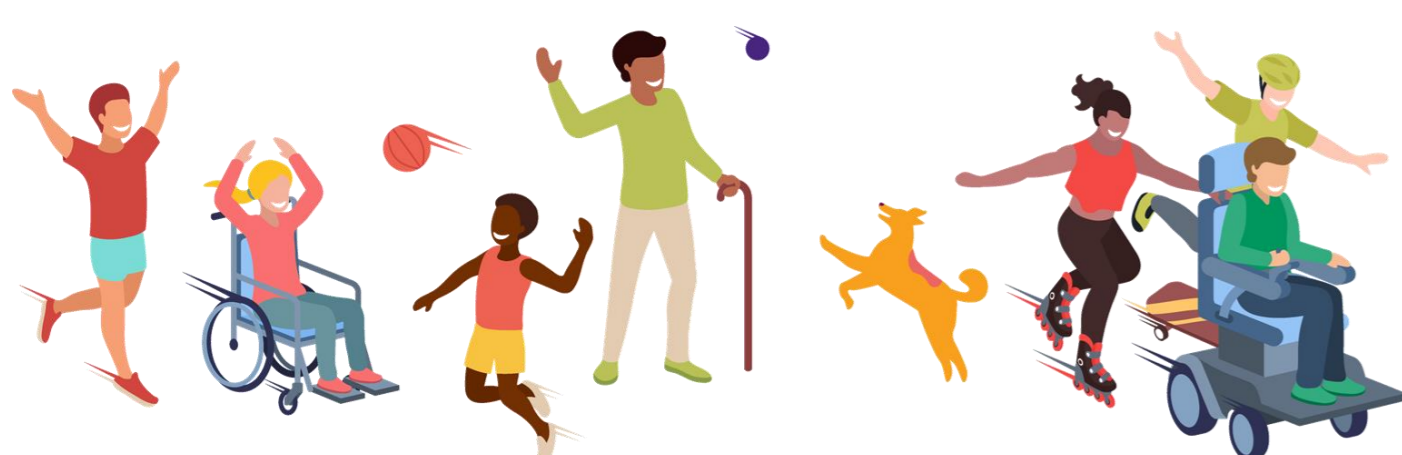
### What did we find out?

... SMA type classification does not always match mobility level or severity of symptoms.

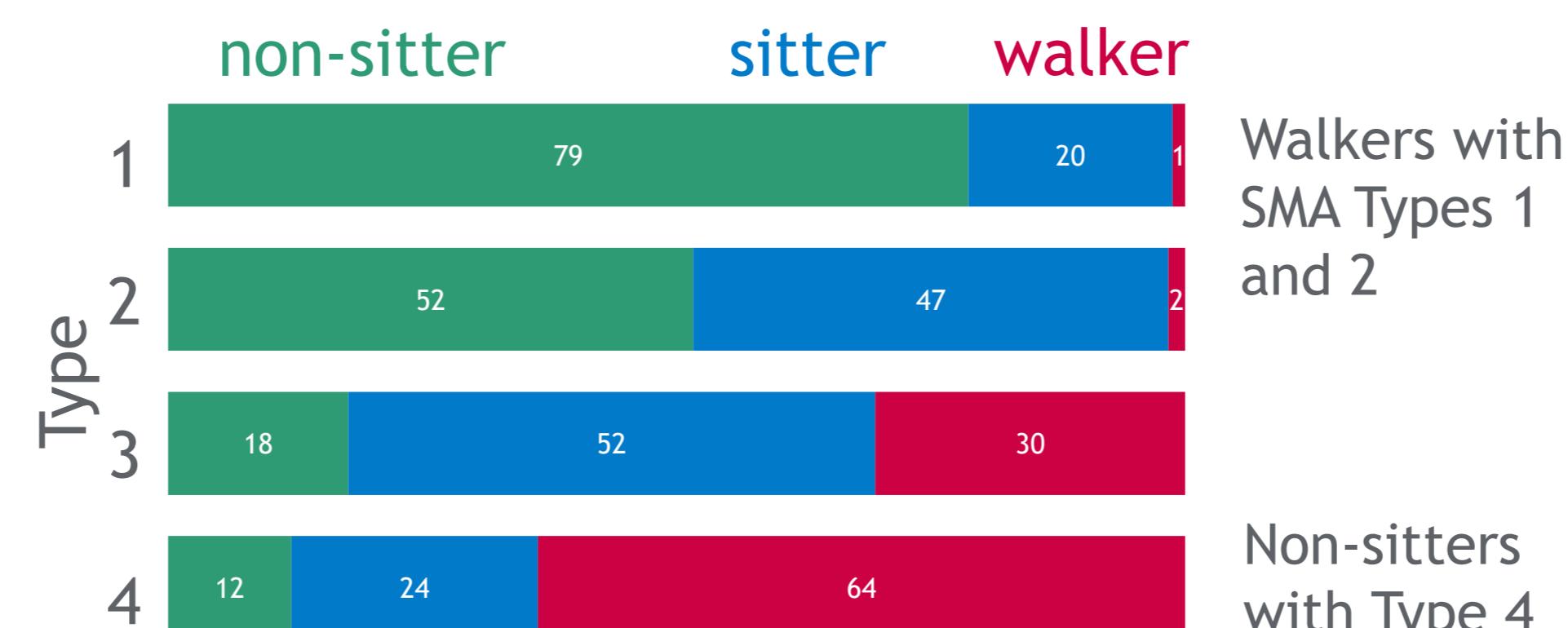
...and assessments used in clinical trials should represent what matters to you and what makes a difference in your daily lives.

... involving the SMA community in all stages of drug development is beneficial.

... almost 100% of the participants considered stabilization of their condition a treatment success.



Different mobility levels appear across all SMA types



Walkers with SMA Types 1 and 2

Non-sitters with Type 4

From 'Understanding European patient expectations towards current therapeutic development in spinal muscular atrophy', published in *Neuromuscular Disorders*, 31(5) 2021; Gusset, et al. (open access)

#### Acknowledgements

We appreciate the help from national SMA organisations for their contribution to the survey elaboration as well as its dissemination, and all the participants for sharing their experiences.

SMA Europe funds high quality research and conducts patient-focused research in SMA. SMA Europe works closely with patients and caregivers, patient advocacy groups, scientists, clinicians, the pharmaceutical industry and regulators to extend clinical trial and treatment access throughout Europe. **All together. One goal.**

“Stopping my SMA from getting worse is so important for me. I just want to preserve the few things left that allow some independence, like using a power wheelchair and my phone - and my ability to speak. That buys time for even better advances in medicine in the future.”

EUPESMA-2019 participant



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