Thank you!

A summary of the EUPESMA-2019 survey results

What is EUPESMA-2019: European Patient Expectation Survey SMA

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

Why was it needed

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

Who took part

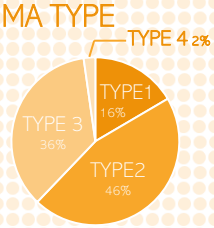
All kinds of people affected by SMA, with both unique and shared experiences, with all SMA types and mobility levels across 26 European countries

A huge thank you to every patient, parent and carer who took part in this survey. Your input will provide vital evidence to guide future drug development and support broad access to all SMA therapies in the future

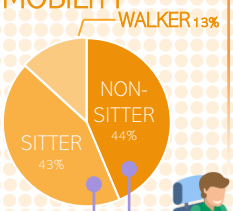
– The SMA Europe organisation

1474
responses
Patients, parents, caregivers
0–81 years old

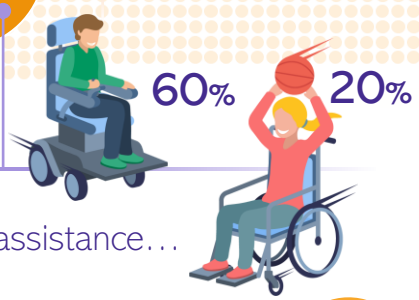
SMA TYPE



MOBILITY



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

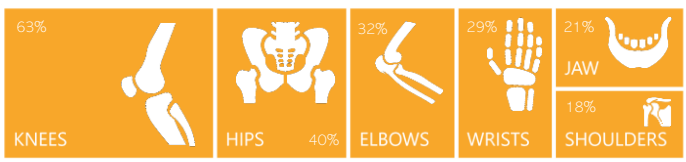


Half of the participants had scoliosis

7 out of 10 did not need breathing assistance…

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

CONTRACTURES



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

From the authors of '*Understanding European patient expectations towards current therapeutic development in spinal muscular atrophy*', published in Neuromuscular Disorders 2021

Nicole Gusset, Caroline Stalens, Eva Stumpe, Lori Klouvi, Alexandre Mejat· Marie-Christine Ouillade, Mencía de Lemus

What did we find out? >>>

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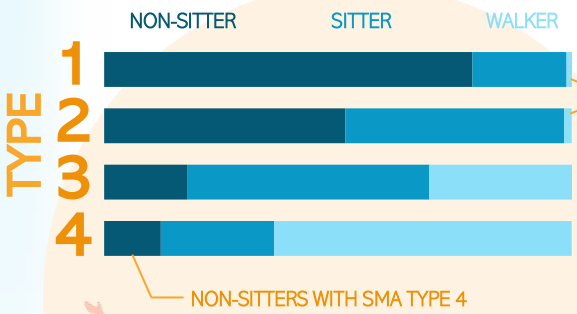
SMA type classification does not always match mobility level or severity of symptoms...

DIFFERENT MOBILITY LEVELS APPEAR ACROSS ALL SMA TYPES



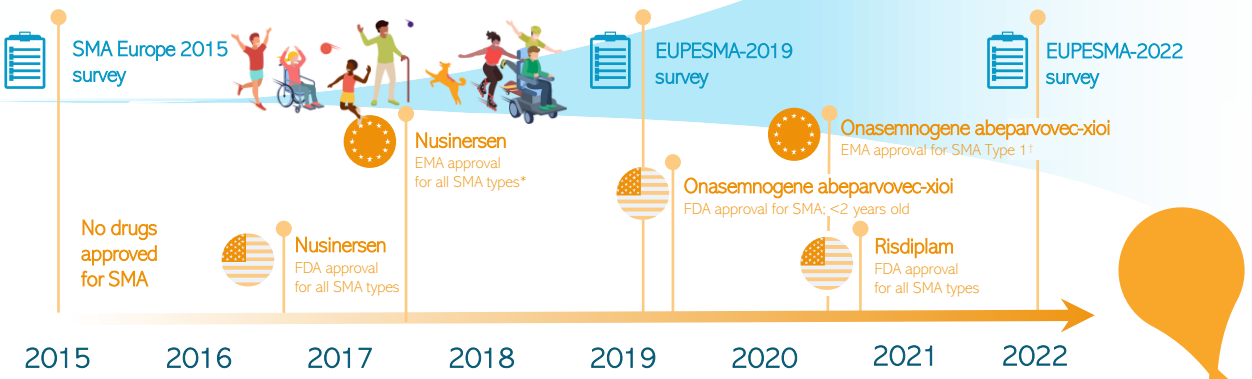
> *Stopping my SMA from getting worse is so important for me. I just want to preserve the few things left that allow some independence, like using a power wheelchair and my phone – and my ability to SPEAK. That buys time for even better advances in medicine in the future*
>
> – EUPESMA-2019 participant

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

ALMOST **100% OF THE PARTICIPANTS** CONSIDERED STABILIZATION OF THEIR CONDITION A TREATMENT SUCCESS

And most importantly, involving the SMA community in all stages of drug development is vital

SMA EUROPE FUNDS HIGH QUALITY RESEARCH AND CONDUCTS PATIENT-FOCUSED RESEARCH IN SMA, AND NOW WORKS CLOSELY WITH PATIENTS AND CAREGIVERS, PATIENT ADVOCACY GROUPS, DOCTORS, THE PHARMCEUTICAL INDUSTRY AND REGULATORS TO EXTEND CLINICAL TRIAL AND TREATMENT ACCESS THROUGHOUT EUROPE



*5q SMA in paediatric and adult patients with some variations in reimbursement across countries, e.g., for Type 1, 2, 3, 4 (excluding IV) and in some cases including age restrictions such as <18 years old. †Or have up to three copies of *SMN2*

> *Our goal must be to gather evidence and drive research further to personalize medicine, so that each individual patient may have access to the therapy that provides individual optimal therapeutic benefit.*
>
> – Dr Nicole Gusset, mother of a daughter with SMA Type 2
> Lead Author and President of SMA Europe