The SMA Daily Life Study – an experience sampling study examining patient-centric outcomes in the daily lives of individuals living with SMA

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What is the aim?

Explore patient-relevant outcomes

- **Fatigue**, **pain**, and **mental health** seem to play an important role in the general **well-being** of patients with SMA, and because they might be valuable as outcome measures for new treatment assessments.
- Research from fields outside of SMA show that these factors have a high level of **between-person**, but also **within-person** variability.
- It remains unclear how these constructs are manifested in **the daily lives of SMA patients**.

Study details

Who will take part?

- ~ 150 from ~ 5 European countries

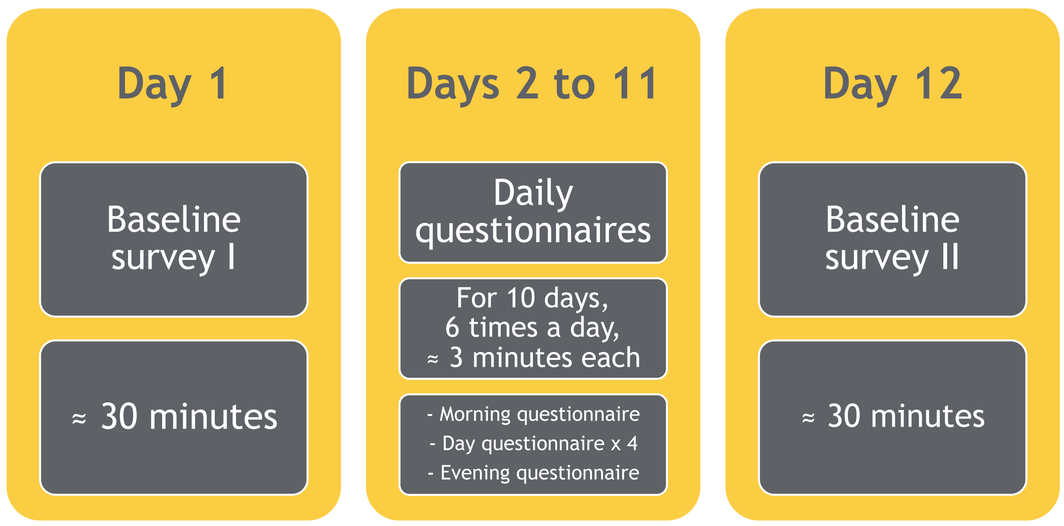
How will we measure?

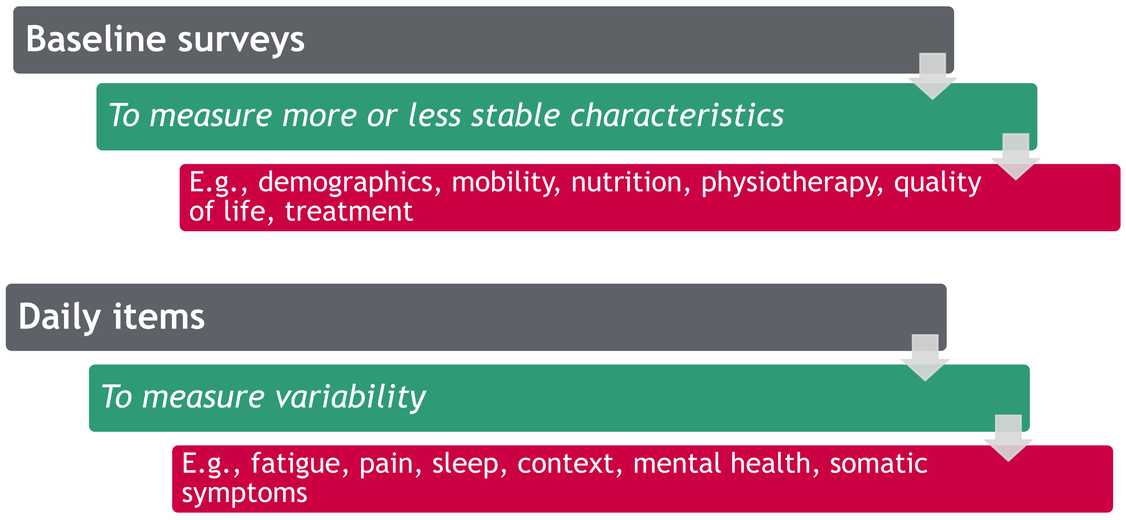
- Experience Sampling Method (ESM).
  - Participants are prompted multiple times a day for several consecutive days to respond to an electronic questionnaire through a smartphone application.

What will we measured?

- **Psychological, social and contextual variables**.
  - Repeated assessments of such constructs at the momentary level.

How we will measure patient relevant outcomes?





What do we expect?

- The results from this study will help us:
  - to better understand how patient-relevant outcomes are manifested in the daily lives of patients,
  - to measure patient-relevant outcomes, taking into account both between-person differences and within-person changes.

Acknowledgements

We would like to thank CureSMA Industry Collaboration and Tilburg University for their involvement. Also, we deeply appreciate the input from the SMA Europe Adult Committee.

Spinal Muscular Atrophy (SMA) is a rare genetic and progressive neuromuscular condition occurring in approximately 1 in 6,000 to 10,000 live births.

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