



SMA EUROPE STEPS IN THE OUTCOME MEASURES ARENA

SMA¹ Europe is an umbrella organisation that includes 17 SMA research and patient organisations from 15 countries across Europe. As patient advocates, SMA Europe works to ensure that the patient's voice is heard wherever decisions are taken that affect patients and where it can provide added value.

SMA is facing today a promising future with already one commercialised therapy and several other approaches being tested in clinical trials. The development of treatments reflecting the patients needs is a main priority of SMA Europe. A key to successfully bring effective therapies to patients as fast as possible includes the selection of the most appropriate measures to identify change in the disease progression.

Providing the perspective of patients and families is invaluable to the development of clinically meaningful outcomes that are accepted by health authorities and reimbursement decision makers. Patient Centered Outcome Measures (PCOMs) are defined as tools to determine efficacy of a treatment, and how a disease and its progression impacts the lives of patients by placing the patients at the center of the development of what has to be measured.

SMA Europe is committed to applying its resources to designing pivotal outcome measures for SMA, shaped in a patient-centric way. We believe that the only way to achieve this goal is through a close partnership with researchers, clinicians, authorities and industry. An open dialogue should pave the way to shape PCOMs that have a broad acceptance as tools for research on the disease and on therapies. Once trials have been successfully completed, health and reimbursement authorities have the responsibility of granting access to treatment. Tools that adequately reflect the impact of a therapy on a patient's well being will support these authorities to make sound decisions, also taking into account the patients's perspective.

SMA Europe is convinced that the patient's input should not be only restricted to classical Patient Reported Outcome Measures (PROMs), but clinical outcomes should also include the patient perspective as reflected in PCOMs. The inclusion of clinical measures, that are relevant to the daily lives of patients and have a significant impact on their general well being, into natural history or other databases is crucial. This is why SMA Europe wants to broaden the traditional role of patient input to the definition of PROMs only and aims to establish an open dialogue in the design of registries and clinical databases, and the important variables that need to be captured in them.

SMA Europe has created a task force to guide to contribute to the selection of the best tools to prove efficacy in a way that is meaningful to patients. This task force is chaired by SMA Europe president, and implements the goals agreed by the full Board of SMA Europe.

In a first step, SMA Europe intends to identify planned and/or existing initiatives, and offering our collaborative support by providing the European patient's voice.

For more information, please contact proms@sma-europe.eu

¹ SMA: spinal muscular atrophy