

**SMA  
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# **Global SMAAdvocacy Event**

## **Activity Report**

**13 March 2024  
Ghent, Belgium**

[www.sma-europe.eu](http://www.sma-europe.eu)

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## Disclaimer

This document reports information collectively created and discussed during the Global SMAAdvocacy Event held in Ghent, Belgium on 13 March 2024.

SMA Europe does not take responsibility for the content produced hereby, which is a mere reflection of the discussions reported to have taken place during the event.

## List of abbreviations

HCP	Healthcare professionals
HTA	Health technology assessment
NBS	Newborn screening
SMA	Spinal muscular atrophy
SOC	Standards of care

## Sponsor acknowledgement

A kind thank you to our sponsors:



# The Global SMAAdvocacy Event

## Overview and goals

On 13 March 2024, SMA Europe hosted the 2024 Global SMAAdvocacy Event that brought together shy of 70 SMA patient advocates from 63 patient organisations, representing 50 countries and 6 continents.

The Global SMAAdvocacy Event addressed an emerging need among SMA patient organisations to create global synergies, learn from each other, and build stronger international advocacy networks, with the objective to:

- Share experiences, successes, and challenges in SMA patient advocacy
- Collaboratively build knowledge: share best practices in access to medicine and care, identify gaps and hurdles, develop solutions
- Identify future priorities and strategies
- Build capacity among patient advocates through mutual and collaborative learning.
- Create and foster relationships among patient advocates and patient organisations worldwide

## A collaborative event

To ensure a hands-on and solution-oriented design, The Global SMAAdvocacy Event was ran as a “SMAckathon” (after hackathons in computer engineering) in which participants worked in groups to find actionable solutions to concrete issues.

The design of this event was directly guided by the needs of patient advocates in SMA. With participants’ feedback, we identified eight urgent challenges in SMA advocacy:

- Equity in access to SMA medicines
- Reimbursement of SMA medicines
- Reimbursement and access to Standards of Care (not medicines)
- National registries
- Access to diagnosis and newborn screening
- Supporting SMA-specific research
- Influencing policy
- Patient involvement and engagement in bench-to-bedside processes

Participants explored each challenge, working from the present status in different countries to possible, actionable solutions. This report contains a summary of the insights gathered in each group.

## SMAcademy

The Global SMAAdvocacy Event launched SMA Europe's **SMAcademy**, an initiative that aims to build capacity among patient advocates in SMA to increase their efficacy and impact in achieving their goals to improve the lives of people living with SMA.

Patient advocates need to have profound and up-to-date knowledge and skills in a variety of areas, including an understanding of SMA, related medicines and care, medicine development and regulatory processes, policy and access, and beyond. Yet, patient advocates also have valuable hands-on experience that is often transmitted in informal, unstructured, and dispersed ways. For this reason, SMAcademy promotes learning not only from experts across a variety of fields, but also from other patient advocates' experiences across diverse geographic and social settings.

Our motto is: **Co-create. Peer exchange. Expert Opinion.**

Participants in the Global SMAAdvocacy Event are strongly invited to follow our next online and in-person learning opportunities and to continue to feedback on their most pressing learning needs.

For further information, please visit the [Global SMAAdvocacy Event webpage](#).

For any questions, please contact **Alice Larotonda**, Community Research and Education Manager, at [alice.larotonda@sma-europe.eu](mailto:alice.larotonda@sma-europe.eu).

# Equity in access to SMA medicines

Equity in access to SMA medicines is a main concern throughout the globe. The workgroup included individuals from several European, Asian, Pacific and Africa countries representing a range of access situations.

## Current status

While in some countries patients can access all three currently available medicines for SMA, other countries have access to none. In the cases where access was available, restrictions to access based on criteria like age persisted. Clinical trials and compassionate use programs still constitute, altogether, the only hope to receive SMA medicines in some geographic settings.

## Goals

The group agreed on a common future goal: **full access to all SMA medicines for all people living with SMA**. Discussions further pointed out the importance of:

- Expediting and streamlining approval processes
- Ensuring continuity in access
- Access to combination therapies
- Removal of access restrictions based on age, type, start and stop criteria, use of respiratory relief
- Further accumulation and circulation of evidence
- Access to specialized equipment and assistance

## Action plan

The group's action plan addressed different areas of intervention, including the generation of Real-World Evidence to inform policy, the direct involvement of HCPs, the collective negotiation of more affordable prices, and the implementation of risk-sharing strategies among all stakeholders.

# Reimbursement for SMA medicines

Reimbursement for SMA medicines is essential to remove disparities in access to SMA medicines. The workgroup's diversity highlighted differences in reimbursement across the globe and their concrete consequences on access.

## Current status

Reimbursement for SMA medicine is inconsistent throughout the globe but also within local contexts, depending on factors such as individual countries' economies, policies, and health systems. Moreover, a variety of issues, from pricing to data availability, can constitute obstacles to ensuring reimbursement.

## Goals

The group agreed on the main common goal that **no person living with SMA should pay for SMA through personal funds or crowdfunding**. Instead, the goal is to achieve reimbursement for all, possibly including the opportunity to choose between therapies as well as combination therapies.

## Action plan

Raising and creating awareness about the vital importance of accessing reimbursed SMA medicines is crucial to leverage the support of policy and the general public. To achieve this, the group crafted a list of multistakeholder strategies that would leverage different messaging, adapted to local social and cultural contexts.

# Reimbursement and access to Standards of Care

The creation of updated and complete Standards of Care applicable to individuals across the SMA spectrum is sorely needed and constitutes a priority for people living with SMA all over the globe. The workgroup represented a diversity of international experiences in reimbursement and access to Standards of Care, but all advocates found the current guidelines lacking.

## Current status

The global landscape of access and reimbursement of SOC is punctuated by disparities. The group reported issues with a persistent lack of knowledge among HCPs as well as patient and families, as well as barriers within healthcare systems, ranging from poor access to medical equipment, physical therapy, or mental health support, to suboptimal services for the transition to adult care.

## Action plan

The group suggested a variety of strategies that may facilitate the creation and ultimately the fruition of the best SOC for all people living with SMA. The pillar of these actions is to establish a **new global SOC reference**, co-created with patients. Education towards the adoption of common standards and raising awareness would be fundamental to support this process.



# National Registries

Evidence is fundamental to best guide new and current care for people living with SMA. To this end, registries are a fundamental, yet challenging tool. The participants represented countries in which registries are present, are in the building process, or absent.

## Current status

There is a great need for information regarding SMA, however, many are the challenges faced in collecting it consistently and thoroughly. Current registries are often fragmented, contain limited information, or information that is not relevant to patients. Furthermore, they face a systematic lack of resources.

## Goals

If we are to improve the lives of those living with SMA, **data is vital**. However, this data needs to be **thorough, consistent, inclusive, and patient-relevant**. The creation of a consistent, global system of data sharing is fundamental to fully bring to fruition the power of data.

## Action plan

The proposed action plan revolves around the creation of a solid base of data that could be shared internationally. Registries would have to find consensus on the data collected, in collaborations with HCPs and patients. Awareness raising actions are also needed to highlight the importance of data in the patient community.

# Access to diagnosis and newborn screening

Diagnosis and newborn screening are fundamental to promptly identify and initiate care for people living with SMA. Global disparities in access to diagnostics are deep, and workgroup participants contributed to understand challenges and strategies they implemented in local settings.

## Current status

Diagnosing SMA is still a challenge in many countries around the world, with long pathways to diagnosis and poor awareness among HCPs. Newborn screening is fundamental to speed time-to-diagnosis. While screening programs and pilots are in place in some settings, access is still limited worldwide, punctuated by national and regional differences, and hindered by lengthy political processes for approval.

## Goal

The group agreed that the permanent **implementation of NBS programs worldwide** is fundamental. Raising awareness among front-line HCPs for symptom detection is also crucial.

## Action plan

The proposed action plan builds on four strategies: preparing the community by raising awareness, presenting and proposing evidence of the importance of newborn screening, facilitating and supporting political processes towards approval, including through international collaborations.

# Supporting SMA-specific research

Continued research is of vital importance for the SMA community. Patient organisations have had an important role in supporting and funding SMA-specific research, yet this comes with significant financial challenges and not all organisations can afford to directly support the research they find important.

## Current status

Scientific research requires significant amounts of resources, as well as the continued interest of the scientific community and related industry. The present landscape of patient organisation involvement is very variable, including supporting research through collaborations with specialised healthcare centres and industry, patient advising, and direct fundraising. Many of the activities feel punctual, with no guarantee of long-term continuity.

## Goal

**Guaranteeing continued interest in SMA-specific research** is important. To this goal, raising awareness among patients, patient advocates as well as the scientific community is a central mission.

## Action plan

The proposed action plan revolves around two main strategies: raising awareness and raising funds. The group suggested a variety of action points towards these strategies, with the common thread of improving communication about the importance of research and making research in SMA attractive to a variety of stakeholders like government, universities, and clinical centres.

# Influencing policy

The group presented a global landscape characterised by high fragmentation and regional variations on patient organisation's capacity to influence policy. Growing this capacity, however, is a priority to meet advocacy goals in favour of people living with SMA.

## Current status

Patient organisations' capacity to influence policy varies greatly, in particular, in relation to negotiating access and reimbursement for SMA medicines. These differences are exacerbated by the absence of tailored laws for disease-specific treatment access, shared HTA principles, and regulation of medicines' prices.

## Goal

The group established that great benefit would derive from having rules or **guidelines** shared at a **regional or global level** to regulate the access to and pricing of SMA medicines as well as best care practices for SMA, and maximise data collection and sharing efforts.

## Action plan

The proposed action plan include strategies based on creating and sharing evidence to support advocacy efforts, for instance, by expanding and coordinating access data collection initiatives like [OdySMA](#) to a global level, forming global registries, or global professional networks to elaborate new SoC.

# Patient engagement in drug development processes

The group, composed of industry representatives, worked on strategies to optimise patient engagement and involvement in all phases of drug development to optimise patient-relevance and patients' priorities.

## Current status

Industry faces a variety of obstacles to patient engagement, from patient reticence to collaboration to compliance challenges. There is a felt need to expand patient engagement efforts, particularly beyond Europe.

## Goals

Participants agreed that **a unified approach is needed** to increase the patient community's comfort with industry collaborations, increase the perceived value of patient engagement and keep the momentum going.

## Action plan

Strategies proposed include fostering collaboration between companies as well as between industry and patient organisations, as well as internal work to understand and improve compliance regulations to allow for better patient engagement.

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