SMA EUROPE

All together. One goal.

# Annual report 2025

**Publisher**
SMA Europe e.V.
Im Moos 4
79112 Freiburg
Germany

**Responsible for content according to § 55 para. 2 RStV**
Nicole Gusset
SMA Europe e.V.
Im Moos 4
79112 Freiburg
Germany

**Publication title**
Annual report 2025

**Visual content and design**
Emilia Debska, SMA Europe
Elena Diez, external designer

**Image credits**
Miguel M. Serrano
ReFOCUS Media Labs

**Year of publication**
2026

**Publication frequency**
Annual

**Contact**
Email: secretariat@sma-europe.eu
Website: www.sma-europe.eu

**Register information**
Registered Association (e.V.)
VR 700122

**VAT ID**
DE359313359

**Liability for content**
The contents of this publication have been prepared with the utmost care. However, no guarantee is given for the accuracy, completeness, or timeliness of the information provided.

**Liability for links**
This publication may contain links to external websites. SMA Europe e.V. has no influence on their content and assumes no liability for them.

**Copyright**
All content (texts, images, graphics) is subject to copyright. Any use beyond the limits of copyright law requires prior written consent of the respective rights holder, unless otherwise indicated.

**Editorial independence**
The editorial content of this publication is produced independently. Signed contributions reflect the views of the respective authors.

# Index

**07** About this report

**12** Governance and organisational framework

**18** Delivery by strategic area

**28** Cross-cutting partnerships, stakeholder engagement

**29** Community and membership

**34** Outreach and representation

**42** Financial overview and sustainability

**48** Annex

# About this report





## Our vision and mission

SMA Europe is a non-profit umbrella organisation of spinal muscular atrophy (SMA) patient organisations from across Europe. We work to bring effective treatments and optimal care to everyone living with SMA.

Together, through greater understanding, we will create a better world for all those living with SMA.
**All together. One goal.**

## Purpose and scope

This document is the Annual report of SMA Europe. It provides an official account of the organisation's activities during the reporting year and fulfils its reporting obligations as a non-profit organisation. The report focuses on governance, accountability, and the responsible use of resources, documenting what SMA Europe governed, delivered, and resourced in pursuit of its mission.

The report does not aim to provide detailed thematic analysis or an in-depth presentation of programme outcomes. Such content, including community-generated evidence and policy-oriented insights, is published separately through SMA Europe's thematic publications, including the One Goal Series.

# Message from our president

**Yasemin Erbas** President, SMA Europe

As President of SMA Europe, I am pleased to present our Annual report for 2025.

Being a membership-based organisation, SMA Europe derives its legitimacy from its member organisations, represented through the General Assembly (GA). Ensuring that our association remains aligned with the needs and priorities of people living with SMA is therefore essential. Our commitment to patient relevance, and to the lived realities and needs of the SMA community, is central to SMA Europe's work and guides its priorities and activities, with a collaborative, community-driven approach and a focus on impact.

The past years have seen continued changes in the SMA landscape, which have had a significant impact on our organisation which was initially founded to accelerate the development of effective treatments for SMA. Today we have several treatments, but unmet needs persist and access to both treatments and care remain a challenge across the European SMA community. At the same time, the SMA community has grown and diversified with new organisations emerging in response to changing needs at national levels.

These developments were further reflected in SMA Europe's work and priorities in 2025. Today, SMA Europe brings together 30 member organisations from 28 countries, each operating within a different reality of SMA. In order to remain effective across these diverse and complex needs in a rapidly changing context, SMA Europe has expanded in scope and capacity and has therefore evolved from a patient-driven organisation to one that remains patient-led while being more strongly supported by professional staff. This evolution opens many new possibilities and allows us to remain responsive and agile.

As SMA Europe continues to evolve, maintaining strong alignment with community priorities remains a central focus. In this context, in the past year, we have further strengthened the organisation's governance and operational structure, as illustrated in this report. The Board has maintained oversight of these developments, ensuring alignment between strategic priorities, organisational capacity, financial resources, and impact.

A first priority was to further ensure continued community involvement and patient-relevance across our priorities and activities. Therefore, we have put in place additional structures, including dedicated committees, advisory structures and working groups for strategically important topics. These structures support meaningful involvement from across the community, ensure that different perspectives are heard and our work remains relevant, and allow for effective engagement and coordination of staff members.

Another key priority for the Board has been the advancement of the 2026–2030 strategy. As the landscape is evolving fast, it is important to adopt a strategy that not only responds to current needs but also anticipates future developments and gaps. Through an extensive participatory process, we have finalised a new strategy that reflects the need for a more structured and anticipatory approach, and we look forward to its implementation in 2026.

In the context of our new governance framework, we have also, together with our CEO, established a new performance framework around key performance indicators. This framework strengthens the link between strategic objectives, implementation, and outcomes, and allows for greater focus, as well as clearer and more transparent visibility of results and impact. We are confident that this development will support SMA Europe in delivering on its strategic priorities and in successfully navigating continued evolution to a professionally supported organisation.

An exciting new development in 2025 was our 1st International Clinical Care Symposium. While our work has historically focused strongly on supporting fundamental and translational research, the availability of treatments has made it increasingly clear that high-quality care is essential to ensure that people living with SMA can benefit optimally. At present, many aspects of care still require further development, and access to high-quality care remains uneven across Europe. This symposium, which is based on topics identified by the community as clinical priorities, responds directly to this need and reflects the importance of continued progress in standards of care, as well as more equitable access to care.

Finally, as reflected in our financial report, SMA Europe has a healthy and stable financial position. However, as we enter the next strategic cycle, we will place more emphasis on diversification of our income, which is important to safeguard our independence and legitimacy. This is also closely linked to our need to continue supporting research, which lies at the heart of our mission and is essential for sustained progress for people living with SMA. Ensuring that we can continue to fund research will be critical to generating new knowledge and, ultimately, to developing new solutions for our SMA community. This will remain a key focus for SMA Europe in the next years.

On behalf of the Board, I would like to thank our GA for their support, as well as our CEO, staff, volunteers, and partners for their contribution to SMA Europe's mission. We look forward to continuing our collaboration in creating a better world for all those living with SMA.

**Yasemin Erbas**
President, SMA Europe

# Message from our CEO

**Nicole Gusset**
CEO, SMA Europe

The past year has been a period of reflection, organisational development, and strategic preparation for SMA Europe. While maintaining momentum across all areas of our work, 2025 was defined by a strong focus on strengthening our foundations and positioning the organisation for its next phase of evolution.

In response to the evolving SMA landscape and the resulting organisational and strategic demands, SMA Europe has continued to strengthen its role as a coordinating and structuring organisation.

A central focus of the year was the development of SMA Europe's 2026–2030 strategy, building on extensive engagement with our members, community, and stakeholders. In parallel, we prepared the organisation for its implementation by strengthening internal processes, aligning the staff team, and developing the tools required to support delivery. This included the introduction of a KPI-based monitoring approach, execution, and ongoing performance tracking across the organisation. These steps reflect a deliberate shift towards stronger strategic execution, ensuring that priorities are translated into coordinated and measurable action.

At the same time, SMA Europe continued to advance its work across research, therapy and care, policy, and capacity building. These efforts were supported by the active engagement of our member organisations, the contributions of volunteers and experts, and collaboration with partners across Europe and beyond. Delivering this work required sustained coordination, strong internal capacity, and careful management of partnerships and risks across different areas of activity.

Ensuring coherence across these activities and translating strategic priorities into coordinated action across the organisation was a central focus of the year. This was supported by clear prioritisation and disciplined allocation of resources, alongside continued investment in internal capacity and coordination.

This report reflects SMA Europe's commitment to transparency and accountability. It focuses on how the organisation has governed its work, implemented its mandate, and managed its resources during the reporting year. Detailed thematic analysis and community-driven evidence are presented separately through our publications, including the One Goal Series, allowing this report to remain focused on its governance purpose.

Looking ahead, SMA Europe enters a new strategic cycle with strengthened governance structures, well-defined priorities, and a continued commitment to working collaboratively and guided by priorities of the SMA community. Building on stablished practices, we will continue to reinforce community-led priorisation, co-creation, and structured engagement across our activities. The coming years will require sustained effort, adaptability, and disciplined prioritisation to ensure that progress in SMA translates into meaningful and equitable outcomes for all those affected.

I would like to thank our members, Board, staff, volunteers, partners, and all those who contribute to SMA Europe's work for their continued commitment and collaboration.

**Nicole Gusset**
CEO, SMA Europe

# Governance and organisational framework

## Legal status and mandate

SMA Europe is a non-profit umbrella organisation registered in Germany and operating in accordance with German non-profit law. The organisation is established for public benefit purposes and acts in line with its statutes and applicable legal and regulatory requirements.

The statutory purpose of SMA Europe is to promote the health, healthcare and wellbeing of people living with SMA, and to promote science and research in all areas of life affected by SMA, from diagnosis to treatment, care, and daily life activities.

The organisation operates across Europe and represents a network of national SMA patient organisations and neuromuscular disease organisations. Its members share the mission of SMA Europe and participate in the governance of the organisation through the GA, which serves as the highest decision-making body.

## Governance structure

The governance of SMA Europe is based on a clear separation of responsibilities between its governing, supervisory, and executive functions, in line with its statutes and recognised good governance principles.

The GA is the highest governing body of SMA Europe. Composed of delegates from our member organisations, it exercises ultimate authority over statutory matters, including the approval of annual plans and budgets, the election of Board members, and amendments to the Statutes. The GA provides the foundational mandate of the organisation and ensures that SMA Europe's strategic direction remains aligned with the priorities of its member organisations.

The Board is responsible for the strategic direction and overall oversight of the organisation, in alignment with the mandate defined by the General Assembly. Acting in the best interest of SMA Europe and in accordance with its statutory mandate, the Board exercises supervisory responsibility and provides strategic guidance to the CEO, while ensuring sound financial and organisational governance and long-term sustainability.

The CEO holds executive responsibility for the organisation. The CEO leads the organisation's strategy implementation in accordance with the strategic direction defined by the Board, provides organisational leadership, manages day-to-day operations of the organisation. In this role, the CEO, represents SMA Europe externally and holds power of attorney in accordance with the statutes and applicable law. The CEO is responsible for translating strategic decisions into operational action, ensuring effective, lawful, and responsible management of the organisation, and leading the staff team in the delivery of SMA Europe's mission.

The staff team supports the delivery of the mission through the implementation of projects, programmes, and organisational functions. Staff operate under the leadership of the CEO and contribute professional expertise across different areas.

In addition, volunteers may contribute to the implementation of projects and initiatives, including in coordinating roles, within defined operational structures and under the overall responsibility of the executive management.

Committees are established by decision of the Board and support the work of the Board and the organisation by providing structured expertise and stakeholder input in defined areas. They work closely with the CEO and staff team and contribute to strategic and organisational deliberations. Their mandates, composition, and reporting lines are defined through formal terms of reference, and their input is reported to the Board.

In addition, project-specific sounding boards or working groups may be established to support individual projects or initiatives. These sounding boards operate at an operational level under the responsibility of the respective project leads and provide targeted input, feedback, and expertise relevant to the project scope. They do not hold governance functions.

Decision-making processes are designed to ensure transparency, accountability, and appropriate checks and balances. Decisions are taken at the appropriate governance or executive level in accordance with the statutes, internal regulations, and delegated authorities.

## Governance activities during the year

During the reporting year, the Board met on 13 occasions. Board meetings focused on fulfilling the responsibilities of the Board for strategic oversight, organisational governance, and financial stewardship.

The GA convened in Athens, Greece on 26 April 2025 for the General Assembly meeting. The GA adopted seven formal resolutions, including the approval of the annual plan and budget, the discharge of the Board, and other statutory decisions in accordance with the organisation's statutes. In addition, the delegates to the GA were updated during three Delegates Calls held during the year.

During the reporting period, one election and one appointment took place. This was the re-election of our Treasurer, Eva Stumpe, by the GA, as well as the appointment of Mencia de Lemus as Co-Chair of the Treatment Committee (TC) by the Board.

During the year, changes in membership were formally acknowledged in accordance with the statutes. This included changes in the membership of two organisations, which became full members. Member organisations also designated new delegates to represent them in the GA, reflecting updates in national representation for SMA Hungary Foundation, FSMA Iceland, SMA Turkey Foundation, and Muskelsvindfonden (Denmark).

In addition to statutory matters, the Board engaged in strategic discussions throughout the year, with a particular focus on the development of the Strategy 2026–2030 of SMA Europe, a process that was initiated in early 2024 and advanced further during the reporting year. The development of the 2026–2030 strategy was informed by structured input from member organisations and delegates, ensuring alignment with the priorities of the SMA community. In 2025, the Board held several online strategic discussions and participated in two dedicated strategy workshops, including one held on 24 April 2025, prior to the Annual Meetings in Athens, Greece and a second workshop following the International Clinical Care Symposium in Milan, on 31 August 2025. The latter involving both Board members and the staff team. Following the Annual Meeting, a member survey was conducted to systematically gather feedback and weight the proposed strategic areas, supporting an inclusive and evidence-informed approach to strategic decision-making. In December 2025, the high-level strategic framework was presented to delegates of SMA Europe during a virtual Delegates Call.

In parallel, the CEO led the development of a Key Performance Indicator (KPI) framework for 2026–2030 based on discussions with the Board. The document was reviewed by the Board in October and was designed to support the operationalisation and monitoring of the approved strategic objectives. A staff retreat held in late October 2025 was used to work with the staff team on the KPI framework and on initial implementation planning, ensuring alignment between strategic objectives, operational planning, and future reporting.

## Policies, compliance, and risk management

During the reporting year, SMA Europe adopted five new internal policies and updated three existing policies to strengthen corporate governance, ensure legal and regulatory compliance, and support organisational efficiency. All policies were approved in line with established governance procedures and apply across the organisation.

SMA Europe continues to systematically develop its internal policy framework to support long-term operational sustainability and resilience in a changing regulatory environment.

SMA Europe is committed to the responsible and lawful handling of personal and sensitive data. During the reporting year, the organisation maintained continuous oversight of data protection and ethics matters through ongoing monitoring of applicable legislation and the effective operation of internal controls, supported by external guidance where appropriate.

Conflicts of interest are managed through formal policies and mandatory disclosure requirements. Board members, committee members, management, and staff are required to declare potential conflicts in accordance with organisational rules. During the reporting year, numerous conflicts of interest declarations were reviewed, with no material conflicts identified.

## Governance development in 2025

During 2025, SMA Europe began laying the governance foundations for the organisation's next strategic phase. This preparatory work focused on strengthening oversight structures, clarifying roles and responsibilities across Board and executive functions, and aligning governance practices with the priorities of the forthcoming 2026–2030 strategy.

Key measures included enhancing financial oversight and risk monitoring processes, refining reporting structures to support more timely and transparent decision-making and initiating the integration of a KPI framework into regular governance cycles. These steps were designed to ensure that strategic planning, implementation, and evaluation are clearly connected and supported by reliable data and accountable processes.

In parallel, SMA Europe reviewed its governance policies and procedures to ensure continued compliance with statutory and non-profit requirements, and to reinforce organisational resilience. Attention was also given to succession planning and leadership continuity across Board, staff, and volunteer structures, recognising that stable governance capacity is essential for long-term sustainability.

These actions taken in 2025 provide the governance platform for the implementation phase of SMA Europe's 2026–2030 strategy, ensuring that future growth is accompanied by robust oversight, transparency, and responsible stewardship of resources.

# Delivery by strategic area

# Research

**Through its Research pillar, SMA Europe promotes patient-relevant research that addresses unmet needs identified by the SMA community. The organisation works to ensure that research priorities, funding mechanisms, and scientific exchange are aligned with community perspectives and contribute to meaningful progress for people living with SMA.**

## Governance and oversight

Research activities are overseen through established governance structures, including the Scientific Advisory Board (SAB). The SAB supports SMA Europe in identifying and promoting high-quality research initiatives that help ensure treatments reach patients rapidly, safely, and sustainably. The SAB carries out its activities in alignment with the mandate and strategic objectives of SMA Europe, upholding the highest ethical standards, and commitment to patient-relevant research.

## Key deliveries in 2025

During the reporting year, SMA Europe advanced its work on community-informed research priorities. The SMA Research Priorities were further disseminated through the submission of a scientific publication, the presentation of posters at scientific meetings, and additional awareness-raising activities aimed at increasing recognition of their relevance among researchers and funders. The identified community-driven priorities also informed the development of the Scientific Congress programme.

SMA Europe continued to manage its research funding activities. This included progressing Call for Research Proposals cycle 12 through the execution of grant agreements with awarded projects, as well as the review of interim and final reports from projects funded under the 11th Call. Preparatory work for the 13th Call was also undertaken, including engagement with prospective funders and activities to raise awareness of the importance of patient-relevant research.

Preparatory work for the International Scientific Congress 2026 continued throughout the year. Activities focused on governance and organisational aspects, including establishment of the scientific programme, compliance related to industry involvement, accreditation processes, financial planning, and overall event management. In parallel, preparatory activities for Early-Stage Researchers and patient advocates events linked to the 2026 Congress were initiated.

In addition, SMA Europe continued the SMArt Horizon initiative, related to projects evaluating the use of spinal cord stimulation in people living with SMA, by supporting in research collaboration and knowledge exchange at European level.

# Therapy and care

**Through the Therapy and Care pillar, SMA Europe works to advance patient-relevance in the development, assessment, and implementation of SMA therapies and care, ensuring that treatment and care pathways reflect the lived experiences, needs, and priorities of people living with SMA and their families.**

## Governance and oversight

Activities under this pillar were led at executive level by the CEO, who held overall responsibility for strategic steering, implementation, and external engagement related to therapy and care.

In relation to the 1st International Care Symposium, governance and implementation responsibilities were structured to ensure scientific quality, transparency, and appropriate community involvement. The selection of abstracts and the development of the scientific content were supported by the SAB through a dedicated medical panel. Overall management and coordination of the symposium were led at executive level by the CEO, supported by a project team and a congress organising committee that included volunteer contributors.

Clinical trial–related activities were supported by the TC, which provided expert patient input on issues related to clinical trial design, participation, and the lifecycle of medicines. The TC contributed advisory perspectives to the engagement of SMA Europe with industry partners and other stakeholders in this area.

Regulatory and assessment-related activities were supported by the EMA Committee, which contributed patient perspectives to our positioning and engagement in regulatory processes, including interactions related to medicines assessed at European level.

Community research activities, including patient experience surveys and related analyses, were implemented through a dedicated project team involving staff and volunteers. This team supported participatory data collection, analysis, and dissemination in line with our community-led research principles.

## Key deliveries in 2025

During the reporting year, SMA Europe continued to generate and analyse patient experience data related to therapy and care through the European Patient Experience Survey on SMA (EUPESMA). A new survey on treatment expectations was conducted and analysed, with preparatory work for scientific publications, conference posters, and thematic dissemination through the One Goal Series. In addition, the first edition of the One Goal Series based on our survey on clinical trial experiences was published, translating patient experience data into accessible and relevant advocacy messages.

Findings and insights generated under this pillar were disseminated through poster presentations and participation in external workshops and scientific meetings, organised by a range of stakeholders. This included participation in workshops convened by organisations such as the ENMC, including a workshop on cognitive issues in SMA, as well as a fatigue-focused workshop held in the United States.

A key milestone under this pillar was the delivery of the 1st International Care Symposium, which provided a dedicated platform to address care-related topics relevant to people living with SMA across Europe. The symposium brought together clinical, patient, and stakeholder perspectives and represented a major step in strengthening structured dialogue on SMA care. Following the symposium, work was initiated on workshop-related outputs, including the preparation of a workshop report and the development of an emergency care template, drawing on existing models and practices from different countries. These outputs aim to support knowledge transfer and practical implementation beyond the event itself.

SMA Europe further strengthened its work on clinical trial and treatment information and access through the revision of the SMA Europe Clinical Trials Hub and the launch of the SMA Europe Clinical Trial Finder tool, supporting improved access to up-to-date trial information for the SMA community and other stakeholders.

In parallel, SMA Europe collaborated with Semantic Hub in a preliminary study on the patient journey of families with newly diagnosed children, focusing on experiences from diagnosis to treatment initiation. This work aimed to improve understanding of emerging care pathways for a new generation of families and to inform future activities in this area.

Throughout the year, the TC engaged in advisory activities with industry partners and other stakeholders, providing patient-informed input across different stages of medicine development. SMA Europe also contributed to regulatory positioning, including engagement related to products assessed by the EMA, in line with its role as a patient organisation representing community perspectives.

# Healthcare systems, policy and access

**Through this pillar, SMA Europe works to improve equitable access to diagnosis, treatment, and care for people living with SMA across Europe. The organisation addresses structural barriers within healthcare systems and supports evidence-informed policy discussions at national and European levels.**

## Governance and oversight

Activities under this pillar were led at executive level by the CEO, who held overall responsibility for strategic steering, implementation, and external engagement related to healthcare systems, policy, and access.

The Newborn Screening (NBS) Alliance operates under a dedicated Steering Committee which provides strategic direction for the initiative. Day-to-day activities and reporting are managed through the executive management, ensuring alignment with broader objectives of SMA Europe.

OdySMA is implemented through defined project management arrangements and supported by a project-level sounding board that provides operational input and stakeholder perspectives. These structures support effective delivery while remaining clearly separated from formal governance functions.

## Key deliveries in 2025

During the reporting year, SMA Europe continued to strengthen its work on newborn screening and early diagnosis through the NBS Alliance. Activities included support to national entities, participation in European policy events, and the organisation of a social media campaign marking Newborn Screening Day. A webinar marking the 5th anniversary of the NBS Alliance was held, and preparatory work was undertaken for an ENMC meeting. In addition, a new White Paper was published and made available through translation into multiple languages to support dissemination and policy uptake.

Work under the OdySMA initiative focused on generating and disseminating evidence related to care pathways and access. This included the presentation of publications and posters, the collection and sharing of real-life experiences, and the organisation of a workshop focused on care. Further development and use of digital tools continued, including the clinical trial finder, supporting access to information relevant to people living with SMA and other stakeholders. OdySMA supported national entities and SMA Europe member organisations by providing data, tools, and insights to inform national-level discussions, advocacy activities, and stakeholder engagement related to care and access.

In addition, SMA Europe undertook a preliminary, exploratory assessment of the early patient journey for families of children diagnosed through newborn screening, drawing on selected experiences from diagnosis to treatment initiation. This work was intended to identify initial patterns and gaps to inform future, more systematic activities. Building on these early insights, further structured work in this area is planned for 2026.



# Communication and outreach

**Through the Communication and Outreach pillar, SMA Europe ensures that its work, priorities, and community perspectives are communicated in a clear, accessible, and evidence-informed manner. Communication activities support awareness-raising, knowledge transfer, and engagement with a wide range of stakeholders, while remaining grounded in SMA Europe's strategic objectives.**

## Governance and oversight

Communication and outreach activities were led at executive level by the CEO, with implementation carried out by the staff team and external partners where appropriate. Editorial direction and messaging were aligned with the strategic framework and publication ecosystem, including the One Goal Series.

Awareness and outreach activities are implemented through defined project management arrangements and supported by a project-level sounding boards (Communications WG and Youth Group) that provide operational input, hands-on engagement, and stakeholder perspectives. These structures support effective delivery while remaining clearly separated from formal governance functions.

## Key deliveries in 2025

During the reporting year, SMA Europe implemented a range of awareness and engagement activities designed to connect community needs, research, and policy-relevant themes. This included the awareness campaign "Connecting the Dots", which featured research grant awardees and community members highlighting unmet needs and their relevance for patient-centred research. SMA Europe continued also to engage younger members of the community through Youth Group SMArt TV episodes, providing a dedicated format for peer-to-peer exchange and visibility of youth perspectives.

To strengthen internal and external communication, SMA Europe established a weekly internal newsletter and a monthly external newsletter, supporting structured information flow, transparency, and regular engagement with members and stakeholders.

In addition, work was undertaken to update SMA Europe's visual identity and branding, with a focus on improving accessibility and coherence across communication channels. This included the design and rollout of the "One Goal Series" editorial line, providing a unifying narrative framework across publications, campaigns, and outreach activities.

# Capacity building

**Through the Capacity Building pillar, SMA Europe works to strengthen the capabilities of its member organisations, network, and internal structures. This includes supporting learning, exchange, and leadership development, as well as building the organisational foundations required for sustainable and effective action.**

## Governance and oversight

Capacity-building activities were led at executive level by the CEO and implemented through the staff team, with support from advisory structures where appropriate. Learning and training activities were informed by a multi-stakeholder advisory board, which provided input to programme development without holding governance responsibilities.

Governance and leadership-related capacity-building activities were aligned with SMA Europe's broader governance framework and internal policies, as described in the Governance section of this report.

## Key Deliveries in 2025

During the reporting year, SMA Europe continued to develop its learning offer through SMAcademy with webinars and workshops. Advanced work was conducted on the development of a structured training for SMA patient advocates. These activities aimed to strengthen advocacy skills, organisational capacity, and shared understanding across the SMA Europe community. Preparatory work also continued for the second edition of the Global SMAdvocacy Event, supporting longer-term capacity-building and international networking objectives.

The SMA Europe Annual Meetings served as a key capacity-building moment, combining statutory functions with interactive workshops designed to support exchange, learning, and collective reflection among members.

SMA Europe encouraged peer exchange among member organisations through a range of structured and informal activities, including via Our SMA Europe platform. These exchanges facilitated knowledge sharing, mutual learning, and collaboration across the network. Activities were informed by participant feedback and ongoing reflection.

Internally, SMA Europe invested in organisational capacity building as a maturing organisation. This included staff learning and development, the optimisation of internal systems, and the establishment of core processes. Preparatory work was undertaken to improve the organisation's IT infrastructure, including the implementation of a Customer Relationship Management (CRM) system.

Capacity building also extended beyond formal membership, supporting network strengthening and peer exchange among volunteers and contributors engaged in SMA Europe's activities. In parallel, internal work continued on organisational development and strategic planning, including contributions to the preparation and refinement of the organisation's strategic direction.

# Cross-cutting partnerships, stakeholder engagement

Throughout the reporting year, SMA Europe invested in the development, maintenance, and stewardship of partnerships that support its mission across its area of work. This included the establishment and management of partnership agreements, regular coordination with existing partners, and engagement aimed at identifying opportunities for future collaboration.

Partnership relationships were managed under the responsibility of the CEO ensuring alignment with SMA Europe's strategic objectives and governance framework. The President contributed to this work through active engagement in selected partnerships and stakeholder interactions, supporting relationship-building at a strategic level.

SMA Europe also engaged with a range of stakeholders to explore potential areas for future collaboration, contributing to network development and strengthening relationships across the SMA ecosystem.

See the Annex for the full list of our partners and collaborators.

# Community and membership





SMA Europe is a membership-based umbrella organisation composed of national patient organisations, led by people living with SMA and their families, who put the voice of those living with SMA at the heart of everything they do. They advocate and campaign at national level while also engaging and influencing all stakeholders and decisions at European level, through SMA Europe.

Only through true representation of the SMA community will the voices of people living with SMA and their families be heard, and will their needs be met.

## Member organisations and geographic scope

During the reporting year, our membership comprised 30 organisations representing 28 countries across Europe. Membership is open to non-profit SMA patient organisations that share mission and values of SMA Europe and meet the criteria defined by the Board and are aligned with the statutes.

Changes in membership during the year were formally acknowledged in accordance with statutory procedures. This included changes in the membership of two organisations, which became full members. These developments reflect the evolving composition and geographic reach of the SMA Europe network.

## Delegates and participation

Member organisations participate in governance of SMA Europe through the GA, via their designated delegates. During the reporting year, four member organisations appointed new delegates, reflecting changes in national representation. These updates were formally recorded and ensured continued alignment between SMA Europe and its members. The organisations who appointed new delegates were:

- FSMA Iceland with Auður Kolbra Birgisdóttir
- Muskelsvindfonden, Denmark with Mette Bendix Klausen
- SMA Foundation Hungary with Flora Bery-Janusko and Andrea Orbán
- SMA Turkey Foundation with Can Akgün

## Volunteers

In addition to its formal membership, SMA Europe benefits from the engagement of volunteers who contribute across multiple areas of the organisation's work. Volunteers were involved in project teams, advisory committees, working groups and event organisation, supporting both delivery capacity and peer exchange. In some cases, volunteers take on active roles in the coordination and delivery of specific initiatives, working in close collaboration with staff and within defined project structures.

## Board, staff, and organisational roles

Our community is further supported by its Board, staff team, and various committees and working groups, whose roles and responsibilities are described in the Governance and Organisational Framework section of this report. Together, these structures enable coordinated action, accountability, and continuity across the organisation.

# Board

**Yasemin Erbas**
President.
SMA Belgium

**Olga Germanenko,**
Vice-President.
SMA Family Foundation
Russia

**Eva Stumpe**
Treasurer, Deutsche
Gesellschaft für
Muskelkranke, Germany

**Marie-Christine Ouillade**
AFM Téléthon, France

**Stefan Bos.**
Vereniging Spierziekten
Nederland, The
Netherlands

**Simona Joveska.**
Stop SMA North
Macedonia

**Jakub Tomczyk.**
Fundacja SMA, Poland

# Staff

**Nicole Gusset**
CEO

**Theo Soultanos**
Operations Manager

**Emilia Debska**
Communications and
Marketing Manager

**Federica Fontana**
Research and Medical
Manager

**Laura Gumbert**
Healthcare System and
Access Manager

**Alice Larotonda**
Community Research
and Education Manager

**Carolina Pachecoy**
Operations Support
Manager

# Outreach and representation

# Events

During the reporting year, SMA Europe hosted and participated in a range of events supporting its strategic objectives and governance responsibilities. Events hosted by SMA Europe included statutory meetings and thematic events, while participation in external events contributed to representation, exchange, and visibility at European and international level.

Details on event content and outcomes are reported under the relevant strategic pillars. This section provides an overview of events from an accountability and representation perspective.

## Events hosted by SMA Europe

**Annual Meetings**
24-26 April 2025, Athens, Greece
The event was attended by more than 50 people, including SMA Europe delegates, industry representatives and other invited guests.

**1st International Clinical Care Symposium on SMA**,
29-30 August 2025, Milan, Italy
The event brought together nearly 200 participants from around the world, on-site and virtually.

## SMAcademy workshops and webinars

**Understanding the new EU Joint Clinical Assessment and its possible implications**
January 2025
Speakers: Paolo Morgese and Tresia Bolt.

**Empowering advocacy & advancing adult SMA care: benchmarking report workshop**
March 2025
Speaker: Anne-Pierre Pikaert.

**Design, launch, impact: how to run a successful awareness campaign**
May 2025
Speaker: Emilia Debska.

**Modern approaches to managing orthopaedic challenges in SMA**
November 2025
Speakers: Michail Kokkinakis and Luca Labianca.

## Webinars hosted by SMA Europe

**Screen at birth, save lives — 5 Years of the European Alliance for Newborn Screening in Spinal Muscular Atrophy webinar**
11 December 2025, online.

## SMA Europe governance-related meetings

**2025 General Assembly meeting**
26 April 2025, Athens, Greece, hybrid
Attended by 27 delegates.

**Delegates call- summer break**
10 July 2025, online
Attended by 20 delegates.

**Delegates call – updates**
4 November 2025, online
Attended by 27 delegates.

**Delegates call – festive season breakdown**
16 December 2025, online
Attended by 22 delegates.

## Contribution to external events

**285th ENMC International Workshop on SMA and the brain**
31 January 2024 – 2 February 2025, Hoofddorp, The Netherlands
Talk presented by **Yasemin Erbas** (President, SMA Europe) and Portia Thorman (SMA Europe Delegate for SMA UK).

**DIA Congress Basel**
18 March – 20 March 2025, Basel, Switzerland
Attended by **Nicole Gusset**, CEO SMA Europe.

**ENMC General Assembly**
4 April – 6 April, Hoofddorp, The Netherlands
Attended by **Nicole Gusset**, CEO, SMA Europe.

**Cure SMA 2nd International SMA Patient Advocacy Group Meeting**
28 June 2025, Anaheim, USA
Invited speaker: **Nicole Gusset**, (CEO, SMA Europe) "From dreams to reality: how research makes it possible", attended by several other SMA Europe delegates, among others.

**Cure SMA 2nd International SMA Patient Advocacy Group Meeting**
28 June 2025, Anaheim, USA
Invited speaker: **Yasemin Erbas** (President, SMA Europe), "Towards a new disease description for SMA", attended by several other SMA Europe delegates, among others.

**Cure SMA Annual SMA Research & Clinical Care Meeting and the Annual SMA Conference**
26-29 June 2025, Anaheim, USA
Attended by **Yasemin Erbas** (President, SMA Europe), **Nicole Gusset** (CEO, SMA Europe), **Federica Fontana** (Research and Medical Manager, SMA Europe), and by several other SMA Europe delegates.

**NBS for rare diseases: How can EU lead on the global stage? Meeting at the European Parliament hosted by the three MEPs Tomislav Sokol (Croatia), Sirpa Pietikäinen (Finland) and Billy Kellerher (Ireland)**
23 September 2025, Brussels, Belgium
Attended by **Marie-Christine Ouillade**, Chair of the SMA NBSA.

**Revisiting nomenclature, mechanisms and assessment of fatigue and fatigability in SMA: Towards enhancing patient outcomes**
24 October - 26 October 2026, New York, USA
Talk 'SMA Europe Fatigue Experience' presented by **Yasemin Erbas**, President of SMA Europe.

**7 years of the SMA Drug programme. How does Poland rank in the treatment of Spinal Muscular Atrophy compared with other European Countries?**
4 November 2025
Talk presented by **Nicole Gusset** (CEO, SMA Europe).

# Publications and knowledge outputs

## Peer-reviewed articles

**Erbas Y**, Finkel RS

**Ad astra per aspera: treatment challenges and opportunities for children with spinal muscular atrophy and tracheostomy**

Gene Ther. 2026 Jan;33(1):84-86. Epub 2025 Nov 25
https://doi.org/10.1038/s41434-025-00581-2

**Erbas Y**, Servais L, Shieh PB, Goedeker NL, Waldrop MA, Bo R, Raju D, Benguerba K, Reyna SP, Wolff D, Finkel RS

**Trach and treat: Safety and motor outcomes following onasemnogene abeparvovec in patients with spinal muscular atrophy and tracheostomies in the RESTORE registry**

J Neuromuscul Dis. 2025 Nov 11:22143602251395173
https://doi.org/10.1177/22143602251395173

Gómez-Andrés D, Farrar MA, Alvarez-Molinero M, Garcia-Uzquiano R, Brusa C, Baranello G, Quijano-Roy S; 285th ENMC Workshop participants (including **Erbas Y.**)

**285th ENMC international workshop: SMN-associated neurodevelopmental disorder: type 1 spinal muscular atrophy and the brain**
31st January - 2nd February 2025, Hoofddorp, The Netherlands

Neuromuscul Disord. 2026 Mar:60:106331. Epub 2025 Dec 30 https://doi.org/10.1016/j.nmd.2025.106331

Weiß C, Vill K, Baumann M, Bernert G, Blaschek A, Eisenkölbl A, Flotats-Bastardas M, Friese J, Ganter C, Goldhahn K, Hahn A, von der Hagen M, Hartmann H, Hasselmann O, Horber V, Husain RA, Illsinger S, Jacquier D, Johannsen J, Köhler C, Kölbel H, Kolodzig M, Klein A, Pechmann A, von Moers A, Müller-Felber W, Rauscher C, Schara-Schmidt U, Schreiber G, Schwartz O, Sproß J, Stettner GM, Stoltenburg C, **Stumpe E**, Trollmann R, Wiegand G, Wilichowski E, Kirschner J, Ziegler A; collaborators and members of the INTEGRATE ATMP consortium

**Delphi consensus on gene therapy of spinal muscular atrophy with onasemnogene abeparvovec in Germany, Austria and Switzerland-part I-systematic literature review and existing evidence**

J Neuromuscul Dis. 2025 Nov 24:22143602251387019
https://doi.org/10.1177/22143602251387019

## Scientific posters by SMA Europe

Erbas Y., Larotonda, A. & Gusset, N.

**Experiences of Clinical Trial Participation in SMA: Insights from a Pan-European SMA Europe survey**
Presented at the Cure SMA Annual SMA Research & Clinical Care Meeting, 25-29 June 2025, Anaheim, California (USA)

Fontana F., Christie-Brown V., Claus P., Debska E., Gillingwater T. H., Erbas Y. & Gusset N.

**Identifying Research Priorities in SMA: A European Study on the Perspectives of Healthcare Professionals and Patients**
Presented at the Cure SMA Annual SMA Research & Clinical Care Meeting, 25-29 June 2025, Anaheim, California (USA)

Gusset, N., Debska, E., Fontana, F. & Erbas Y.

**Towards a Research Agenda for SMA**
Presented at the Cure SMA Annual SMA Conference, 26-29 June 2025, Anaheim, California (USA) and at the 1st International Clinical Care Symposium on SMA, 29-30 August 2025, Milan, Italy

Gusset, N., Gumbert, A., Larotonda, A. & Erbas Y.

**OdySMA – Tracking access to SMA treatment and care through quantitative and qualitative data**
Presented at the Cure SMA Annual SMA Research & Clinical Care Meeting, 25-29 June 2025, Anaheim, California (USA)

## Publications by members of SMA Europe

Plueschke K, Jonker C, Kankanen H, Vetter T, Sepodes B, Naehrlich L, Hillert J, Crane G, Straus S, Foggi P, Martin S, Kyriakopoulou C, Mol P, Day F, Breen K, Bennett N, **de Lemus Belmonte M**, Bennett S, Verpillat P, Roes KCB, Cochino A, Schaefer F, Hernández-Rivas JM, McGettigan P, Arlett P.

**Optimizing Patient Registries for Regulatory Decision Making - Key Learnings From an HMA/EMA Multistakeholder Workshop**

Clin Pharmacol Ther. 2025 Sep;118(3):551-560. Epub 2025 Jun 2
https://doi.org/10.1002/cpt.3733

Cattinari MG, Pascual-Pascual SI, **de Lemus M**, Medina J, Dumont M, Rebollo P, Vázquez-Costa JF.

**Preliminary psychometric validation of patient-reported outcomes relevant to individuals with spinal muscular atrophy and their caregivers**

Orphanet J Rare Dis. 2025 Jun 4;20(1):274
https://doi.org/10.1186/s13023-025-03832-y

Cattinari MG, **de Lemus M**, Dumont M, Tizzano E.

**[Spinal Muscular Atrophy: The Reality of the Adult Patient in Spain]**

Rev Neurol. 2025 Mar 27;80(2):33462
https://doi.org/10.31083/rn33462

Jonker CJ, Plueschke K, Breen KC, **de Lemus Belmonte M**, Verpillat P, Pacurariu A.

**Learnings from a registry-based cohort study for spinal muscular atrophy disease**

Neuromuscul Disord. 2026 Feb:59:106332.
Epub 2025 Dec 30
https://doi.org/10.1016/j.nmd.2025.106332

Cattinari M. G., **de Lemus M.**

**Beyond SMN: What's Next? Broadening the Focus on Spinal Muscular Atrophy**
Presented at the 1st International Clinical Care Symposium on SMA, 29-30 August 2025, Milan, Italy

Cattinari M. G., **de Lemus M.**

**Emergency information card for patients with Spinal Muscular Atrophy (SMA): An evidence-based tool for acute care in rare and complex diseases**
Presented at the 1st International Clinical Care Symposium on SMA, 29-30 August 2025, Milan, Italy

# Publications and knowledge outputs

## One Goal series

Larotonda, A., Erbas, Y. & Gusset, N. 2025

**The European Patient Experience Survey on SMA Clinical Trials. One Goal Series - Together we prioritise.**

ISSN 3134-6626.

See the publication

## Other publications

SMA Europe (2025)

**Abstract book. 1st International Clinical Care Symposium on SMA: Medical Emergencies in SMA: pathophysiology, prevention, and response.**

See the publication

## Media coverage

**SMA Europe announces the awardees and projects shortlisted through its call for research no.12**
Rare Revolution Magazine, 28 February 2025

**SMA Europe provides funding for five new research projects in SMA**
SMA News Today, 3 March 2025

**Spinal cord stimulation: A new perspective for SMA research**
Rare Revolution Magazine, 23 May 2025

**Navigating Patient-Focused Innovation: One Company's Journey with the SMA Community**
Global Forum, July 2025 issue

**SMA community shines light on SMA as part of awareness month**
SMA News Today, 1 August 2025

**Interview with Nicole Gusset, Rare Revolution Magazine**
Beyond the Breakthroughs" edition August 2025

**New SMA awareness campaign aims to link research, patient needs**
SMA News Today, 5 September 2025

**SMA Europe launches new clinical trials finder to support access to clinical trials and accelerate progress in SMA treatment**
Rare Revolution Magazine, 30 September 2025

**SMA Europe online tool helps patients find clinical trials**
SMA News Today, 10 October 2025

## Websites

**SMA Europe website: SMA Europe**
Home shares the latest updates on projects, initiatives, updates on projects, initiatives and source of news and relevant resources for our SMA Community.

See the website

**Clinical Trials Finder: SMA Europe**
Clinical Trials Finder which is directly connected to clinicaltrials.gov and is calibrated to only retrieve data associated with Spinal Muscular Atrophy, updating new data instantly.

See the website

**OdySMA: OdySMA**
Highlights progress in access to SMA treatment across Europe, alongside real-life stories from people living with SMA in diverse countries.

See the website

**SMA Newborn Screening Alliance: NBS Alliance**
Provides the updates on newborn screening for SMA across Europe.

See the website

# Financial overview and sustainability





## Financial governance

Financial governance at SMA Europe is based on clear oversight mechanisms, defined responsibilities, and transparent decision-making processes, in line with the organisation's statutes and applicable non-profit requirements.

The Board exercises oversight of the organisation's financial management, including regular review of financial reporting, monitoring of income and expenditure, and consideration of financial risks and sustainability. Financial information is prepared by the executive management and presented to the Board to support informed oversight and decision-making. Accounting services are provided by Armin Pfeuffer Dipl.-Kfm, and the organisation's statutory audit is conducted by Bansbach GmbH, providing independent verification of financial reporting and compliance.

The annual budget is prepared by the executive management, reviewed by the Treasurer and then submitted to the Board for review prior to approval by the GA, in accordance with statutory procedures.

Budget implementation is monitored throughout the year, and material deviations are reported through established governance channels and addressed through appropriate management or Board action. Together, these processes ensure prudent stewardship of SMA Europe's resources and accountability to its members and stakeholders.

| **Accounts** | 01.01.2025 | 31.12.2025 |
|---|---|---|
| HVB Bank | 2'240'527.35 € | **2'139'395.55 €** |
| PayPal | 3'074.87 € | **0 €** |

| **Revenue sources** | **amount** |
|---|---|
| Patient organisations | **268'500.00 €** |
| Donations and grants | **135'729.74 €** |
| Corporate funding | **566'000.00 €** |
| Services and events | **36'301.77 €** |
| **Total revenue** | **1'006'531.51 €** |

| **Expenses** | **amount** |
|---|---|
| **OPERATING EXPENSES** | |
| Staff | 48'313.65 € |
| Volunteers | 5'282.44 € |
| Logistics | 74'244.79 € |
| Material | 813.35 € |
| Services received | 15'309.61 € |
| Other operating expenses | 10'455.14 € |
| **Total operating expenses** | **154'418.98 €** |
| **DIRECT PROJECT EXPENSES** | |
| Communication and outreach | 70'491.90 € |
| Capacity building | 137'659.49 € |
| Research | 406'869.46 € |
| Therapy and care | 130'655.77 € |
| Healthcare, policy and access | 210'542.58 € |
| **Total project expenses** | **956'219.20 €** |
| **Subtotal** | **1'110'638.18 €** |
| **Total expenses** | **1'095'079.18 €** |

| | |
|---|---|
| **Result 2025** | **-88'547.67 €** |

Explanations:

**Patient organisations:**
Membership fees and earmarked contributions from members for the Call for Research

**Donations and grants**
Grants received from Cure SMA

**Corporate funding**
Financial support received through corporate funding. In 2025, we have received funds from Asuragen, Biogen, F.Hoffmann-La Roche Ltd, ImmunoIVD, Novartis, Scholar Rock, and ZenTech

**Services and events**
Revenue generated by our International Clinical Care Symposium, and our TC and individual volunteer activities through which we raise the patient voice and advocate for the needs of our community

**Miscellaneous**
Refunds

**Volunteers**
Volunteer work, including travel expenses

**Logistics**
International Sceintific Congress, Annual General Meeting, Workshops, Staff Retreat and travel expenses of staff and Board members

**Services received**
Microsoft 365, software, website, bank fees, accounting services, insurances, external pay-roll services, and other external services

**Other operating expenses**
Membership fees to other organisations, staff and volunteer external trainings, taxes

**Communication and outreach**
Direct project expenses on Awareness and Communication Activities including the Awareness Campaign

**Capacity building:**
SMAcademy, the Global SMAdvocacy Event, and Supporting Families in Crisis Situations

**Research**
Call for Research, the Research Agenda Project and SMArt Horizon.

**Therapy and care**
Community Research Projects such as the EUPESMA Survey Series, Focus Groups, Clinical Trial readiness activities

**Healthcare, policy and access**
NBS Alliance and OdySMA

**Audit of the income surplus statement (Einnahmenüberschussrechnung) for the 2024 financial year**

Our finances are audited by Bansbach GmbH. On April 30, 2026, they concluded that the income and expenditure statement for the period from January 1, 2025, to December 31, 2025, complies with the requirements of Sections 64 et seq. of the German Fiscal Code (AO) in conjunction with Section 4 (3) of the German Income Tax Act (EStG).

# Organisational sustainability

## Staffing model

The organisational model of SMA Europe combines a professional staff team with the active involvement of Board members, other volunteers, and community contributors. This structure supports flexible and efficient delivery while maintaining strong links to the community.

Board members contribute through regular Board meetings and ongoing engagement across strategic areas. In addition to their formal governance role, Board members support organisational development and provide strategic input, drawing on their expertise and connection to member organisations.

As a fully virtual organisation, SMA Europe places emphasis on effective internal communication, coordination, and inclusive working practices across geographically distributed teams. Staff are supported through onboarding, performance management, and appropriate employment practices.

Depending on the nature of activities, staff, volunteers, and delegates contribute in complementary roles, including both coordination and support functions within defined project structures. In some cases, volunteers take on coordinating roles in the delivery of specific initiatives, working in close collaboration with staff and within established organisational frameworks.

The organisation's work is further supported by committees and advisory structures, which contribute expertise and stakeholder perspectives to relevant areas, reinforcing structured input and alignment across activities.

## Operational resilience

During the reporting year, SMA Europe continued to strengthen its operational resilience through the development and refinement of internal processes, systems, and organisational structures. This included work to improve coordination across activities, further develop internal procedures, and prepare for the implementation of key systems, including the planned introduction of a CRM system. In parallel, efforts were made to support consistent delivery across a growing portfolio of activities and to strengthen the organisation's capacity to operate effectively within a fully virtual environment.



# Outlook to 2026

**Looking ahead, SMA Europe will focus on consolidating its organisational foundations, strengthening governance and financial sustainability, and advancing the transition into the 2026–2030 strategic cycle. Building on preparatory work undertaken in previous years, the coming period will mark the implementation phase of the organisation's new strategy. This outlook is shaped by a dynamic external environment, including evolving regulatory, funding, and healthcare system contexts across Europe.**

## Governance priorities

A central priority will be to ensure an orderly transition into the 2026–2030 strategic cycle, supported by clear oversight arrangements, defined mandates, and alignment between strategy, implementation, and evaluation. The organisation will integrate its KPI framework into routine governance and management processes, supporting transparency, performance monitoring, and organisational learning, and adaptive decision-making.

SMA Europe will continue to reinforce oversight of financial sustainability, including the implementation of a diversified fundraising and partnership approach. Maintaining independence, transparency, and compliance across funding relationships will remain a core governance responsibility.

Attention will also be given to leadership continuity and organisational stability through retention and succession planning across Board, staff, and volunteer structures including delegates.

## Organisational focus

Operationally, SMA Europe will prioritise the implementation of the 2026–2030 strategy through coherent planning, prioritisation of activities, and allocation of resources across all strategic pillars.

The organisation will continue to invest in internal capacity, including staff development, process optimisation, and the rollout of key digital systems. Strengthening coordination, knowledge retention, and data-informed decision-making will remain key priorities.

SMA Europe will further strengthen its network by supporting member organisations, volunteers, and peer exchange across Europe, reinforcing collective capacity at both European and national levels.

## Financial outlook and sustainability

Financial planning will be closely aligned with the implementation of the 2026–2030 strategy, ensuring that resources are directed toward priority objectives while safeguarding long-term sustainability. SMA Europe will continue to diversify income streams and monitor funding dependency in order to maintain strategic autonomy.

At the same time, the organisation will strengthen resilience by linking financial planning with workforce stability, capacity development, and prudent resource management. Through ongoing monitoring and governance oversight, SMA Europe aims to ensure sustainable growth and responsible stewardship of resources.

# Annex





## Acknowledgements

**SMA Europe appreciates the donations received from all its funders. Ensuring a sustained variety of funding is key to minimise potential conflicts of interest and to ensure the sustainability of the organisation.**

**SMA Europe would like to thank the following partners for their highly valued support in 2024:**

### SMA Europe's Member Organisations (in alphabetical order of countries)

SMA Austria
SMA Belgium
Muscular Dystrophy Association Cyprus (MDA Cyprus)
SMÁci, z. s. (Czech Republic)
Muskelsvindfonden (Denmark)
SMA Finland
AFM-Téléthon (France)
Deutsche Gesellschaft für Muskelkranke (DGM)/ Initiative SMA (Germany)
Muscular Dystrophy Association Hellas (MDA) (Greece)
SMA Foundation (Hungary)*
FSMA (Iceland)*
SMA Ireland
Families of SMA Israel
Famiglie SMA (Italy)
Prinses Beatrix Spierfonds (Netherlands)
Vereniging Spierziekten Nederland (Netherlands)
Stop SMA North Macedonia
Fundacja SMA (Poland)
Associação Portuguesa de Doentes Neuromusculares (APN) (Portugal)
Asociația SMACare (Romania)
SMA Family Foundation Russia
SMA Serbia
SMA Slovakia
FundAME (Spain)
Nätverket för spinal muskelatrofi (NSMA) (Sweden)
SMA Schweiz (Switzerland)
SMA Benimle Yürü (Turkey)
Turkey SMA Foundation
SMA UK
Children with SMA (CSMA) (Ukraine)

* Became full members in 2025.

### SMA Europe SAB

A special acknowledgement and 'Thank you' to our SAB. Our SAB is composed of an international group of neuroscientists and neurologists with particular expertise in SMA research, and advises us on our scientific congresses, clinical trials initiatives and on the selection and appraisal of the research grants we award.

Our SAB members in 2025:

Tom Gillingwater, Chair, UK
Stefania Corti, vice-Chair, Italy
Melissa Bowerman, UK
Claudio Bruno, Italy
Peter Claus, Germany
Richard Finkel, USA
Ewout Groen, The Netherlands
Cécile Martinat, France
Christian Simon, Germany
Charlotte Sumner, USA
Ludo Van der Pol, The Netherlands
Ulrika Kreicbergs, Sweden

### Treatment Committee

Co-chairs: Thomas Koed Doktor and Mencía de Lemus

### Adult Committee

Co-chairs: Lisbeth Doktor and Laetitia Ouillade

### EMA Committee

### Partner organisations

Cure SMA (US)
Cure SMA India
EAMDA
EMA (European Medicines Agency)
ENMC
EURO-NMD
Eurordis
SMA Foundation (US)
Treat-NMD

### Industry partners

Argenx
Biogen
Bio-Techne (Asuragen)
F. Hoffmann-La Roche AG
ImmunoIVD
Novartis
Scholar Rock
ZenTech (LaCar)

### Other partners

161 Consulting Ltd - Michelle Levene
Alliance of Regenerative Medicines (ARM)
Bansbach GmbH
Betterplace
Ciompala, Mateusz
Copicentro las Tablas
Gardner Puzelli Media
Ebicus B.V.
Euractiv
Fundacja ReFocus Media Labs
Hernández, Elena Díez
Jana Popova
Karakaya, Seda
Kephren Publishing
Krishna Consultancy – Ankita Batla
MediCongress Services N.V.
Patvocates
Pickaert, El-Anne-Pierre
Pfeuffer, Armin Dipl.-Kfm
Rastogi, Akshat
Semantic Hub
Tóth, Tímea
Winheller

# List of abbreviations

**CRM**
Customer Relationship Management

**CT(s)**
Clinical Trial(s)

**ECR(s)**
Early Career Researcher(s)

**EUPESMA**
European Patient Experience Survey

**GA**
General Assembly

**NBS**
Newborn Screening

**PSP**
Priority Setting Partnership

**RD**
Rare Disease

**SAB**
Scientific Advisory Board

**SMA**
Spinal muscular atrophy

**TC**
Treatment Committee

SMA EUROPE

www.sma-europe.eu
communications@ sma-europe.eu

Follow us at:
Instagram | Facebook | LinkedIn | Bluesky

© 2026 SMA Europe. All rights reserved.