SMA
EUR
OPE

Annual report 2024

All together. One goal.

Index

Foreword

From needs to knowledge to impact: how research drives our spiral of progress

Dr. Nicole Gusset CEO SMA Europe

At SMA Europe, everything we do, whether in research, clinical trials, access, advocacy or improving care, is driven by one mission: to improve the lives of all people living with spinal muscular atrophy (SMA) across Europe. We see our work not as a checklist of separate tasks, but as a continuous, upward-moving spiral, where each activity builds on the last, informs the next, and brings us closer to meaningful, lasting change. This spiral model allows us to respond, adapt, and evolve alongside the needs of the community we serve.

At the heart of this spiral is the SMA community itself. It is the lived experiences, challenges, and priorities of people living with SMA that guide everything we do. That is why the process always begins with needs, not assumptions. Through our Priority Setting Partnership, we worked together with people living with SMA, families, and healthcare professionals to define what matters most. These priorities now shape our research agenda and drive the direction of our actions.

Research is the first pathway we take to begin addressing those needs, a starting point where ideas are explored, knowledge is built, and potential solutions are uncovered. Without it, progress cannot begin. But research alone is not enough. If left in the lab, even the most promising discoveries remain out of reach. That is why SMA Europe not only supports patient-relevant science, but also builds the bridge needed to turn research into real-world impact, so that our shared dreams can become tangible realities.

Our Call for Research Proposals, a competitive and transnational grant programme, ensures that research aligned with community priorities gets funded and advanced. Since its launch, more than 50 high-impact projects have been supported; in 2024 alone, five new projects were funded, each responding to an unmet need identified by the community itself.

To ensure research translates into effective treatments, we actively shape the clinical trial ecosystem. In 2024, we concluded our Clinical Trial Site Readiness assessment, which mapped infrastructure needs in sites across Europe and highlighted where investment is needed to ensure SMA remains a research priority. We also analysed the results of EUPESMA, our European Patient Experience Survey in SMA, to better understand people's expectations and experiences around clinical trials. These insights now inform the work of our Treatment Committee, which advises pharmaceutical and biotech partners on how to design trials that are not only scientifically rigorous, but also accessible, inclusive, and meaningful to those who participate.

To support this work, we began developing a Critical Assessment Guide to help patient advocates evaluate trial protocols from a community relevance perspective. We also ensured that the broader SMA community stayed informed about ongoing trials and developments, empowering families to make informed decisions about participation. In 2025, EUPESMA will expand further to explore treatment experiences, including perceptions of new therapies, combination treatments, and the sensitive area of treatment discontinuation, gathering knowledge that can guide better care, shared decision-making, and advocacy. Importantly, patient experience data is increasingly recognised as a valuable source of evidence to inform regulators and reimbursement authorities, helping to shape decisions about treatment approval, access, and funding. Through EUPESMA, we aim to ensure that the voices of people living with SMA actively contribute to how therapies are evaluated and made available.

Our commitment to meaningful progress also includes investing in people. Through initiatives like SMAcademy and our Global SMAdvocacy event, we are building a strong, informed network of patient advocates who can lead change within their own countries and contribute to a stronger “patient voice”. These are not isolated events, they are part of a long-term effort to build capacity across the community. Much of this work focuses on how to move from needs to knowledge to impact, ensuring that advocates are equipped to act effectively and sustainably. In 2025, alongside ongoing webinars and workshops, we will finalise a formal SMA advocate training programme and the SMA Journal Club, providing structured learning opportunities to deepen understanding of research, access, care innovation, and evolving health systems frameworks.

One of the most powerful moments in 2024 was the Global SMAdvocacy Event in Ghent, where advocates from all six continents came together to share experiences, align strategies, and reinforce a unified global voice for SMA. Our strength as a European organisation is amplified when we connect to the wider global movement, because progress does not stop at borders, and neither should solidarity.

All of this is only possible because SMA Europe stands on a strong foundation. Today, we are a robust, well-governed organisation, supported by solid legal and financial structures, an experienced and motivated team of staff, and a committed group of volunteers. Over the past three years, we have worked hard to build internal systems that are not only efficient and transparent, but also sustainable, enabling us to act with agility and impact.

Our drive for impact runs through every initiative we lead. Through our Scientific International Congress on SMA, we bring together more than 1’000 participants, from early career researchers to leading clinicians and scientists, and patient advocates, to share knowledge, build partnerships, and advance the field. Through OdySMA – A Quest to Access, we systematically track disparities in access to diagnosis, treatment, and care across our member countries, while equipping local advocates with data and tools to push for national change. Through the launch of our International Clinical Care Symposium, we are working to address urgent, life-threatening gaps in care—especially during acute crises such as respiratory or metabolic emergencies.

We also know that raising awareness builds connection and power. Campaigns like #WeAreUnique and #WeAreOne have given voice to the diversity, resilience, and unity of our community, offering powerful stories, visual narratives, and artistic expressions that bring SMA into the public spotlight while reinforcing our shared identity. These campaigns were also deeply rooted in the needs and hopes of our community. They helped frame research as a source of possibility and progress, showing how it can transform hope into reality. By highlighting the ongoing importance of research, they helped educate and engage the community in a topic that often feels abstract or far removed from daily life. In doing so, the campaigns fostered a deeper understanding of why supporting research remains essential and help to build long-term commitment across the SMA community to stand behind the science that serves them.

From the first spark of research to the moment solutions reach an individual, from empowering advocates to shifting healthcare systems, SMA Europe's work reflects a spiral of continuous progress. Every insight, every partnership, and every action contribute to the next turn of that spiral. We never stop, because the needs of the SMA community are evolving, and so must we.

This is not a cycle with an end. It is a living commitment, one that renews itself with every innovation, every act of advocacy, and every expression of solidarity. Progress is not the task of one organisation alone. It is the shared responsibility of every individual, every member organisation, and every voice within our community. At SMA Europe, we are proud to be the platform that brings this collective strength together, to support, connect, and empower, but we cannot drive change alone. Lasting impact can only be achieved when we move forward together. And we will always strive for more, because the people we serve deserve nothing less.

All together. One Goal.
In solidarity,

N. Gusset

Dr. Nicole Gusset,
CEO SMA Europe

2024 highlights

#WeAreOne

456,402

views

+90

volunteers supporting SMA Europe's initiatives and projects

+40

publications, outreach contributions and media appearances

30

SMA organisations from 28 European countries

+70

patient advocates from 6 continents attending our Global SMAdvocacy event

+1000

participants at the 4th Scientific International Congress on SMA in Ghent”

+70

volunteers actively participating in SMA Europe’s projects, committees and sounding boards.

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. Only through true representation of the SMA community – the unique wants, experiences and aspirations of the people behind the condition – will their voices be heard and their needs be met.

All together. One goal.

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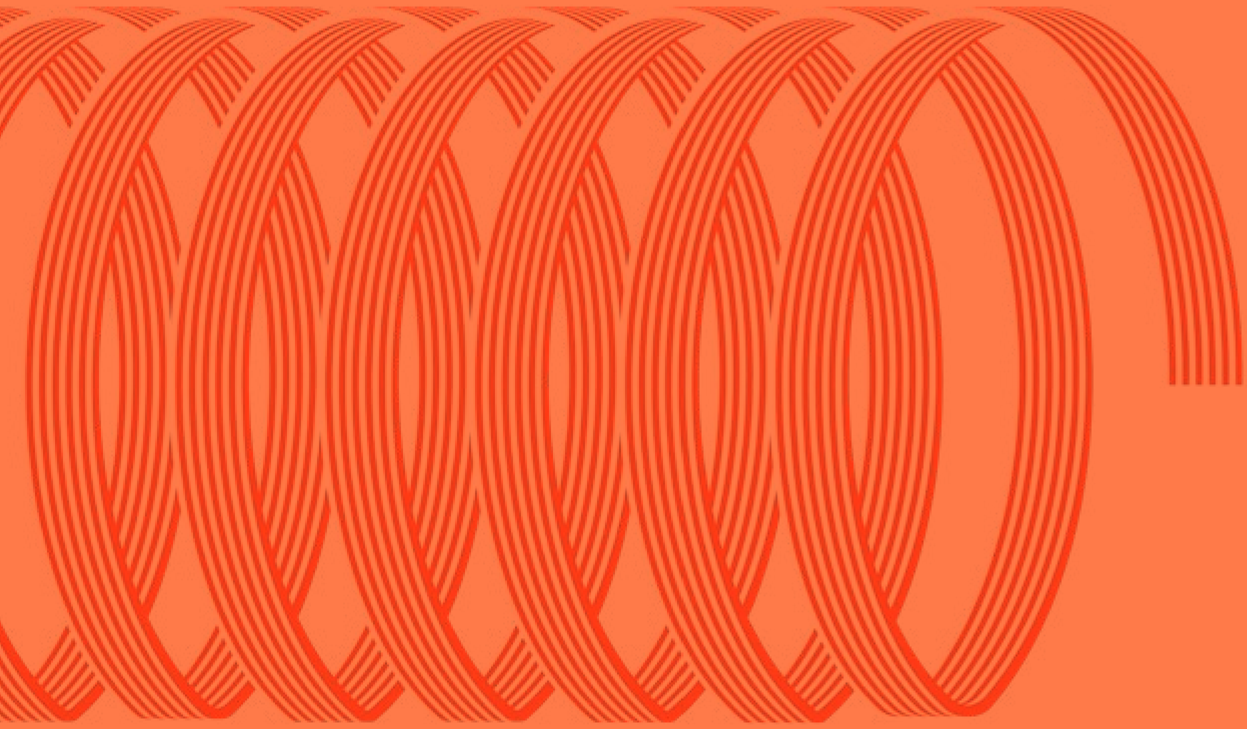
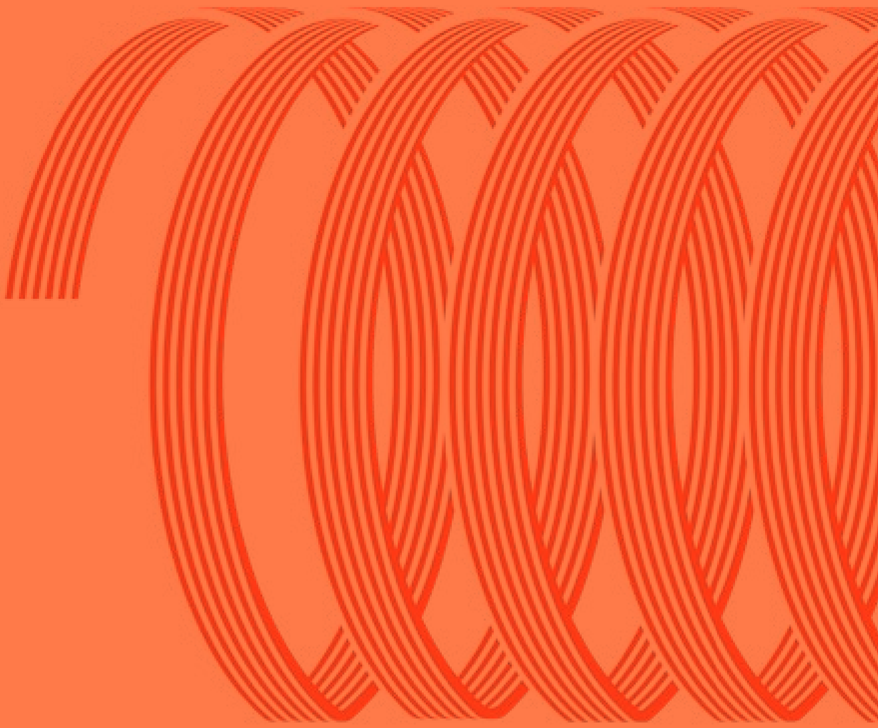


Our main projects and activities



> "At SMA Europe, we are dedicated to advocating for those affected by Spinal Muscular Atrophy, ensuring that every voice is heard, and every family has access to the support they need. Together, we are driving progress in research, treatment, and awareness—because no one should face SMA alone."

Jakub Tomczyk, Member of the board SMA Europe.

Routed in our mission, our 2024 projects are organised under **our strategic pillars.**

Research

Promote patient-relevant SMA research.

Research agenda of SMA Europe: priority setting partnership

Call for Research Proposals no.12

SMArt Horizon

Therapy and care

Accelerate progress in the diagnosis, treatment, and care of people living with SMA.

Clinical trials activities

EUPESMA

Healthcare system, policy and access

Improve access to diagnosis, optimal treatment, and care for all people living with SMA in Europe.

OdySMA – A Quest to Access

SMA NBS Alliance

Capacity building

Strenghten the organisations' efficiency, sustainability and capacity to take collective action and advocate on both national and international levels.

Annual meetings with general assembly

Our SMA Europe

SMAcademy

Communication and outreach

Ensure all the communications initiatives and actions to serve as the SMA Europe's voice for SMA patients and rely on evidence-based advocacy.

2024 Awareness campaign: One Community. Shared Dreams.

Events

Strenghten the organisations' international presence and facilitate creation of new sinergies among our stakeholders.

Global SMAdvocacy event

International Scientific Congress on Spinal Muscular Atrophy

#WeAreUnique Awareness Campaign Vernissage during the 4th International Scientific Congress on Spinal Muscular Atrophy in Ghent

Research

Research agenda of SMA Europe: priority setting partnership

Through a Priority Setting Partnership (PSP) process in collaboration with the James Lind Alliance, this project identified the top 10 unanswered research questions about SMA that are a priority to people living with SMA, their caregivers and healthcare professionals. An expert panel further ranked these questions in eight research areas. By engaging with a wide range of stakeholders, including research funders, clinicians, and industry representatives, these results enable SMA Europe to advocate for a unified approach that directs limited resources towards addressing the most pressing needs of those living with SMA.

— The top research priorities identified by the SMA community provides a tool to guide research efforts towards the most pressing needs of those living with SMA. —

What makes this project relevant to our SMA Community?

This project is relevant to the SMA community because it provides a tool for all key stakeholders to align future research efforts with the most pressing unmet needs identified by the community and health care professionals, ensuring that limited resources are directed where they can deliver greatest impact.

Main achievements in 2024

Initiated scientific discussions around the Top Research Priorities for SMA. SMA Europe hosted a dedicated workshop titled "Towards a research agenda for SMA" during the 4th Scientific International Congress on SMA. Further visibility was achieved through poster presentations at the Cure SMA 2024 Annual SMA Conference in Austin, TX, USA and at the Motor Neuron Diseases Workshop in Turin, Italy.

Launched a viral communication campaign on the Top 10 Research Priorities across all external and internal channels, successfully raising awareness and intending to mobilise the members and community support, including financial backing for research aligned with these priorities.

2025 Focus

Strengthening awareness and engagement through presentations at key scientific forums, including presenting a poster titled "Identifying Research Priorities in SMA: A European Study on the Perspectives of Healthcare Professionals and Patients", at the Cure SMA 2025 Annual SMA Research and Clinical Care Meeting.

Publishing the identified Top Research Priorities in a peer-reviewed scientific journal to support researchers in using these insights to shape their research focus and strengthen funding applications.

Embedding research priorities into the design of the 5th Scientific International Congress on SMA and the framework of the 13th Call for Research Proposals, ensuring they serve as a compass for future scientific and funding agendas.

TOP 10 FUTURE RESEARCH PRIORITIES

TOP 10 FUTURE RESEARCH PRIORITIES
#1
Regenerating motoneurons

TOP 10 FUTURE RESEARCH PRIORITIES
#3
Rebuilding and strengthening damaged muscles

TOP 10 FUTURE RESEARCH PRIORITIES
#4
Exploring metabolism and diet

TOP 10 FUTURE RESEARCH PRIORITIES
#6
Understanding the impact of SMA on the body

TOP 10 FUTURE RESEARCH PRIORITIES
#7
Developing innovative therapies such as spinal cord stimulation

TOP 10 FUTURE RESEARCH PRIORITIES
#9
Finding solutions to manage fatigue

TOP 10 FUTURE RESEARCH PRIORITIES
#10
Leveraging technology and assistive devices


TOP 10 FUTURE RESEARCH PRIORITIES

#2

Identifying biomarkers


TOP 10 FUTURE RESEARCH PRIORITIES

#5

Tailoring physiotherapy


TOP 10 FUTURE RESEARCH PRIORITIES

#8

Advancing treatments for the skeletal system

Call for Research Proposals no.12

SMA Europe is unique in supporting cutting-edge scientific research on all aspects of SMA. No European organisation launches a research call focusing exclusively on spinal muscular atrophy. Launched every two years, the Call for Research aims to support research proposals that address the needs of people living with SMA and demonstrate a clear pathway to impacting those in the short or longer term. The projects are evaluated by SMA Europe's Scientific Advisory Board, which consists of neuroscientists and neurologists with specialised expertise in SMA research, along with external SMA expert reviewers. Including this 12th round, the Call for Research Proposals has funded 53 projects, with a total amount exceeding 6 million €.

> — The Call for Research Proposals initiative supports scientific and medical research in SMA, aiming to address the unmet needs of individuals living with SMA and to lead to new solutions. —

What makes this project relevant to our SMA Community?

The "Call for Research" is a vital mechanism through which SMA Europe channels funding into high-impact, high-quality, patient-relevant science. It prioritises research that addresses knowledge gaps in SMA pathophysiology and shows clear potential to lead to meaningful therapeutic advances—whether short- or long-term. This approach ensures that the community's resources are directed towards innovations that matter most to people living with SMA.

Main achievements in 2024

Launched the Community Review Panel. Starting with the 12th edition, a Community Review Panel, composed of patient experts or caregivers with a strong background in research and/or patient advocacy, was formally integrated into the evaluation process, strengthening the alignment of funding decisions by incorporating patients' priorities.

Applied a priority-driven funding strategy. Guided by the outcomes of the Priority Setting Partnership, the priorities for the 12th Call for Research were:

- Reviving the motor unit in patients with SMA with a specific interest for the role of neuromuscular junction (dys)function;
- Biomarkers within and beyond the neuromuscular system;
- How metabolism is affected in SMA and how this might influence peripheral organs.

Awarded five innovative research grants. Through the 12th Call for Research, SMA Europe with its funding organisations supported five outstanding research teams across Europe:

- Dr. Sorana Ciura, Institute Imagine (France)
- Dr. Nathalie Didier, INSERM (Institut national de la santé et de la recherche médicale, France)
- Dr. Morgan Gazzola, I-Stem (Institute for Stem Cell Therapy and Exploration of Monogenic Diseases, France)
- Prof. Dr. Simon Parson, University of Aberdeen (UK)
- Dr. Gabriella Viero, National Research Council (Italy)

The 12th Call for Research proposals was generously co-funded by AFM-Téléthon, Initiative SMA, FundAME, SMA Schweiz, SMA UK, Fundacja SMA, and SMA Europe. This demonstrates the strong collaboration within the SMA community and reflects the very mission on which SMA Europe was founded: to jointly support research that benefits all the SMA community.

2025 Focus

Reviewing and refining evaluation procedures. Building on the experience gained from the 12th Call for Research, SMA Europe will revise the Community Review Panel process to enhance collaboration and alignment between the Scientific Advisory Board and the community reviewers, ensuring that scientific excellence and patient priorities come together to identify the most impactful projects.

Mobilising resources for the 13th Call for Research. SMA Europe will advocate to member organisations to secure the necessary funding to launch the 13th Call. This reaffirms SMA Europe's ongoing commitment to advancing research for SMA, a condition that, despite all the progress, still has many unmet needs and lacks a cure.

SMArt Horizon

The SMArt Horizon aims at identifying innovative, “out of the box” therapeutic strategies for SMA that go beyond existing treatments and traditional targets. By actively scanning diverse research fields, including regenerative medicine, precision medicine, and cutting-edge technologies like AI, robotics, and nano-technologies, SMArt Horizon aims to identify groundbreaking approaches that can then be tailored specifically to SMA with the ultimate goal of modifying disease progression, restoring function and thereby fundamentally improving quality of life for those affected. The project is a collaborative initiative of SMA Europe and the SMA Foundation and is largely financially supported by FundAME through its Calendar Funds, Spierziekten Nederland and SMA Schweiz.

What makes this project relevant to our SMA Community?

SMArt Horizon represents a pivotal shift towards pioneering solutions beyond conventional treatments. What makes this project relevant to our community is its focus on identifying new avenues of therapy that complement existing treatments.

Main achievements in 2024

Co-funded the innovative study “Characterization of the effects of spinal cord stimulation on arm and hand kinematics and motoneuron firing rates in people with non-ambulatory SMA”, led by Dr. Marco Capogrosso (University of Pittsburgh)

2025 Focus

Driving the advancement of a promising study on upper limb spinal cord stimulation, with the aim of ensuring that, if proven safe and effective, it can be rapidly made available to the entire SMA community.

Entering a re-assessment phase to evaluate the potential for identifying additional high-impact projects and how to secure new funding to support these.

— SMArt Horizon is a collaborative partnership, aimed at identifying innovative, “out-of-the-box” therapies for SMA to modify diseased progression and restoring function. —

Therapy and care

Clinical trials activities

Clinical trials are a critical step in turning scientific discoveries into approved treatments that can improve and extend the lives of people living with SMA. For our community, clinical trials are not just a scientific process, they represent access, equity, and hope. SMA Europe plays a key role in ensuring that these trials are designed, communicated, and implemented with the patient perspective at their core.

Our involvement spans multiple stages of the clinical trial process. We gather insights from people with SMA through community surveys (e.g. EUPESMA) and workshops to understand their expectations for future treatments and clinical trial conditions. We also support education and transparency by providing tools, such as awareness videos, webinars, and media updates, so that the community is informed and empowered to engage in clinical research. Through our Treatment Committee, we advise pharmaceutical companies and start-ups on trial design, helping to make protocols more inclusive, feasible, and aligned with patient needs. We also work to support fast and fair recruitment via our upcoming Clinical Trial Finder (2025) and by strengthening site readiness through data collection. These efforts help ensure that SMA remains a priority in a competitive research environment and that innovations can reach those who need them most.

SMA Europe is collaborating on some of these efforts with the Cure SMA Industry Collaboration.

What makes the activities relevant to our SMA Community?

In 2024, SMA Europe worked closely with a wide range of partners, including member organisations, community, pharmaceutical industries, healthcare professionals, and regulators, to ensure that clinical trials are designed, communicated, and implemented with the community's needs at the centre.

A key foundation of this work was the clinical trial community survey, which gathered real-world insights into the experiences and expectations of trial participants. These findings highlighted critical areas for improvement, such as reducing the burden of participation and improving trial accessibility. The results will inform the work of our Treatment Committee, which engages with industry to advise on the development of more inclusive, patient-relevant trial protocols. To strengthen this influence further, we began developing a critical assessment guide, a practical tool that will help both our Treatment Committee and member organisations evaluate trial designs and advocate for protocols that reflect patient priorities and support equitable access to future treatments.

At the same time, we remained committed to keeping the community informed, providing regular updates on new developments in the clinical trial landscape. This ensures that individuals and families are equipped to make informed decisions about trial participation—empowering them to actively engage in shaping the future of SMA research. Together, these activities represent a comprehensive and coordinated effort to align clinical research more closely with the lived realities and hopes of our community.

Main achievements in 2024

Concluded the Clinical Trial Site Readiness assessment, collecting data from sites across Europe to understand capacity challenges and infrastructure gaps. The results will guide future planning and ensure SMA remains an attractive condition for clinical research investment.

Initiated the development of a guide for Treatment Committee members, focused on how to critically assess aspects of clinical trial design from a patient relevance perspective. The process began with a prioritisation workshop to define key focus areas.

Provided structured advice to industry partners, advocating for more inclusive, patient-friendly trial designs through Community Advisory Boards facilitated by the Treatment Committee.

Analysed results from a community survey on clinical trial experiences, highlighting participant needs and preferences to reduce burden and support faster, more sustainable recruitment across Europe.

Regularly updated the community on new developments in the clinical trial landscape through our Clinical Trial Hub and through our communication channels, ensuring individuals and families were well-informed and empowered to make educated decisions about trial participation.

2025 Focus

Publishing and disseminating a first-of-its-kind booklet, combining findings from the Clinical Trial Site Readiness assessment and the community survey on clinical trial experiences. This publication will serve as an advocacy tool to improve trial conditions and site support.

Presenting research findings at scientific congresses and in peer-reviewed publications, expanding the visibility of patient-generated data and insights.

Developing the first chapters of the "Guide to Critical Assessment" for patient advocates, supporting more targeted and informed contributions to trial planning and evaluation.

Expanding the use of Community Advisory Boards in collaboration with industry partners, ensuring that trial designs are aligned with patient needs and do not compromise future treatment access or reimbursement pathways.

IN FOCUS

Paving the way for smarter, more inclusive clinical trials

SMA Europe is committed to accelerating access to safe and effective treatments for everyone living with spinal muscular atrophy (SMA). A central part of this mission is supporting meaningful patient involvement in clinical research and promoting innovative clinical trial designs that are better aligned with the realities of rare disease communities.

As described by Dr. Nicole Gusset (Gusset, Regulatory Affairs Watch, 2024), traditional clinical trials often struggle to meet the needs of rare conditions like SMA, where populations are small, diverse, and outcomes complex. Complex and adaptive trial designs, such as protocol flexibility, external control arms, and the use of real-world data, can reduce the burden on participants, speed up development, and improve trial relevance. These approaches broaden eligibility, support cross-border participation, and allow researchers to more efficiently evaluate potential therapies across the full spectrum of the condition.

In addition, decentralised clinical trials (DCTs) help shift elements of participation closer to home, reducing travel and physical demands for individuals and families. When paired with mobile technologies, like wearables and remote monitoring, clinical research becomes more patient-centred, capturing real-time and more meaningful data. This is particularly important in SMA, where traditional outcome measures may not reflect what truly matters to the community.

To ensure these innovations benefit patients, SMA Europe advocates for early and continuous patient involvement in the trial process. By working closely with researchers, regulators, and industry partners, SMA Europe helps define patient-relevant endpoints, ensures informed participation, and promotes ethical, inclusive research practices. In doing so, SMA Europe plays a vital role in reshaping the future of clinical research, making it smarter, faster, and more aligned with the lived experience of people with SMA.

EUPESMA

EUPESMA (European Patient Experience Survey in SMA) is a recurring survey initiative that captures the evolving experiences and expectations of people living with SMA across Europe every second year. By collecting systematic, patient-led data on key topics, EUPESMA helps shape SMA Europe's advocacy agenda and informs external stakeholders about what truly matters to the community.

Furthermore, patient experience data is increasingly recognised by regulators and reimbursement authorities as a credible source of evidence to guide access and policy decisions. EUPESMA positions the SMA community as an active contributor in shaping how clinical research is evaluated and translated into real-world outcomes.

— The community would like to see more clinical trials in SMA and is willing to participate, provided the opportunity. Patient-centric clinical trial designs are key to the success of clinical trial recruitment and participation. —

EUPESMA 2023

What makes the activities 2024 relevant to our SMA Community?

The 2023 edition focused on clinical trials, exploring perceptions, expectations, and challenges related to trial participation, both from individuals with SMA and their caregivers, including those with and without trial experience. This data is especially relevant today, as more treatments for SMA become available and the role of clinical trials shifts. Understanding whether the community still sees value in participating in trials, and under what conditions, what challenges they are faced with, is critical to designing trials that are ethical, inclusive, and responsive to patient needs.

The findings provide insight not only into the experiences of those who have taken part in trials but also help us understand why some individuals have not, whether due to practical barriers, lack of information, or personal choice. These perspectives are critical to shaping more accessible opportunities in the future. In addition, successful clinical trials depend on efficient recruitment, high retention, and trust. The results of this survey will help inform strategies to improve all three, ultimately supporting the goal of bringing research results to the people who need them most.

Main achievements in 2024

Strengthened the evidence base on what enables or prevents trial participation in SMA, providing clear insights to shape patient-relevant and friendly trial design and engagement strategies by collecting survey data.

Amplified the community voice in reporting to key stakeholder including sponsors such as the Cure SMA Industry Collaboration.

Positioned SMA Europe as a key knowledge partner, translating community perspectives into practical recommendations for improving clinical trial frameworks by preparing a community Insights Report, designed to make complex survey data accessible and actionable for advocates, researchers, and sponsors.

2025 Focus

Driving advocacy by publishing our Insights Report, using the data from the survey findings to influence how clinical trials are designed and promoted, ensuring trials reflect real-world expectations and enable broader participation.

Supporting dialogue with stakeholders, by publishing the insights report and by reinforcing the importance of patient experience in clinical research evaluation.

Embedding insights into SMA Europe's broader clinical trial work, including clinical trial site readiness and Treatment Committee guidance, ensuring a feedback loop between community input and clinical research planning.

EUPESMA 2025

What makes the activities 2024 relevant to our SMA Community?

EUPESMA 2025 focuses on treatment experiences and expectations related to SMA medicines, involving people living with SMA, their caregivers, and healthcare professionals. As the treatment landscape evolves and new therapies emerge, understanding the real-world perspectives of those directly affected is essential.

This survey helps trace a comprehensive picture of the current treatment situation, including challenges faced by specific subpopulations (e.g. people using invasive ventilation) and experiences in less commonly discussed scenarios, such as treatment interruption. It also provides insight into the treatment decision-making process, capturing who is involved, what factors are considered, and where gaps in information persist. Importantly, the survey explores expectations and future wishes, including satisfaction with current therapies, interest in switching treatments, and hopes for upcoming options.

By gathering this patient experience data, SMA Europe aims to inform more responsive clinical practices and shape evidence-based patient advocacy, ensuring that emerging strategies reflect what truly matters to the SMA community. These findings will also serve as a valuable source of evidence for discussions with regulators, healthcare providers, and industry stakeholders, particularly in an era where treatment choice, personalisation, and informed decision-making are increasingly central.

Main achievements in 2024

Laid the groundwork for a high-impact evidence base, ensuring that the survey reflects the diverse realities of the SMA community by conducting literature review and facilitating focus group discussions with SMA Europe's Treatment Committee.

Engaged the community and clinical stakeholders early, by incorporating pilot feedback, resulting in a survey instrument that captures relevant, nuanced, and unmet needs.

Enabled broad participation by translating the survey into 17 languages, removing linguistic barriers and supporting representation across Europe.

Positioned the project as a cornerstone for future advocacy on treatment equity, switching, and personalisation.

2025 Focus

Securing ethics committee approval to ensure robust, ethical handling of participant data.

Generating insights from people living with SMA, caregivers and clinicians across Europe, that challenge assumptions, spotlighting the lived realities of treatment today, including satisfaction, challenges, and areas for improvement.

Elevating patient voices in SMA treatment policy, using the findings to influence decisions by stakeholders and **empower national advocacy**, by providing national organisations with clear data to support their discussions on access, personalisation and treatment choice.

Publish a community-focused Insights Report, offering accessible analysis and clear messaging to guide both policy and public engagement.

— EUPESMA 2025 will offer a unique look into how people living with SMA experience, choose, and evaluate treatments, highlighting what works, what is missing, and what truly matters. The results will guide advocacy for more personalised, informed, and equitable treatment strategies across Europe. —

Healthcare system, policy and access

OdySMA –
A quest to access

SMA Europe is committed to improving access to timely diagnosis, optimal treatment, and quality care for all individuals living with SMA across Europe. However, the journey to access is complex and varies greatly between countries. Understanding the different healthcare systems, assessing the current access landscape in each member country, and equipping advocates to engage effectively at the national level are all essential steps to drive change.

To address these challenges, SMA Europe launched OdySMA – a quest to access: an initiative that systematically identifies and tracks barriers to healthcare access across European countries and the entire SMA spectrum. OdySMA not only raises awareness about these obstacles but also advocates for solutions by highlighting best practices and sharing experiences from across Europe and calling for research to fill gaps.

A core tool of the initiative is the OdySMA platform, which serves as a central information hub and enables the monitoring of over 21 benchmark indicators to:

- Analyse, consolidate, and publish comparative data on access to diagnosis, treatment, and care in all SMA Europe member countries.
- Visualise disparities across countries and SMA subgroups to identify access gaps and promote targeted solutions.

Beyond data, OdySMA focuses on building advocacy capacity. The initiative equips patient advocates with tools, practical training, and one-on-one support to help them develop and implement effective national advocacy strategies. This ensures that advocates not only have access to the right information but also know how to use it for maximum impact.

OdySMA also integrates SMA Europe's broader efforts in healthcare systems, access, and policy by expanding our network in these fields and ensuring that the voice of the SMA community is heard in critical European or global level discussions.

— No one is left behind. —

What makes this project relevant to our SMA Community?

OdySMA directly strengthens the advocacy work of patient organisations across Europe by providing visualised data, practical tools, and a centralised knowledge hub. This supports national campaigns for better access to medicines and care, while also raising awareness about successful advocacy models in countries such as Denmark and the Netherlands.

By incorporating real-life stories, OdySMA brings attention to critical and often overlooked challenges in access to care — including gaps in adult services, ventilation support, and trial participation and diagnosis. These stories humanise the data, making it more relatable and powerful for stakeholders and policymakers to understand the unique situation of people living with SMA.

Our training sessions and supporting materials enable patient advocates to confidently use OdySMA in their own national contexts. Meanwhile, academic and policy engagement, such as presentations and posters at scientific and healthcare-related congresses, universities, and at the European Parliament, ensures that SMA-related access gaps remain visible to decision-makers and researchers alike.

Main achievements in 2024

Launched the Adult Care Benchmarking Report during Rare Disease Month, highlighting disparities in adult SMA care across member countries. The report highlights the processes and barriers within the national healthcare systems and includes concrete recommendations for national and European-level stakeholders and serves as a powerful advocacy tool for driving systemic change.

Integrated the first real-life stories alongside quantitative data to reveal critical nuances that numbers alone cannot capture. These stories illuminated the complex realities of people living with SMA, highlighting issues such as access inequalities, gaps in psychosocial support, and the disconnect between clinical standards and lived experiences across countries.

Strengthened visibility through high-level presentations, by sharing our insights in academic and policy forums, we amplified the voice of the SMA community and put patient needs at the centre of cross-sector discussions. Highlights included:

- **Top Scoring Poster Award** at ECRD 2024, recognizing the relevance of our contribution within the rare disease community.
- Participation in the 4th International Scientific Congress on SMA in Ghent and in the “Caring in Society” SMA Conference in Lisbon.
- **Guest lecture at Brown University** (USA), where real-life stories from the OdySMA project were used in a medical anthropology course to demonstrate the power of storytelling in patient advocacy.

Developed of additional advocacy tools, including a clinical trial authority list, an overview of the access situation for ventilated individuals with SMA and a Fact Sheet of the EU's Joint Clinical Assessment (JCA) process. And data collection on reimbursement pathways and crowdfunding models, with the aim to enable members to better navigate their national healthcare systems.

Expanded our network by becoming a member in the European Commission's HTA Stakeholder Network, and by partnering with other non-for-profit organisations in advocacy efforts on the Joint Clinical Assessment process and ATMPs (e.g. Call to Action coordinated by Alliance of Regenerative Medicines).

2025 Focus

Strengthening the advocacy capacity of SMA patient advocates by providing dedicated training on how to leverage the Adult Care Benchmarking Report as a national advocacy tool, with the aim of turning its recommendations into concrete policy change.

Supporting clinical trial recruitment in Europe by developing and launching an interactive, user-friendly platform with mapped clinical trials across Europe and globally. Integrated into both the OdySMA platform and SMA Europe's website, this tool will help the community better understand ongoing trials and accelerate access through improved participation.

Raising awareness about existing gaps in access to medicines, care, and diagnosis by continuing to collect and publish real-life stories, while also highlighting best-practice examples from the community to showcase what is working well across countries.

Enhancing the tools available to patient advocates by developing new resources based on the results of our reimbursement and crowdfunding survey, helping members to navigate national access systems more effectively.

Assessing the strategic potential of OdySMA as an advocacy tool by analysing how it can be further developed to create impact across all stakeholders at both national and European levels.


SMA
EUR
OPE
All together
One goal
SMA
EUR
OPE

SMA NBS Alliance

SMA NBS Alliance aims to bring together stakeholders to demand that NBS programmes in all European countries include a test for SMA for all new-born children. The objective is to decrease the time it takes for a child born with SMA to be diagnosed and to assist patient organisations in their efforts.

What makes this project relevant to our SMA Community?

When SMA NBSA started the work, no newborns were screened for SMA in Europe.

Since SMA NBSA started, many countries have added SMA to their national neonatal screening programmes, and some others have an active pilot programme (in some cases for the whole country, and in others at least for certain regions).

Main developments in 2024

Active support to national entities like Greece, Romania, Cyprus, contact with Spain and some support outside Europe (Morocco)

Workshop at the SMA Europe scientific congress in Ghent (March 2024)

Social media campaign for NBS day

Member survey on best-practices about how to support newly diagnosed families

EU policy: Participation in events: ERN meeting in Paris (February 2024), EUCOPE meeting (March 2024), Screen4Rare meeting at the EU parliament (November 2024) and participation at the NBS working group of Eurordis

Webinar about SMN (E Tizzano): “The evolving of SMA Genetics on the new therapeutic era” (10.04.2024)

Update of SMA NBSA White Paper

Launch webinar to present the updated White Paper (12.12.2024)

List of scientific literature on website available: https://goo.su/FznnGb

Next steps in 2025

Continue to give support to national entities on support

List and identify a contact in the different countries without SMA NBS to propose help

Participation at EURO NMD meetings and working groups (e.g. Eurordis working group)

Dissemination of survey results

Application for an ENMC meeting to propose best practices after a positive screening

Small update of the White Paper to Version 2.1 and referencing the White Paper

Translations of the White Paper incl. Integration into existing designs

Regular update of the website

An event and a scientific paper to present what the Alliance has achieved and to look towards the future of NBS in SMA

— Newborn screening and early treatment are crucial to improve health outcomes in SMA. —

Link to White Paper

Capacity building

Annual meetings with general assembly

What makes this event relevant to our SMA Community?

SMA Europe's Annual General Meeting (AGM) is more than an annual gathering, it is a cornerstone of both our governance and our community. At the heart of the event lies the General Assembly (GA), our organisation's highest governing body, which plays a formal and statutory role in shaping the strategic direction of SMA Europe. As a German-registered non-profit, this structure ensures transparency, accountability, and active member participation in key decisions, such as Board elections, budget and annual planning approvals.

This formal process is embedded within the broader context of the Annual Meetings, which provide a dynamic and collaborative environment for member organisations to come together, share experiences, and engage with SMA Europe's ongoing work. The meetings are an essential space to foster solidarity, mutual understanding, and trust, strengthening the ties that unite our network and ensuring that SMA Europe's actions remain grounded in the lived realities of its members.

To complement the years when our multistakeholder Scientific Congress does not take place, we extend invitations to selected external guests and collaborators to join our AGM every second year. This approach helps ensure that opportunities for connection, dialogue, and shared learning with external partners continue consistently across the SMA landscape.

By bringing both governance and collaboration into one space, the Annual Meetings allow our members to inform, shape, and energise the work we do all together.

Annual General Meeting 13-14 June 2024, Munich Germany

Main achievements in 2024

Brought together over 20 delegates and patient advocates from across Europe in Munich, Germany (13–14 June), including strong representation from our German member organisation.

Facilitated strategy development, including a dedicated Board strategy meeting.

Hosted thematic workshops on core areas such as OdySMA, NBS Alliance, Critical Thinking in SMA R&D and operational sustainability and development.

Held a successful hybrid General Assembly, which included the resignation of the former President to assume the role of CEO, the election of Dr Yasemin Erbas as the new President, the expansion of the Board to seven members, and the welcoming of new members and delegates to the SMA Europe network.

Annual General Meeting 24-26 April 2025, Athens, Greece

2025 Focus

Hosting the 2025 AGM and GA Meeting in Athens (24–26 April), bringing together member organisations for collaborative planning, exchange, and decision-making.

Facilitating key workshops, including a moderated session with industry partners on updating the SMA disease description, and a Board-led discussion on the future of topics such as the NBS Alliance, daily living with SMA, and European health policy.

Encouraging peer learning and exchange through a World Café session and interactive speed-dating discussions on key areas such as fundraising to support initiatives like the Call for Research and the future of this research programme.

Supporting accessible and practical learning through a Kahoot quiz on SMA, a role-play session on real-life communications scenarios, and hands-on demo of the OdySMA tool.

Reinforcing community connection through structured and informal networking opportunities, strengthening trust and unity across the SMA Europe network.

— An opportunity for our members to meet, interact and exchange beyond the ordinary virtual setting and make an extraordinary difference. —

Our SMA Europe

“Our SMA Europe” is an initiative aimed at building together a stronger, independent, sustainable, and impactful community of SMA organisations across Europe, together. The initiative focuses on understanding the diverse needs of our members, and of each other, and equipping us all with tools to share experiences, exchange best practices, and collaborate effectively across borders.

Recognising the wide range of contexts, expertise, and organisational sizes within our network, Our SMA Europe fosters effective communication. It ensures that every member organisation remains informed and that every voice is heard, especially when we, as SMA Europe, shape our priorities and actions as an umbrella organisation. By facilitating regular knowledge exchange, identifying emerging topics, and aligning around shared priorities, this initiative strengthens our collective ability to respond to challenges and seize opportunities as a unified European community.

— This initiative enables a deeper understanding of the diverse needs within the SMA Europe network and equips us with the tools to foster effective internal communication and collaboration, allowing each organisation to maximise its impact, avoid duplication of efforts, and build on shared strengths. —

What makes this project relevant to our SMA Community?

Based on the results of interviews that we conducted to assess the needs of our members, we implemented a well-structured information-sharing framework that helps ensure that national SMA organisations remained well-informed, empowered, and connected. By providing tailored advocacy tools, timely updates, and peer-to-peer exchange opportunities, the initiative strengthens the capacity of member organisations to respond to emerging challenges, advocate effectively, and collaborate across borders. The flexibility of communication channels, such as newsletters, sounding boards, and real-time messaging, allow for both alignment and day-to-day (peer-to-peer) support, helping to build a more unified and impactful SMA community across Europe and beyond.

Main achievements in 2024

Created and coordinated dedicated Sounding Boards, offering structured opportunities for patient advocates to inform and guide SMA Europe's initiatives, ensuring our actions remain community-driven and responsive to our priorities.

Launched SMA Europe's first fully curated Annual Report, providing members and stakeholders a comprehensive overview of our collective achievements, impact and future direction.

Established a WhatsApp Community, segmented into topic-specific groups to facilitate real-time dialogue, peer support, and knowledge exchange among patient advocates across Europe.

Focus 2025

Strengthening collaboration among member organisations, with a dedicated focus on facilitating peer learning, knowledge sharing, and exchange of best practices.

Further enhancing information exchange, including the launch of a monthly external newsletter to reach a broader audience, such as the scientific community, healthcare professionals, and industry partners, with insights from across the SMA Europe network.

Introducing weekly and monthly newsletters, designed to keep Delegates, volunteers and other stakeholders informed with timely community updates, upcoming opportunities, and developments in the field.

Link to
our song

SMAcademy

SMAcademy is SMA Europe's new initiative that aims to build capacity among patient advocates in SMA by offering targeted training and learning resources.

SMAcademy programmes are designed in close collaboration with the community to ensure that evolving needs are met.

What makes this project relevant to our SMA Community?

SMAcademy is SMA Europe's core initiative to build a stronger, more informed, and sustainable community of patient advocates. It responds to a simple truth: meaningful progress for people with SMA is only possible if those most affected are empowered to lead, shape, and advocate for change at every level.

Through structured training, targeted learning resources, and innovative formats, SMAcademy equips advocates with the tools they need to navigate complex topics, from research and clinical trials to treatment access, best practices in care, health economics and policy, digital health, real-world evidence, and rare disease frameworks. Co-created with and for the community, SMAcademy is rooted in real needs and lived experience. It also fosters international collaboration, helping to unify advocacy voices and build connections across the European and global SMA landscape.

SMAcademy is a cornerstone of SMA Europe's spiral of progress: where needs lead to knowledge, and knowledge fuels impact.

> **— SMAcademy provides opportunities for patient advocates in SMA to hone their advocacy skills and increase their effectiveness in achieving advocacy goals for people living with SMA all around the globe. —**

Main achievements in 2024

Laid the foundation for the first structured training programme for SMA patient advocates, including a curriculum outline and learning roadmap based on real needs identified by the community.

Conducted a scoping review and gap analysis of existing patient education programmes, and aligned the design of SMAcademy with principles of sustainability and relevance.

Engaged the community in co-design, through needs assessments, speed-dating sessions at the AGM, and an SMAcademy Pillars Workshop to shape content and priorities.

Partnered with EUPATI, a leader in patient education, to ensure alignment with best practices and access to wider learning opportunities.

Expanded ongoing learning access through topic-specific webinars on science, clinical care, policy, and regulatory frameworks relevant to SMA advocacy.

2025 Focus

Finalising the SMA Patient Advocate Training Programme, the first systematic capacity-building course for SMA advocates, designed to prepare confident, informed changemakers. The programme is set for launch in 2026.

Introducing innovative and engaging learning formats, including the pilot SMA Journal Club (in collaboration with the Treatment Committee) and the SMAcademy Kahoot Game, a gamified approach to SMA knowledge.

Delivering targeted webinars and workshops on advanced advocacy areas, ensuring advocates are well-prepared to engage with emerging challenges in research, treatment access, clinical trial design, and health policy.

Strengthening the advocacy network by connecting learners across countries, facilitating peer exchange, and reinforcing the shared goal of improving lives through empowered action.

Communication and outreach

2024 Awareness campaign: One Community. Shared Dreams.

Awareness campaigns are a vital part to achieve our mission. They help elevate the voices of people living with SMA, increase public and stakeholder understanding of the condition, and mobilise support for the most urgent unmet needs, especially in research, care, and access. These campaigns not only strengthen visibility and advocacy on a European level but also empower our member organisations with shared narratives and adaptable tools to drive awareness in their own national contexts.

What makes this project relevant to our SMA Community?

The 2024 Summer Awareness Campaign, #WeAreOne, aimed to stress the fact that we are all one community and we have many shared dreams. In a landscape where people living with SMA represent different generations, live different national realities, and require different level of care and support, this campaign delivered a clear and unifying message: no matter our individual journeys, we are united by one shared dream: to build a better world for everyone affected by SMA. By centring this message, the campaign aimed to re-affirm one collective identity across countries and experiences, and reminded the community that solidarity remains one of our strongest tools for driving change.

To ensure the campaign spoke not only to the heart but also to key priorities, SMA Europe brought together people living with SMA and respected opinion leaders from the research and clinical communities to co-create the content and direction. This collaboration not only amplified the urgency of continued, patient-relevant research in a condition where there is no cure, but also demonstrated the power of dialogue between lived experience and scientific expertise. The campaign's tools, such as a professionally produced documentary, photography, music, and reusable materials, were designed to be inclusive, adaptable, and impactful. Member organisations across Europe were able to tailor and share content that resonated locally while reinforcing a Europe-wide narrative of unity, resilience, and purpose. In doing so, #WeAreOne became more than a campaign, it became a symbol of who we are, and what we can achieve together.

Main achievements in 2024

Produced a professional photography series, capturing the diversity, resilience, and unity of our community, creating visuals content for future campaign materials and communications.

Created the official SMA Europe Song, with lyrics written by a person living with SMA and performed by our Turkish delegate, a powerful expression of identity and unity

Produced a professional documentary film spotlighting the continued urgency of SMA research. The film and its accompanying "video pills" serve as a powerful tool for advocacy and can support external funding applications, also of researchers, highlighting the gaps in the current SMA research landscape.

Launched and coordinated the awareness campaign across all communication channels during August 2024 and reaching the SMA community and stakeholders across the globe.

Created a viral spin-off viral campaign highlighting SMA Europe's Top 10 Research Priorities, directly linking the awareness campaign to our research agenda and reinforcing calls of continued investment in research.

2025 Focus

Launching the 2025 Awareness Campaign titled "Connecting the Dots", which will align the call for continued, sustainable, patient-relevant SMA research with the announcement of projects selected under SMA Europe's 12th Call for Research.

Delivering a dedicated SMAcademy webinar empowering a wider group of patient advocates by sharing knowledge on how to plan, produce, and coordinate multichannel awareness campaign.

— Key social media data:
805'000 Impressions
456'402 Views
491'961 Reach —

Link to
our video

Events

Global SMAdvocacy event

What makes this event relevant to our SMA Community?

The Global SMAdvocacy Event provides a vital platform for SMA patient advocates from across the world to connect, exchange strategies, and work together toward shared goals in advocacy, access, and care. In a global landscape where access to SMA treatment and care remains deeply unequal, this event fosters much-needed mutual learning and solidarity, helping advocates amplify their voices and impact at national and regional levels.

By creating space for in-person collaboration, the event strengthens networks across borders, encourages shared problem-solving, and promotes collective advocacy that transcends geographic boundaries. The opportunity to learn from each other, from real-world experiences of other organisations, facing similar or very different challenges, helps patient advocates build more effective, context-sensitive strategies and unlock creative solutions.

> — Learning from each other's experience, SMA patient advocates are stronger. The Global SMAdvocacy Event empowers SMA advocates across continents to turn shared challenges into collective solutions, building a stronger, more connected voice for people living with SMA worldwide. —

Main achievements in 2024

Brought together 70 patient advocates representing 63 organisations from 50 countries and 6 continents, making it one of the most globally diverse gatherings in SMA advocacy to date.

Designed and delivered a patient-driven workshop, developed through direct input from participants to address the most urgent challenges in SMA advocacy worldwide.

Facilitated a solution-oriented working session, enabling participants to co-create actionable approaches to shared advocacy issues.

Produced two reports, including a public-facing summary available on SMA Europe's website and a detailed version for participants, helping to spread learnings and preserve outcomes for future action.

2025 Focus

Preparing and resourcing the 2026 edition of the Global SMAdvocacy Event, including:

- Securing funding to enable broad participation and travel support
- Identifying and engaging global advocacy leaders and emerging voices
- Designing an agenda that reflects community-defined priorities and fosters collaboration

Leveraging insights from the 2024 workshop, ensuring participant-led recommendations inform future advocacy actions and international collaborations.

Strengthen the global advocacy network by maintaining engagement between editions and fostering continued collaboration and knowledge-sharing among international SMA advocates.

Link to
the event
website

International Scientific Congress on Spinal Muscular Atrophy

SMA Europe is committed to advancing patient-relevant research in spinal muscular atrophy through its projects, funding initiatives, and strategic collaborations. One of its key scientific initiatives is the Scientific International Congress on SMA, which is held every two years. The congress brings together an international and multidisciplinary community of scientists, healthcare professionals, and patient advocates to present and exchange breakthrough ideas in SMA research, with a strong emphasis on the patient relevance of their findings. The event also serves as a catalyst for fostering existing collaborations and sparking new ones across disciplines and borders. In its most recent edition, the congress welcomed over 1,000 participants from more than 70 countries, establishing it as a truly global forum for SMA research and care.

4th International Scientific Congress on Spinal Muscular Atrophy, 2024

What makes this campaing relevant to our SMA Community?

The 4th Scientific International Congress on SMA, held in March 2024, reinforced its position as a leading platform for scientific excellence and collaboration in the SMA field. With a programme driven by cutting-edge research and high academic standards, the event showcased the latest breakthroughs in SMA research. A strong emphasis was placed on advancing interdisciplinary collaboration by bringing together scientists, healthcare professionals, and patient advocates from around the world. The inclusion of lay summary slides in scientific presentations helped bridge the communication gap between researchers, clinicians, and patient representatives, while multistakeholder workshops and a dedicated session between early career researchers and advocates created space for mutual understanding and deeper alignment on patient-relevant research priorities.

Through the 2024 congress, SMA Europe fostered meaningful dialogue across all stakeholder groups in the SMA field. The event featured respected, internationally renowned speakers and offered a vital platform for emerging scientific talent. It served not only to share cutting-edge research but also to inspire scientific progress in a condition that, despite recent advances, remains without a cure. Together, the scientific and advocacy communities critically examined key developments in SMA research and care. By facilitating the exchange of scientific evidence and clinical experience, the congress created unique opportunities to improve treatment, therapy, and long-term care outcomes for people living with SMA.

In 2024, SMA Europe took additional steps to ensure that the patient voice was not only heard but actively integrated into the congress. Through initiatives such as the #WeAreUnique Vernissage, a dedicated SMA Europe booth, and the presence of global advocates, the event underscored the power of collaboration. For the first time, a Community Award for the most patient-relevant research, selected by a community panel, was presented alongside the traditional scientific awards, recognising research that resonates most with those affected by SMA. Additional highlights included the Global SMAdvocacy event and the SMA Europe meets Early Career Researchers session, both of which strengthened the international network of researchers and advocates working together to improve outcomes for those living with SMA. By promoting events that bring all stakeholders together, SMA Europe continues to demonstrate our the dual commitment to scientific excellence and deep community relevance, fostering a stronger understanding of community needs and enabling g their integration into future research efforts which aim to address critical gaps and drive the development of meaningful, lasting solutions.

Main achievements in 2024

Successfully hosted the 4th Congress from 14 and 16 March 2024, bringing together over 1000 participants from six continents, with all seats sold out.

Presented 28 podium talks, 12 flash poster presentations, and 179 scientific posters showcasing the latest research and developments in the SMA field.

Conducted four expert-led parallel workshops on the research agenda of SMA, stem cells, newborn screening and insights from other diseases, coordinated by leading scientists in the field.

Awarded early career researchers for best presentation and best posters, and, for the first time, presented a Community Award for the most patient-relevant poster, selected by a panel of community representatives.

Organised three high-impact pre-congress events on 13 March 2024, each designed to elevate engagement, dialogue and awareness.

- Global SMAdvocacy event
- SMA Europe meets ECRs event
- #WeAreUnique Vernissage 13 March 2024 and exposition.

> — The goal of the SMA Europe's scientific congress is to bring together an international and multidisciplinary group of scientists and healthcare professionals. Our organisation provides a venue for them to present and exchange their breakthrough ideas relating to SMA, especially considering the patient-relevance of their findings, and to cement existing and stimulate new collaborations. —

5th International Scientific Congress on Spinal Muscular Atrophy, 2026

Focus 2025

Organising the 5th Scientific International Congress on SMA in the Convention Centre in Budapest, Hungary, which will bring together the global research and clinical community to address emerging needs and research gaps in the field.

Extending the event from 3 to 4 days, allowing for deeper engagement, additional scientific content and enhanced opportunities for networking and collaboration

- **Hosting in-depth workshops on Day 1**, covering emerging and cross-cutting topics that complement the core scientific programme—broadening the scope without compromising scientific excellence.
- **Opening the Congress with a Networking event on the evening of Day 1** to foster early connections.

Identifying the most urgent research priorities in SMA through a process guided by our Scientific Advisory Board and informed by the outcomes of SMA Europe's priority-setting project, ensuring the congress addresses the evolving needs of the community.

Attracting leading scientists and offering a dedicated platform for early career researchers, reinforcing our commitment to nurturing the next generation of SMA experts and promoting transdisciplinary collaboration.

Strengthening early career researcher involvement, with tailored sessions, visibility opportunities, awards and interaction with senior researchers and advocates.

Enhancing the integration of the patient voice, by inviting participants of the **Global SMAdvocacy event** to join the congress, facilitating structured exchanges between advocates and researchers, and embedding community perspectives throughout the scientific programme.

Celebrating the 20th anniversary of SMA Europe during the congress, marking two decades of collaborative progress in science, advocacy and community building, while looking ahead to shape a future of continued impact and innovation.

Introducing the SMA Europe Lifetime Achievement Award, recognising outstanding, long-term contributions to SMA research, care, or advocacy.

#WeAreUnique Awareness Campaign Vernissage during the 4th International Scientific Congress on Spinal Muscular Atrophy in Ghent

What makes this campaing relevant to our SMA Community?

The #WeAreUnique Vernissage offered a rare and meaningful opportunity for researchers, many of whom do not regularly engage directly with people living with SMA, to gain insight into the lived experience of the community they serve. By creating an emotional connection and humanising the scientific work, the exhibition helped raise awareness, foster empathy, and strengthen the bond between the research community and individuals with SMA. This kind of connection is essential to sustaining motivation, aligning research priorities with real needs, and ultimately accelerating impactful research in our field.

Main achievements in 2024

Presented the #WeAreUnique Vernissage to the international research and advocacy community during the 4th International Scientific Congress on SMA. The exhibition was based on the 2023 Summer Awareness Campaign, the first one professionally designed and promoted awareness initiative by SMA Europe, highlighting 11 beautiful stories of different people who live with SMA across Europe.

Officially opened the Vernissage on the pre-congress day, 13 March 2024, offering a powerful visual and narrative experience to researchers, clinicians, and advocates creating a shared space for connection and reflection.

> — The space of the Vernissage, placed strategically close to the scientific posters' area and SMA Europe's booth, became the "meeting point" for many of the attendees of the Congress. Several posts on social media were published with the Vernissage in the background, and we received very positive feedback from the scientific community while being grateful for the opportunity to learn more about people living with SMA in person. —

We are sustainable. We are SMA Europe.

Operational sustainability: strengthening the foundation for community impact

What makes this project relevant to our SMA Community?

Behind every impactful activity, whether in research, care, policy, or access, stands an organisation capable of delivering it with focus, transparency, and accountability. For SMA Europe, strengthening our internal capacity is not an end in itself, it is how we ensure that the needs, rights, and hopes of people living with SMA continue to be met through meaningful, sustained action. Whether it is building a virtual team across borders, enhancing financial and digital systems, fostering good governance, or growing our network of members and partners, all of these efforts serve one purpose: to make our work more responsive, reliable, and resilient.

By investing in people, improving our operations, communicating more clearly, maintaining high governance standards, and planning strategically for the future, we are laying the groundwork for stronger advocacy, smarter collaboration, and better outcomes. In a rapidly evolving landscape of treatments, care options, and policy debates, our ability to deliver consistently and credibly is what allows us to stay focused on what matters most: the lived experience of our community and the ambition to improve every aspect of life with SMA.

Main achievements in 2024

1. People — Investing in skills and motivation

At SMA Europe, people are at the centre of everything we do. Our impact depends not only on a capable staff team, but also on the engagement of our volunteers, Board members, and community contributors across Europe. To deliver long-term results, we work to ensure that everyone involved, whether employed, elected, or volunteering, has the support, clarity, and conditions they need to contribute meaningfully.

Operating as a fully virtual organisation adds both flexibility and responsibility. We invest in maintaining strong internal communication, nurturing cohesion across borders, and building a respectful, inclusive environment. For staff, this means consistent performance support, onboarding, and fair employment practices. For volunteers, this means recognising their time and expertise, offering clear introductions to their topics, and providing the materials and guidance they need, while expecting active engagement and co-responsibility.

SMA Europe is committed to fair, appreciative volunteer involvement: we respect their time, offer structured opportunities for input, and maintain transparent, reliable agreements. For SMA Europe Board members, their involvement is ensured through regular monthly Board meetings, together with the CEO, and active engagement across strategic areas. Their contributions are supported with practical measures such as travel reimbursement, documentation, and accessible communication channels to enable their governance role to be fulfilled effectively.

Key achievements 2024

- Continued to offer structured support for staff through regular 1:1s, weekly team meetings, and an annual staff retreat to strengthen cohesion in our virtual setting.
- Introduced annual reviews and supported staff through a fair, transparent HR framework.
- Established a recruitment and onboarding process, leading to the competitive and successful recruitment of new staff members.
- Ensured fair, compliant freelance agreements for all external collaborators.
- Reinforced our position as a reliable employer with functioning payroll and performance evaluation systems.
- Strengthened a respectful and appreciative volunteer environment, offering clear on-boarding, task guidance, and fair expectations for involvement.
- Ensured regular engagement of SMA Europe's Board, including monthly meetings with the CEO, strategic involvement in decision-making processes, and logistical support for their roles, such as travel cost reimbursement.

2. Operational and financial management — Balancing growth with mission

For a mission-driven organisation like SMA Europe, financial discipline and strategic investment must go hand in hand. While prioritising programmatic spending remains core to our work, we also recognise the importance of investing in internal capacity, such as digital infrastructure, staff and volunteer development, and fundraising systems, to ensure long-term sustainability. As a virtually operating organisation, we continuously review how our systems and digital tools support smooth and secure collaboration, project management, and cost efficiency. Building resilience and efficiency across operations is essential to support our growing community and programme portfolio.

Key achievements 2024

- Improved operational workflows by formalising processes, using low-barrier digital tools, and reducing manual tasks without overcommitting to costly automation.
- Developed standardised templates for internal workflows and project management.
- Conducted relevant staff trainings
- Improved financial tracking and planning to maintain transparency and better support strategic decision-making.
- Managed administrative operations in a lean, sustainable model suited to our virtual structure

3. Transparency and communication — Earning trust of donors through clarity

Transparent, regular, and accessible communication is fundamental to strengthening trust across our network. As expectations shift, especially among donors and partners, we must continue to invest in tools, training, and processes that enable updates and simplified engagement. This includes thoughtful donor communication, streamlined digital platforms, and consistent (financial) reporting. Virtual collaboration requires extra clarity, frequency, and consistency in both internal and external communications, from aligning teams across borders to informing stakeholders and supporters worldwide. Clear communication also supports stronger internal coordination and keeps members, stakeholders, and supporters informed and empowered.

Key achievements 2024

- Advanced work on communication structures, laying the foundation for clearer internal communication and improved external visibility.
- Advanced our internal communication by offering consistent channels for coordination across the virtual team of volunteers and staff.
- Used diverse platforms to ensure stakeholder visibility into our work and impact.

4. Governance and compliance — Managing risk, enabling confidence

Good governance is the backbone of credibility and organisational health. For SMA Europe, this means more than compliance with regulations, it is about anticipating risks, embedding strong data and cybersecurity practices, and ensuring Board oversight remains active and informed. In a European context, data protection and privacy (e.g. GDPR compliance) must be embedded into our daily operations. Virtual operations bring an added focus on secure digital systems, data access protocols, and clear documentation of responsibilities and procedures. At the same time, we continue to address evolving tax and regulatory responsibilities as a German-registered NPO. Being proactive in these areas protects not only our organisation's continuity but also the trust placed in us by our community.

Key achievements 2024

- Maintained compliance with legal and fiscal requirements relevant to our work as a German-registered non-profit.
- Established key internal documents including the Governance Manual, Board Charter, Staff Handbook, Volunteer Charter, Treatment Committee Charter, Travel Policy, Email Policy
- Successful succession planning of the Board by expanding the Board to broaden expertise and ensure continuity, including a handover from the former President to the new one and with the former president moving to the CEO role.
- Created and continued sounding boards and committees composed of community members and external experts (e.g. Medical Panel, NBS Steering Committee) to inform our work with diverse perspectives.

5. Organisational development and strategic planning — Building capacity for sustainable growth

Our impact relies on a clear strategic vision, an engaged network, and the internal capacity to act on both. In 2024, SMA Europe made important strides in strengthening its long-term positioning by growing its membership base, formalising planning structures, and deepening stakeholder engagement. These efforts ensure we remain agile, community-driven, and prepared for the evolving landscape of rare disease advocacy.

Key achievements 2024

- Recruited three new member organisations (Slovakia, Austria, and Israel), and onboarded many new volunteers from across Europe.
- Continued to work closely with more than 70 volunteers from 30 countries, fostering a vibrant and skilled community of contributors.
- Established and piloted a tailored project management framework to ensure consistency, clarify roles, and support quality delivery across initiatives.
- Facilitated succession planning at the leadership level, supporting a smooth transition in Board leadership.
- **Strengthened external engagement** by developing new partnerships, fostering dialogue with relevant stakeholders across sectors, and establishing a clear operational process for interacting with industry partners
- Used stakeholder outreach to build a more stable network for information exchange and cross-sectoral learning.

2025 Focus: operational sustainability

In 2025, SMA Europe will continue to strengthen its organisational foundation by embedding sustainability principles across all areas of operation—financial, structural, environmental, and human. We aim to enhance internal collaboration through the rollout of a dedicated internal communications framework and reinforce external trust through a clearer, more visible communications strategy. Strengthening and diversifying our funding base will remain a priority, ensuring that key initiatives and capacity-building efforts remain viable long-term. We will also focus on building strategic partnerships and alliances across sectors, expanding our ability to share knowledge, scale impact, and serve the SMA community through a collaborative, resilient, and future-ready organisation.

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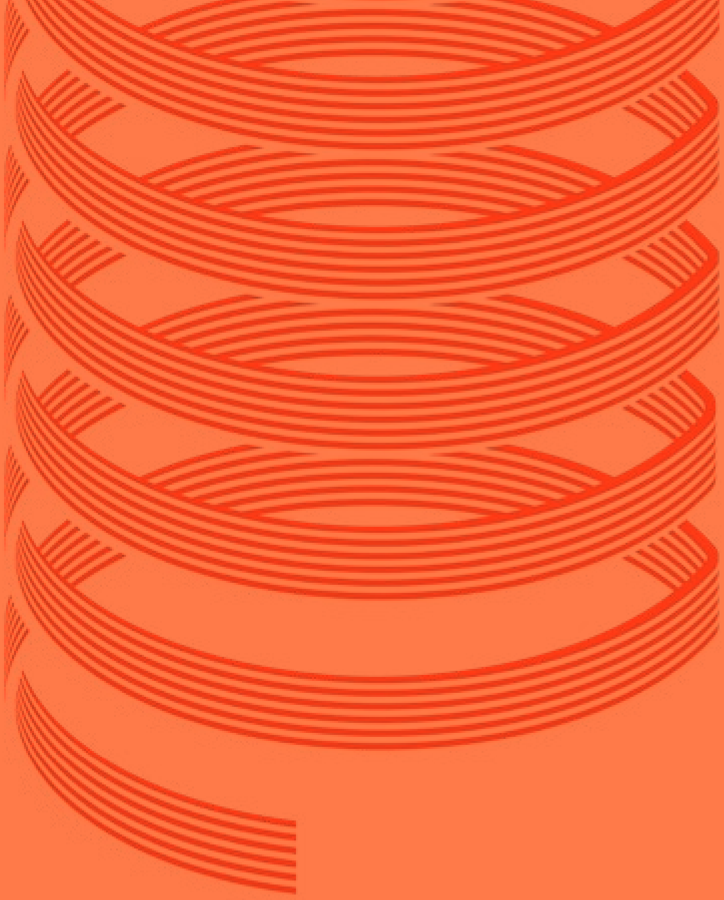


Special feature

SMA Slovakia

> “My name is Kristína Čurgali. I’m 40 years old, happily married to a wonderful and supportive husband, and the proud mother of two amazing.”

Kristína Čurgali, President and co-founder of SMA Slovakia

About Kristína and her journey with SMA.

My name is Kristína Čurgali. I'm 40 years old, happily married to a wonderful and supportive husband, and the proud mother of two amazing, yet sometimes delightfully cheeky boys.

Motherhood has reshaped my perspective on many things, including spinal muscular atrophy (SMA). Like so many parents in similar situations, I've experienced the full spectrum of grief – denial, anger, bargaining, depression, and, finally, acceptance.

Our journey with SMA began when my eldest son, Jakub, was diagnosed with SMA type 2 at 19 months old – just as my younger son, Lukáš, was born. Lukáš is a carrier. That moment marked the beginning of our SMAordinary family journey. It was also the moment I realised that if I could help even one parent navigate the challenges of SMA, I could make the world a better place for my son – a better place for all SMA patients and their families.

With my academic background and the scarcity of reliable information on SMA in Slovakia, I immediately threw myself into research, reading every paper I could find – often between breastfeeding sessions, diaper changes, and the joyful chaos of raising two toddlers.

Today, Jakub attends a mainstream primary school, and I've returned to my career as an assistant professor at one of the country's largest universities. At the same time, I'm also on a journey of self-discovery – learning who I am beyond being a SMA-Mother.

As a family, our greatest goal is to ensure that SMA doesn't overshadow our children's happiness, independence, and learning opportunities. SMA may be a part of our lives, but it doesn't define us. Instead, it has strengthened our resilience, deepened our empathy, and connected us to an incredible community.

As for my personal life, my husband and I share a deep love for Italian and Greek cuisine, long conversations with friends at our countryside cottage, and a well-earned glass of wine at the end of the day. Once, we dreamed of giving our children the world. Now, we dream of giving them the world they deserve.

About the SMA patient organisation in Slovakia

SMA Slovakia – Aliancia rodín is a young and growing organisation, proudly celebrating its first anniversary. It was founded in response to an increasing need for a united voice to support, connect, and advocate for families living with SMA in Slovakia.

In just one year, we have made remarkable strides in building a strong and supportive community. Our network now includes families, pharmaceutical companies, and healthcare professionals such as neurologists, orthopaedic surgeons, physiotherapists, and physiatrists.

One of our proudest achievements was organising TOGETHER IN SMA 2024, a successful three-day social event that brought the Slovak community together.

We have also hosted an educational event for Slovak and Czech SMA families, featuring renowned physiotherapist Chiara Mastella and orthopaedic technician Romano Salsi. Additionally, we are actively working to establish partnerships with patient organizations in Iceland and Norway, fostering international collaboration.

Slovakia is home to approximately 70 SMA families, with 35 of them already part of SMA Slovakia. As we continue to grow, our mission remains clear: to empower and support the SMA community through advocacy, education, and meaningful connections.

About the SMA Community in Slovakia

Slovakia has a public healthcare system where most medical costs are covered by public health insurance. A major breakthrough came on January 1st, 2024, with the introduction of fully funded newborn screening for SMA.

Babies identified through this screening who have 2–3 copies of the SMN2 gene are eligible for gene therapy. Additionally, all three types of SMA treatment are fully reimbursed by insurance companies, provided patients meet the required medical criteria.

Currently, three SMA patients in Slovakia are not receiving any treatment – one by personal choice, while the other two do not meet the eligibility criteria of requiring less than 16 hours of ventilation per day.

In Slovakia, SMA patients have access to a number of fully-funded medical devices, and partial financial assistance is available for specialized SMA wheelchairs, lifts, home modifications, and wheelchair-adapted vehicles.

About your membership in SMA Europe

From the very beginning, I closely followed almost everything related to SMA on social media – that's how I first discovered SMA Europe. When the idea of establishing a patient organisation in Slovakia started to take shape, I had the opportunity to meet Nicole and Theo during an online call organised by a third party. One thing led to another, and here we are today—a small organisation from a small country, now part of something much bigger.

For me, the greatest value of being a member of SMA Europe is the sense of unity, collaboration, and shared purpose. We all fight for the same goal – not just to treat and cure SMA, but to make the world a better place.

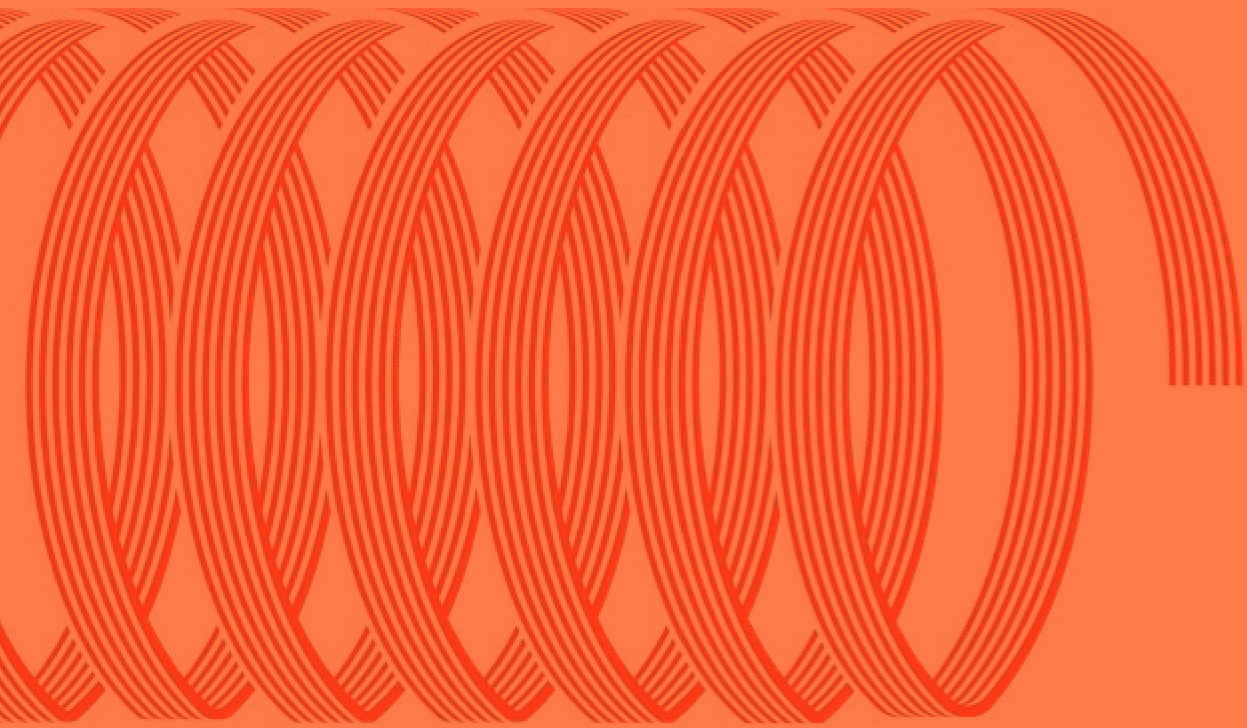
> “Living with SMA does not diminish one’s abilities, value, or potential. By championing inclusivity, securing equal opportunities, and ensuring lifelong care, I believe we can build a future where every individual with SMA can thrive, not just survive.”

The future of SMA and SMA Community

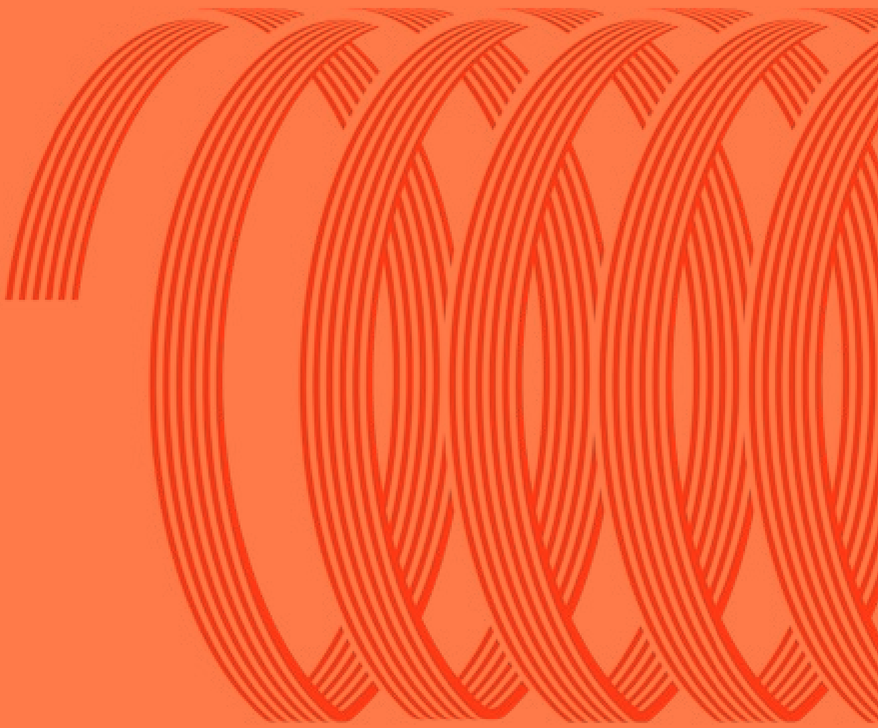
We are now entering a new era for the SMA community. With effective treatments now widely available across Europe, newborn screening programmes being implemented, and promising new therapies on the horizon, we stand on the brink of a future where no child has to experience the full impact of this condition.

However, significant challenges remain – many countries still struggle with the affordability of treatment, and only a handful provide comprehensive support for independent living, assistive technologies, and employment opportunities for people with disabilities.

Living with SMA does not diminish one’s abilities, value, or potential. By championing inclusivity, securing equal opportunities, and ensuring lifelong care, I believe we can build a future where every individual with SMA can thrive, not just survive.



Our team



Our team

Board

Dr. Nicole Gusset
President, resigned in June 2024.

Dr. Yasemin Erbas
President, elected in June 2024.
SMA Belgium

Olga Germanenko,
Vice-President, elected in June 2024. SMA Family Foundation Russia

Eva Stumpe
Treasurer, Deutsche Gesellschaft für Muskelkranke, Germany

Marie-Christine Ouillade
Re-elected in June 2024.
AFM Téléthon, France

Stefan Bos.
Elected in June 2024.
Vereniging Spierziekten Nederland, The Netherlands

Simona Joveska.
Elected in June 2024.
Stop SMA North Macedonia

Jakub Tomczyk.
Elected in June 2024.
Fundacja SMA, Poland

Staff

Dr. Nicole Gusset
CEO

Theo Soultanos
Operations Manager

Emilia Debska
Communications and Marketing Manager

Vanessa Christie-Brown
Research Manager
(Left in July 2024)

Federica Fontana
Research and Medical Manager (Joined in October 2024)

Laura Gumbert
Healthcare System and Access Manager

Alice Larotonda
Community Research and Education Manager

Carolina Pachecoy
Operations Support Manager

Our community is Us

Committees, working groups and sounding boards of SMA Europe

"It's been an honour to become co-chair to Véronique van Assche in the Adult Committee. Véronique has taught me a great deal about how SMA Europe is working and the collaboration between different groups. I enjoy the knowledge sharing and find it very important to succeed - both on a national level and globally. In the future, I hope that together with SMA Europe, its member organisations and through a diversity of advocacy actions and initiatives, we can make a difference for newborn screening on an international level and end the use of stop criteria for the adult population so no person living with SMA is left without access to SMA medicines."

Liesbeth Doktor, Co-chair of the Adult committee. SMA Europe

SMA Europe Scientific Advisory Board (SAB)

Our Scientific Advisory Board is composed of an international group of scientists and neurologists with particular expertise in SMA research. The SAB advises us on our scientific congresses and on the selection and appraisal of the research grants we award.
The SAB is chaired by Prof. Tom Gillingwater.
Link to the website

SMA Europe adult committee

This committee is composed of a group of adults living with SMA, from member and non-member countries, who provide advice to various SMA Europe activities, on relevant topics for adults living with SMA.
The Adult Committee is chaired by Lisbeth Doktor and Laetitia Ouillade.
Link to the website

EMA committee

Group of experienced patient experts who engage with EMA activities on behalf of SMA Europe as an EMA-eligible organisation.
The EMA Committee is chaired by Mencia de Lemus.

SMA Europe treatment committee

Group of experienced patient experts, from member countries, who engage in independent discussions on treatment related topics to support the development of news, positions and publications.
The committee is chaired by Thomas Koed Doktor.
Link to the website

Working groups and sounding boards of SMA Europe

To ensure that our activities and work processes are relevant to patients, SMA Europe also counts with the support of the Communications Working Group, SMA Europe Youth Group and different sounding boards, composed by our Project Leads and volunteers from the SMA international community. This allows us to gather feedback in an uncomplicated, agile yet structured way related to specific projects or topics. Thank you to all volunteers who give their time and commitment to all the initiatives they support. Our work would not be possible without your ongoing engagement and support.

Our publications and outreach

SMA Europe editorial publications:

Authorship SMA Europe, F. Hoffmann-La Roche Ltd., Bartels, B., Bos, S., Erbas, Y., Gietka, J., Gray, C., Kostera-Pruszczyk, A., Laforet, P., Lofra, R. M., Sansone, V., Vázquez, J., Vissing, J., Walter, M., Hungarian Association of Muscular Dystrophy Patients, Hall and Partners, Weber Shandwick Brussels, Brandao, S., Negri, D., Schober, K., Gumbert, L., Gusset, N., and Van Assche, V. (2024, February).

Title **“Care for adults living with SMA in Europe: a benchmarking report.”**

RDD 2024, OdySMA.

Authorship SMA Europe (2024, March).

Title **Abstract Book**, Ghent on the 4th International Scientific Congress on Spinal Muscular Atrophy. ISSN: 3079-7551

Event 4th Scientific International Congress on Spinal Muscular Atrophy. 12-14 March. Ghent, Belgium.

Authorship Authorship: SMA NBS Alliance (2024, December).

Title **SMA Newborn Screening White Paper Update: How far we’ve come.**

Journal articles:

Autors Gumbert, L., Larotonda, A., Erbas, Y., and Gusset, N. (2024).

Title **Meeting abstracts from the 12th European Conference on Rare Diseases and Orphan Products.** Orphanet Journal of Rare Diseases, 19(S1), 447, s13023-024-03293–03299.

Source https://doi.org/10.1186/s13023-024-03293-9

Autors Gusset, N. (2024).

Title **Embracing innovation: A patient advocacy perspective on evolving trial designs.** Regulatory Affairs Watch, 6(9), 31–33.

Source https://doi.org/10.54920/SCTO.2024.RAWATCH.9.31

Autors Malherbe, H. L., Jain, R., Antoniadou, V., Ouillade, M.-C., Gil Cardozo, D. F., Gumus, G., Kitikiti, N. S., McKay, L., and Wang, C. M. (2024).

Title **Patient organizations: Advocating for timely newborn screening and improved quality of life.** Rare Disease and Orphan Drugs Journal, 3(3).

Source https://doi.org/10.20517/rdodj.2024.11

Autors Martí, Y., Ribero, V. A., Batsonb, S., Mitchellb, S., Gornia, K., Gusset, N., Oskouie, M., Servaisf, L., Deconinckh, N., McGrattanj, K. E., Mercuril, E and Sutherland, C. S. (2024).

Title **A Systematic Literature Review of the Natural History of Respiratory, Swallowing, Feeding, and Speech Functions in Spinal Muscular Atrophy (SMA).** Journal of Neuromuscular Diseases, 11(5), 889–904.

Source https://doi.org/10.3233/JND-230248

Autors Tătaru, E.-A., Ouillade, M.-C., Chan, C.-H., and Pearce, D. A. (2024).

Title **Incorporating a new disease in the newborn screening programs in Europe: The spinal muscular atrophy case study.** Rare Disease and Orphan Drugs Journal, 3(3), 26. Journal of Neuromuscular Diseases, 11(5), 889–904.

Source https://doi.org/10.20517/rdodj.2024.08

Poster presentations:

Author	Sully, K., Chiriboga, C., Duong, T., Erbas, Y., Glascock, J., Gusset, N., Muni-Lofra, R., O'Connell, C., Vázquez-Costa, J. F., Walter, M. C., Guittari, C. J., Cochrane, J., Townson, L. D., Kufakwaro, J., Marciniak, A., Riley, D., and Nam, J. (2024).
Title	**Delphi consensus panel on assessing clinically meaningful treatment outcomes in adults living with spinal muscular atrophy (SMA).** (2024)
Event	4th Scientific International Congress on Spinal Muscular Atrophy, 14-16 March, Ghent, Belgium.

Author	Christie-Brown, V., Staley, K., Pachecoy, C., Kinsella, S., Muni-Lofra, R., Mayhew, A., Seliverstov, Y., Pattni, J., Howarth, K., Nørdstrom, M., Van Assche, V., Popova, J., Velkovski, J., Meyers, M., Germanenko, O., Schwersenz, I., Rucinski, K., van Rijswijck, H., Erbas, Y., and Gusset, N. (2024).
Title	**Identifying the top 10 unanswered research priorities for spinal muscular atrophy.**
Event	4th Scientific International Congress on Spinal Muscular Atrophy, 14-16 March, Ghent, Belgium.
Contributors	Fontana, F., Larotonda, A.

Author	Gusset, N., Gumbert, L., Larotonda, A. and Erbas, Y. (2024).
Title	**OdySMA – a quest to access: an SMA Europe advocacy tool.**
Event	4th Scientific International Congress on Spinal Muscular Atrophy, 14-16 March, Ghent, Belgium.

Author	Assche, Van V., Gumbert, L., Brandao, S., Negri, D., Schober, K. and Gusset, N. (2024).
Title	**Benchmarking care for adults living with spinal muscular atrophy (SMA) in Europe: A call to action from SMA Europe.**
Event	4th Scientific International Congress on Spinal Muscular Atrophy, 14-16 March, Ghent, Belgium.

Poster presentations:

Author Erbas, Y., Larotonda, A., Vedernikova, E., Jongerling, J., and Gusset, N. (2024).

Title **The SMA Daily Life Study – An Experience Sampling Study Examining Patient-Centric Outcomes in the Daily Lives of Individuals Living with SMA.**

Event SMA Europe's 4th Scientific International Congress, 14-16 March 2024, Ghent, Belgium.

……

Author Erbas, Y., Larotonda, A., Vedernikova, E., Jongerling, J., and Gusset, N. (2024).

Title **Tracking the presentation of fatigue in daily life for people living with SMA through the Experience Sampling Method.**

Event SMA Europe's 4th Scientific International Congress, 14-16 March 2024, Ghent, Belgium.

……

Author Gusset, N., Gumbert, L., Larotonda, A. and Erbas, Y. (2024).

Title **OdySMA – a quest to access: an SMA Europe advocacy tool.**

Event European Conference on Rare Diseases and Orphan Products ECRD 2024, 15-16 May 2024, online and in Brussels, Belgium.

……

Author Debska, E., Gusset, N. (2024).

Title **#WeAreUnique: a participatory approach towards the creation of a multi-stakeholder awareness campaign on living with Spinal Muscular Atrophy in Europe.**

Event European Conference on Rare Diseases and Orphan Products ECRD 2024, 15-16 May 2024, online and in Brussels, Belgium.

……

Poster presentations:

Author Erbas, Y., Larotonda, A., Vedernikova, E., Jongerling, J., and Gusset, N. (2024).

Title **The SMA Daily Life Study – An Experience Sampling Study Examining Patient-Centric Outcomes in the Daily Lives of Individuals Living with SMA.**

Event European Conference on Rare Diseases and Orphan Products ECRD 2024, 15-16 May 2024, online and in Brussels, Belgium.

……

Author Erbas, Y., Larotonda, A., Vedernikova, E., Jongerling, J., and Gusset, N. (2024).

Title **Title: The SMA Daily Life Study – An Experience Sampling Study Examining Patient-Centric Outcomes in the Daily Lives of Individuals Living with SMA.**

Poster present. CureSMA Annual SMA Research and Clinical Care Meeting, 26-29 June, Anaheim, California, USA.

……

Author Erbas, Y., Larotonda, A., Vedernikova, E., Jongerling, J., and Gusset, N. (2024).

Title **Title: The SMA Daily Life Study – An Experience Sampling Study Examining Patient-Centric Outcomes in the Daily Lives of Individuals Living with SMA.**

Event Poster Presentation: CureSMA Annual SMA Research and Clinical Care Meeting, 26-29 June, Anaheim, California, USA.

……

Author Christie-Brown, V., Staley, K., Pachecoy, C., Kinsella, S., Muni-Lofra, R., Mayhew, A., Seliverstov, Y., Pattni, J., Howarth, K., Nørdstrom, M., Van Assche, V., Popova, J., Velkovski, J., Meyers, M., Germanenko, O., Schwersenz, I., Rucinski, K., van Rijswijck, H., Erbas, Y., and Gusset, N. (2024).

Title **Identifying the top 10 unanswered research priorities for spinal muscular atrophy.**

Event Motor Neuron Diseases: Understanding the Pathogenetic Mechanisms to Develop Therapies III Hybrid Workshop, 8-9 November 2024, Turin, Italy.

Contritutors Fontana, F., Larotonda, A.

Presentations, workshops and others:

Contributor	Ouillade, M.-C. (2024, February).
Title	**Presentation in the Newborn screening session. Topic: "Patients perspective."**
Event	7th ERN EURO-NMD Annual Meeting, Paris, France.

Contributor	Erbas, Y. (2024, June).
Title	**Presentation of data from Novartis' RESTORE registry on "Treatment response in children living with SMA who have a tracheostomy."**
Event	Cure SMA Annual SMA Research and Clinical Care Meeting. Austin, Texas, USA.

Contributors	Contributors: Gusset, N., Gumbert, L., and Brandão, S. (2024, June).
Title	**Advocating for better care and support systems for adults living with SMA: a European benchmarking report.**
Event	Caring in Society SMA Conference 2024. Lisbon, Portugal.

Contributor	Gusset, N. (2024, June).
Title	**Generating Health-Related Quality of Life in Rare Conditions.**
Event	HTAi 2024 Annual Meetings. Seville, Spain.

Contributor	Germanenko, O. (2024, August).
Title	**Empowering the Global SMA Ecosystem.**
Event	SMArtCon2024 Clinical Care Symposium, the Second National Conference on Spinal Muscular Atrophy. India.

Contributor	Soultanos, T. (2024, August).
Title	**SMA Europe Overview and Activities.**
Event	3rd National SMA Camp. Eskişehir, Türkiye.

Presentations, workshops and others:

Contributors Larotonda, A., and Gumbert, L. (2024, October).

Title **“Humanizing” the OdySMA, a quest to access: Ethnographic storytelling in non-profit advocacy.**

Event Lecture at Brown University. Rhode Island, United States.

Contributors Larotonda, A. (2024, September).

Title **Collaborative dialogue - Working together for enhancing patient involvement through education.**

Event EUPATI Annual General Meeting 2024, Roundtable discussion. Brussels, Belgium.

Contributors Gusset, N. (2024, November).

Title **How to cure the incurable? The promise of advanced therapies and overcoming patient access challenges.**

Event Evening reception co-hosted by MEP Stine Bosse and MEP Romana Jerkovic. European Parliament, Brussels, Belgium.

Authorship De Waele, L., Levene, M., Brites, J., and Ouillade, M.-C. (2024, December).

Title **SMA Newborn Screening White Paper Update: How far we’ve come.**

Event SMAcademy Webinar.

Media coverage:

Title **SMA Europe launches “Care for adults living with SMA in Europe: a benchmarking report.”**

Source Euractiv, 29 February 2024.
https://pr.euractiv.com/pr/sma-europe-launches-care-adults-living-sma-europe-benchmarking-report-260334

Title **SMA Europe launches “Care for adults living with SMA in Europe: a benchmarking report.”**

Source Rare Revolution Magazine, 29 February 2024.
https://rarerevolutionmagazine.com/sma-europe-launches-care-for-adults-living-with-sma-in-europe-a-benchmarking-report/

Title **SMAdvocacy. The journey towards advocacy and hope.**

Source Rarity Life Magazine, 6 March 2024.
https://issuu.com/raritylife/docs/rarity_life_issue_9/72

Title **Call for Research nr. 12.**

Source Treat NMD Newsletter, 29 March 2024.
https://www.treat-nmd.org/sma-europe-launches-call-for-research/

Title **SMA Europe launches its 12th call for research.**

Source Rare Revolution Magazine, 3 April 2024.
https://rarerevolutionmagazine.com/sma-europe-launches-its-12th-call-for-research/

Title **Whatever happened to the world’s most expensive drug?**

Source swissinfo.ch, 23 April 2024.
https://www.swissinfo.ch/eng/multinational-companies/whatever-happened-to-the-worlds-most-expensive-drug/75388576

Media coverage:

Title **Digital Spotlight on SMA.**

Source Rare Revolution Magazine, 4 May 2024.
https://rarerevolutionmagazine.com/digital_spotlights/sma/

Title **SMA Europe launches “real-life stories” to show how access to treatment and care impacts people living with spinal muscular atrophy.**

Source Rare Revolution Magazine, 22 May 2024.
https://rarerevolutionmagazine.com/sma-europe-launches-real-life-stories-to-show-how-access-to-treatment-and-care-impacts-people-living-with-spinal-muscular-atrophy/

Title **The Power of Collaboration.**

Source Rarity Life Magazine, 18 June 2024.
https://www.samebutdifferentcic.org.uk/raritylife

Title **SMA Europe unites international voices in the new documentary: One Community. Shared Dreams.**

Source Rare Revolution Magazine, 31 July 2024.
https://rarerevolutionmagazine.com/sma-europe-unites-international-voices-in-the-new-documentary-one-community-shared-dreams/

Title **New SMA Europe documentary aims to raise awareness on research.**

Source SMA News Today, 3 September 2024.
https://smanewstoday.com/news/new-sma-europe-documentary-aiming-raise-awareness-research/

Title **The role of patient organisations in implementing newborn screening for rare diseases.**

Source Share4Rare blog, 5 November 2024.
https://www.share4rare.org/news/role-patient-organisations-newborn-screening-rare-diseases

Media coverage:

Title **Access to spinal muscular atrophy (SMA) treatment in Denmark: a success story of how persistence can win in the end.**

Source Rare Revolution Magazine, 15 November 2024.
https://rarerevolutionmagazine.com/access-to-spinal-muscular-atrophy-sma-treatment-in-denmark-a-success-story-of-how-persistence-can-win-in-the-end/

Title **SMA Europe inviting patients to talk of how treatment has changed life.**

Source SMA News Today, 17 December 2024.
https://smanewstoday.com/news/odysma-patients-talk-how-sma-treatment-changed-life/

Title **SMA Europe announces the awardees and projects shortlisted through its call for research no.12.**

Source Rare Revolution Magazine, 28 February 2025.
https://rarerevolutionmagazine.com/sma-europe-announces-the-awardees-and-projects-shortlisted-through-its-call-for-research-no-12/

Title **SMA Europe provides funding for five new research projects in SMA.**

Source SMA News Today, 3 March2025.
https://smanewstoday.com/news/sma-europe-provides-funding-five-sma-research-projects/

Our supporters and partners

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)

Partner organisations

Cure SMA (US)

Cure SMA India

EAMDA

EMA
(European Medicines Agency)

ENMC

EURO-NMD

Eurordis

SMA Foundation (US)

Treat-NMD

Our supporters and partners

Industry partners

Biogen

Biohaven

Bio-Techne (Asuragen)

F. Hoffmann-La Roche Ltd

ImmunoIVD

LaCAR MDX Technologies

NMD Pharma

Novartis

Revvity

Scholar Rock

Other partners

161 Consulting Ltd - Michelle Levene

Alliance of Regenerative Medicines (ARM)

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Newus

Patvocates – Anne-Pierre Pickaert

Piagmo Teknoloji Limited

Pfeuffer, Armin Dipl.-Kfm

Rare Revolution Magazine

Rarity Life Magazine

Rastogi, Akshat

Saupe, Laura

Smart soc. Coop Impresa Sociale - Francesco Dragone

Tóth, Tímea

SMA Europe Scientific Advisory Board (SAB)

A special acknowledgement and 'Thank you' to our Scientific Advisory Board (SAB). Our SAB is composed of an international group of neuroscientists and neurologists with particular expertise in spinal muscular atrophy (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 are:

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

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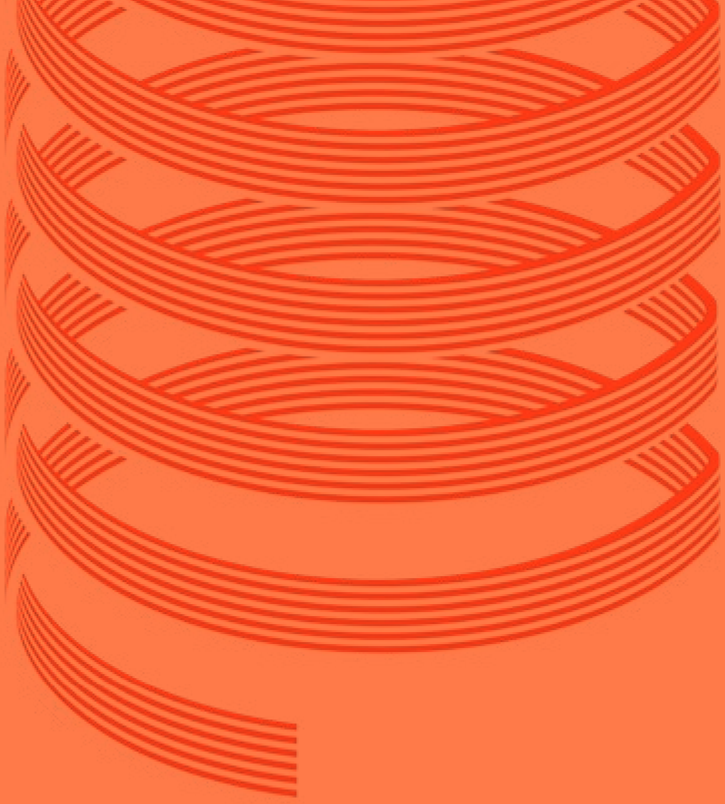

Our finances

Funds in

1’313’961.88 €

Funds out

1’049’766.08 €

Account balance	01.01.2024	31.12.2024
HVB Bank	1’975’250.57 €	**2’240’527.35 €**
PayPal	3’074.87 €	**3’074.87 €**

Revenue sources	amount
Patient organisations	**321’142.55 €**
Donations and grants	**153’970.42 €**
Corporate funding	**380’000.00 €**
Services and events	**455’352.35 €**
Miscellaneous	**3’496.56 €**
Total revenue	**1’313’961.88 €**

Expenses	amount
Operating expenses	**305’268.32 €**
Staff	84’520.18 €
Volunteers	7’265.42 €
Logistics	199’530.71 €
Material	211.19 €
Services received	7’636.10 €
Other operating expenses	6’104.72 €
Direct project expenses	**744’497.76 €**
Communication and outreach	61’596.29 €
Capacity building	146’461.51 €
Research	181’241.72 €
Therapy and care	180’762.87 €
Healthcare, policy and access	174’435.37 €
Total expenses	**1’049’766.08 €**

Explanations:

Patient organisations: This item records both membership fees and earmarked contributions from members for the Call for Research and SMArt Horizon.

Donations and grants: This item records grants received from Cure SMA.

Corporate funding: This item records financial support received through corporate funding. In 2024, we have received funds from Asuragen, Biogen, Biohaven, ImmunoIVD, LaCAR MDX, Novartis (Novartis Gene Therapies), Revvity, F. Hoffmann-La Roche Ltd, Scholar Rock.

Services and events: This item records revenue generated through our International Scientific Congress and our Treatment Committee activities through which we raise the patient voice and advocate for the needs of our community.

Miscellaneous: This item records refunds.

Volunteers: This item records expenses related to volunteer work, including travel expenses.

Logistics: This item records expenses related to the International Scientific Congress, Annual General Meeting, Workshops, Staff Retreat and travel expenses of staff and Board members.

Services received: This item records operating expenses on Microsoft 365, software, website, bank fees, accounting services, insurances, external pay-roll services, and other external services.

Other operating expenses: This item records expenses on membership fees to other organisations, staff and volunteer external trainings, taxes, reserves and tax emergency fund.

Communication and outreach: This item records direct project expenses on Awareness Activities including the Awareness Campaign and the Annual Report.

Capacity building: This item records direct project expenses on SMAcademy, the Global SMAdvocacy Event, Supporting Families in Crisis Situations.

Research: This item records direct project expenses on the Call for Research, the Research Agenda Project (Priority Setting Partnership Project) and SMArt Horizon.

Therapy and care: This item records direct project expenses on the Community Research Project such as the EUPESMA Survey Series, Focus Groups, Clinical Trial readiness activities.

Healthcare, policy and access: This item records direct project expenses on the 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 25, 2025, they concluded that the income and expenditure statement for the period from January 1, 2024, to December 31, 2024, 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).

List of abbreviations:

SMA	Spinal muscular atrophy
CT(s)	Clinical Trial(s)
ECR(s)	Early Career Researcher(s)
EUPESMA	European Patient Experience Survey
GA	General Assembly
PSP	Priority Setting Partnership
RD	Rare Disease
SMA NBSA	SMA Newborn Screening Alliance

All the graphics and visual resources included in this document are property of SMA Europe.

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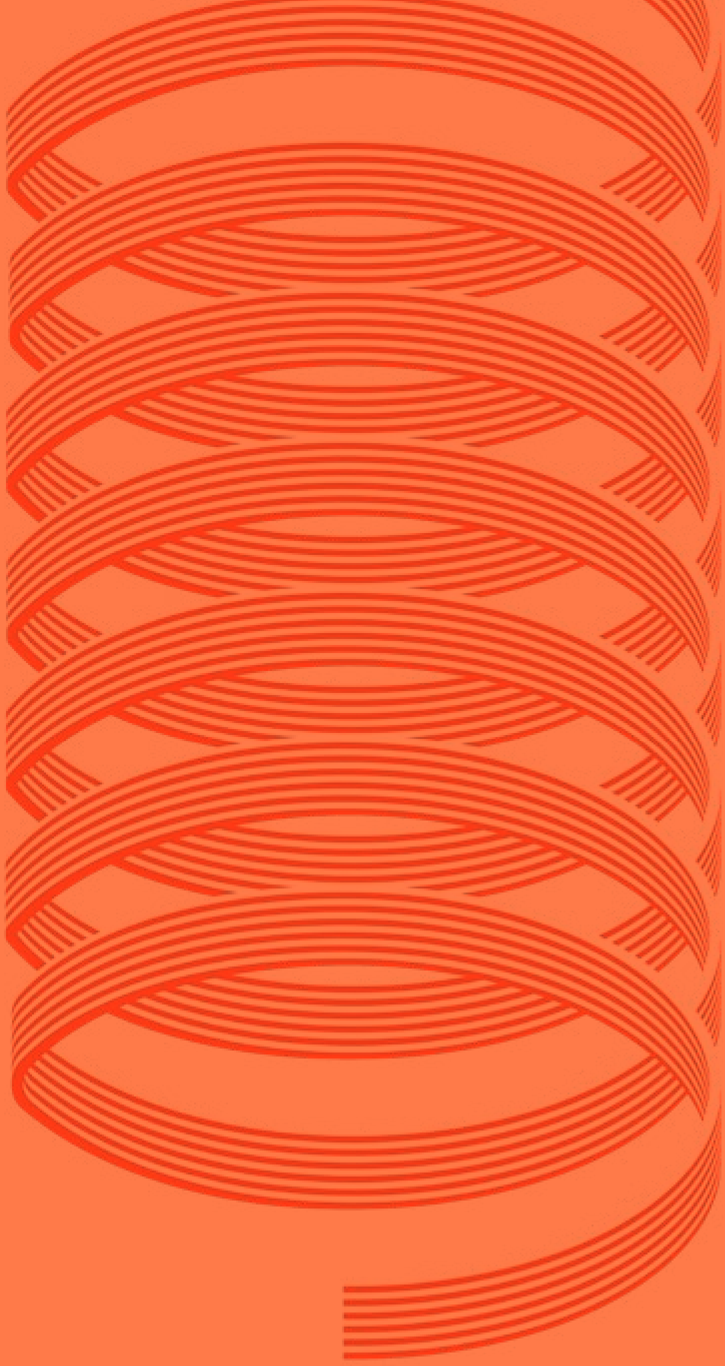

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