SMA
EUR
OPE

Annual report
2023

All together.
One goal.

Index

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

Foreword

It is with great pride that I present SMA Europe's first annual report for 2023. This year marks another milestone in our journey as we have made remarkable strides towards our mission of creating a better world for all those living with Spinal Muscular Atrophy (SMA).

In 2023, we successfully ran 16 projects and initiatives that spanned across our five strategic pillars: Research; Therapy & Care; Healthcare System, Access & Policy; Communication & Outreach; and Capacity Building. These projects are a testament to our commitment to advancing the field of SMA and improving the lives of those living with this condition.

As the first-ever appointed CEO of SMA Europe in early 2023, I have focused on ensuring the strategic sustainability of our organisation. This involves building a robust and effective governance system, securing funding and fostering a sense of unity among our dedicated staff members. We have made significant progress in strengthening the organisation from inside to consolidate external reputation and credibility of SMA Europe.

We have significantly increased our presence on various external communication channels, including social media, allowing us to reach and engage with a wider audience. Furthermore, we have contributed over 20 scientific posters and publications to the scientific community. All in all, this is the foundation for increasing our visibility to raise awareness of SMA, the needs of our community and the advancement of solutions.

At the heart of our achievements lies a profound sense of community. We are a mission-driven organisation, deeply committed to working in partnership with our member organisations, our international SMA partner organisations and all our stakeholders. Together, we are making significant progress towards a world where those living with SMA can thrive.

Our work would not be possible without the invaluable contributions of our members and of our many volunteers, including those serving on our Board and in our four committees. Their support and expertise have been crucial in driving our projects forward and ensuring their relevance to the SMA community.

Foreword

However, as a community, we are also facing significant challenges. Access to diagnosis, care and treatment remains heterogeneous across Europe and globally, with many individuals living with SMA still without access to essential medicines and care. Additionally, there are knowledge gaps surrounding the available treatments, as we do not yet fully understand which therapy or combination works best for whom. An emerging new phenotype of early treated children, alongside issues faced by the chronic population receiving treatment, presents further complexities that we are only beginning to unravel.

In 2023, we diligently worked on addressing these challenges and are committed – all together - to continuing our efforts towards finding solutions in the future.

Thank you for being part of this journey. Your support and dedication are what make our successes possible. As we look to the future, I am confident that, together, we will continue to make a meaningful impact and drive positive change for the SMA community.

With gratitude and hope,

N. Gusset

Dr. Nicole Gusset
CEO, SMA Europe

2023 highlights

We successfully ran

16 projects

in which **+ 50 volunteers** and **9 staff** members contributed.

We had

1 new member

(MDA Cyprus) joining SMA Europe.

We appointed our

1st CEO

to ensure the **strategic sustainability** of our organisation.

We counted on

4 Committees

and **1 Working Group** to support our projects and ensure their relevance to our community.

More than

20 posters

and **publications** were presented and published at different renowned international events and platforms.

We had

6138 followers

on our social media (+25% compared to 2022).

← →

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.

> “Since we became a member in 2017, the joint work of all the members has already yielded meaningful results at European level. And we have had the opportunity to establish contacts and interactions with key stakeholders, which help us to work more effectively in all areas, including those needed to solve the challenges of Russian patients and families.”
>
> **Olga Germanenko,** SMA Family Foundation Russia

OUR PROJECTS

“**United, at the European level and in collaboration with all stakeholders,** we will achieve our vision of creating a better world for all those living with SMA. This is our reason for being, above all else. It infuses everything we do and is what we invite others to help us achieve.”

Nicole Gusset, CEO & President of SMA Europe

Routed in our mission, our 2023 projects are organised under our five strategic pillars.

Research

Promote patient-relevant SMA research.

SMA Research Agenda: Priority Setting Partnership (PSP)
4th Scientific International Congress on SMA
Call for Research Proposal
SMArt Horizon
SMA Europe meets early career researchers (ECRs)

Therapy and Care

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

Clinical Trial Site Readiness in Europe
EUPESMA 2023 – European Patient Experience Survey
SMA Daily Life Study

Healthcare System, Policy and Access

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

OdySMA
Adult Care Benchmarking
SMA Newborn Screening Alliance (NBSA)

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
Global SMAdvocacy Event

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.

Awareness Campaign 2023

Our projects

SMA Research Agenda: PSP
4th Scientific International Congress on SMA
Call for Research Proposal
SMArt Horizon
SMA Europe meets Early Career Researchers
Clinical Trial Site Readiness
EUPESMA 2023
SMA Daily Life Study
OdySMA
Adult Care Benchmarking
SMA Newborn Screening Alliance
Annual Meetings with General Assembly
Our SMA Europe
SMAcademy
Global SMAdvocacy Event
Awareness Campaign 2023

> **The results will allow SMA Europe to drive towards an SMA research agenda and prioritise the real needs of all living with SMA.**

Description

Pillar: Research.

This project identifies the top 10 unanswered research questions about SMA that are a priority to people who live with SMA, their caregivers and healthcare professionals.

The results will allow SMA Europe to drive towards an SMA research agenda with all relevant stakeholders and to push scientists, service providers and funders to recognise and prioritise the needs of those living with SMA.

The PSP is run in collaboration with the James Lind Alliance.

Main developments in 2023

The top 10 priorities for SMA research, from the perspective of people who live with SMA, their caregivers and healthcare professionals were agreed in a predefined thorough process with two large-scale surveys that were translated into several European languages and culminated in a multi-stakeholder face-to-face workshop in Amsterdam.

Next steps

— A workshop towards an SMA Research Agenda to be held during the 4th Scientific International Congress on SMA between scientists, clinicians and patient advocates.

— The educational campaign to be developed and raise awareness about the research agenda in 2024.

— Academic publication and dissemination of the results of the defined 10 priorities.

What makes this project relevant to our SMA Community?

This project is relevant to the SMA community because it allows for the prioritisation of ongoing and future needs, and to construct the future SMA research agenda.

The PSP project is a tool to guide future research efforts towards a focus on unmet needs identified by the community to ensure that resources are used as effectively as possible.

Link to the website

Our projects

The goal of our scientific congress is to bring together an international and transdisciplinary group of scientists, healthcare professionals and patient advocates.

Description

Pillar: Research.

SMA Europe is committed to promoting patient-relevant SMA research through its projects, funding initiatives and collaborations.

The goal of our scientific congress is to bring together an international and transdisciplinary group of scientists, healthcare professionals and patient advocates. The event provides a venue for them to present and exchange their breakthrough ideas relating to SMA, especially also considering the patient relevance of their findings, and to cement existing and stimulate new collaborations.

This is the fourth congress organised by SMA Europe.

Main developments in 2023

- Preparing the scientific programme of the event, together with the members of our Scientific Advisory Board incl. assessment of scientific abstracts and selection of speakers.
- Planning, coordinating and implementing of all necessary aspects of the congress incl. sponsorships, logistics, social programme and communications.

Next steps

- 4th Internacional Scientific Congress on SMA in Ghent: 14 – 16 March 2024.

Events linked to the congress:

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

What makes this project relevant to our SMA Community?

Our scientific congresses feature highly respected, internationally renowned speakers. Equally important, they are a platform for talented young researchers.

Together with healthcare professionals and patient advocates, they discuss, debate and dissect significant new developments and advancements relating to SMA.

By providing an opportunity to exchange scientific evidence and clinical experiences, these events have a unique opportunity to lead to ever-improved treatment and care for patients living with SMA.

Our projects

SMA Research Agenda: PSP
4th Scientific International Congress on SMA
Call for Research Proposal
SMArt Horizon
SMA Europe meets Early Career Researchers
Clinical Trial Site Readiness
EUPESMA 2023
SMA Daily Life Study
OdySMA
Adult Care Benchmarking
SMA Newborn Screening Alliance
Annual Meetings with General Assembly
Our SMA Europe
SMAcademy
Global SMAdvocacy Event
Awareness Campaign 2023

This initiative funds the identification of targets for effective treatments that address the needs of all who live with SMA.

Description

Pillar: Research.

The Call for Research Proposal is an initiative set up to promote and sustain fundamental and clinical research in SMA in Europe.

Including this 11th round, the Call for Research Proposal has funded 48 projects and other initiatives.

Main developments in 2023

6 grants were awarded:

- Marina Boido, Neuroscience Institute Cavalieri Ottolenghi (NICO).
- Haiyan Zhou, University College London (UCL).
- Oliver Gruss, Rheinische Friedrich-Wilhelms-Universität Bonn.
- Simon Parson, University of Aberdeen.
- Peter Claus, SMATHERIA gGmbH.

SMA Europe members who financially contributed to the Call for Research Proposal are:

AFM Téléthon, Deutsche Gesellschaft fuer Muskelkranke (DGM), FundAME, Muskelsvindfonden, Nätverket för spinal muskelatrofi (NSMA), SMA Belgium, SMA UK and SMA Schweiz.

Next steps

Launch of Call for Research Proposal nr. 12 in spring 2024.

What makes this project relevant to our SMA Community?

The primary aims of this initative are to find therapies and elucidate the basic pathophysiological processes of the condition.

SMA Europe is one of the only organisations that funds research on spinal muscular atrophy. Therefore, our Call for Research Proposal fills a crucial gap in this field, ensuring that the needs of the community in treatment development and care, are addressed and met.

Link to the website

Our projects

SMA Research Agenda: PSP
4th Scientific International Congress on SMA
Call for Research Proposal
SMArt Horizon
SMA Europe meets Early Career Researchers
Clinical Trial Site Readiness
EUPESMA 2023
SMA Daily Life Study
OdySMA
Adult Care Benchmarking
SMA Newborn Screening Alliance
Annual Meetings with General Assembly
Our SMA Europe
SMAcademy
Global SMAdvocacy Event
Awareness Campaign 2023

Description

Pillar: Research.

The SMArt Horizon aims at identifying new innovative, "out-of-the-box" therapies for people living with SMA, especially for the chronic population.

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 which can then be tailored specifically to SMA.

The project is a partnership between SMA Europe, its members and the SMA Foundation. It is also financially co-supported by FundAME through its Calendar Fund, SMA Schweiz and Vereniging Spierziekten Nederland.

> **The SMArt Horizon aims at identifying new innovative, "out-of-the-box" therapies for people living with SMA.**

Main developments in 2023

- Individuals from 10 member organisations were involved to scan their countries for promising research. During monthly meetings, findings were shared and assessed by the project team with attendees from the SMA Foundation and SMA Europe.
- Various research centres (e.g., AFM Téléthon, France; EPFL, Switzerland) with interesting projects and concepts were visited.
- One research project was selected to be financially supported: "Characterisation of the effects of spinal cord stimulation on arm and hand kinematics and motor neuron firing rates in people with non-ambulatory SMA".

Next steps

Support of selected study of Marco Capogrosso:

- Financial support.
- Advise on how to ensure that the study remains patient-relevant.

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.

Link to the website

Our projects

Description

Pillars: Research and Capacity Building.

SMA Europe meets Early Career Researchers is an international event geared towards fostering the relationship and understanding between ECRs and SMA patient advocates.

Main developments in 2023

— Coordination and planning of the event.

— Coordination of the programme that consists of:

  - Roundtables on lived experiences and geographical differences, science communication and advocacy; feedback on talks, science communication and its role in patient advocacy.
  - Flash talks and Q&As.

Next steps

— The event on 13th March 2024, with 16 ECTs and 8 patient advocates.

— Feedback surveys, reports and dissemination of the results.

— Buddying system for those who want to further exchange knowledge and discuss topics.

What makes this project relevant to our SMA Community?

This initiative allows:

— ECRs to engage with the broader SMA community and to bridge the gap between their laboratory work and the lived experiences of the condition they are working on.

— Patient advocates to understand the current research landscape and research processes. It gives them tools and arguments for lobbying policymakers and health organisations for sustained research funding. It also allows them to advocate for their unmet needs with future leaders in research.

This event allows for knowledge exchange between Early Career Researchers and Patient Advocates.

Our projects

The outcomes of the project will inform the Clinical Trial Handbook for Patient Advocates and Recommendations for SMA patient-friendly Clinical Trials 2024.

Description

Pillar: Therapy and Care.

The Clinical Trial Site Readiness initative was initatied in 2018 within the Cure SMA Industry Collaboration. In 2023, we worked towards understanding if there had been a change in European clinical trial site capacity to run trials now that disease-modifying treatments are available. It also sought to equip trial sites with resources to support their ability to run or expand trials, in a patient-relevant way.

This programme includes the preparation and sharing of already available education materials to help sites and teams further understand SMA, from a patient perspective.

Main developments in 2023

Conduction of the survey and interviews with clinical trial sites:

- 32 / 140 sites across 14 countries responded. Results indicate that additional staff is required, as well as equipment and infrastructure.
- The conclusions also point out that SMA Europe can play an impactful role by serving as a go-between for industry and sites to increase the capacity of those countries where access to treatment is still limited.

Next steps

- Clinical Trial Handbook for Patient Advocates and Recommendations for SMA patient-friendly Clinical Trials.

What makes this project relevant to our SMA Community?

The Clinical Trials Site Readiness initative is relevant because there is an expanding pipeline of potential therapeutic agents currently being developed with a limited number of clinical trial sites.

It was anticipated that clinical trial sites across Europe might have challenges while taking on more trials to test these compounds, so SMA Europe sought to understand what these might be.

The project allows to think about initiatives that could be undertaken to alleviate these challenges and increase site capacity as well as lead to more effective, patient-relevant solutions.

Our projects

EUPESMA gathers experiences and expectations from people living with SMA to help us build a landscape of the evolving needs of our community.

Description

Pillar: Therapy and Care.

The EUPESMA gathers experiences and expectations from people living with SMA to help us build a landscape of the evolving needs of our community.

The results of these surveys inform our organisation's advocacy efforts and help us advise stakeholders on the necessary changes to best serve and improve the lives of people living with SMA. The surveys are designed together with our community.

Our EUPESMA 2023 survey focused on SMA and Clinical Trials.

Main developments in 2023

- A qualitative workshop at the Annual Meetings in Prague was conducted to guide survey design.
- The survey questionnaire was designed with feedback from SMA patient advocates and people living with SMA.
- The survey was translated into 14 European languages in collaboration with SMA Europe's volunteers.
- The survey was distributed and promoted by members of SMA Europe.

Next steps

- Data analysis and elaboration of results.
- Development of posters for presentation at relevant conferences.
- Development of one manuscript for scientific publication.
- Lay Summary report.

What makes this project relevant to our SMA Community?

The EUPESMA 2023 on clinical trials helps us understand the expectations towards clinical trials and the readiness to participate in clinical trials, now that three medicines for SMA are available. Data on experiences and expectations around their participation will support our advocacy efforts in future site selection and patient-friendly clinical trial design.

Link to the website

Our projects

Results from the SMA Daily Life lead to the development of valid assesment tools for patient-relevant outcomes.

Description

Pillar: Therapy and Care.

Factors like fatigue, pain, and mental health affect the daily wellbeing of people living with SMA and may be valuable outcomes measures when assessing treatment.

These patient-relevant outcomes are gaining increasing attention in the field of SMA, including as endpoints in clinical trials.

Designed in close collaboration with people living with SMA, the SMA Daily Life Study assesses patient-relevant outcomes with an innovative method that allows to track how these measures change in the day-to-day of individual patients.

Main developments in 2023

— Pilot and the final design of the SMA Daily Life Study.
— 47 people living with SMA completed this intensive study, providing more than 2245 data points.
— First results presented with a poster at the 2023 CureSMA Conference.
— Two abstracts were accepted for poster presentations at the 4th Scientific International Congress on SMA in Ghent.
— The participants were provided with the opportunity to receive first-hand access to their data, selecting the information that was most relevant to them. These participants received their data in the form of a personalised infographic.

Next steps

— Two scientific posters to be presented at SMA Europe's International Scientific Congress, ECRD 2024 and 2024 Cure SMA Conference.
— Publication of results in a peer-reviewed scientific journal.

What makes this project relevant to our SMA Community?

Results from the SMA Daily Life Study, strong of a patient-relevant approach, allow us to obtain unique insights that can guide the development of more accurate and valid assessment tools for patient-relevant outcomes.

Link to the website

Our projects

SMA Research Agenda: PSP
4th Scientific International Congress on SMA
Call for Research Proposal
SMArt Horizon
SMA Europe meets Early Career Researchers
Clinical Trial Site Readiness
EUPESMA 2023
SMA Daily Life Study
OdySMA
Adult Care Benchmarking
SMA Newborn Screening Alliance
Annual Meetings with General Assembly
Our SMA Europe
SMAcademy
Global SMAdvocacy Event
Awareness Campaign 2023

Description

Pillars: Healthcare System, Policy and Access.

With our initative "OdySMA – a quest to access" SMA Europe's goals are to identify hurdles to access to healthcare and initiate advocacy as well as policy-shaping initiatives to overcome those.

OdySMA aims to reveal the "quest to access" of people living with SMA by mapping, visualising and centralising knowledge around access issues and by driving advocacy around it.

Main developments in 2023

— Launch of the OdySMA platform in February 2023 (Rare Disease month).

— Constant data updates and support of our members and individuals living with SMA.

— Training and workshop on OdySMA for SMA Europe member organisations at SMA Europe Annual Meetings in Prague.

— Abstract submission to congresses in 2024, including the 4th Scientific International Congress on SMA Europe in Ghent.

Next steps

— Ongoing optimisation of the OdySMA access data.

— Combining quantitative data with qualitative data by including "real-life stories" into the OdySMA platform.

— Adult Care Benchmarking with SMA project's data to be integrated into the OdySMA platform.

— Training for patient advocates on how to build successful advocacy strategies.

— Assessment of member experience: "Pathways to access to medicines, including crowdfunding".

What makes this project relevant to our SMA Community?

The aim behind the collected and visualised data, tools and centralised knowledge is to support patient advocates to identify, understand and overcome variations in access to medicines and care in Europe. It is paramount for successful advocacy for access to medicines and care.

Link to the website

> **OdySMA is a initiative to reveal the "quest to access" of people living with SMA.**

Our projects

SMA Research Agenda: PSP
4th Scientific International Congress on SMA
Call for Research Proposal
SMArt Horizon
SMA Europe meets Early Career Researchers
Clinical Trial Site Readiness
EUPESMA 2023
SMA Daily Life Study
OdySMA
Adult Care Benchmarking
SMA Newborn Screening Alliance
Annual Meetings with General Assembly
Our SMA Europe
SMAcademy
Global SMAdvocacy Event
Awareness Campaign 2023

Description

Pillars: Healthcare System, Policy and Access.

The objective of the project “Benchmarking care for adults living with SMA in Europe” is to provide the SMA community with an evidence-based resource that can be used to engage relevant stakeholders, increase awareness of the care challenges faced by adults living with SMA, and to call for policy change.

The outcomes of the project are the development of one, general benchmarking report and 22 country short reports.

Main developments in 2023

In 2023, the data for the report was collected. The following methodology was applied:

- Rapid literature review.
- Online structured survey with clinical experts treating adults living with SMA.
- Semi-structured phone calls with SMA Europe Members.

Next steps

- Launch of the full report and the 22 country short reports on Rare Disease Day 2024.
- Presentation of the project to MEP Stelios Kympouropoulos.
- Webinar for SMA Europe Members presenting the project ahead of the launch of the reports.
- Launch of the respective subpage on OdySMA describing the project and dissemination of the reports.
- Integration of the data on OdySMA.
- Training for patient advocates on how to use the report to build successful national advocacy strategies.

What makes this project relevant to our SMA Community?

Adults make up an estimated 50% of the SMA population, and with the increasing availability of innovative treatments that improve quality of life and survival rates, those numbers are set to grow.

Through the evidence collected in this project, SMA Europe aims to raise awareness and eventually improve the situation of adults living with SMA.

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Our projects

SMA Research Agenda: PSP
4th Scientific International Congress on SMA
Call for Research Proposal
SMArt Horizon
SMA Europe meets Early Career Researchers
Clinical Trial Site Readiness
EUPESMA 2023
SMA Daily Life Study
OdySMA
Adult Care Benchmarking
SMA Newborn Screening Alliance
Annual Meetings with General Assembly
Our SMA Europe
SMAcademy
Global SMAdvocacy Event
Awareness Campaign 2023

The goal of SMA Newborn Screening Alliance is to decrease in European countries the time it takes for a child born with spinal muscular atrophy to be diagnosed.

Description

Pillars: Healthcare System, Policy and Access.

SMA Europe established the SMA NBSA to bring together all the stakeholders who share the vision and are willing to work together towards making SMA newborn screening in all European countries a reality.

Main developments in 2023

- December 2023: 55% of the babies born in the EU are screened for SMA and more than 65% of European newborns including Russia and Turkey are screened.
- Re-design of the SMA NBSA website.
- Signature of the open letter “Resolving health inequalities in newborn screening across Europe” to Commissioner Kyriakides together with Members of EURORDIS, ERN-NMD, Screen4Care and the University College London.

Next steps

- Assess the situation regarding newborn screening for SMA in the different countries and define, which steps need to be taken to improve access to screening.
- Work with countries where there is partial or no implementation of NBS (e.g. Romania, Greece, etc.).
- Individual support to Greece and North Macedonia for implementing SMA NBS.
- Webinar for Members: The evolving of SMA Genetics in the new therapeutic era by Eduardo Tizzano M.D., Ph.D.
- Participation at the EUCOPE Expert Session 9 - ‘Navigating Diagnostics Through the Lens of the Pharmaceutical Package and Beyond’ at the European Parliament in March 2024.
- Scientific publications, update of the White Paper.

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).

Link to the website

Our projects

SMA Research Agenda: PSP

4th Scientific International Congress on SMA

Call for Research Proposal

SMArt Horizon

SMA Europe meets Early Career Researchers

Clinical Trial Site Readiness

EUPESMA 2023

SMA Daily Life Study

OdySMA

Adult Care Benchmarking

SMA Newborn Screening Alliance

Annual Meetings with General Assembly

Our SMA Europe

SMAcademy

Global SMAdvocacy Event

Awareness Campaign 2023

These meetings offer an opportunity to SMA Europe's members to shape and contribute to our activities according to their needs.

Description

Pillars: Capacity Building, Communication & Outreach.

SMA Europe's Annual Meetings with GA offer our member organisations the opportunity to come together, discuss all matters relevant for SMA Europe and network with each other.

During the event, member organisations contribute actively to our projects and are updated on the status of the various projects and initiatives which SMA Europe is spearheading. The Annual General Meetings close with the GA where our delegates make key decisions on various matters concerning the organisation according to their statutory obligations.

Main developments in 2023

More than 25 of our delegates joined the meetings in Prague in person, from April 27 – 29 along with other stakeholders of our organisation. SMA Europe invited also 15 industry partners' representatives from Europe and the United States to a Pharma Day.

Outcomes of the Annual Meetings in Prague:

- Hybrid GA took place successfully.
- Several workshops were held with our members on different topics. We also updated them on the progress of our initiatives and projects.
- Fruitful Treatment Committee Advisory Boards with various industry partners took place.
- Industry partners, patient advocates and other guests actively participated in our multi-stakeholder scoping SMAcademy workshop, and received updates on SMA Europe's projects.

Next steps

Annual Meetings in Munich, 13 – 14 June 2024.

What makes this project relevant to our SMA Community?

These meetings offer an opportunity to SMA Europe's members to shape and contribute to our activities according to their needs.

The attendees have also the chance to discuss in person the issues facing the community and strengthen the networks and bonds between each other.

Link to the website

Our projects

SMA Research Agenda: PSP
4th Scientific International Congress on SMA
Call for Research Proposal
SMArt Horizon
SMA Europe meets Early Career Researchers
Clinical Trial Site Readiness
EUPESMA 2023
SMA Daily Life Study
OdySMA
Adult Care Benchmarking
SMA Newborn Screening Alliance
Annual Meetings with General Assembly
Our SMA Europe
SMAcademy
Global SMAdvocacy Event
Awareness Campaign 2023

Description

Pillars: Capacity Building, Communication & Outreach.

The project aims at developing a stronger, independent, sustainable, and impactful community of SMA organisations in Europe.

Its focus is to ensure that SMA Europe understands the needs and expectations of the member organisations.

It also helps to create a sustainable ecosystem where different expectations are met through the active participation of our members, always under the inclusion and diversity criteria.

Main developments in 2023

- Design of a pre-questionnaire to understand the main topics of interest for our members, that should be covered during the in-depth interviews phase of the project. It was completed by 18 member organisations.
- Presentation of the results of the preliminary feedback gained from the pre-questionnaire delivered during our Annual Meetings in Prague.
- In-depth interviews with 19 member organisations were designed and conducted starting November 2023.
- Two “Learning from each other” sessions were held during our regular Delegate Calls with success stories from Spain (Awareness Campaign #CarolinaAyúdame aimed at improving FundAME’s social and political influence in Spain) and Greece (“SMA Journey – Patient experience” project).

Next steps

- Finalise data collection and report.
- Action plan to be developed in 2025 with some initiatives already implemented in 2024.

What makes this project relevant to our SMA Community?

The members of SMA Europe comprehend a variety of contexts and diversity when it comes to the expertise and size of each organisation. This project aims at securing that every voice is heard and that we can design our actions, also on the operational level, to ensure the regular exchange of knowledge, topics of interest and uncovered needs of our members.

This project reinforces the position of SMA Europe as the platform that brings its member organisations together.

Our projects

SMA Research Agenda: PSP
4th Scientific International Congress on SMA
Call for Research Proposal
SMArt Horizon
SMA Europe meets Early Career Researchers
Clinical Trial Site Readiness
EUPESMA 2023
SMA Daily Life Study
OdySMA
Adult Care Benchmarking
SMA Newborn Screening Alliance
Annual Meetings with General Assembly
Our SMA Europe
SMAcademy
Global SMAdvocacy Event
Awareness Campaign 2023

Description

Pillar: Capacity Building.

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.

Main developments in 2023

- Feasibility assessment.
- Multistakeholder workshop held during the Annual Meetings in Prague.

Next steps

- Building SMAcademy: definition of the scope, programmes, and evolving learning objectives of SMAcademy in collaboration with the patient advocates’ community.
- Creation and dissemination of learning opportunities, such as webinars, podcasts and videos.
- Creation of an information hub with SMAcademy resources.

What makes this project relevant to our SMA Community?

This project allows SMA patient advocates to become more effective in advocating for a better world for people living with SMA.

Bringing learners together, SMAcademy also fosters networks among patient advocacy organisations and with stakeholders on a global level.

> **SMAcademy is SMA Europe’s new initiative that aims to build capacity among patient advocates in SMA.**

Our projects

> **The Global SMAdvocacy Event aims to provide a much-needed opportunity for patient advocates to exchange experiences, learn from each other, and strategise together.**

Description

Pillars: Capacity Building, Communication and Outreach.

SMAcademy will be officially launched with the Global SMAdvocacy Event.

The event will run a day before the 4th Scientific International Congress on SMA in Ghent and aims to bring together patient advocates from across the globe.

Main developments in 2023

— In 2023 we raised funds and opened a call for applications for our launch event, the Global SMAdvocacy Event.

Next steps

— Assignation of scholarships to attend the event in person, to ensure that patient advocates from around the globe have equal opportunities to join.

— Identification of the most urgent challenges in SMA advocacy through our application process and direct polling of the participants.

— Design and coordination of a full day of hands-on workshops to work towards solutions to the identified challenges.

What makes this project relevant to our SMA Community?

The Global SMAdvocacy Event aims to provide a much-needed opportunity for patient advocates to exchange experiences, learn from each other, and strategise together towards shared goals in SMA patient advocacy.

The community can benefit from the value of meeting in person to network and forge new synergies among advocates from different parts of the world.

Our projects

Social media

103.664
Impressions

12.312
Interactions

459
New followers

Website

+5.100
Users

+17.000
Visits

392
Visits on the campaign page

Description

Pillars: Capacity Building, Communication & Outreach.

The 2023 #WeAreUnique Campaign was the first awareness campaign fully curated by the organisation since its creation in 2007 and focused on the stories of people who live with SMA, across Europe.

The campaign shows how diverse our community is while we keep fighting for the same goals and face similar challenges when it comes to access to treatment, diagnosis and care standards, across Europe.

Main developments in 2023

- Participative workshop held during Annual Meetings in Prague to ensure that the campaign resonates with the expectations of SMA Europe's community.
- Regular meetings with the Communications Working Group to receive pertinent feedback.
- Coordination of the graphic designs for the campaign together with the volunteers who decided to participate. The campaign counted on the participation of 11 individuals living with SMA from different countries that are members of SMA Europe.

Next steps

- Vernissage with the final designs of the campaign held during the 4th Scientific International Congress on SMA in Ghent.
- Awareness Campaign 2024 to be focused on the importance of the research in SMA.

What makes this project relevant to our SMA Community?

The #WeAreUnique 2023 campaign's scope was to tell the real stories of people living with SMA and showcase what people in the community feel defines them the most. It also unified all their testimonials under one, common umbrella: SMA Europe as their reference patient organisation in Europe.

This initiative raised awareness of our SMA community and ensured its mid and long-term impact through materials that are to be reused again during the 4th Scientific International Congress on SMA. It also helped to unify our communications channels and ensured their future sustainability in terms of outreach and engagement.

Link to the website

OPERATIONAL EFFICIENCY AND SUSTAINABILITY

Our vision **to create a better world for all affected by SMA** involves several steps and initiatives. One of our strategic objectives towards building our long-term operational capacity, has been to strengthen SMA Europe's effectiveness, sustainability and capacity to take collective action.

We have worked towards increasing the organisation's sustainability in terms of governance, human, financial and organisational resources, and have invested into strengthening the organisation from the inside by gathering feedback from members and staff, having a well-functioning Board, diversifying and securing funding sources.

Moreover, to operate as a leading non-profit organisation that exemplifies sustainability, contributing to environmental preservation, the wellbeing of our community, and economic resilience, means we strive to embed and integrate sustainable practices across all operations, enhance our impact on the SMA community while minimising our environmental footprint.

We have already taken **some steps in the direction of achieving our sustainability goals** and we plan to continue pursuing this path to ensure as positive an impact on the environment and the community while maintaining economic resilience and integrity, as possible.

In 2023, we devoted significant resources (human and financial) in the direction of improving our operational effectiveness and ensuring our long-term sustainability by:

- Working on policies, procedures and templates to allow more effective implementation, including branding materials.
- Improving further the remote working setting for staff and volunteers, e.g. regular meeting(s), regular staff meeting and regular 1:1 meetings).

  In November 2023, following a year of exciting projects and new opportunities, the SMA Europe staff team had the chance to get together in Switzerland to regroup and spend some quality time together. During those few days, we had the time to take stock of- and reflect on everything we achieved during 2023, how we see our organisation, and exchanged ideas on how to address any challenges and improve upon the quality of our outcomes to ultimately achieve our shared goals. Gathering the team in a quiet and serene setting offered us the unique opportunity to enhance our team cohesion and spirit, through various fun activities and discussions. Most importantly, we managed to reenergise so we can position ourselves better to serve the SMA community, all while having the privilege of admiring the Alpine beauty!
- Holding quarterly Delegates' Calls to update our members on the progress of various projects, as this has proven an effective tool for keeping our members updated and staying in touch with each other outside any face-to-face meetings.
- Working on maintaining, improving and leveraging to the maximum our MS 365 system and other digital tools.
- Exploring additional digital options and solutions that would allow us to integrate sustainable tools into our operations and enable us to continue to pursue our sustainability goals.
- Working on a consistent and sustainable reporting system.
- Working on human resource procedures, incl. implementation of employees in six different European countries and recruiting new members to the team, our CEO in Switzerland and our Outcome Research Manager in Germany.
- Improving our financial tracking and reporting systems.
- Developing and structuring the Board in a more effective manner.
- Improving credibility, reputation and increased satisfaction with members and external stakeholders.

Moving forward, SMA Europe will continue to strive to achieve its long-term sustainability goals by focusing, among others, on the following areas:

- **Sustainable Procurement:** Prioritise purchasing from suppliers who adhere to sustainable practices.
- **Operational Efficiency:** Streamline operations to reduce waste and enhance resource efficiency.
- **Circular Economy Practices:** Emphasise reducing, reusing, and recycling materials in all projects and operations.
- **Adopt Green Technologies:** Utilise renewable energy sources, eco-friendly materials, and energy-efficient equipment.
- **Leverage Digital Tools:** Use digital tools for virtual meetings, reducing the need for travel and physical resources.
- **Feedback Mechanisms:** Create avenues for continuous feedback and suggestions from staff and stakeholders.
- **Adaptive Management:** Stay flexible and responsive to new sustainability challenges and opportunities.
- **Transparency:** Regularly report progress to stakeholders through annual sustainability reports and other communication channels.

SMA Europe continues working on both an internal and external communications strategy and its implementation, to ensure the work done and impact achieved is also well communicated to the outside and understood by both the membership and key stakeholders. This will consolidate the external reputation and credibility of SMA Europe and increase satisfaction of members, Board and staff.

SMA Europe has **also reinforced its staff and volunteer base through the establishment of various committees, to enable further engagement** such as in a larger number of decision-making bodies, scientific opinion-making committees, and other healthcare relevant specialists' committees. SMA Europe remains patient-relevant, is driven by people affected by SMA, is flexible and will also be innovative in its tools to work in a pan-European environment by fostering partnerships, alliances and consortiums.

SPECIAL FEATURE

> “In the next 5 – 10 years, I hope to see a stronger global community of SMA Europe members.”

Joseph Ioannou Vice-President of MDA Cyprus

Joseph Ioannou

Joseph Ioannou is the Vice-President of Muscular Dystrophy Association Cyprus (MDA Cyprus). We have spoken with him to better understand both his interests and the challenges faced by the organisation of this small country bathed by the Mediterranean.

Please tell us who you are. Personally, and professionally. We want to know more about you. What are your hobbies. What you enjoy most... and what you do in your everyday life

My name is Joseph Ioannou, and I'm 37 years old. Despite having SMA-LED, I navigate life with patience and resilience. Since the last election of MDA Cyprus, I am a Vice-President of our organisation.

Despite everyday challenges, I find solace and joy in a diverse array of hobbies and interests. Weekends are my sanctuary, where I immerse myself in the electrifying world of football, whether it's cheering on my favourite team, OMONOIA NICOSIA, from the stands, or joining friends on the field for a spirited match. Beyond the roar of the crowd, I'm a huge fan of Marvel world, with a cherished collection of comics and small figures that never fail to ignite my imagination.

In 2018, I got married to a beautiful woman, who has been my partner in all of life's adventures. Together, we embark on enriching experiences to explore different cultures and forge meaningful connections with people from all walks of life. Whether we stroll through bustling markets or marvel at the grandeur of historical landmarks, we're always eager to embrace the richness the world has to offer.

As a Computer Engineering graduate, I've ventured into the dynamic world of technology with my own small business, FIX IT CODE, based in Cyprus. While I thrive in the fast-paced environment of entrepreneurship, I also wear multiple hats as the owner, General Manager, and Technical Support Specialist.

Please tell us more about MDA Cyprus, its structure and goals.

The MDA Cyprus was founded in 1986, and its members include children and adults from all over Cyprus. The objectives of the Muscular Dystrophy Association are to secure and provide medical care, mobility aids, and support to its members, to promote measures for active participation of the state and society in all areas related to Muscular Dystrophy, and to organise lectures and events to inform members about research or other issues concerning them.

The rare conditions dealt with by the Muscular Dystrophy Association of Cyprus include Duchenne and Becker Muscular Dystrophy, Myasthenia Gravis, conditions of the Motor Neurons such as ALS and SMA, conditions of the Peripheral Nerves such as Charcot-Marie-Tooth disease, and generally almost all categories of neuromuscular diseases. The Association closely collaborates with the Cyprus Institute of Neurology and Genetics, where it is also housed. At the Institute, both research and care coexist, creating an excellent research and medical center.

MDA Cyprus uses the services of the Institute to provide patients and their families with the best possible medical care in Cyprus. In collaboration with the Cyprus Institute of Neurology and Genetics, the Muscular Dystrophy Association of Cyprus coordinates the Cyprus TELETHON, an annual international philanthropic event.

At MDA Cyprus, there are 8 people with SMA, 3 children from 1 to 7 years old, 3 people 27 – 36 years old, and 2 people from 60 – 65. People with SMA in Cyprus as we know are 11 now, we are trying to get in touch with as many as possible.

> **“Beyond the roar of the crowd, I’m a huge fan of Marvel world, with a cherished collection of comics and small figures that never fail to ignite my imagination.”**

What is the situation of the SMA Community in Cyprus?

In Cyprus, there is no newborn screening for a certain condition. Therefore, the diagnosis is typically confirmed only if someone is aware of the symptoms and undergoes genetic testing at the Institute of Neurology and Genetics in Cyprus. Once the diagnosis is made, the neurologist requests treatment from the Ministry of Health, which is then approved and administered to the patient.

Patients are also provided with supportive services, such as visits to doctors of various specialties, diagnostic tests, physiotherapy, and technical means for daily living (such as wheelchairs, special beds, and mattresses), through the National Health System of Cyprus.

Additionally, financial assistance is provided to help patients acquire an adapted car, disability benefits for personal employment assistants, medical equipment (such as ventilators and suction devices), and consumables.

How did you come across SMA Europe as an organisation? What was the reason for you and your national organisation to be interested in joining SMA Europe?
As a Muscular Dystrophy Association with patients living with SMA, we are seeking to find a community where we can exchange knowledge and learn from other countries with the same disease. Being from a small country, we must have access to treatments and understand how other countries are organising themselves to tackle SMA. While searching online, I came across your organisation and spoke to my board about becoming a member. Luckily, they have accepted the proposal.

> **“MDA Cyprus uses the services of the Institute to provide patients and their families with the best possible medical care in Cyprus.”**

How do you see the future of SMA Europe in 5 – 10 years?
I understand that the most important aim for all members is to raise awareness and understanding of SMA and to promote access to high-quality care and quality of life for individuals with SMA and their families. This also includes promoting scientific research, supporting access to new therapies, developing healthcare infrastructures, and advocating for newborn screening.

In the next 5 – 10 years, I hope to see a stronger global community of SMA Europe members, taking even more effective actions to promote research, enhance support for individuals living with SMA and their families, and achieve greater influence in policy and society to improve living conditions and promote equality.

OUR TEAM

Our team

Board

Nicole Gusset
President, SMA Schweiz, Switzerland

Yasemin Erbas
Vice-President, SMA Belgium

Eva Stumpe
Treasurer, Deutsche Gesellschaft für Muskelkranke, Germany

Marie-Christine Ouillade
AFM Téléthon, France

Olga Germanenko,
SMA Family Foundation Russia





Staff

Nicole Gusset
CEO

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Operations Manager

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Laura Gumbert
Healthcare System and Access Manager

Carolina Pachecoy
Operations Support Manager

Alice Larotonda
Community Research and Education Manager

Anjani Kalra
Outcome Research Manager

Anna Motyl
Freelance Research Officer

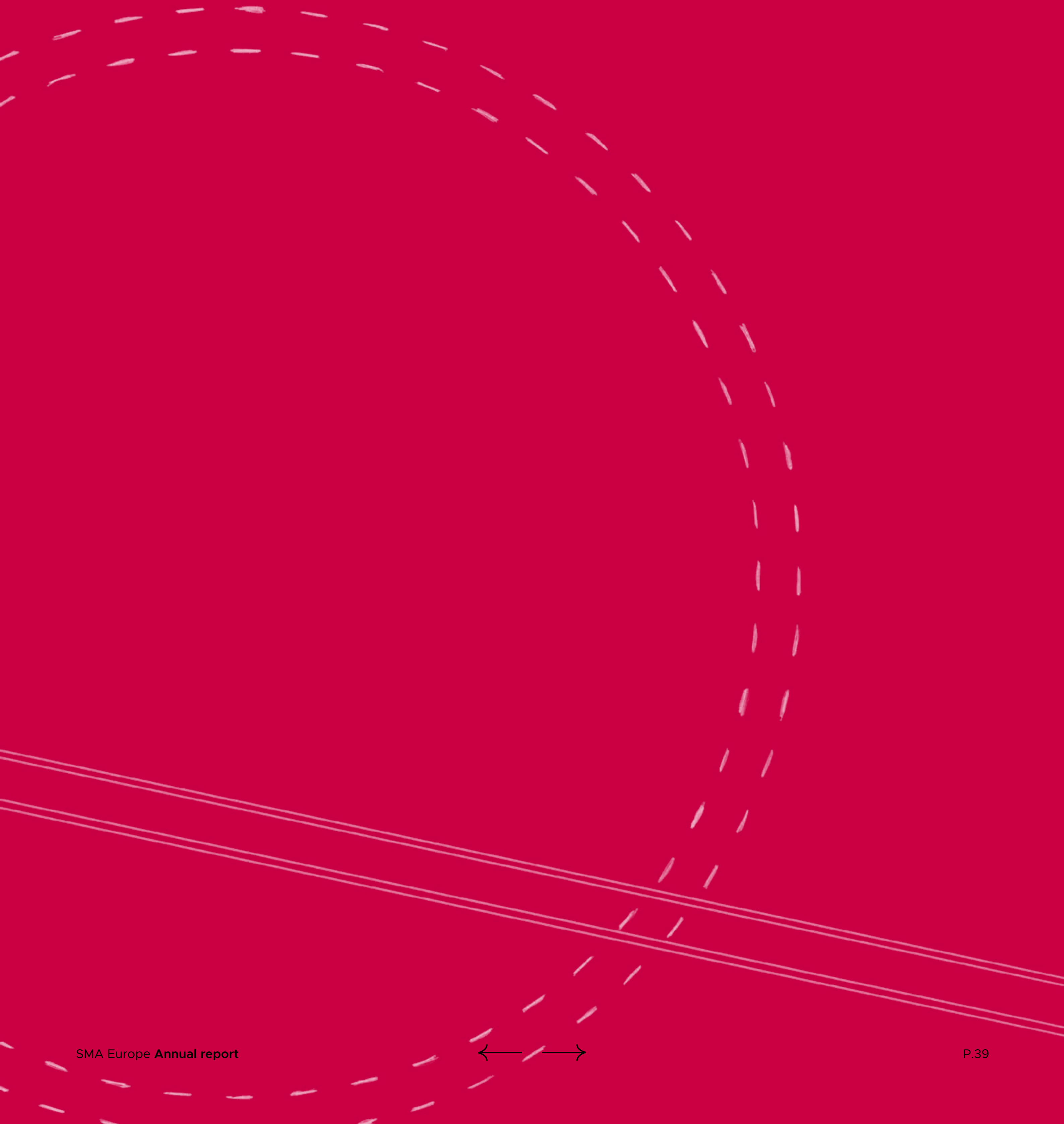








OUR COMMITTEES



Our committees

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 Véronique van Asche.
Link to the website

EMA Committee

Group of experienced patient experts, who engage in bringing the voice of the community to EMA committees and meetings.
The EMA Committee is chaired by Mencía de Lemus.

SMA Europe Treatment Committee

Group of experienced patient experts who engage in independent discussions on treatment related topics to support the development of news, positions and publications. In 2023, the Treatment Committee had Standing Committees and/or Advisory Boards with Biogen, Novartis Gene Therapies, Roche and Scholar Rock.
The Treatment Committee is chaired by Thomas Doktor, PhD.
Link to the website

Communications Working Group

The Communications Working Group is composed of patient advocates who live with SMA. The group provides perspectives and advice on the communications activities of SMA Europe and ensures that the actions are aligned with the needs and expectations of our SMA community.

OUR PUBLICATIONS AND OUTREACH

Journal articles

Authors Aponte Ribero, V., Martí, Y., Batson, S., Mitchell, S., Gorni, K., **Gusset, N.**, Oskoui, M., Servais, L., & Sutherland, C. S. (2023a).

Title **Systematic literature review of the natural history of spinal muscular atrophy.**

Source Neurology, 101(21)
https://doi.org/10.1212/wnl.0000000000207878

Authors König, L. M., Krukowski, R. A., Kuntsche, E., Busse, H., **Gumbert, L.**, Gemesi, K., Neter, E., Mohamed, N. F., Ross, K. M., John-Akinola, Y. O., Cooper, R., Allmeta, A., Macedo Silva, A., Forbes, C. C., Western, M. J.

Title **Reducing intervention- and research-induced inequalities to tackle the digital divide in health promotion.**

Source Int J Equity Health 22, 249 (2023)
https://doi.org/10.1186/s12939-023-02055-6

Authors Senn, K.C., Thiele, S., **Gumbert, L.**, Krause, S., Walter, M.C., Nagels, K.H.

Title **Inclusion body myositis—health-related quality of life and care situation during phases of the “patience journey” in Germany: results from a qualitative study.**

Source Health Qual Life Outcomes 21, 111 (2023)
https://doi.org/10.1186/s12955-023-02196-w

Authors **Gumbert, L., Erbas, Y.**, Schmieder, F. **Gusset, N.**

Title **The OdySMA initiative — an online tool for people living with Spinal Muscular Atrophy (SMA), patient advocacy groups, and decision-makers to identify and overcome barriers regarding access to treatment and care.**

Source Orphanet Journal of Rare Diseases 2023: P38 in: Meeting abstracts from the 11th edition of the European conference on Rare Diseases & Orphan Products (ECRD) 2022. Orphanet J Rare Dis 18 (Suppl 1), 118 (2023)
https://doi.org/10.1186/s13023-023-02707-4

Authors Harrington, A., Corazza, A., Arnold, P., **Gusset, N., Ouillade, M.-C.**, Kelly, S., Pistollato, M., Jones, C.

Title **Addressing inequalities in SMA care: a SMA European policy and access tracker.**

Source Orphanet Journal of Rare Diseases 2023: P20 in: Meeting abstracts from the 11th edition of the European conference on Rare Diseases & Orphan Products (ECRD) 2022. Orphanet J Rare Dis 18 (Suppl 1), 118 (2023)
https://doi.org/10.1186/s13023-023-02707-4

Journal articles

Authors	Clay, I., Peerenboom, N., Connors, D. E., Bourke, S., Keogh, A., Wac, K., Gur-Arie, T., Baker, J., Bull, C., Cereatti, A., Cormack, F., Eggenspieler, D., Foschini, L., Ganea, R., Groenen, P. M., **Gusset, N.**, Izmailova, E., Kanzler, C. M., Leyens, L., … Hoffmann, S. C. (2023).
Title	**Reverse engineering of digital measures: Inviting patients to the conversation.**
Source	Digital Biomarkers, 28–44. https://doi.org/10.1159/000530413

Authors	Aponte Ribero, V., Martí, Y., Batson, S., Mitchell, S., Gorni, K., **Gusset, N.**, Oskoui, M., Servais, L., & Sutherland, C. S. (2023).
Title	**Systematic literature review of the natural history of spinal muscular atrophy.**
Source	Neurology, 101(21). https://doi.org/10.1212/wnl.0000000000207878

Authors	Walter, M. C., Laforêt, P., van der Pol, W. L., Pegoraro, E., Attarian, S., Bartels, B., Gorni, K., Goemans, N., **Gusset, N.**, Hodgkinson, V., Hagenacker, T., Kirschner, J., Klein, A., Kostera-Pruszczyk, A., Lochmüller, H., Marini-Bettolo, C., Mercuri, E., Muni-Lofra, R., Ouillade, L., … Vissing, J. (2023).
Title	**254th ENMC International Workshop. formation of a European network to initiate a European data collection, along with development and sharing of treatment guidelines for adult SMA patients. Virtual meeting 28 – 30 January 2022.**
Source	Neuromuscular Disorders, 33(6), 511–522. https://doi.org/10.1016/j.nmd.2023.03.011

Authors	Rovira-Moreno, E., Abulí, A., Muñoz-Cabello, P., Codina-Solà, M., Baillès, E., **de Lemus, M.**, Darras, B. T., & Tizzano, E. F. (2023).
Title	**The diagnosis communication process in spinal muscular atrophy: A cross-cutting view of the new challenges facing the therapeutic era.**
Source	Genetics in Medicine Open, 1(1), 100825. https://doi.org/10.1016/j.gimo.2023.100825

Posters

Authors **Christie-Brown, V.**, Staley, K., Kinsella, S., Muni-Lofra, R., Mayhew, A., Seliverstov, Y., Pattni, J., Howarth, K., Nordstrom, M., **Van Assche, V., Popova, J.**, Velkovski, J., Meyers, M., **Germanenko, O.**, Schwersenz, I., **Rucinski, K., van Rijswijck, H., Pachecoy, C., Erbas, Y. & Gusset, N.** (2023).

Title **Identifying the top 10 unanswered research priorities for spinal muscular atrophy. SMA Europe, Freiburg, Germany.**

Poster presentation Annual SMA Research & Clinical Care Meeting, Orlando, Florida, USA.

Authors Tizzano, E., **Christie-Brown, V.**, Baranello, G., **Germanenko, O.**, Gray, A., Krstic, M., Lilien, C., Patel, H., Servais, L., Scoto, M. (2023).

Title **Clinical trial readiness for spinal muscular atrophy: experience of an international educational-training initiative.**

Poster presentation Annual SMA Research & Clinical Care Meeting, Orlando, Florida, USA.

Authors **Christie-Brown, V.**, Motyl, A., Cuesta, M., Krstic, M., Khosla, S., **Gusset, N.**, Broekgaarden, R. (2023).

Title **SMA clinical trial readiness in Europe.**

Poster presentation Annual SMA Research & Clinical Care Meeting, Orlando, Florida, USA.

Authors **Gumbert, L., Erbas, Y., Gusset, N.** (2023).

Title **Odysma - a quest to access: an SMA Europe advocacy tool.**

Poster presentation Annual SMA Research & Clinical Care Meeting, Orlando, Florida, USA.

Authors **Gusset, N. & Erbas, Y.** (2023).

Title **A decision for life - pathway to treatment decisions in newly diagnosed families with spinal muscular atrophy.**

Poster presentation Annual SMA Research & Clinical Care Meeting, Orlando, Florida, USA.

Authors **Erbas, Y., Larotonda, A.**, Vedernikova, E., Jongerling, J., Gusset, N. (2023).

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

Poster presentation Annual SMA Research & Clinical Care Meeting, Orlando, Florida, USA.

Posters

Authors	**Erbas, Y. & Gusset, N.** (2023).
Title	**The need for evidence-based treatment decisions in spinal muscular atrophy type 0.**
Poster presentation	Annual SMA Research & Clinical Care Meeting, Orlando, Florida, USA.

Authors	**Erbas, Y.**, Vedernikova, E., **Kalra, A., Gusset, N.** (2023).
Title	**Eupesma-2021: European patient experience survey in SMA to understand patient expectations towards physiotherapy and nutrition.**
Poster presentation	Annual SMA Research & Clinical Care Meeting, Orlando, Florida, USA.

Authors	Proud, C.M., Mercuri, E., Finkel, R.C., Kirschner, J., De Vivo, D.C., Muntoni, F., Saito, K., Tizzano, E.F., Desguerre Roy, I., **Gusset, N.**, Benguerba, K., Raju, D., Faulkner, E., Servais, L. (2023).
Title	**Combination disease-modifying treatment in spinal muscular atrophy: a proposed classification for future analyses.**
Poster presentation	Annual SMA Research & Clinical Care Meeting, Orlando, Florida, USA.

Authors	Servais, L., Day, J.W., De Vivo, D.C., Kirschner, J., Mercuri, E., Muntoni, F., Proud, C.M., Shieh, P.B., Tizzano, E.F., Quijano-Roy, S., Desguerre, I., Saito, K., Faulkner, E., Benguerba K.M., Raju, D., LaMarca, N., Sun, R., **Gusset, N.**, Anderson, F.A., Finkel, R.C. (2023).
Title	**Real-world Outcomes in Patients with Spinal Muscular Atrophy Treated with Onasemnogene Abeparvovec as Monotherapy: Findings from the RESTORE Registry.**
Poster presentation	Annual SMA Research & Clinical Care Meeting, Orlando, Florida, USA.

Authors	Petridis, F., **Gumbert, L., Van Assche, V.**, Kavanagh, L., Abdelnour, R., Gorni, K., **Gusset, N.** (2023).
Title	**Benchmarking care for adults living with spinal muscular atrophy (SMA) in Europe.**
Poster presentation	9th Congress of the European Academy of Neurology, Budapest, Hungary.

Posters

Authors	Servais, L., Martí, Y., Batson, S., Mitchell, S., Aponte Ribero, V., Gorni, K., Oskoui, M., **Gusset, N.**, Mercuri, E., McGrattan, K., Deconinck, K., Sutherland, CS. (2023).
Title	**Respiratory outcomes associated with the natural history of patients with Types 1–3 spinal muscular atrophy (SMA).**
Poster presentation	15th European Paediatric Neurology Society (EPNS) Congress, Prague, Czech Republic.

Authors	Martí, Y., Servais, L., Aponte Ribero, V., Batson, S., Mitchell, S., Gorni, M., **Gusset, N.**, Oskoui, N., Sutherland, CS. (2023).
Title	**Motor function outcomes associated with the natural history of patients with Types 1–3 spinal muscular atrophy (SMA).**
Poster presentation	15th European Paediatric Neurology Society (EPNS) Congress, Prague, Czech Republic.

Authors	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., & Nam, J. (2024).
Title	**Delphi consensus panel on assessing clinically meaningful treatment outcomes in adults living with spinal muscular atrophy (SMA) (P2-11.007).**
Source	Neurology, 102(17_supplement_1) https://doi.org/10.1212/wnl.0000000000205716

Presentations Workshops & Others:

Participants	**Ouillade, M-C.** (2023, February).
Title	**Time to act: Building a pharmaceutical framework fit for rare diseases.**
Event	European Parlament Conference. Brussels, Belgium.

Participants	**Van Asche V., De Lemus, M. & Gusset, N.** (2023, April).
Title	**Novel Treatments in SMA - workshop.**
Event	EPFL, Lausanne, Switzerland

Participant	**Gusset, N.** (2023, May).
Title	**Gene Therapies – A wave is coming - are health systems ready? Challenges of SMA patients in Europe and Beyond.**
Location	Side event 76th World Health Assembly, WHO, Geneva, Switzerland

Participant	**Germanenko, O.** (2023, May).
Title	**Gene Therapy webinar - the SMA Case study.**
Event	Beacon Webex.

Participants	**Gumbert, L., Erbas, Y., Gusset, N.** (2023, June).
Title	**OdySMA – a quest to access: an SMA Europe advocacy tool.**
Event	Digital Divide in Health Promotion, International Expert Workshop, University of Bayreuth/Campus Kulmbach, Germany.

Participant	**Gusset, N.** (2023, June).
Title	**Access to Medicines – The Role of Patient Advocacy.**
Event	Cure SMA International Patient Advocacy Group Meeting. Orlando; USA.

Participant	**O'Grady, J.** (2023, August).
Title	**Health technology assessment of the addition of spinal muscular atrophy (SMA) to the National Newborn Bloodspot Screening Programme.**
Event	Expert advisory group. Zoom meeting.

Presentations Workshops & Others:

Participants	**Erbas, Y., & Rucinski, K.** (2023, September).
Title	**Meeting with Commissioner in Autumn 2023 together with other participating organisations: Screen4Rare and Eurordis.**
Event	European Parliament. Brussels; Belgium.

Participants	**European Alliance for Newborn Screening in Spinal Muscular Atrophy** (2023, September).
Title	**Open Letter: "Resolving health inequalities in newborn screening across Europe."**
Location	Brussels, Belgium.

Media Coverage

Title **Letters to my Younger Self: Besnike Mustafi**
Source Rare Youth Revolution, 21 February 2023
https://www.rareyouthrevolution.com/post/letters-to-my-younger-self-besnike-mustafi

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Title **Are we there yet?" Designing communication initiatives based on community needs – the Comms Working Group at SMA Europe**
Source Rare Youth Revolution, 15 March 2023
https://rarerevolutionmagazine.com/are-we-there-yet-designing-communication-initiatives-based-on-community-needs-the-comms-working-group-at-sma-europe/

Title **Rare Odyssey**
Source Rare Revolution Magazine, 15 April 2023
https://editions.rarerevolutionmagazine.com/html5/reader/production/default.aspx?pubname=&edid=96d21e11-98ad-4cd1-9c24-0022245bef9d

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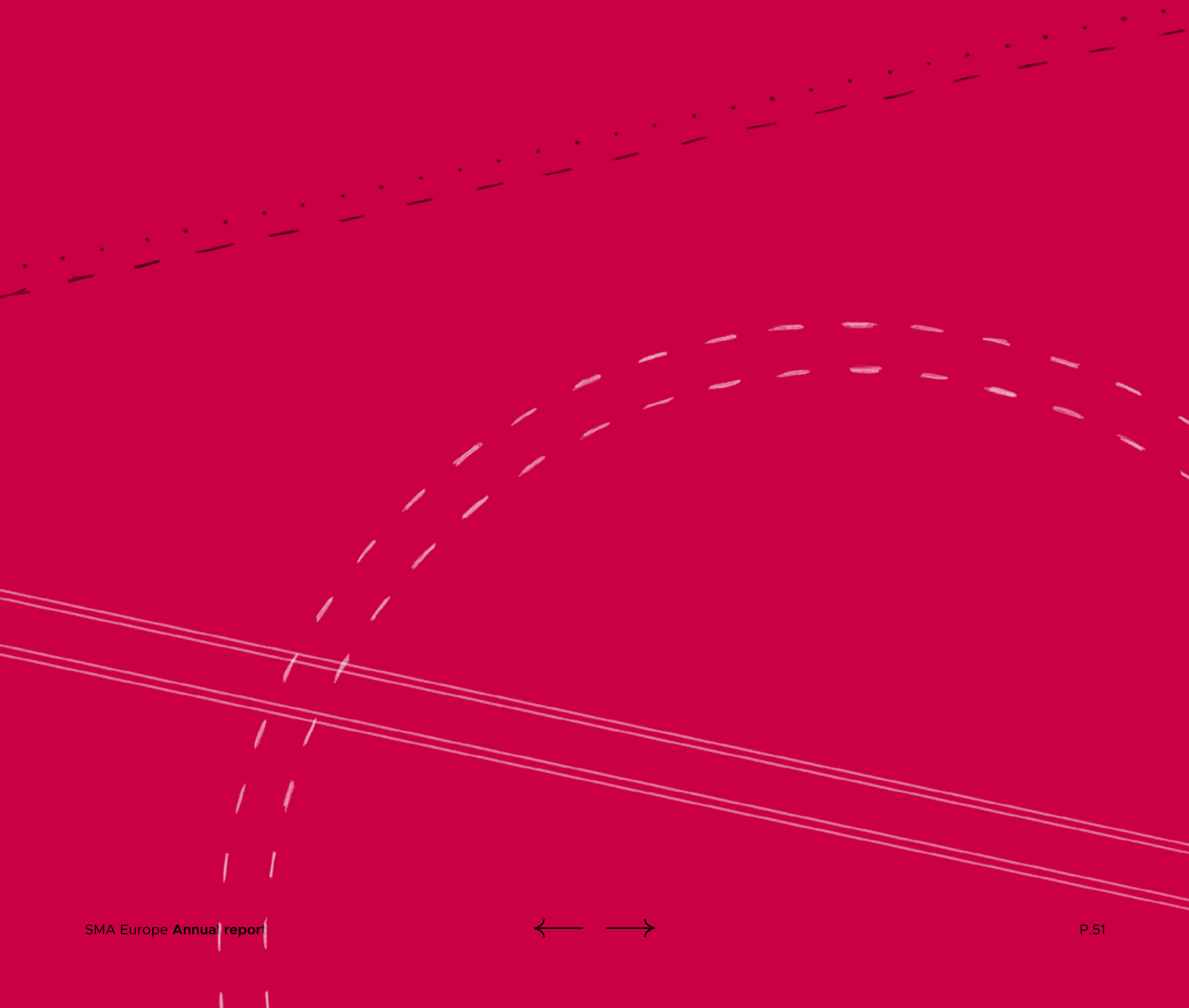
Title **Запрещенный прием**
Source Kommersant, 8 September 2023
https://www.kommersant.ru/doc/6196965

Title **I am Unique**
Source Rarity Life Magazine, 12 December 2023
https://www.samebutdifferentcic.org.uk/raritylife

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 2023:

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

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), Germany

Muscular Dystrophy Association Hellas (MDA), Greece

SMA Foundation, Hungary

FSMA, Iceland

SMA Ireland

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

Asociaţia SMACare, Romania

SMA Family Foundation Russia

SMA Serbia

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

SMA Europe's Partner Organisations

Cure SMA, US

Cure SMA India

EAMDA

European Medicines Agency (EMA)

ENMC

EURO-NMD

Eurordis

SMA Foundation, US

Treat-NMD

Industry Partners

Asuragen (Bio-Techne)

Biohaven

Biogen

ImmunoIVD

LaCAR MDX Technologies

Novartis Gene Therapies

Roche

Scholar Rock

Other Partners

Acumen

Admedicum

Dipl. -Kfm. A. Pfeuffer

Dreaming Fish

Elena Díez Hernández

Gardner Puzelli Media

Krishna Consultancy

MC Copigrafia

Newus

Patvocates

TwoCan Associates

Volition Ltd


CHRISTIAN PFEUFFER
INITIATIVE SMA
MEMBER OF SMA EUROPE
PATIENT ORGANISATION
SMA EUROPE

OUR FINANCES

Funds In
1,232,280.13 €

Funds Out
1,187,013.98 €

Revenue Sources	**Amount**
Corporate Funding	**468,750.00 €**
Asuragen	5,000.00 €
Biogen	154,940.00 €
Biohaven	1,000.00 €
ImmunoIVD	5,000.00 €
LaCAR MDX	8,000.00 €
Novartis Gene Therapies	88,500.00 €
Roche	195,340.00 €
Scholar Rock	10,970.00 €
Patient Organisations	**229,000.00 €**
Services & Event Fees	**341,060.94 €**
Donations & Grants	**188,823.48 €**
Miscellaneous	**4,645.71 €**
Total Revenue	**1,232,280.13 €**

Funds In

1,232,280.13 €

Funds Out

1,187,013.98 €

Expenses	**Amount**
Direct Project Expenses	**1,024,083.54 €**
Operating Expenses	**162,930.44 €**
Staff	109,009.14 €
Volunteers	9,568.64 €
Logistics	16,971.98 €
Materials	127.98 €
Service and Event Fees	21,252.70 €
Other Operating Expenses	6,000.00 €
Total Revenue	**1,187,013.98 €**

Design and Art Direction: Elena Díez | elenadiez.es

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