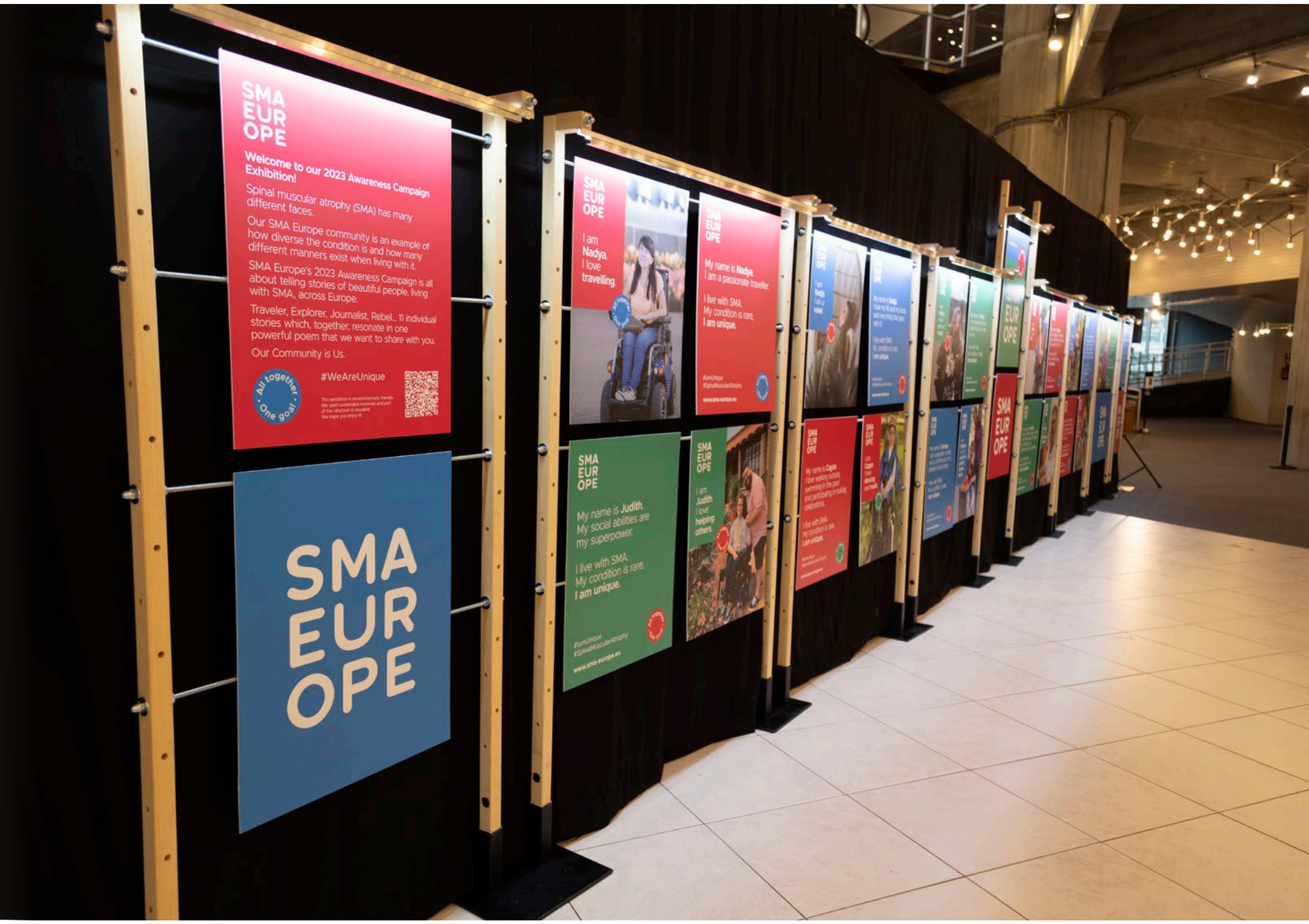
The Power of **COLLABORATION**

SMA Europe's 4th Scientific International Congress on Spinal Muscular Atrophy

In March 2024, SMA Europe hosted its 4th Scientific International Congress on Spinal Muscular Atrophy (SMA) in Ghent, Belgium, which brought together attendees from 6 continents. The congress served as a beacon of hope and unity for the global SMA community, its significance reaching far beyond geographical boundaries and touching the lives of SMA patients, families, clinicians, researchers, advocates and other stakeholders worldwide. The congress provided a unique opportunity for the SMA community to come together, share knowledge and experiences, foster collaboration, and advance efforts to improve the lives of those living with SMA.

We spoke with three dedicated SMA advocates who attended the 4th Scientific International Congress on SMA, as well as the inaugural Global SMAdvocacy event about both their personal SMA journeys and their hopes for the upcoming events. Although their lives might be different they each share a common goal - to improve awareness, access, care and research for SMA.

Yasemin is Vice President of SMA Europe. *"What is special about SMA Europe is that we're all extremely passionate patient advocates, we all come from different countries. Everyone is extremely motivated to fight for the same cause, and they all bring their expertise and*



Photograph by Ceridwen Hughes

their own experiences. This makes us unique, we have this wealth of knowledge within our organisation. We are an extremely strong organisation. To me, SMA Europe means empowerment. It means bringing people together from all over the world to elevate the patient's voice. I think we are going to achieve great things for the SMA community."

Yasemin's SMA journey began back when her son was diagnosed nine years ago, when he was just one month old, and they had been seen at the hospital for some tests. A neurologist with over thirty years of experience expressed 'concern that their son had SMA type 1' but that she was still waiting for the results to come back. *"The first thing that I asked the neurologist was, are there any clinical trials for him? Is there anything that we can try? Maybe try a new treatment or something?"*

The concern for the neurologist was that Yasemin's son was only one month old and they didn't expect him to live much longer than six months. The young family swung between the fear of losing him and the hope that there would be a clinical trial to join. *"It was a very turbulent time. Weeks passed before we got the confirmation that he had SMA, but that he could start a trial as well."*

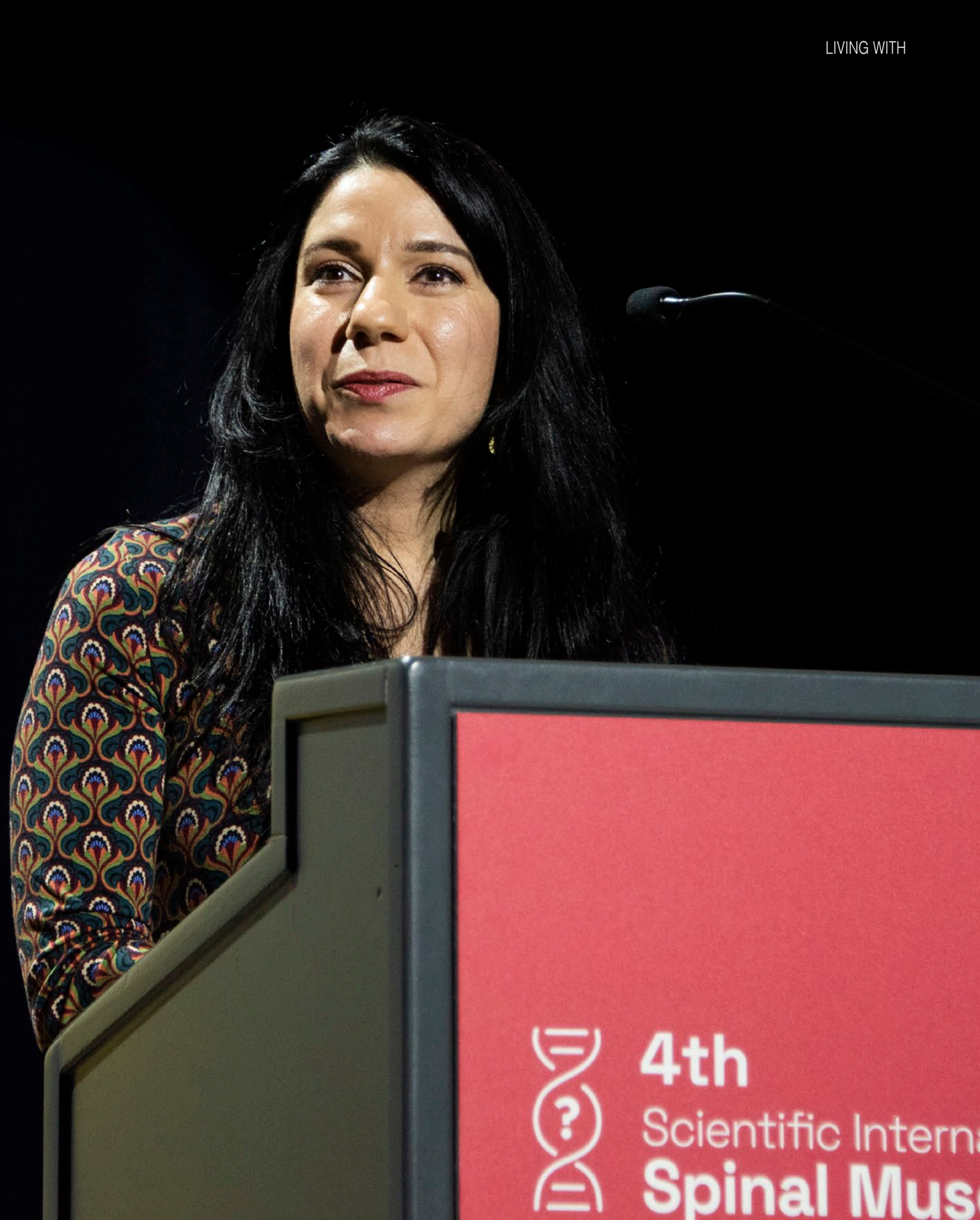
The clinical trial was an important step for them, and the improvements to his health had a huge impact on their son. *"For us, the trial meant the difference between life and death. I think without that my son would not be alive today. He was doing extremely well when on the trial and the treatment he was receiving was a true miracle. He was almost sitting up by himself which for a type 1 child is rare."*

Just before his fourth birthday he was hospitalised, he needed intensive care and was intubated. It was a difficult time for the family and Yasemin had to battle to get the doctors to listen. *"That was the time when I thought I need to be a patient advocate, his treating doctors had given up on him. They decided to move him to a different hospital where they found a pulmonary doctor who listened to them and agreed to try to help."* Eventually, they were able to take him home. *"Our son is full of life. The doctors who did not want to give my son the tracheostomy told us that he would never be able to speak, but he has proven the opposite; he is literally talking all day. I think he is a very happy child, he knows his limits."*

This period was a turning point for Yasemin, and she knew that she needed to challenge the way people view disability and the clinical way the quality of life is viewed. *"There is so much that SMA patients can do, some are living full lives. I met someone online, similar to my son, who also couldn't move a finger. He had a tracheostomy and a ventilator, but he did these incredible road trips with his personal assistants to places like Iran, Kazakhstan, Turkmenistan, and Russia. The things he did were so amazing, and they were things that I, with my functioning body, did not dare to do. This empowered me to fight for our son."*

"I don't know what it is like to live with SMA. If someone tells me that an individuals quality of life is going to be low with a ventilator, who am I to say any different? But, if a person who is living that life tells me that life is beautiful, meaningful and worth it, then they are the only ones who can tell us what is good or not."

Today the importance of having patient organisations like SMA Europe cannot be underestimated in terms of peer support, advocacy and having somewhere to turn for patients, families and supporters. *"It's only through patient organisations that*



Photograph of Yasemin courtesy of SMA Europe

we can collectively elevate the patient's voice. Once we all come together, that's how we grow strong and how we can really make a change."

At the inaugural Global SMAdvocacy event everyone will have a voice, and the hope for Yasmin is that collectively they can achieve much together. *"It's going to be the first time that so many patient advocates from all over the world are going to meet. We know that there are certain things that we can only achieve together, and I hope that we will become closer as an SMA family. That collectively we can become extra motivated to join forces, working together to reach our goals. We know things work differently in different countries, but in the end, by working together, I think we can reach these goals."*

"The treatments that we have today have completely changed the course of the disease for the people who can access them. SMA does not have to be a death sentence, it does not have to be a progressive disease. While we are extremely lucky that we have these treatments, it does not mean that we are there yet. We need research. It's important for us that we keep researchers in the field of SMA. It's also why we are organising this 4th Scientific International Congress on SMA to bring the researchers, clinicians, pharmaceutical companies and the different stakeholders together. Because I believe that it is only by coming together, by talking to each other face to face, by brainstorming together, by being creative together, that it is only then that we will be able to progress."

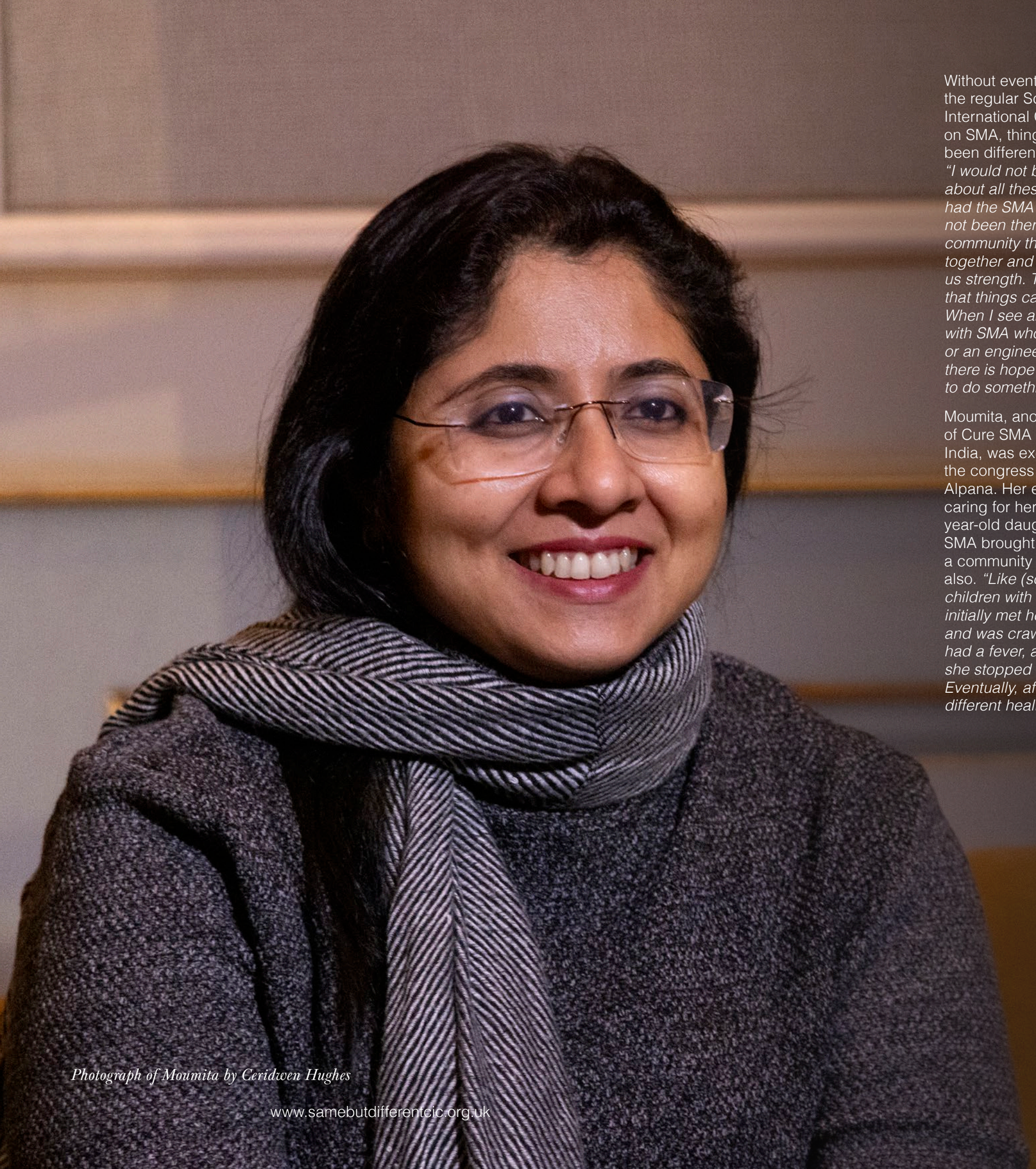
Progress is something that Alpana, a founder and director of patient advocacy at the brilliant Cure SMA Foundation of India, wants for her son, and for all the children in India where healthcare isn't as accessible for many.

"My eleven-year-old son was diagnosed with SMA type 2. We had decided to get some tests for him as he was missing milestones. We got the SMA suggestion from one of my brother-in-law's friends who is a neurologist. It came back as positive and our world came crashing down as a result. My family has a medical background and knew the diagnosis and prognosis of this disease, they told me I needed to be strong for my child. For almost a year, I used to cry and drench my pillows with tears, I went through a very dark phase."

The support of her family made the difference to Alpana and with the encouragement of her brother she attended her very first SMA Conference in Washington DC. This was a real turning point for her. *"Seeing individuals with SMA doing well, with a better quality of life, was a real contrast to what I knew. It was the hope I got then that was the most important thing."*

Inspired, she returned home and set up a Facebook page, hoping to connect with other people from her own country. From those first tentative steps on Facebook ten years ago she, along with other mothers, has helped to build Cure SMA Foundation of India into a vibrant organisation, that has achieved a huge amount for the SMA community nationally. For Alpana attending both the 4th Scientific International Congress on SMA, as well as the inaugural Global SMAdvocacy event, offers many benefits; including the unique opportunity to be in a room full of like-minded people to brainstorm and talk. *"This is truly a global advocacy event covering so much. Reimbursement for treatment is one topic, for example, where we can learn from seeing how other low and middle-income countries have achieved that. We can take this knowledge back to India and it can have a huge impact for us."*

Photograph of Alpana by Ceridwen Hughes



Photograph of Moumita by Ceridwen Hughes

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Without events like the regular Scientific International Congresses on SMA, things may have been different for Alpana *"I would not be here talking about all these things had the SMA community not been there. It is the community that has come together and given all of us strength. There's hope that things can be done. When I see an individual with SMA who's a lawyer, or an engineer, I know there is hope for my son to do something similar."*

Moumita, another founder of Cure SMA Foundation of India, was excited to attend the congress alongside Alpana. Her experiences caring for her thirteen-year-old daughter with SMA brought the need for a community into her life also. *"Like (some) other children with SMA, she initially met her milestones, and was crawling until she had a fever, after which she stopped crawling. Eventually, after visiting different health care centres someone mentioned that it may be a genetic condition. At that time in India genetic tests were not very common. We went to another city for the genetic testing and we got the report with the diagnosis, and the information that SMA was not curable, and that she may not live long."*

"The neurologist told me that she would live for two or three years. I felt numb. My only focus was my daughter, her quality of life and what I, as a mother, could do. I was not ready to give up. I thought 'she may not live long but I will do my best to give her the life that a child should get.'" This determination has driven Moumita onwards, fighting for awareness, inclusivity and more treatments for SMA.

"I was introduced to a doctor who was trained in the UK, at Great Ormand Street Hospital, one of the biggest centres for neuromuscular disorders. She gave me hope, she taught me that it's not important how long she lives for, it is important what quality of life she's living." The communication between doctors in India and the UK helped Moumita and her family immeasurably. *"Slowly our community was created, and, after meeting Alpana and the others on social media, I felt like I was not alone. I had someone ask questions about our child. That is the beauty of a community, that we have someone to guide us through."*

Today her daughter is doing well, and her family have helped build her confidence and zest for life. *"She is very confident, very lively and she has a lot of hope. She wants to study biology, she wants to be a class teacher, she has high hopes of living fully and we have the hope to help her to achieve her hopes and dreams."*

There's still much progress to be made in India, but the national SMA community has achieved a great deal already. *"Initially we had no treatment for this very progressive condition for our daughter, but thankfully we got free access to treatment when she was eleven years old. However it's temporary as we don't know if we will get reimbursement, so access could stop. Treatment access is very important for the stability of the condition."*

"Accessibility is also a barrier. Awareness has increased, but we are a developing country with a huge population and so we have a lot of financial constraints. The Government is trying to make things more accessible, and we're being vocal about improvements for our children, so I believe things will move fast."

For Moumita, the inaugural Global SMAdvocacy event is one of community and mutual interests. It is so important for advocacy that global ideas, technologies, treatments and research are shared to help provide better outcomes worldwide for SMA patients.

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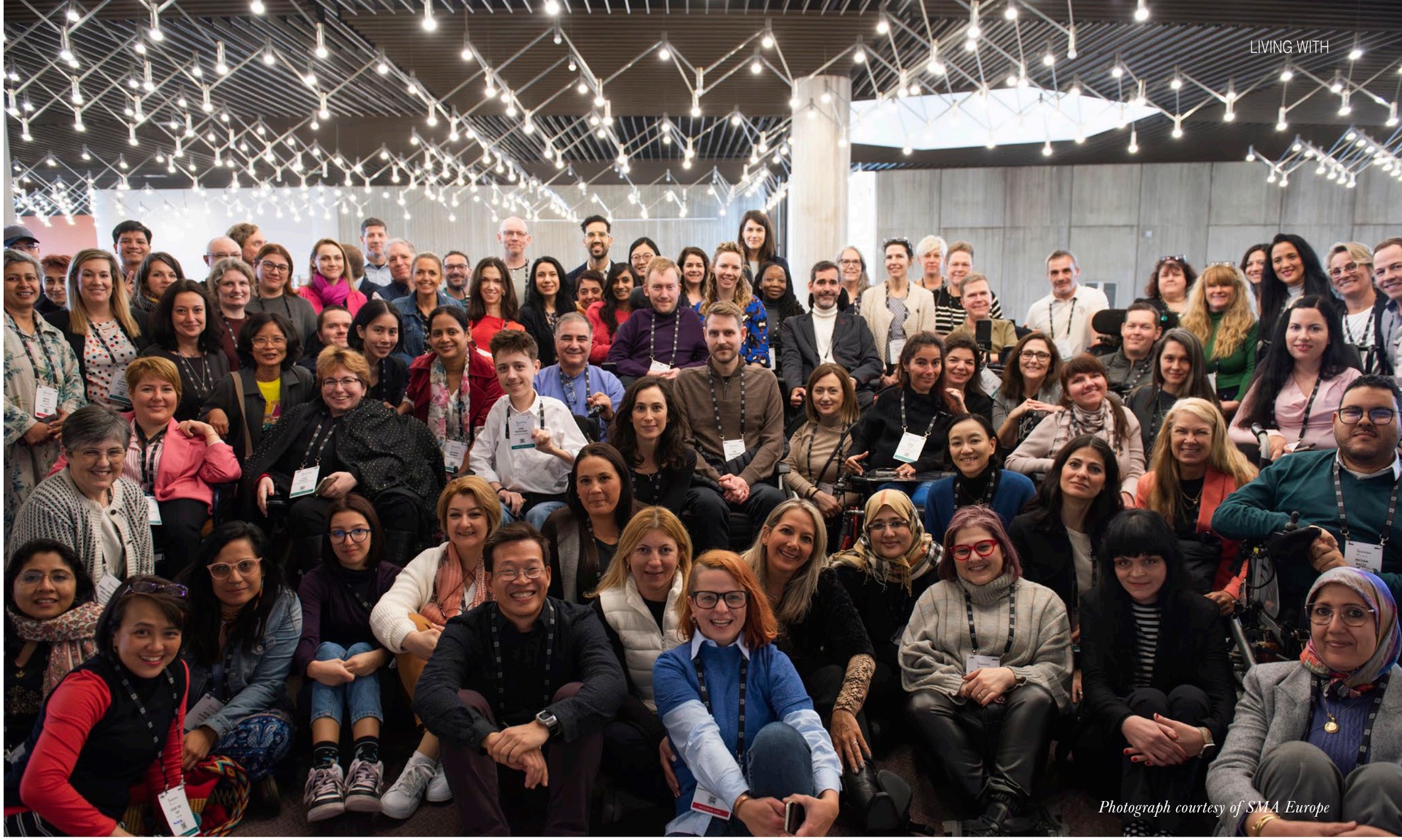
"Ten years back the concept of advocacy was almost not there. But since we started and had exposure to other international organisations, we learned the importance of advocacy in terms of improvement of infrastructure, access to treatment and policy changes. When we meet people from different countries, from different political, and economic situations, we get ideas about how to advocate in our own countries for faster accessibility and availability of treatment."

"India is more in line with the European medical protocols, so when we take back ideas from Europe to our country it's more acceptable to the medical community. The medical community then has the strength to advocate to the policymakers. When we started the organisation, there was nothing for the SMA community in India, and today we have eight to nine centres that SMA patients can access. We have been able to influence the policymakers so that at least one treatment is approved in India and they're focusing on research, which is a big achievement."



Photograph courtesy of SMA Europe

The inaugural Global SMAdvocacy event is aimed at being a platform for the exchange of knowledge and ideas, fostering collaboration and innovation in the pursuit of a cure for SMA. Bringing people together to create a unique environment of shared understanding and common purpose, it is truly a testament to the power of collective action. Despite the diverse backgrounds and circumstances of the participants, they are united in their fight against SMA. They are not just a community; they are a family. Each member, whether a patient, a caregiver, or a supporter, contributes to the strength and resilience of this family.

SMA Europe's 4th Scientific Congress on SMA too serves as a symbol of hope, unity, and determination. It embodies the positivity and common goals of the global SMA family, highlighting the power of collaboration in the fight against SMA. It is a reminder that together, they are stronger, and together, they can make a difference.

About SMA

SMA is a rare genetic and progressive neuromuscular condition occurring in approximately 1 in 6,000 to 10,000 live births.

Characterised by degeneration of nerve cells in the spinal cord (motoneurons), SMA leads to progressive muscle weakness and atrophy. SMA has many faces. SMA is characterised by a wide spectrum of how severely children and adults are affected. The symptoms vary from person to person. SMA may affect daily activities such as breathing, eating, hugging, grabbing, nodding, sitting and walking.

* Information based on the 'About SMA' section of the SMA Europe website.

Contact Information

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