

2023

# International Patient Advocacy Group Meeting



# ACCESS TO MEDICINES

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## The Role of Patient Advocacy

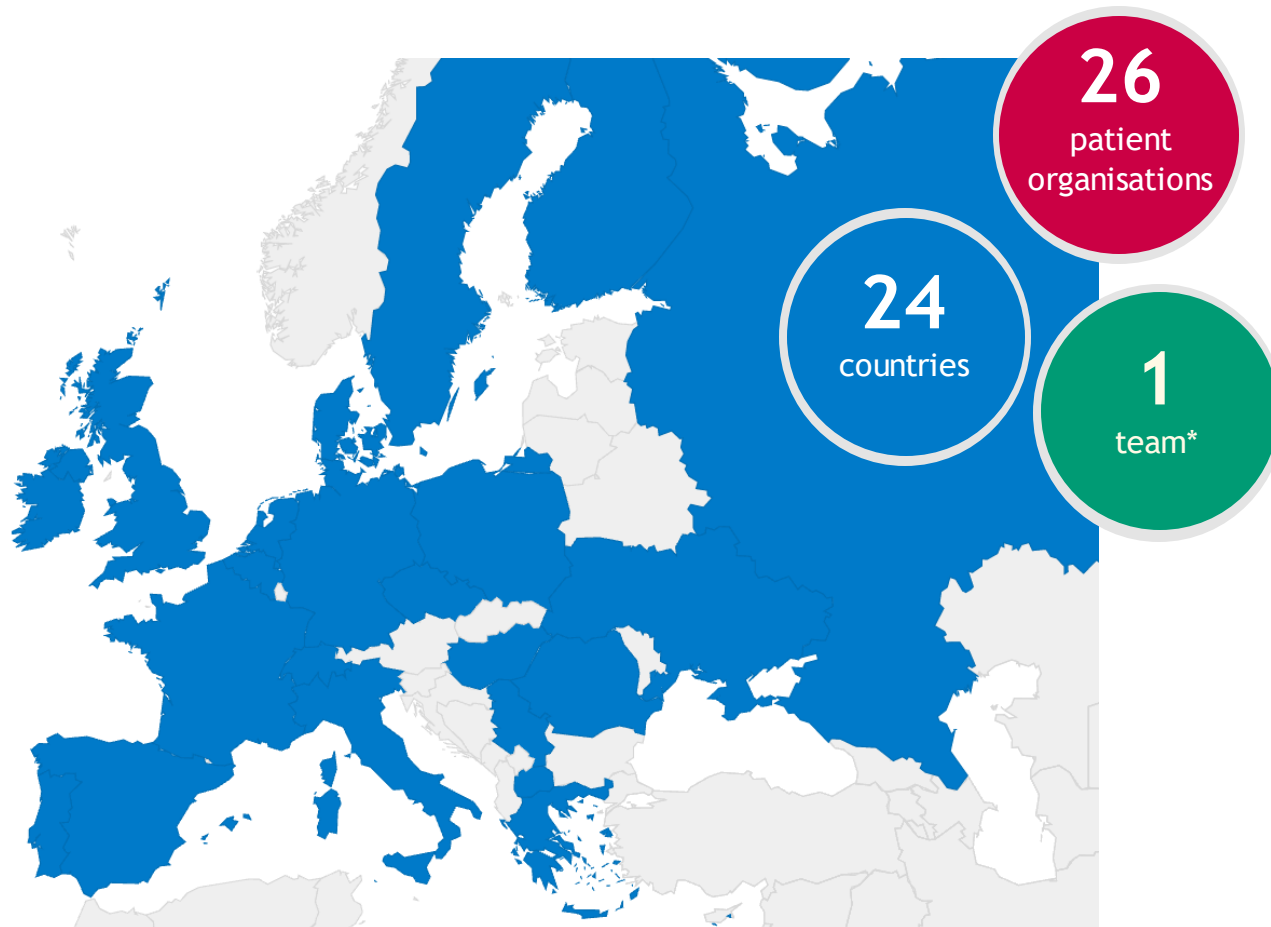
Dr. Nicole Gusset  
President & CEO, SMA Europe

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OPE**

## Who is SMA Europe?



- Umbrella organisation of SMA patient and research organisations from across Europe.
- Founded in 2007
- Delegates of our member organisations form the general assembly. They are volunteers and often living with SMA. They together with our Board, Staff and other Volunteers manage our projects in the areas:
  - Research,
  - Therapy & Care,
  - Healthcare Systems, Policy & Access,
  - Capacity Building, Communications & Outreach



\*many volunteers, 44 Delegates, 5 Board Members, 8 Staff

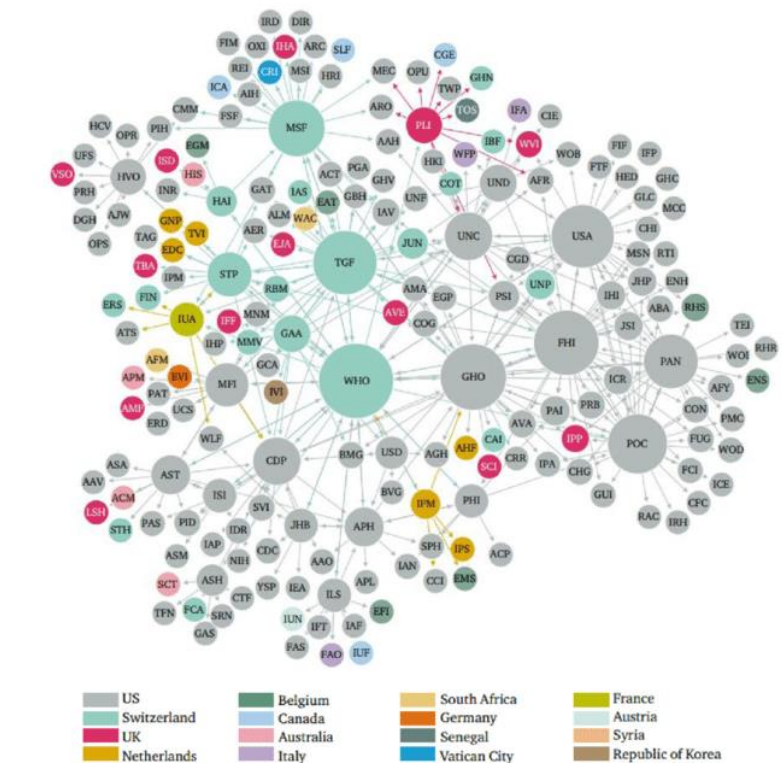


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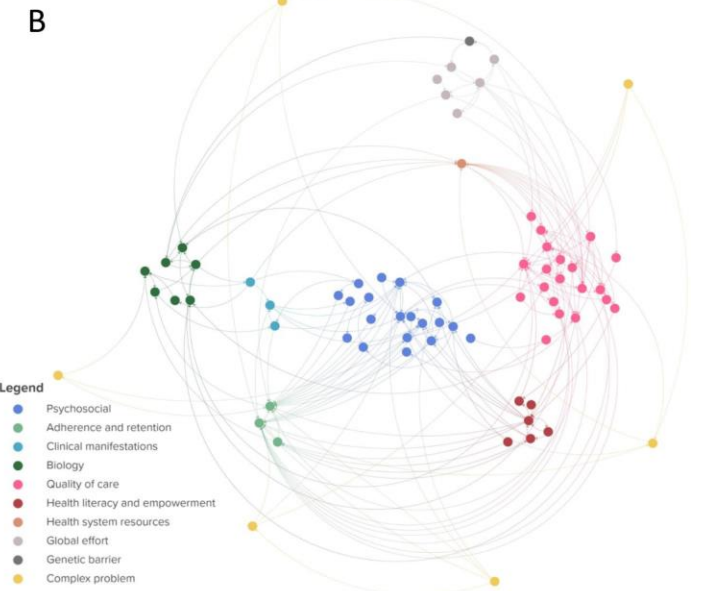
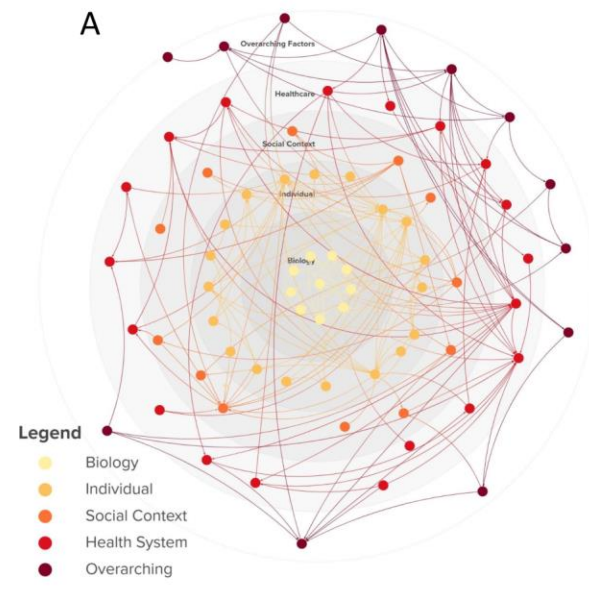
**What can we, as patient advocates, do  
to improve the situation,  
to work towards equitable access for all  
to ensure that no one is left behind?**

# Background: Health Systems

complex, slow, resources-intensive and not ready for innovative treatments for SMA?

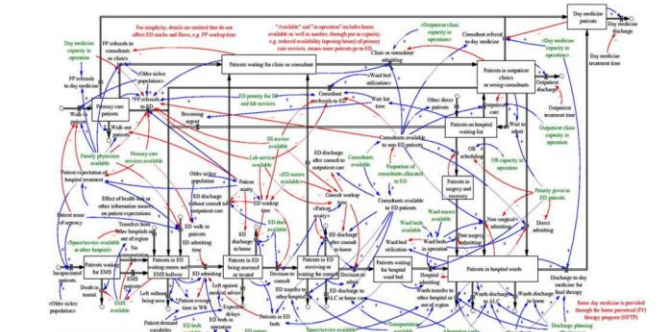
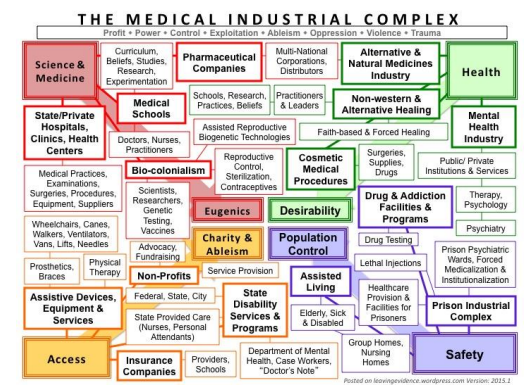


Afshari et al. 2023. Enhancing global health diplomacy for non-communicable diseases: application of the global health network framework. Globalization and Health.



Kiekens et al. 2022. Qualitative systems mapping for complex public health problems: A practical guide. PLoS One.

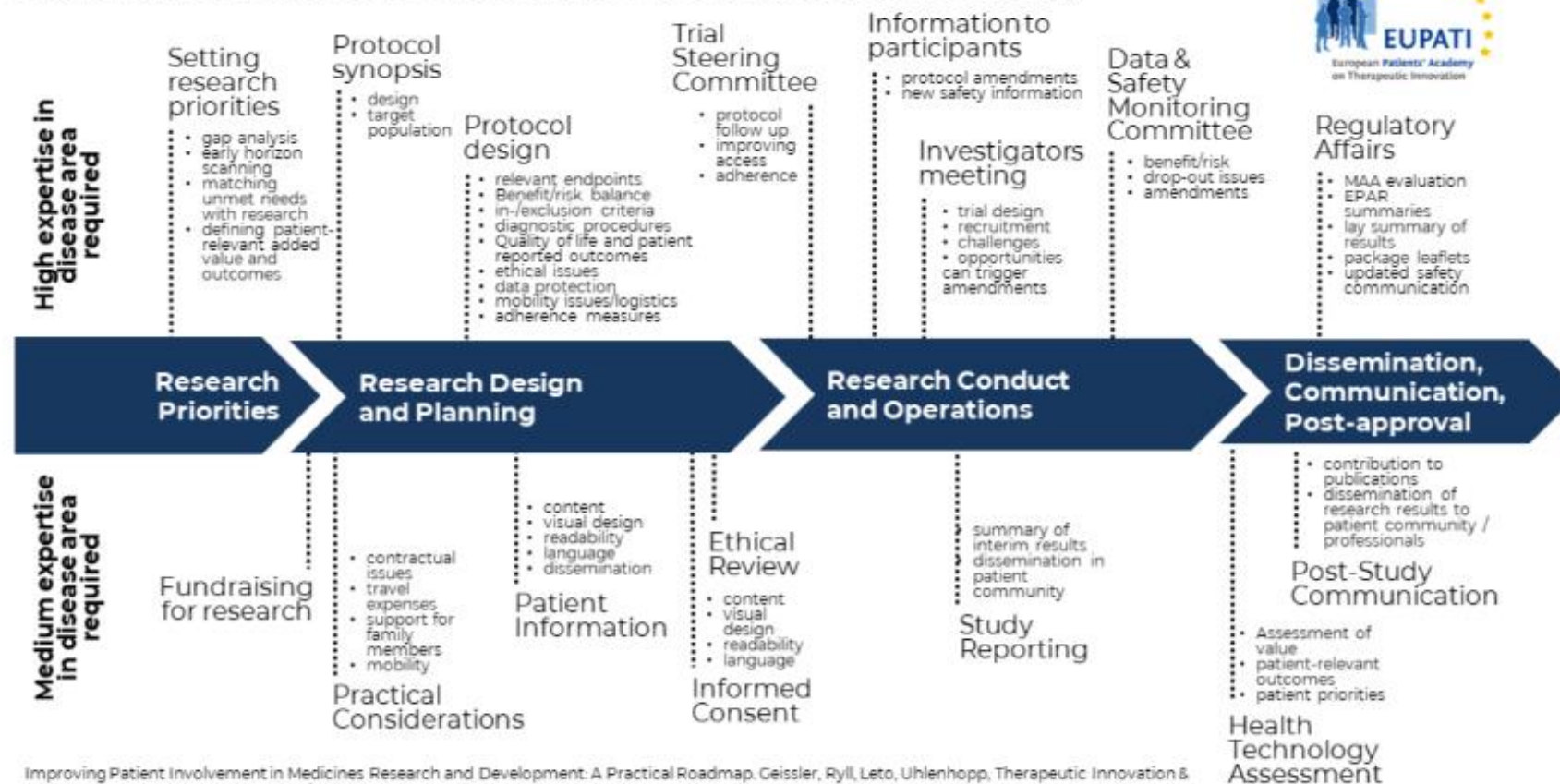
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# Role of Patient Advocates to support Health System Readiness to improve Access

## A) Before a medicine “reaches the market”

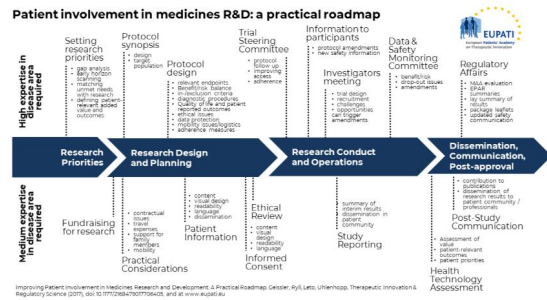
### Patient involvement in medicines R&D: a practical roadmap



Improving Patient Involvement in Medicines Research and Development: A Practical Roadmap. Geissler, Ryll, Leto, Uhlenhopp, Therapeutic Innovation & Regulatory Science (2017), doi: 10.1177/2168479017706405, and at www.eupati.eu

Involvement in R&D as an example: it is one layer of the complex construct only!

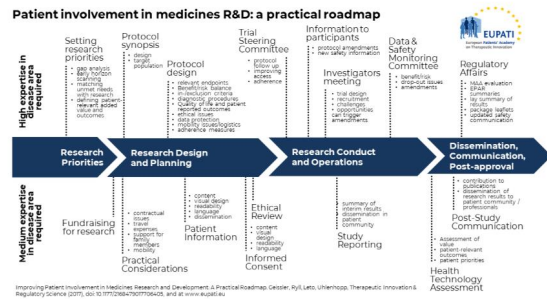
# Role of Patient Advocates: involvement in R&D as an example Research Priorities



- Highlight patient-relevant needs and gaps
- **SMA Research Agenda: SMA Europe priority setting partnership (PSP) initiative**
- **SMARt Horizon: Community-driven Research Scanning**



# Role of Patient Advocates: involvement in R&D as an example Research Design and Planning // Conduct and Operations



- Recruitment and Retention
- Design, assessment and amendment of study protocols (e.g., endpoints, in- / exclusion criteria, ethics, logistics)
- **Clinical Trial Readiness Initiatives** (in collaboration with the Cure SMA IC)
- **SMA Europe Community Surveys**
- **SMA Europe Treatment Committee** (patient experts)
- **SMA Europe EMA Committee**

## Role of Patient Advocates to support Health System Readiness to improve Access

B) After a medicine has “reached the/some market(s)”

### Goal

Access for all those who can benefit from a medicine.

### Objective

Patient advocates successfully advocate for change to improve access.

### Challenges

- **systems leave patients unprotected and alone**
- in-transparency and inconsistency across health systems
- rigid systems using outdated, “static” information
- evidence is lacking and education is missing

# Role of Patient Advocates to support Health System Readiness to improve Access

## B) After a medicine has "reached the/some market(s)" - Evidence

### OdySMA - A Quest to Access

An SMA Europe Access Advocacy Tool

- map and visualise the situation on access to medicines and care in different European countries
- identify access differences across countries and patient subgroups; and link them to the different health systems and economics of countries

<https://odysma.sma-europe.eu/> Visit:

**18**  
EUROPEAN COUNTRIES FAIL TO PROVIDE AVAILABLE LIFE-CHANGING MEDICINE WITHOUT RESTRICTIONS

#### Stabilizing treatment for SMA exists. Pan-European access does not.

OdySMA, inspired by Homer's epic Odyssey adventure, is a bold initiative to reveal the 'quest to access' of people living with Spinal Muscular Atrophy, by mapping, visualising and centralising knowledge and data around access issues.

**78**  
COUNTRY-BY-COUNTRY DATA COMPARISONS

#### Compare access country-by-country

Compare any performance ranking to any other country. How do your access rights compare to those in another European country?

Have you been 'left behind'?

**24**  
COUNTRIES PROFILED ACROSS 21 DATA SETS

#### A benchmark atlas

SMA Europe has rigorously collected and analysed data from multiple benchmark sources to create a unique visualisation of access to medicines & treatment.

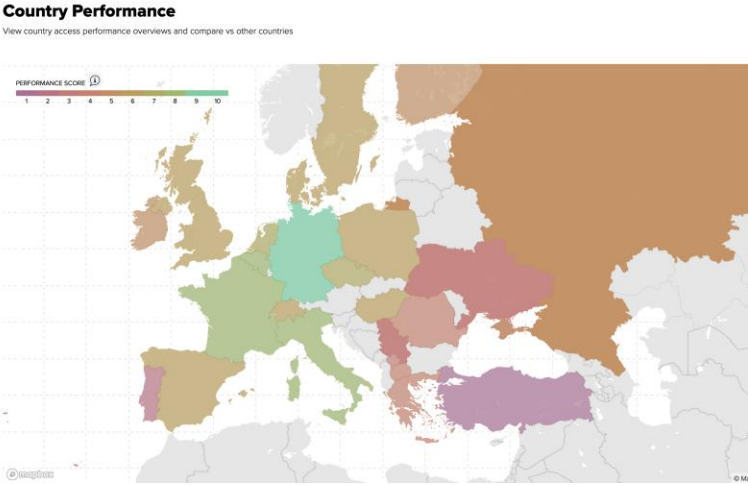
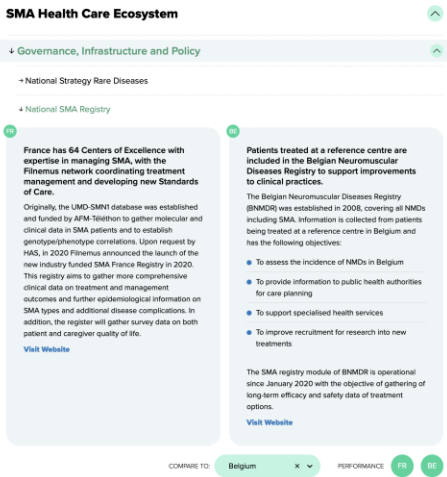
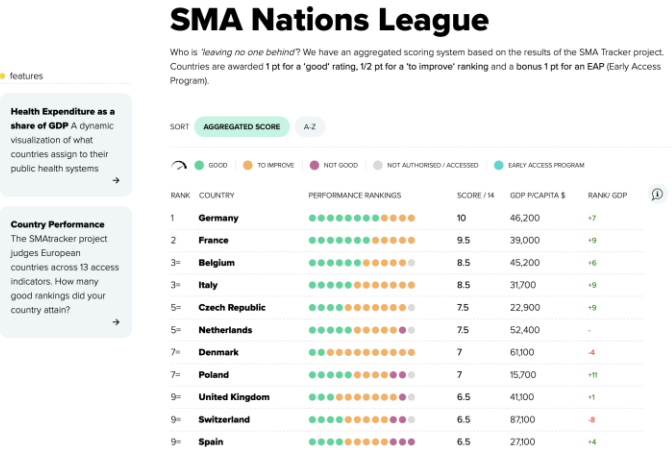
View our data in a series of interactive maps

**12**  
KEY PERFORMANCE RANKINGS

#### How does your country perform?

Check our 'Nations League' scoreboard. Each country is awarded a point for a good score and a 1/2 point for a mid score.

Which countries in Europe are 'leaving no-one behind'?



# Role of Patient Advocates to support Health System Readiness to improve Access

## B) After a medicine has “reached the/some market(s)” - Education

- understand the evidence
- gain the needed skills and
- build successful advocacy strategies to improve access to SMA medicines and care

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**Patients' perspective of access**

Access is based on 5 As:

- **Appropriateness**    Relevance to patient needs
- **Availability**        Existence of health care resources at local, national and EU level.
- **Adequacy**            Quality of care
- **Accessibility**        Ease of access to information on care and to health technologies and care providers
- **Affordability**        Access to care without financial hardship

Source: European Patient Forum Defining and Measuring Access to Healthcare: the Patients' Perspective  
[https://www.eu-patient.eu/globalassets/policy/access/ept\\_position\\_defining\\_and\\_measuring\\_access\\_010316.pdf](https://www.eu-patient.eu/globalassets/policy/access/ept_position_defining_and_measuring_access_010316.pdf)

**Olga Germanenko**    **portia thorman**    **Mencia de Lemus**

Participants in the video call: Mercè Casés, Nicole Gusset, Laura Gumbert, Ferdinand Schmieder, Thomas Doktor, Marie-Christine Oullade S..., Claudia Dumitru, Anne-Pierre Pickaert, Lassi Murto, Anna, Eva Stumpe, Véronique Van Assche.

Many trainings - here one on Access driven by OdySMA

# Impact of Education and SMAcademy

- Complexity of health systems, multifaceted and pace of innovation
  - no one can do it alone!
- Capacity building is the key to unlock change in health systems
  - evidence-based and “patient”-relevant:
    - powerful “patient” advocacy strategies
  - raise voice, build awareness and advocate for change
- **SMA Europe SMAcademy** - educational initiative for SMA patient advocates (kick-off 2024)
  - trusted evidence and information all in one place
  - SMA-specific /relevant, systematic education about on the disease, medicines and care, and the health systems
  - share knowledge and best-practices to develop common and/or own tailored advocacy strategies

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Together, through greater understanding,  
we will create a better world  
for all those living  
with spinal muscular atrophy.

**All together. One goal.**



