

THERE IS A TREATMENT  
APPROVED: WHEN CAN I  
GET IT?  
THE EXAMPLE OF SMA



**SMA Europe**

3rd Multi-Stakeholder Symposium, Eurordis, Brussels 13-14  
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# SPINAL MUSCULAR ATROPHY

- › SMA is a disease that causes severe disability and shortens life expectancy
- › Affects 20k people across Europe.
- › SMA Europe represents patients interests across 16 countries



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# QUESTION FOR THE AUDIENCE

Do you believe patient involvement can be a game-changer in the access to treatments for rare diseases?

1. Yes
2. No



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# TIMELINE SMA



1995: GENE DISCOVERY

2000: 0 pharmas investing  
in SMA

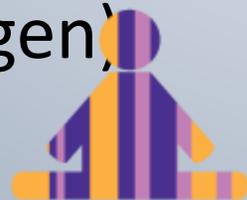
JUNE 2017: SMA IS  
TREATABLE DISEASE

2019 : 16 programmes 6  
drugs on CTs

Source CURESMA

# TREATMENT FOR SMA: SPINRAZA

- › Spinraza can dramatically improve motor function on some of the treated patients
- › Older patients: milder effect or stabilising effect
- › Nov 2018: 20% of European patients treated (source Biogen)



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# CHALLENGES ON THE ACCESS TO TREATMENT

Lack of evidence across the spectrum

Different degrees of efficacy

Patient Reported Outcomes

FRAGMENTATION OF THE EUROPEAN MARKET

LOST GENERATION

Weaker knowledge away from reference centres

Price and reimbursement negotiations

Perceived irrationality of patients

Suboptimal involvement of patients



# QUESTION FOR THE AUDIENCE

Which of these would be more important for patient reps to have?

1. Good understanding of their community's needs and concerns
2. Good dialogue with health authorities
3. Partnership with doctors, researchers and other disease reps
4. Ability to introduce the patient perspective on clinical trial designs and data collection efforts
5. All of the above



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# WHAT DO WE WANT

- › Equal access in timely manner for all who can benefit from treatments
  - Newborns
  - Adults
- › NO CLASS A AND CLASS B PATIENTS
- › Sustainability of treatments



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# HOW CAN WE REACH OUR GOALS

- › Patient reps as equal partners
- › Joint efforts to gather quality data post authorisation
- › Design tools that capture patient relevant changes
- › Better coordination between EMA and national HTAs
- › Work together within the rare disease community



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THANK  
YOU



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