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

SMA Europe

3rd Multi-Stakeholder Symposium, Eurordis, Brussels 13-14 February 2019
SPINAL MUSCULAR ATROPHPY

- 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
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
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).
CHALLENGES ON THE ACCESS TO TREATMENT

Lack of evidence across the spectrum

Different degrees of efficacy

Weaker knowledge away from reference centres

Price and reimbursement negotiations

Percieved irrationality of patients

Suboptimal involvement of patients

Patient Reported Outcomes

LOST GENERATION

FRAGMENTATION OF THE EUROPEAN MARKET
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
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
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
THANK YOU