



ERICA

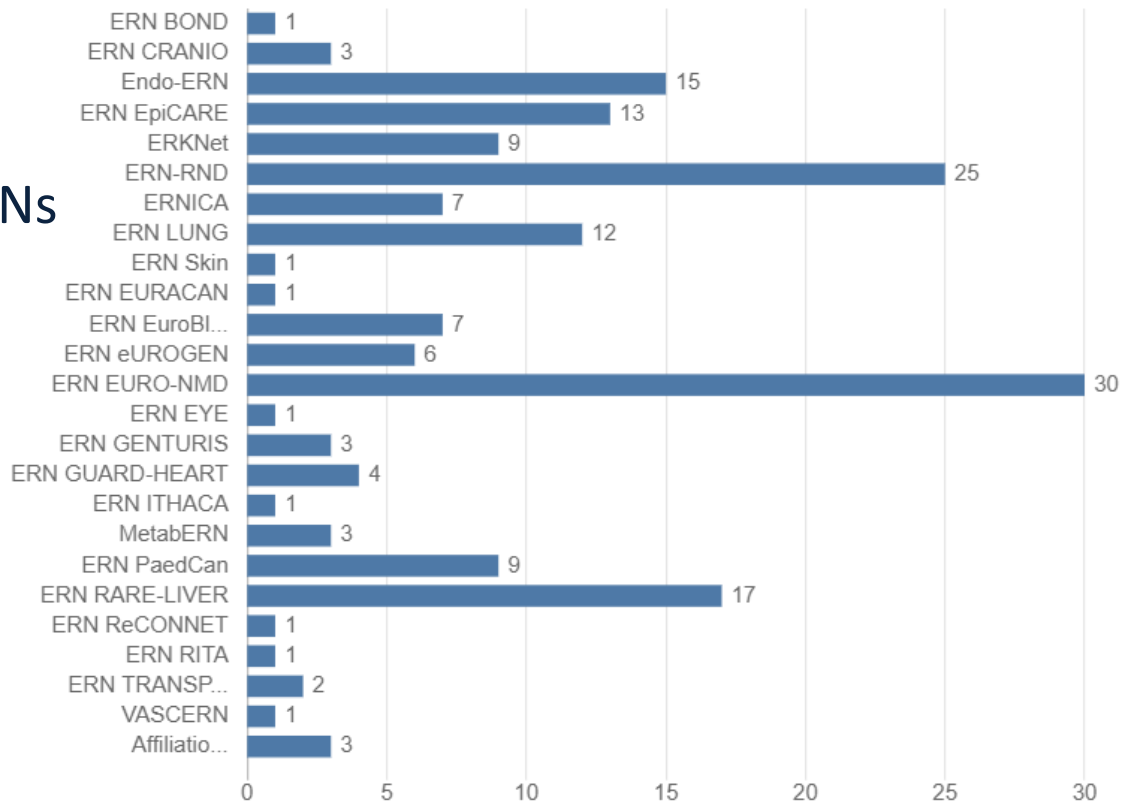
European Rare Disease Research
Coordination and Support Action

Status Quo of ERNs and Clinical Research: Results of ERICA WP4 Survey 2023

1. To provide a more comprehensive state-of-the-art of ERNs and Clinical Research comparing the results of this survey with the previous one delivered in 2018
2. To better understand the priorities and needs of the ERN community specific to clinical research

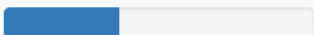




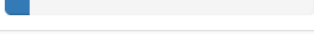
Survey Responses

- 176 responses from 24 ERNs
- ERN coordinators: 42
- HCP members: 134



Does your ERN/HCP has a transversal Working Group for research?

66% have a transversal Working Group on research activities:

| | | Answers |
|--------------------------------|--|---------|
| Basic |  | 66 |
| Diagnostic Research Studies |  | 116 |
| Observational Clinical Studies |  | 151 |
| Interventional Clinical trials |  | 98 |
| Outcome measures |  | 101 |
| Other |  | 14 |

Other comments added:

- translational research;
- retrospective studies;
- evidence-based guidelines for symptomatic treatment;
- epidemiological studies;
- diagnostic and treatment algorithms.

Would you include clinical trials in your research program?

Most (85%) ERN/HCP want to include clinical trials in their activities and they developed some clinical research on the disorders treated by their ERN (74%).

Some survey responses:

- *“We perform clinical research on myotonic dystrophy, painful neuropathies, Duchenne muscular dystrophy and myasthenia gravis.”*
- *“We are characterizing the spreading of the disease and the clinical correlations between C9 expansions and ALS phenotype.”*
- *“We participate in several basic and clinical projects and clinical trials in Parkinson's disease, Huntington's Disease, Deep Brain Stimulation Therapy and other movement disorders as Tourette syndrome.”*
- *“We recently publish paper on Anderson Fabry disease, amyloidosis, ALVC, restrictive CMP.”*

What plans has your ERN/HCP developed to advance clinical research?

- Guidelines
- Diagnostic tools
- Outcome measures PROMs/PREMs
- Registry/digital database to make easier and faster the data collection
- Early genetic diagnosis and counselling
- Increment of the human resources and technology
- Increase collaboration between national clinical centers and laboratories
- Transition from pediatric to adult care

On which areas and fields of research you believe your network has grown over the past 5 years?

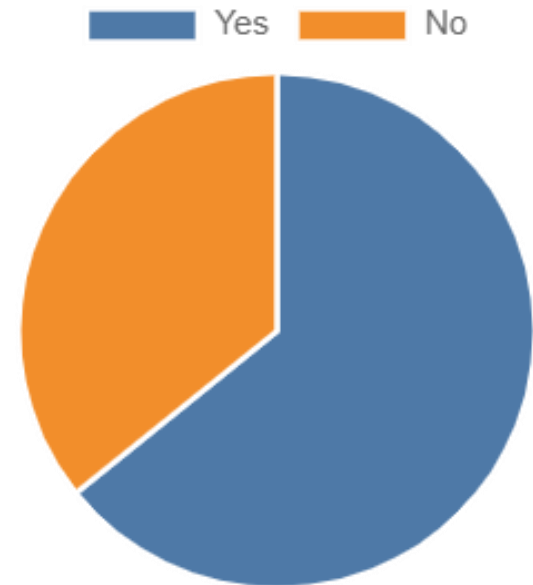
- Diagnostic area
- Fase I study, protocol writing, quality management of trials
- Registries
- Guidelines
- Outcome measures
- Networking

Regarding your ERN's future plans and priorities, which areas and fields of research would you hope to see your ERN address?

- Personalised medicine
- PROMs and PREMs
- Identification of biomarkers
- Resources/funding
- Basic research, diagnostics, clinical trials on treatments or devices
- Natural history studies
- Guidelines
- Registries
- Support disease specific networks

Does your ERN/HCP has any concrete tools or resources (i.e., small population design methodology expertise, database/registry ready for clinical research use, SOPs, site clinical trial readiness, etc.) supporting clinical research in future?

- Database/registries
- SOPs



Please rate the relevance (from very low impact to very high impact) of the following obstacles/ barriers, in terms of hindering clinical research in your ERN:

- Challenges in building relationships with regulatory agencies/authorities



| | | Answers | Ratio |
|-----------|--|---------|---------|
| Very low | | 4 | 2.29 % |
| Low | | 22 | 12.57 % |
| Neutral | | 54 | 30.86 % |
| High | | 61 | 34.86 % |
| Very high | | 34 | 19.43 % |

- Challenges in conducting clinical research that complies with regulatory requirements



| | | Answers | Ratio |
|-----------|--|---------|---------|
| Very low | | 7 | 4 % |
| Low | | 32 | 18.29 % |
| Neutral | | 47 | 26.86 % |
| High | | 59 | 33.71 % |
| Very high | | 30 | 17.14 % |

Separate analysis (ERNs) and (HCPs) for 2 of the questions:

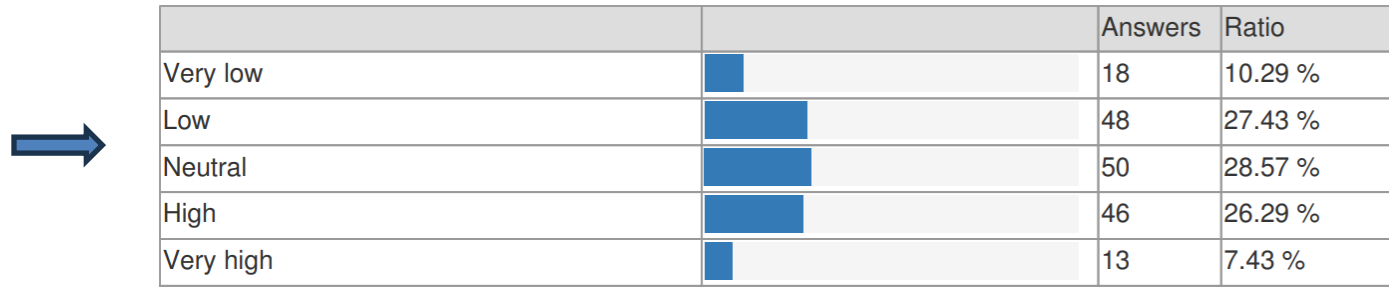
- *Q on barriers -*
- *Q "For clinical trials (CTs), to what extent does your ERN/HCP implement the following practices to involve patient organizations and patients?" -*

would be good to know:

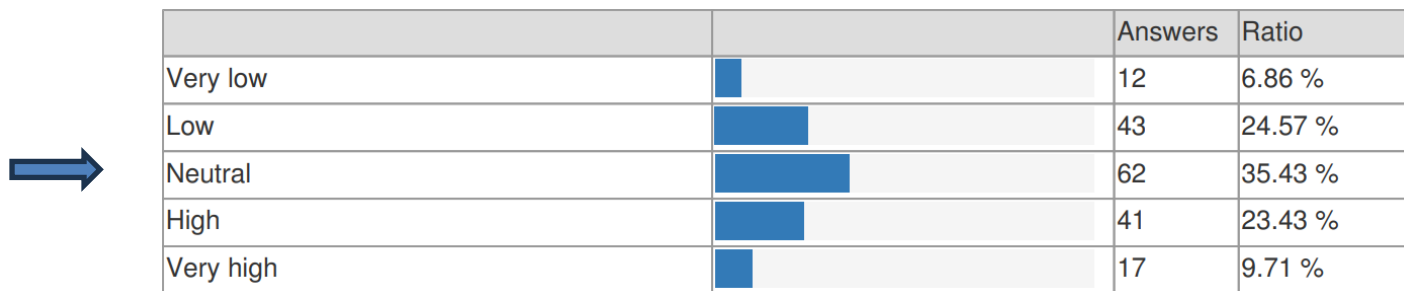
- which ERNs or HCP are rarely/never involving patients and why?
- for the ones who are 'often' doing so how is their experience and what can be improved?

asking the same question for each of the below options.

- Challenges in defining outcomes








- Challenges in building relationships with industry








- Independent Ethics Committee issues



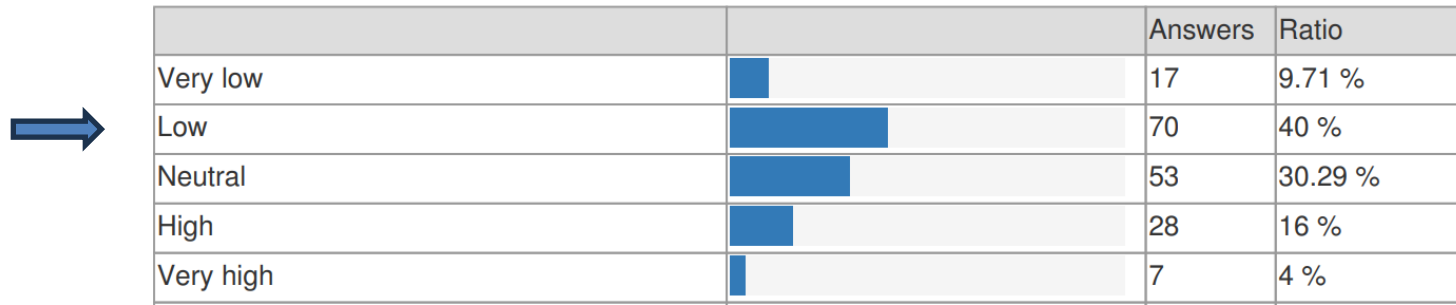
| | | Answers | Ratio |
|-----------|--|---------|---------|
| Very low |  | 13 | 7.43 % |
| Low |  | 41 | 23.43 % |
| Neutral |  | 67 | 38.29 % |
| High |  | 39 | 22.29 % |
| Very high |  | 15 | 8.57 % |

- Lack of suitable PROMs & PREMs

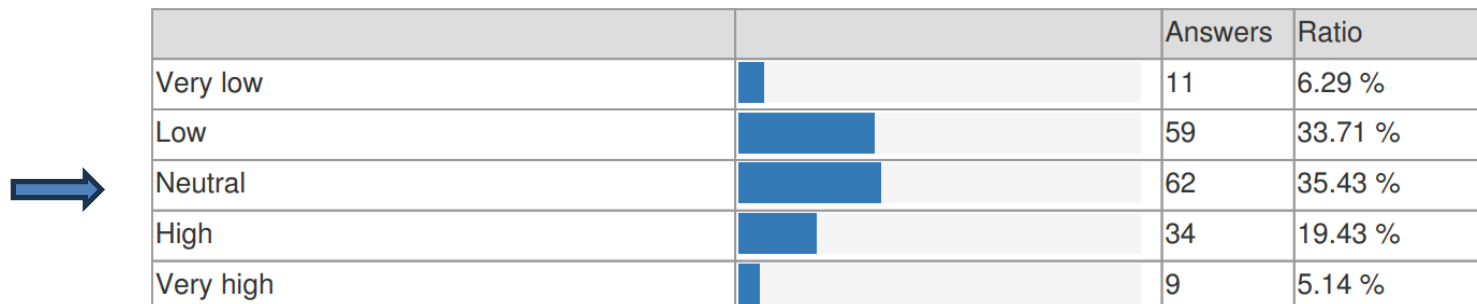


| | | Answers | Ratio |
|-----------|--|---------|---------|
| Very low |  | 10 | 5.71 % |
| Low |  | 34 | 19.43 % |
| Neutral |  | 72 | 41.14 % |
| High |  | 49 | 28 % |
| Very high |  | 10 | 5.71 % |

- Challenges in seeking patient input for research



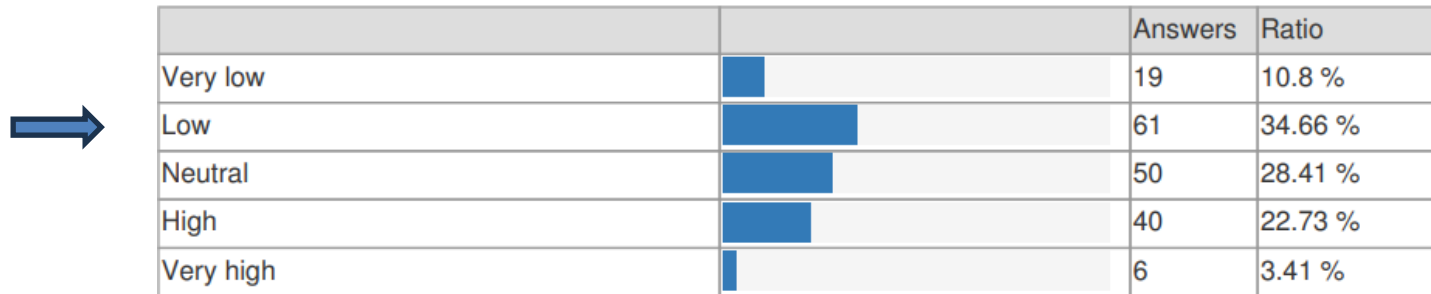
- Challenges in seeking patient input for clinical trial protocol design



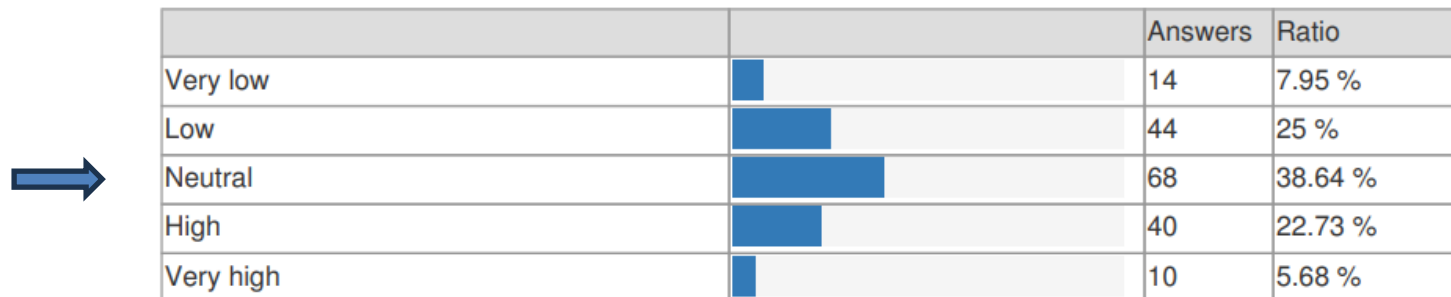
to go more in depth on which type of challenges:

- bc they don't have any contact with any patient organisation?
- due to lack of knowledge?

- Challenges in enrolling patients



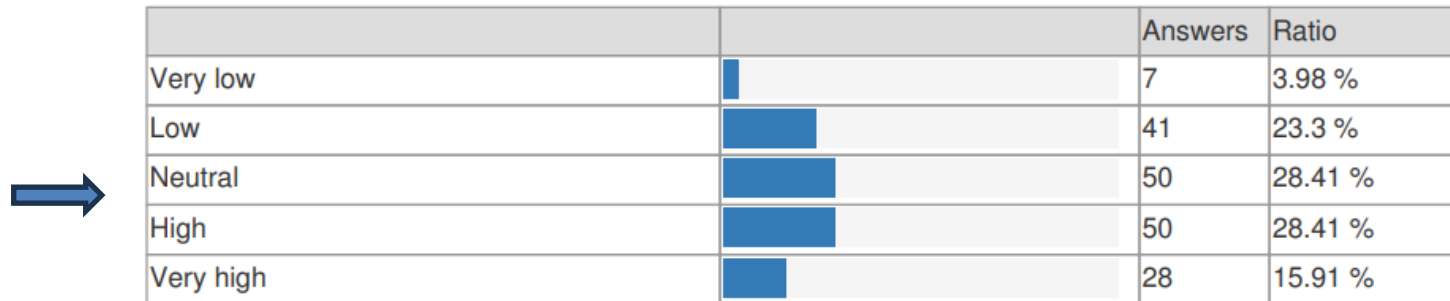
- Challenging in identifying and involving HCPs



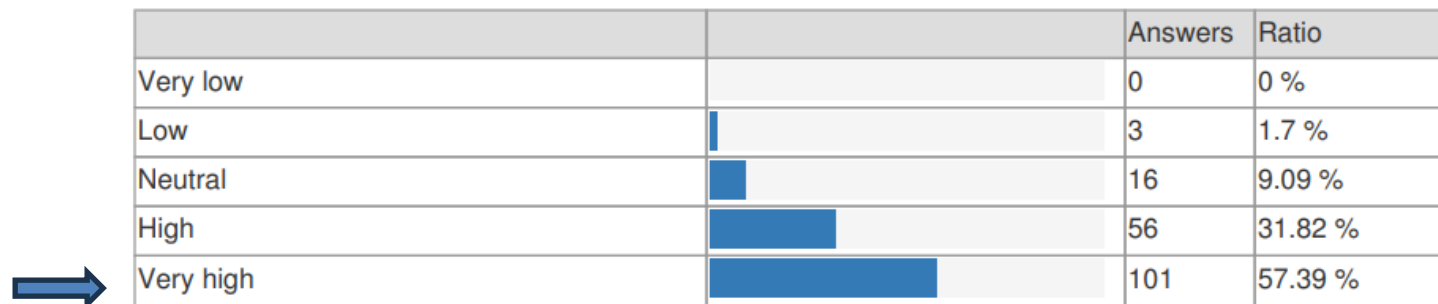
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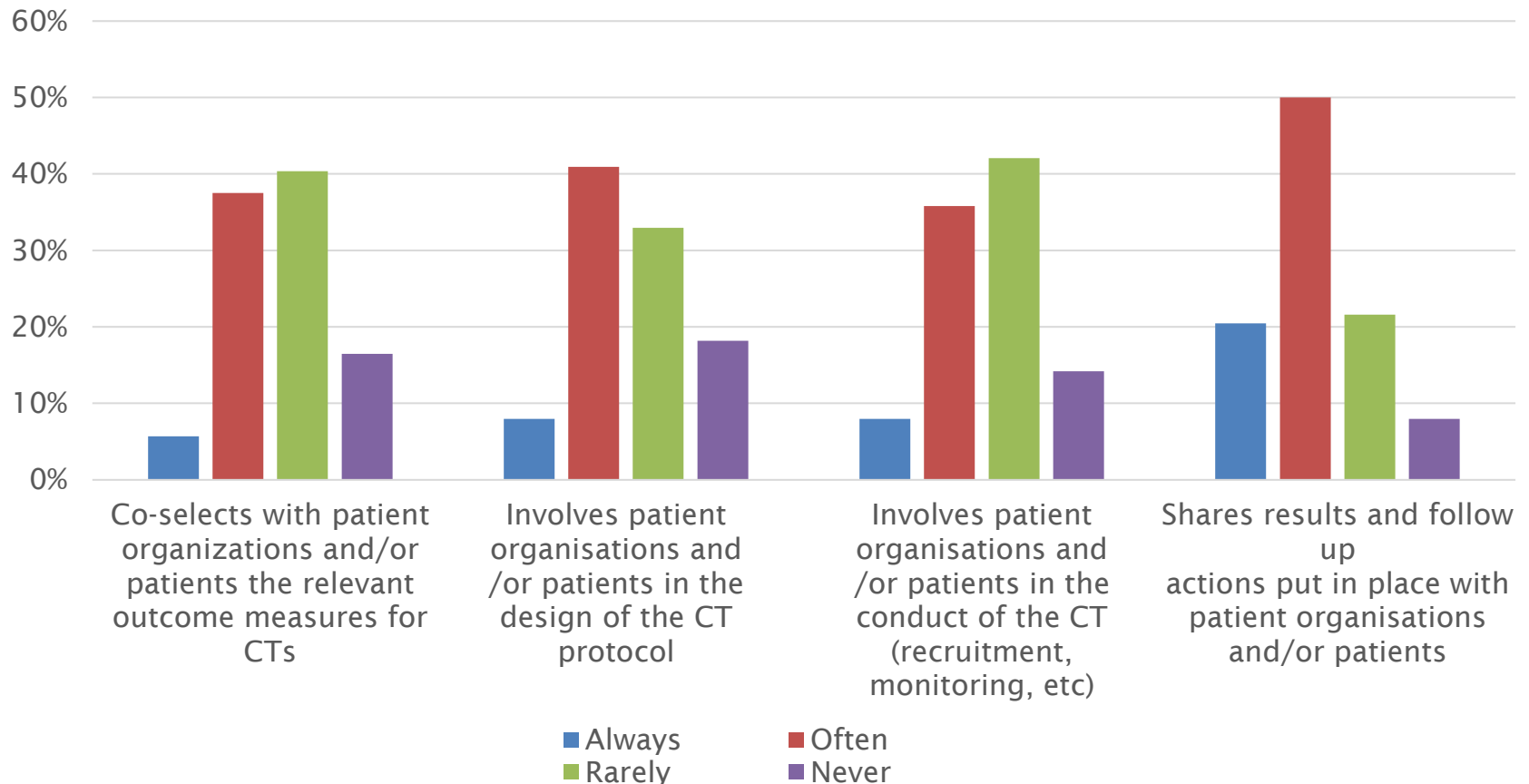
- Issues around clinical data management



- Lack of funding



For clinical trials (CTs), to what extent does your ERN/HCP implement the following practices to involve patient organizations and patients?



THANK YOU
FOR YOUR
ATTENTION

