# Status Quo of ERNs and Clinical Research: Results of the ERN-Wide survey

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# Survey Responses

- 21 responses from 18 ERNs
  - ERN RITA (3); ERN RECONNET (2 replies); ERN EURACAN; ERN GUARD-HEART; ERN EURO-NMD; ERN EYE; ERN ERKNET; ERN ITHACA; MetabERN; ERN SKIN; ERN GENTURIS; VASCERN; ERN TransplantChild; ENDO-ERN; ERN EuroBloodNet; ERN LUNG; ERN EpiCARE

- Capacity in which people completed: How one responds to the survey (i.e. in which capacity) is important : we gave people two options

| | |
|---|---|
| **Representing ERN i.e. person is Coordinator or Group leader or otherwise feels justified in speaking for the ERN** | **17** |
| Prefers to reply in personal capacity as expert representing an HCP (e.g. maybe has not had opportunity to canvas whole ERN) | 4 |

# Q1. Please briefly describe the internal research organisation of your ERN

- Almost all (all except one response) have either a WG or WP or transversal TF for research (sometimes specifically clinical, usually for any research activities)

Selection of comments:

- "We have a working group for research…has not fully committed to actually submitting grants together as a single cohesive group, but already some new collaborations have been formed."
- The ERN has currently 8 thematic groups of diseases, and 3 transversal groups dealing with research:- deep phenotyping - e-health & registries - clinical outcomes. **In each thematic / disease group, a member is in charge of a transversal topic incl research.**
- 'Our ERN has established eight main thematic groups covering the full spectrum of congenital and acquired conditions. Each thematic group has an attached working group for research **from both adult and paediatric backgrounds**.
- We have a 'Research and Clinical Trials Functional Committee' which in 2017 organized a seminar on 'How to build a Clinical Trial Network'

# Q2. Can you briefly summarise what you see to be the added value of your ERN (compared to for instance the projects/isolated activities which occurred pre 2017) in driving forwards Clinical Research for rare and specialised conditions?

- 'The ERN can collect bigger numbers of patients, larger cohorts, develop critical mass" (many mentioned registries and larger population of well-stratified cohorts, to increase research into 'druggable targets' recruit patients, etc. )
- "Development of European level databases, promoting biomarkers and genetic studies and researcher initiated spontaneous therapeutic trials
- "Recruitment of patients in natural history studies or clinical therapeutic trials, collegiality around determination of outcomes and endpoints specific for our diseases
- "May have a bigger coverage of the EU territory and because the best experts are represented it works as a think-tank.'
- 'An ethos of working together via ERN across large geographical areas, mixed expertise e.g. for gene therapy projects"
- Encourage active patient participation to research – better participation of POs to CT decision-making process
- Disease areas have not previously worked closely together; there is tremendous opportunity for cross-fertilisation of ideas, and for creation of new trans-disciplinary networks

# Q3. Has your ERN yet formed any plans to advance clinical research under the scope of the conditions/focus of your Network?

- Many ERNs mentioned conducting Surveys relevant to clinical research activity in their Network, e.g.
  - 'The ERN collects information on Clinical trials where HCPs are involved as well as research projects.
  - 'Survey of research WG members to identify the key unmet need in their specific area'
  - Identification of research gaps, ERN registry, Deep phenotyping classification, CTs ongoing
- 'Establishing CTNs (according to a specific blueprint) is one of our priorities'
- Two cited being part of specific research project - Solve-RD- and a couple mentioned EJP proposal participation
- "Set up of multiple disease-specific registries within different thematic areas as first step to use for future clinical research projects."
- 'We are planning a common natural history study with genotype phenotype correlations on various complications of specific conditions"
- 'Establishment of clinical trial to design a new drug for a specific rare disease.'
- 'Plans to enlarge national studies into European studies.'

# Q4a. Thinking of your ERN's current plans and priorities pertaining to 'Research': please indicate which areas and fields of research YOU believe your Network will focus upon in the first 5 years

- ☐ Public Health
- ☐ Epidemiology/Natural History of Disease
- ☐ Therapeutic Options - Medicines
- ☐ Therapeutic Options - Medical Devices
- ☐ Therapeutic Options - Other (please specify below)
- ☐ Health Technology Assessment (HTA)
- ☐ Quality of Life
- ☐ Socio-Economic
- ☐ Social and Holistic Care
- ☐ Basic/Pre-clinical
- ☐ Animal Models
- ☐ Translational
- ☐ Other

5 – Public Health
18 – Epidemiology
18 – TOs Medicines
4 – TOs Medical Devices
5 – TOs Other
4 – HTA
18 – Quality of Life
5 – Socio-Economic
3 – Social and Holistic Care
6 – Basic/Pre-clinical
3 – Animal Models
14 – Translational

Under 'Other':
3 mention SURGERY
1 '' Gene Therapy
1 '' Prognostic biomarkers
3 '' Diagnostics/Diag Tech
1 '' Radiotherapy
1 '' CPMS

Q4b. Thinking of your ERN's future plans and priorities (after the first 5 years, i.e. 2022 onwards): if resources were not a problem, which areas and fields of research would you HOPE to see your ERN address?

- ☐ Public Health
- ☐ Epidemiology/Natural History of Disease
- ☐ Therapeutic Options - Medicines
- ☐ Therapeutic Options - Medical Devices
- ☐ Therapeutic Options - Other (please specify below)
- ☐ Health Technology Assessment (HTA)
- ☐ Quality of Life
- ☐ Socio-Economic
- ☐ Social and Holistic Care
- ☐ Basic/Pre-clinical
- ☐ Animal Models
- ☐ Translational
- ☐ Other

10 – Public Health
12 – Epidemiology
17 – TOs Medicines
5 – TOs Medical Devices
6 – TOs Other
8 – HTA
14 – Quality of Life
11 – Socio-Economic
6 – Social and Holistic Care
10 – Basic/Pre-clinical
5 – Animal Models
14 – Translational

Under Other:
2 - Surgery
1 -Radiotherapy
2 - Gene Therapy
1 – 'CT on alternative medicine efficacy, nutrition, newborn screening, prevention test at preconceptional levels'
1 – CPMS

# Section 3: Infrastructures/ Resources/Assets/Tools of relevance to clinical research under your ERN

Q5: Thinking about your own Centre – to which of the following does your particular HCP have access at present?

- ☐ Clinical Trial Centre/Unit
- ☐ ELSI (Ethical Legal and Social Issues) Support
- ☐ Dedicated Grants Office
- ☐ Training and Education on the planning and execution of clinical research
- ☐ Other

18 – Clinical Trial Centre/Unit
14 – ELSI support
15 – Dedicated Grants Office
16 – Training and Education
5 – Other

- European Paediatric Research Infrastructure
- Centralised medical ethical committee



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# Q6. Does your ERN have any concrete tools or resources – developed since approval of the Network OR dating to the pre-ERN era- which you foresee supporting clinical research (either in the planning or delivery) in future?

- A couple were unsure here – need to do more mapping still
- Others clearly built upon mature research networks e.g. neuromuscular identified specific assets they will use
- **Many mentioned registries, e .g:**
  - 'Several major registries; a principal focus of the ERN is FAIRifying and aligning these so that they support transversal clinical research activity.
  - We already had some established registers and have initiated some more under the ERN
- Several highlighted research databases specifically for the Network:
  - 'Network-wide research database with ongoing projects listed on the website. Network-wide Core Patient Registry'
  - We are constructing through the network website a on-line repository of members, experts, disease coverage, and facilities available, participation in CT and research projects that will allow: a) Directly update of contents by experts and b) search for partners involved in specific diseases or running research projects in order to engage collaborative actions.'

# Section 4: ERN Grants

Q7. Thinking of your own HCP: which of these funding sources are currently used to support clinical research in the scope of your ERN (e.g. rare eye diseases) (please tick all that apply).

- ☐ International Funding (outside of EU)
- ☐ European Union (e.g. H2020)
- ☐ National Research Council or equivalent
- ☐ Regional Research Funding
- ☐ University
- ☐ Charities or Foundations
- ☐ Industry (Companies)
- ☐ Other

3 – International Funding
18 – European Union
15 – National Research Council
8 – Regional Research Funding
14 – University
13 – Charities or Foundations
15 – Industry (Companies)
2 – Other

'Other'

- Currently, there is no financial support or grants dedicated to ERN-wide research involving all the HCPs concerned with a specific question.

# Section 5: Industry Interactions

**Q8. Thinking now of the pre-ERN era and/or of other networks (not ERNs) you are aware of: please describe any interactions with Industry (Companies) from within your broad disease area, which you feel have facilitated/advanced clinical research.**

- Several examples here of previous engagements with Industry around specific medicines' development
- Several cited how companies would LIKE to work with their ERN, e.g.
  - 'Companies are keen to support certain ERNs in developing registries'
  - 'Companies are definitely interested in using our ERN in finding cohorts for trials, and our current efforts to set up a core registry is expected to facilitate such interactions.'
- One respondent highlighted desire for 'Investigator-initiated studies together with Industry'
- Neuromuscular highlighted way in which Industry has made extensive use of the TREAT-NMD resources namely the registries and CTSR (case study)

# Section 5: Regulatory Interactions

Q9a: Has your ERN had any engagement to-date with the following, to specifically explore how the Network might lead/conduct clinical research? (please tick all that apply)

- ☐ European Medicines Agency
- ☐ National Regulatory Authorities / Competent National Authorities
- ☐ National Health System
- ☐ Ministry of Health/Research/Other
- ☐ Do not know
- ☐ Other

4 – EMA
6 – National Regulatory Authorities
7 – National Health System
8 – Ministry of Health
6 – Do not know
4 – Other

Comments under 'Other'

- Informal discussion but no engagement
- No formal engagement
- Many discussions with all of the above but no engagement
- Too early to say

# Section 6: Needs and Priorities
# Q10: Obstacles…

Q10: Which of the following would you consider to be major obstacles to YOUR ERN facilitating/streamlining/ delivering clinical research? *

- ☐ Lack of funds
- ☐ Lack of clear opportunities at present to engage with Companies
- ☐ Lack of well-stratified patient cohorts for trials/studies, e.g. through appropriate, robust registries
- ☐ Lack of regulatory know-how to organise trials across borders
- ☐ Uncertainty as to how the ERNs can 'lead' or participate to trials and studies (e.g. how far such activities will be delivered entirely within the Network vs how to engage outside)
- ☐ Uncertainty over the best methodologies to conduct trials in small populations
- ☐ Lack of appropriate clinical outcomes/endpoints, etc.
- ☐ Other

19 – Lack of funds
7 – Lack of clear opportunities
10 – Lack of well-stratified patient cohorts for trials/studies
10 – Lack of regulatory know-how
12 – Uncertainty on how ERNs can lead…
4 – Uncertainty over methodologies
3 – Lack of appropriate clinical outcomes
2 - Other

Other:

- Brexit
- Lack of admin support
- Terrible model of co-funding within the health programme

## Q12: Are there any other comments you wish to make -elaborating further on any of the Qs above or raising something not yet mentioned - on the subject of ERNs adding value to clinical research?

- ‘The primary focus of ERNs is clinical care- but for rare diseases, research is an integral part. Its hard to know however, how much research success is actually derived from the ERN grouping, rather than work people are already doing- hopefully as the ERN matures this will become clearer.’
- ‘It is clear that some centers are very collaborative and will share research resources whereas some are still very protective and volunteer little. We have to work on changing this culture or find some way to make sure that ERN members are collaborative.’
- ‘It is important that there is facilitation for the interaction with extra-European consortiums or groups of researchers and extension of clinical trials at an International level