

| Del N° | Deliverable name | WP | Due date (in month) |
|----------------|---|------|---------------------|
| Del17.1 | Results of survey on preferences, needs and resources from the ERNs ecosystem | WP17 | M4 |

Due date: 30.04.2019

Date: 06.06.2019

Version: 1.0

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Deliverable 17.1 - Results of survey on preferences, needs and resources from the ERNs ecosystem

Report

The planned WP17 ERN survey was integrated into an EJP-RD ERN survey that was sent out on 25 March 2017. Responses were collected by 11 April 2019.

The outcome of the survey in general and as regards the WP17 questions is summarized in attachment 1. This has been provided by pillar 2 (Mary Chang and Franz Schäfer).

Questions used for the survey as well as all responses are contained in attachment 2.

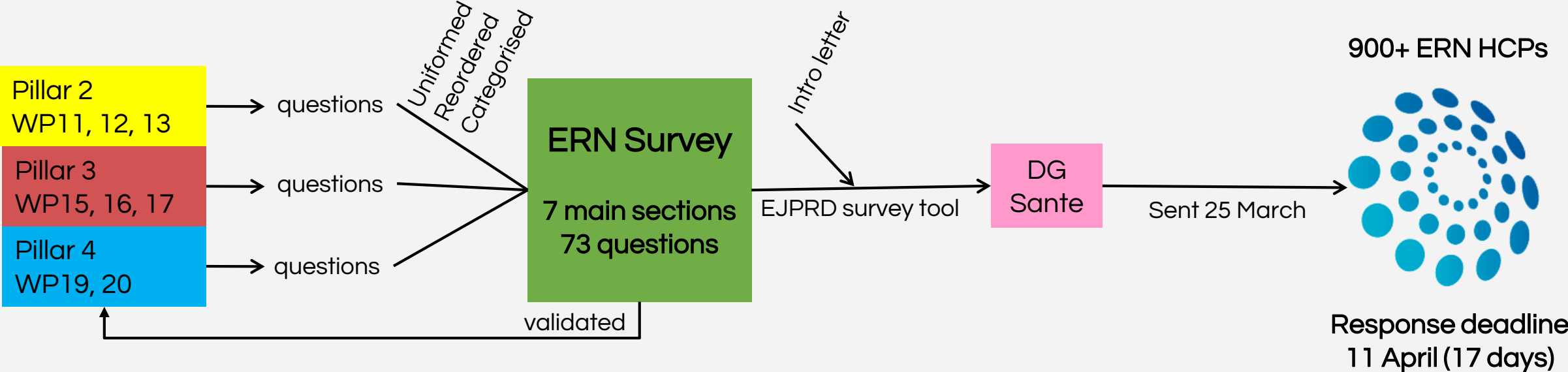
During an analysis meeting of WP17, two training measures were identified that will function as schemes of the ERN research training program to be developed. These are: (i) research fellow exchanges and (ii) training workshops/seminars.

These schemes will further be discussed and specified during the planned focus group meeting on 26 June in Leiden, Netherlands.

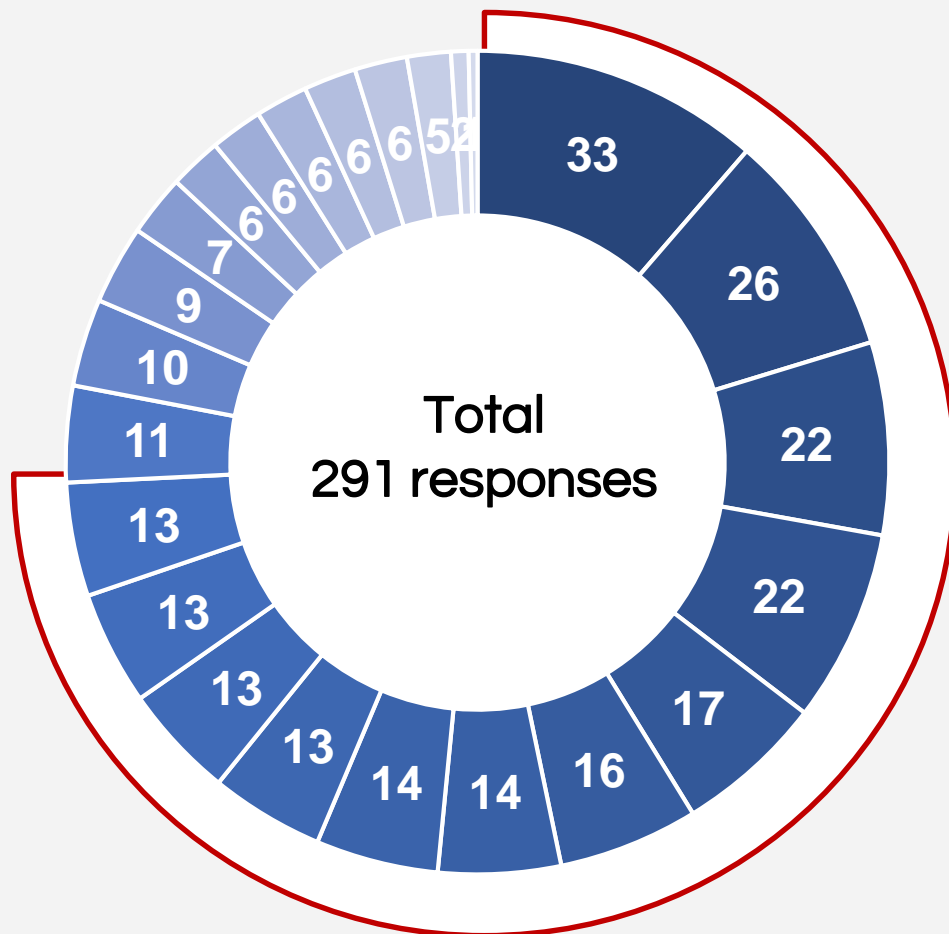
Attachments

- (i) Survey analysis as provided by pillar 2 of EJP-RD
- (ii) Survey questions and data

Set up of ERN Survey 2019



Responses by ERN



- ERN LUNG
- Endo-ERN
- EuroBloodNet
- ERKNet
- MetabERN
- VASCERN
- RITA
- EURO-NMD
- PaedCan
- ITHACA
- eUROGEN
- ERN Skin

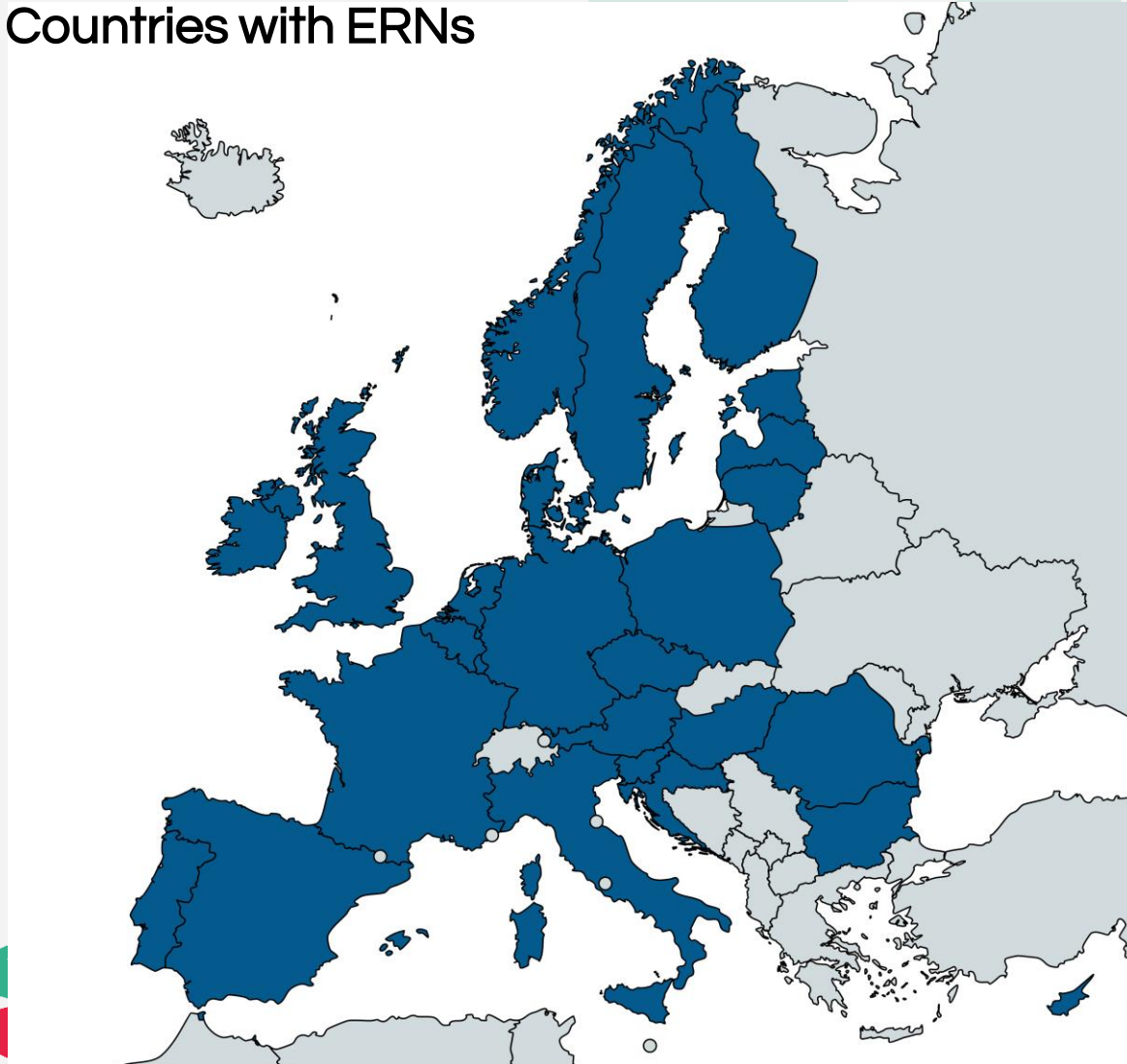
75% of the received responses come from these 12 ERNs

74.2% (216) said YES to secondary contact by EJPRD partners

- ReCONNET
- ERN RARE-LIVER
- BOND
- ERNICA
- TRANSPLANT-CHILD
- EURACAN
- ERN-RND
- EpiCARE
- CRANIO
- ERN EYE
- GUARD-HEART
- GENTURIS

ERN responses by country

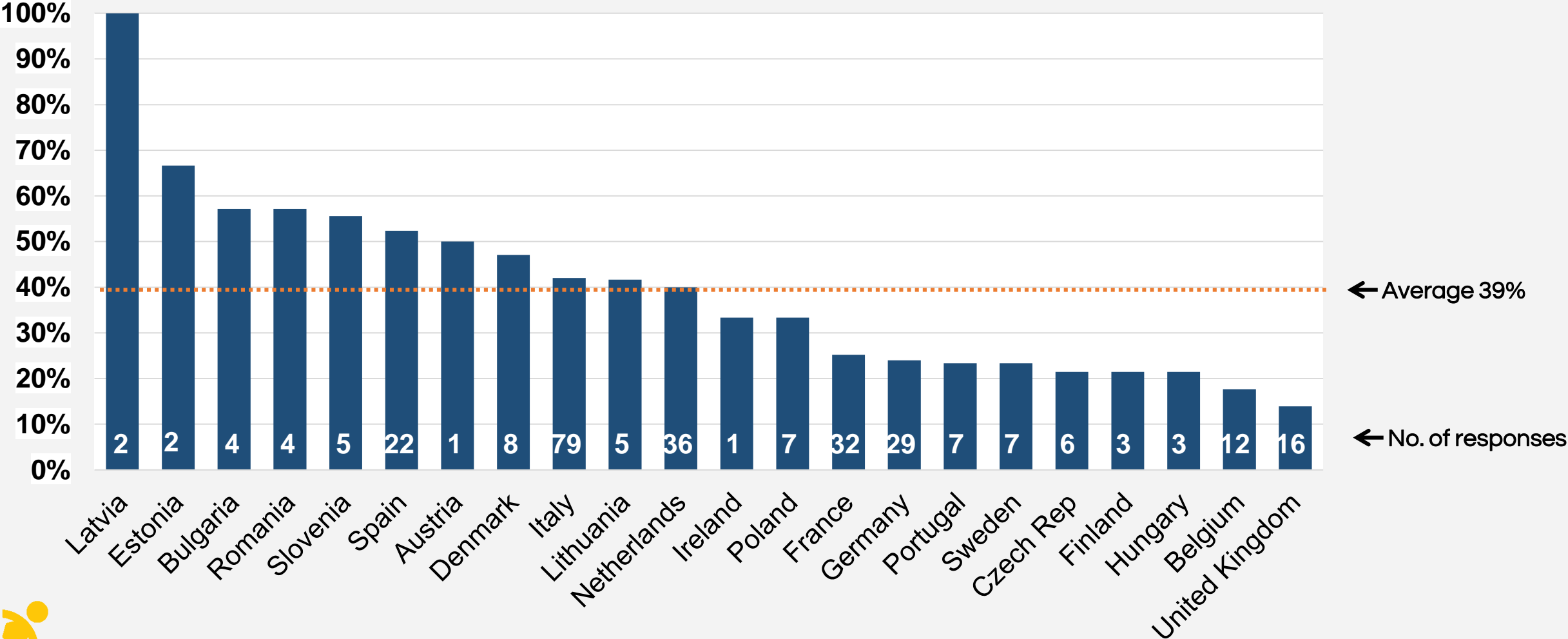
Countries with ERNs



- Responded to survey
- No responses



Responses by country, normalised to **no. of HCPs** in each **country**

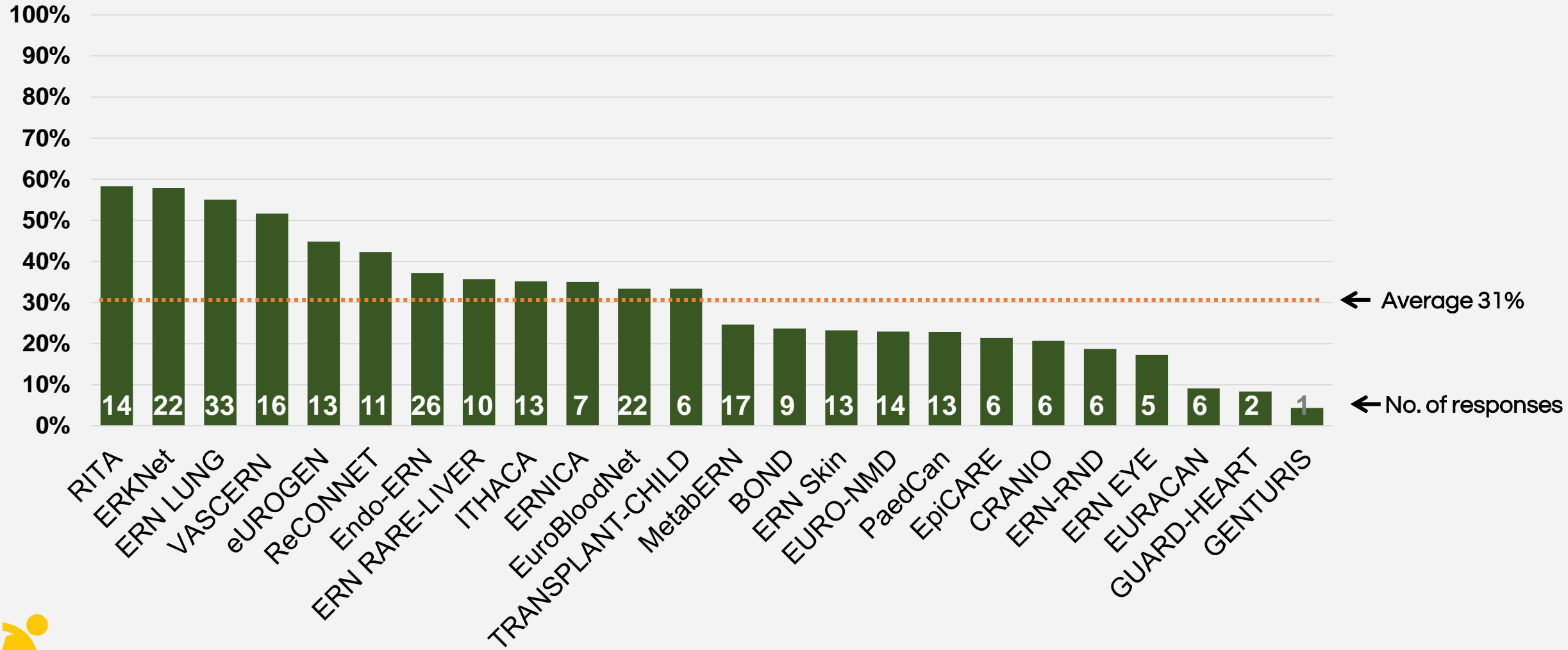


← Average 39%

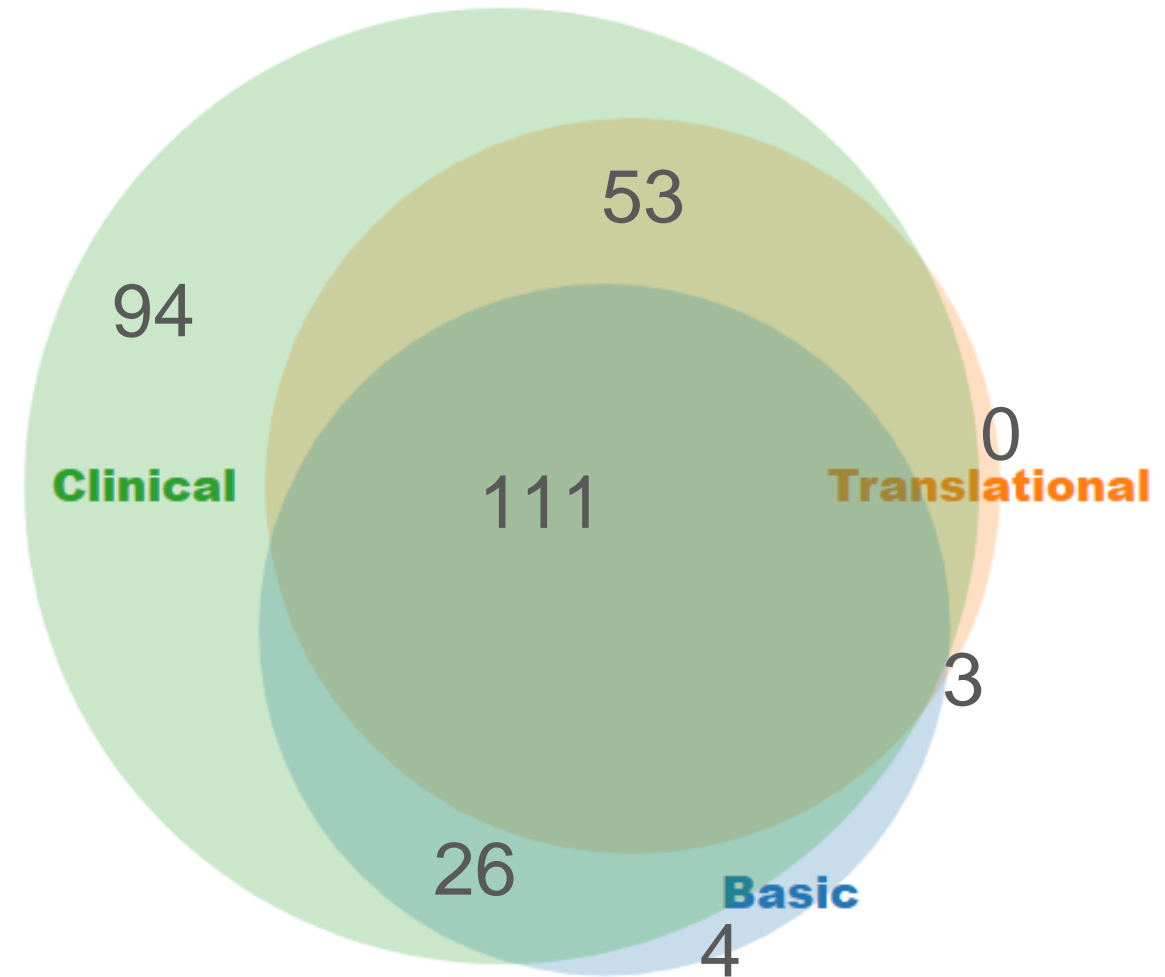
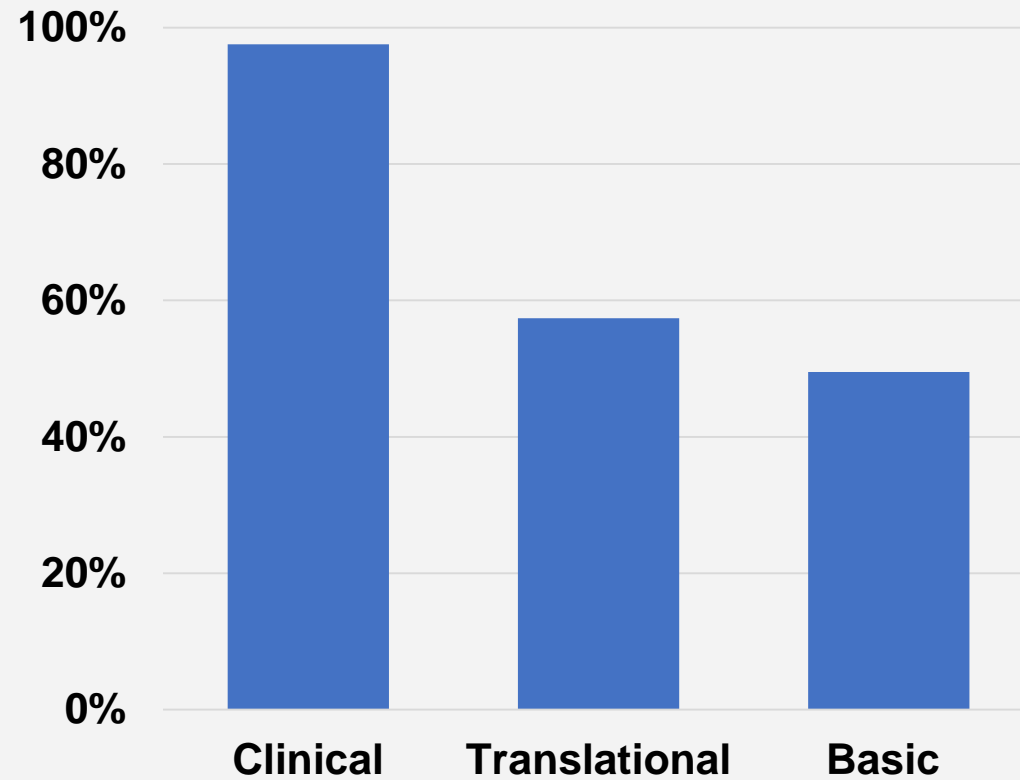
← No. of responses



Responses by ERN, normalised to **no. of HCP** in each **ERN**

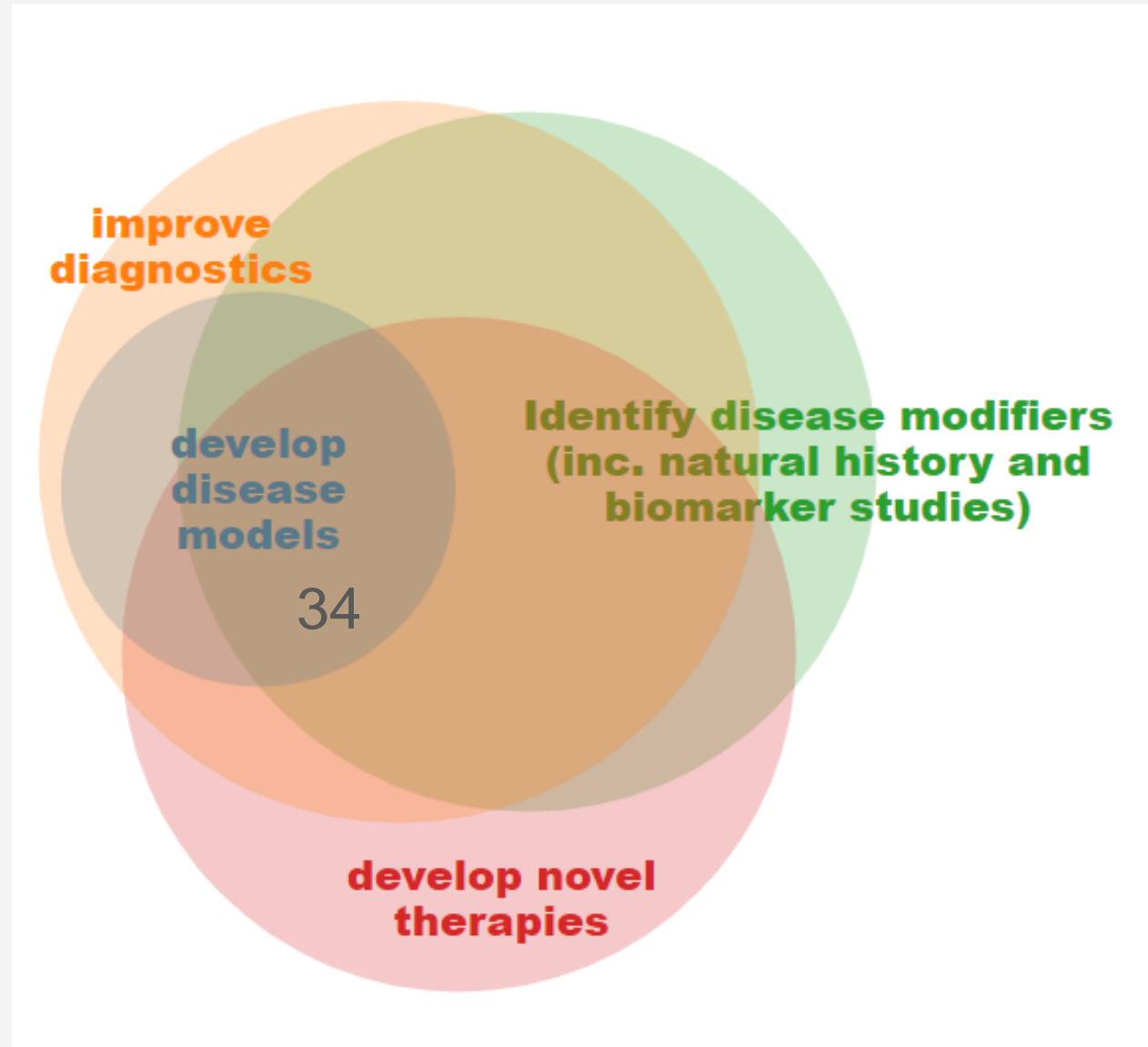
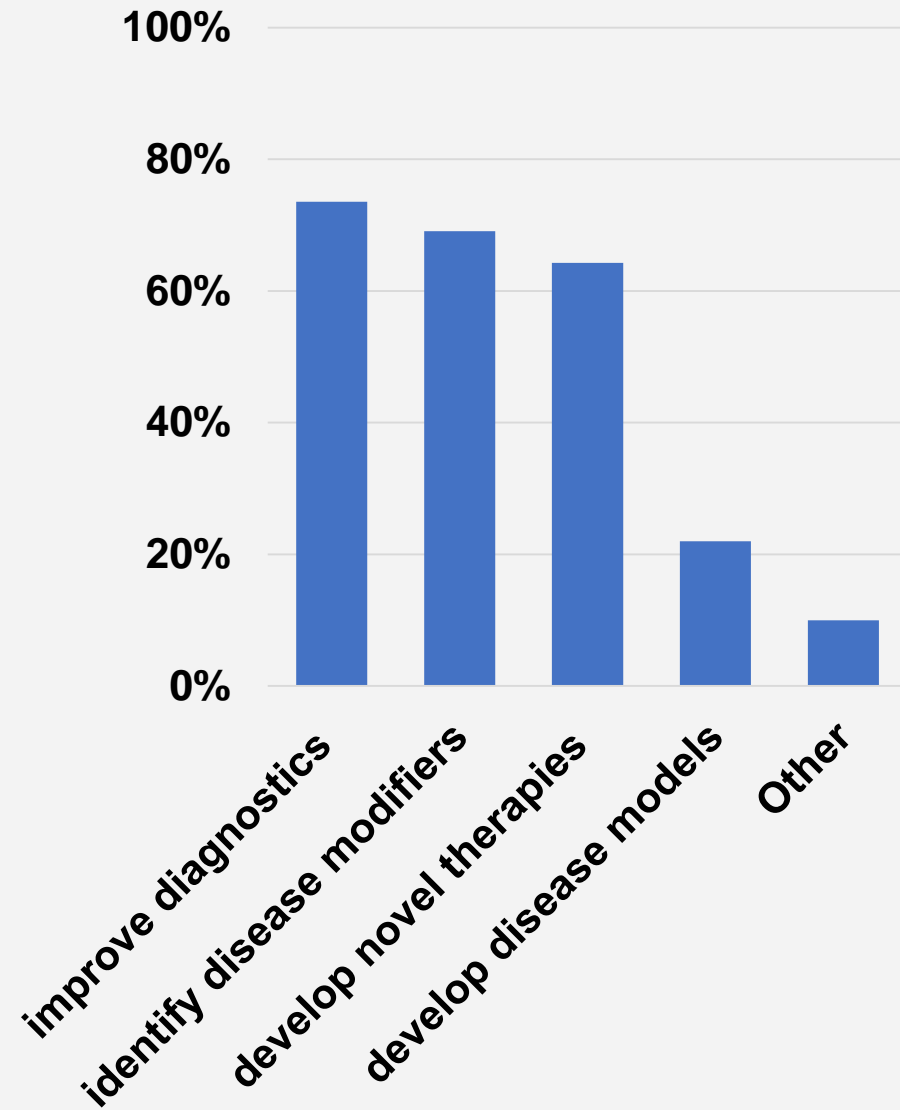


Types of research



- Almost all of the ERN units do clinical research
- Many do all types of research (basic, translational and clinical)

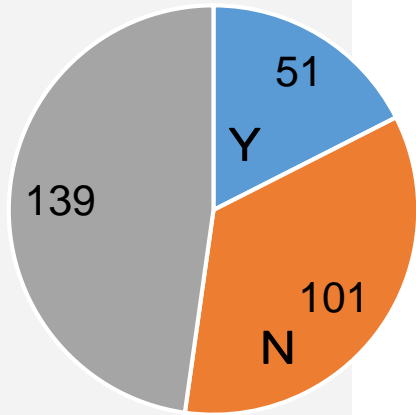
Main purposes of research



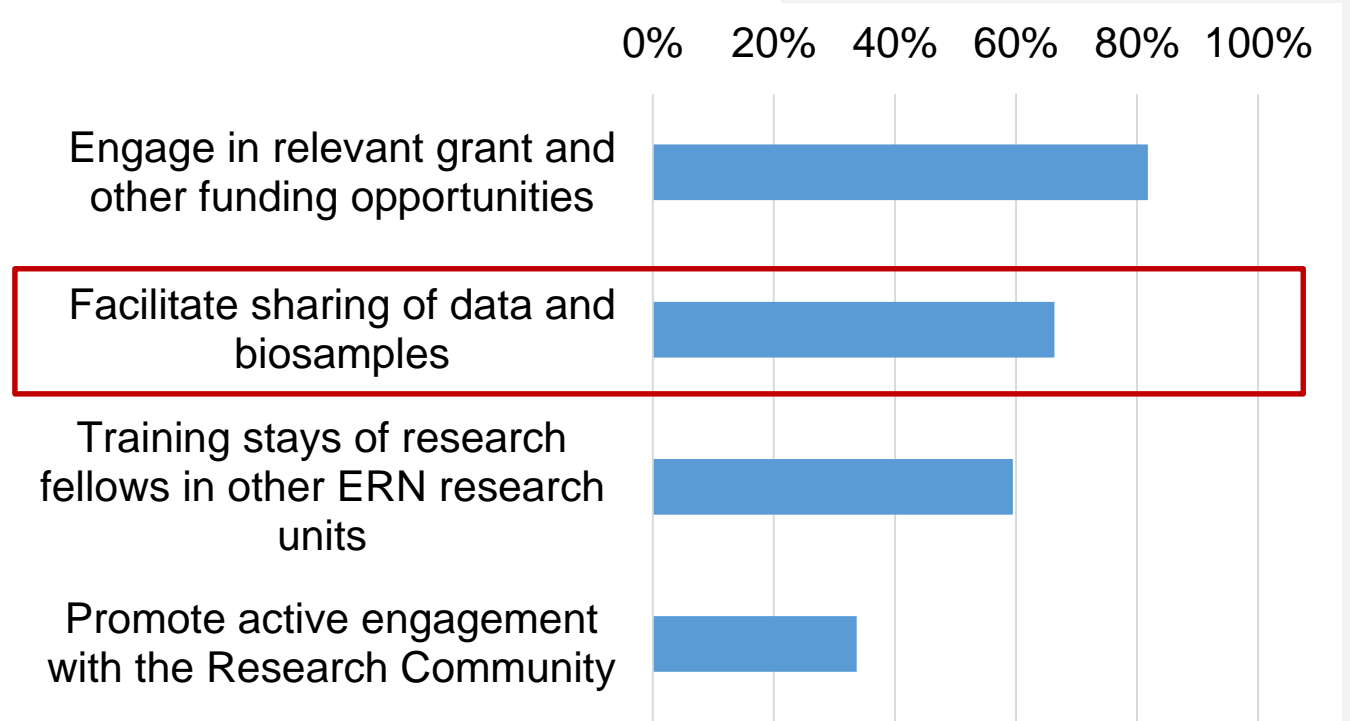
Section F

Training needs (inc patient involvement)

F1. Is there a specific research skills and support training practice currently available in your country or local level that might be of interest and transferable to other ERN HCP?

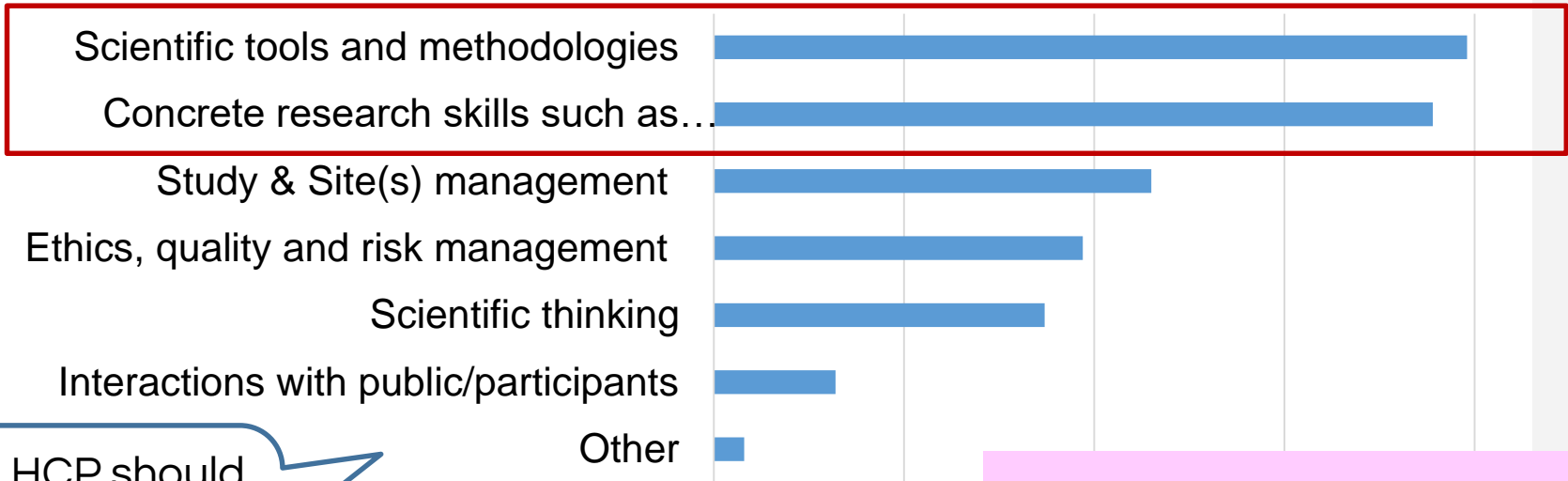


F2. What, in your view, are the most important research support needs to help ERN researchers achieve the goals of the EJP-RD goals (max 3):



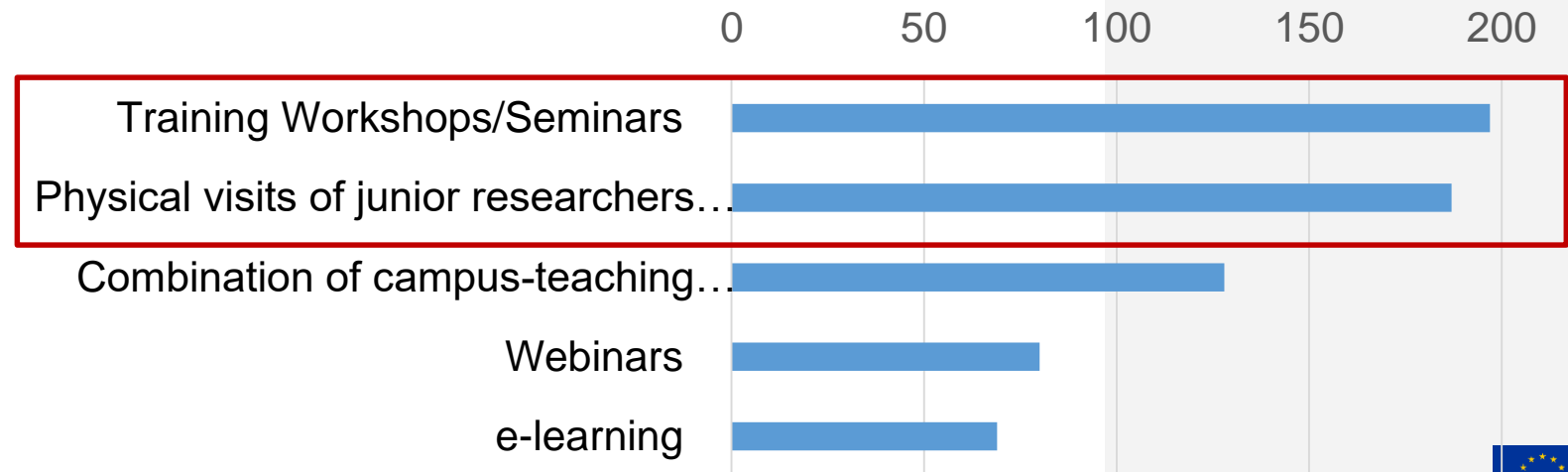
F3. What, in your view, are the most important research skills training domains that need to be addressed to help ERN HCPs raise the level of their research?

All qualified ERN HCP should have these capabilities, as they are part of the selection procedure



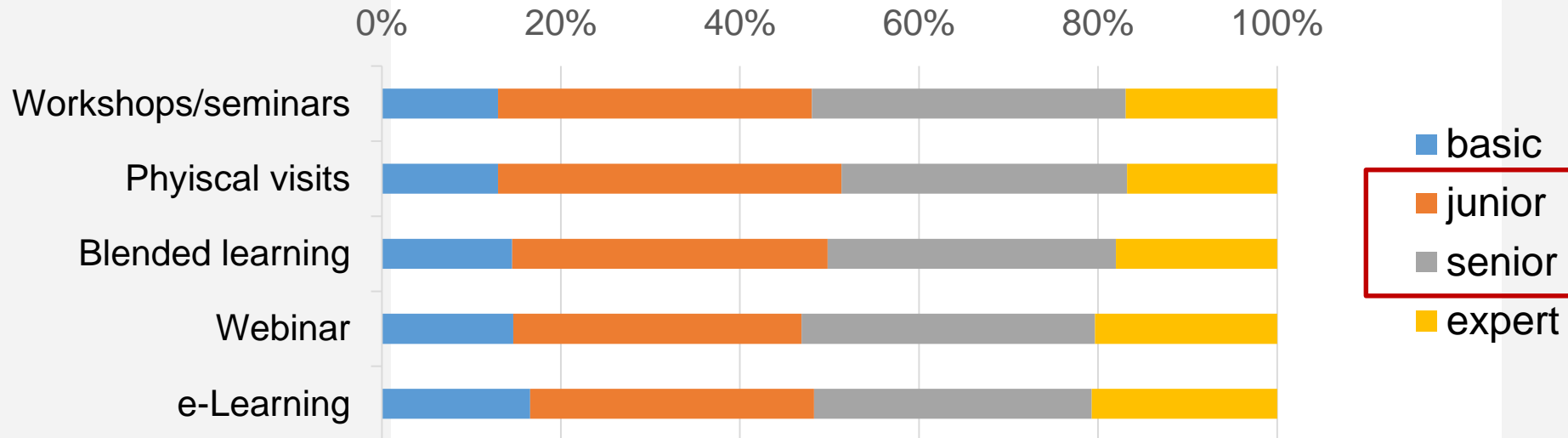
- Interests in technical skills
- Preference for in-person trainings

F4. Which of the following types of training measures would address these domains most efficiently for your group?

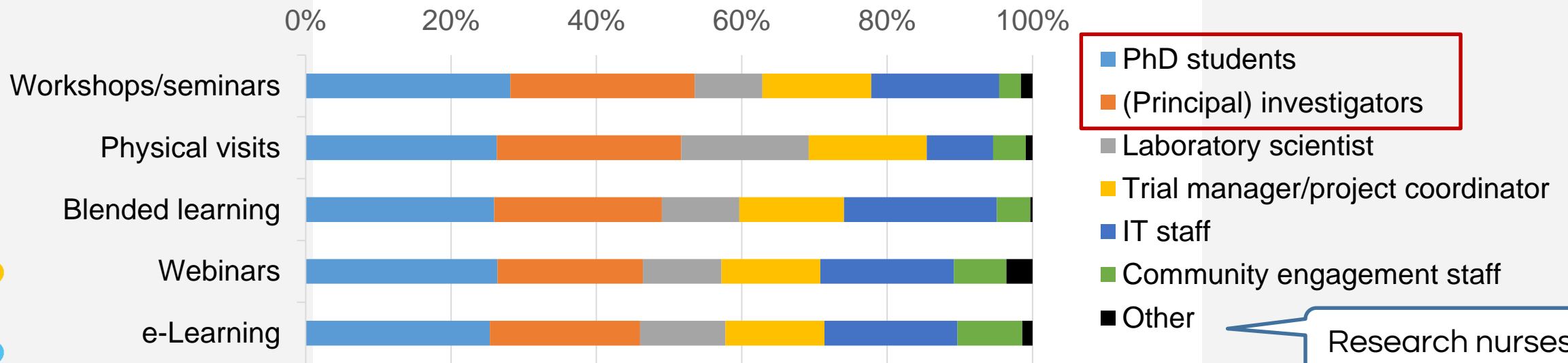


For F2F training methods, preferred group size: <20 participants

What would be the main expertise level of users of these formats?



What would be the main target group for these format in your group?



basic
junior
senior
expert

PhD students
(Principal) investigators

Research nurses



F8. What, in your view, are the most important **opportunity** and **barrier** to equal access to research for countries less or not yet represented in your ERN that can be addressed by research training measures?

OPPORTUNITIES

BARRIERS

Network

Sharing experience

Funding availability

Language

Availability of resource

Heterogeneity

Time to dedicate

Improve research

Standardization

Unequal access to resources

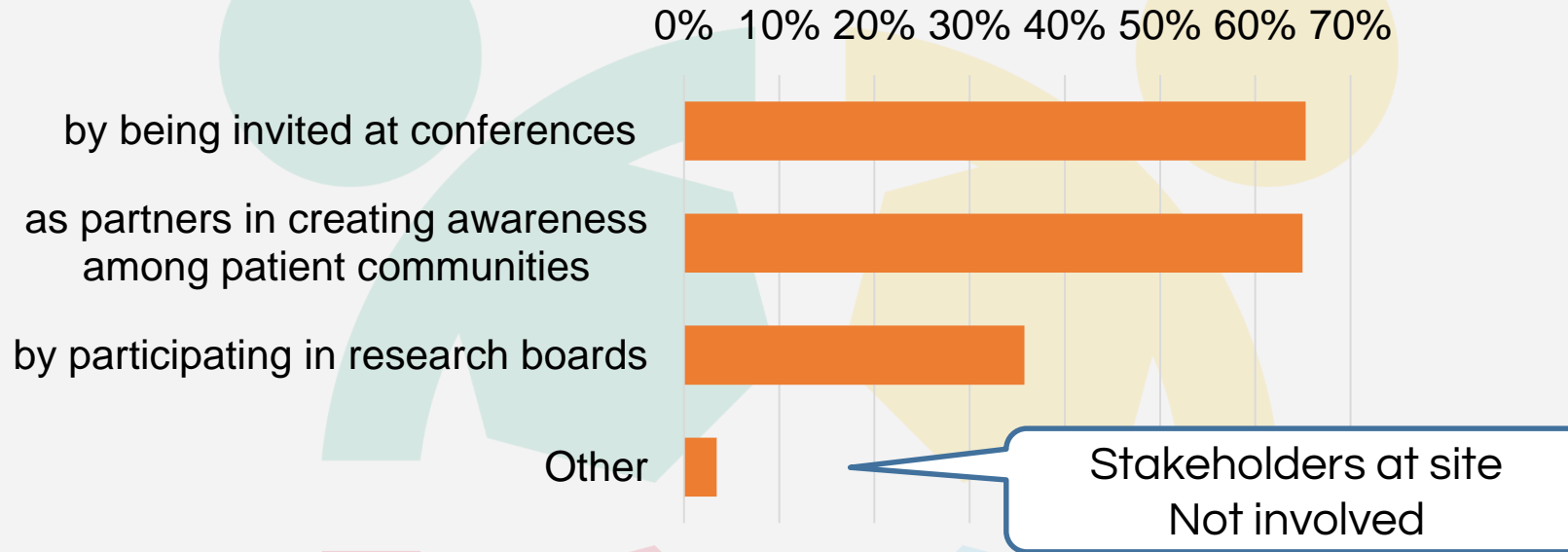
increasing synergies

Sharing experience within ERN

HCP interconnection within ERN is just begging, so results will take time to appear.

differences in resources, non-eligibility of certain countries for some European calls, different funding from national agencies creating inequalities among countries

F6. In what way(s) are patients/patient representatives currently involved as members in your research practice?



OPPORTUNITIES

patient address issues in a different perspective that could eventually enrich the scientific approach to the problem

BARRIERS

geographical distances and psychological involvement
language, lack of experience, lack of patient leaders, different health care system coverage

Attachment 2

Questions for EJP-RD ERN survey

F1. Is there a specific research skills and support training practice currently available in your country or at your local level that might be of interest and transferable to other ERN healthcare providers-

F2. What, in your view, are the most important research support needs to help ERN researchers achieve the goals of the EJP-RD goals (max 3):

F3. What, in your view, are the most important research skills training domains that need to be addressed to help ERN HCPs raise the level of their research- Please pick your top 3!

F4. Which of the following types of training measures would address these domains most efficiently for your group (choose top 3)-

Physical visits: What would be the main target group for this format in your group- [(Principal) investigators, either clinical or non-clinical researchers]

Physical visits: What would be the main expertise level of users of this format:

Training Workshops/Seminars: What would be the main target group for this format in your group- [(Principal) investigators, either clinical or non-clinical researchers]

Training workshops/seminars: What would be the main expertise level of users of this format:

Webinars: What would be the main target group for this format in your group- [(Principal) investigators, either clinical or non-clinical researchers]

Webinars: What would be the main expertise level of users of this format:

e-Learning: What would be the main target group for this format in your group-

e-Learning: What would be the main expertise level of users of this format:

Blended Learning: What would be the main target group for this format in your group- [(Principal) investigators, either clinical or non-clinical researchers]

Blended Learning: What would be the main expertise level of users of this format:

Blended Learning: What would be the most efficient size of training workshops-

F5. In addition to more general training and support needs mentioned above: does your group have any disease group-specific:
training needs-
research support needs-

F6. In what way(s) are patients/patient representatives currently involved as members in your research practice-

F7. What opportunities and barriers do you see to promote patient involvement in the near future-

Major opportunities: Please describe briefly (max 2)

Major barriers: Please describe briefly (max 2)

Please elaborate on how opportunities could be strengthened and barriers overcome:

F8. What, in your view, are the most important opportunity and barrier to equal access to research for countries less or not yet represented in your ERN that can be addressed by research training measures-

Major opportunity:

Major barrier:

F9. Do you think that any form of research skills training and/or research support could help to create this opportunity and overcome this barrier- Please briefly clarify why & how.

Responses from EJP-RD ERN survey

Field summary for F1

F1. Is there a specific research skills and support training practice currently available in your country or at your local level that might be of interest and transferable to other ERN healthcare providers?

| Answer | Count | Percentage |
|---------------|-------|------------|
| Yes (Y) | 49 | 17,44% |
| No (N) | 96 | 34,16% |
| No answer | 136 | 48,40% |
| Not displayed | 0 | 0,00% |

Field summary for F1a

If yes, please specify. What is it and how does it work? What is its added value?

| | | |
|---------------|-----|--------|
| Answer | 42 | 14,95% |
| No answer | 7 | 2,49% |
| Not displayed | 232 | 82,56% |

| ID | Response |
|-----|--|
| 202 | National seminars in idiopathic nephrotic syndrome researches, including all investigators (physicians and saerchers) Disease specific for autoimmune liver disease |
| 232 | Quality of life We have training for new chief and principal investigators, online GCP training |
| 239 | It is useful for trainees and new entrants into clinical research |
| 252 | The Paediatric Rheumatology INternational Trials Organisation (PRINTO) is a not for profit, non governmental, international research network founded 1996. PRINTO initially included 14 European countries (about 90 countries, 654 centres worldwide with 1372 members today), with the goal to foster, facilitate and co-ordinate the development, conduct, analysis, and reporting of multi-centres, international clinical trials and/or outcome standardisation studies in children with paediatric rheumatic diseases (PRD). |
| 280 | Multiple trainings are available, relating to genetics, epidemiology and many other fields of research (see website of Erasmus MC) |
| 289 | Czech branch of ECRIN - CZECRIN, CEPOETA network - cepoeta.org Doctorate in Genetics, Oncology and Clinical Medicine – GenOMeC An International Doctorate program at University of Siena |

Doctorate in Genetics, Oncology and Clinical Medicine (GenOMeC) is an interdisciplinary and International Research Doctorate in genetics and molecular medicine created by a regional network between the three Tuscan Universities (University of Siena, Florence and Pisa) integrating research and educational centres of excellence from all over the world. This link between academic and non-academic basic research and applied research will promote translational medicine and it represents a key element of novelty in the scenario of research, facilitating professional employment of students after PhD. The close interaction between scientific centres of excellence will lead to a sharing of resources, technology platforms, and services in order to develop high quality international scientific projects and bring basic to clinical research. GenOMeC offers cutting-edge research facilities with excellent core facilities for genomics, cell imaging, flow cytometry, bioinformatics, pathophysiology and clinical research, creating an excellent Institution for training and research. The Doctorate is aimed to educate students on the molecular basis and clinical, diagnostic, and therapeutic aspects of monogenic and multifactorial diseases (resulting from the interaction between genes and environment), including cancer.

| | |
|-----|---|
| | GenOMeC intends to address unmet basic and clinical research questions related to rare diseases, in order to increase knowledge in a major medical field that is currently insufficiently covered. In particular, the Doctorate GenOMeC is centred on the study of genetic diseases, with particular focus on hereditary diseases, osteometabolic and connective tissue, metabolism defects, autoimmune and auto-inflammatory diseases, solid and hematological tumors and degeneration, inflammation and cell regeneration processes. Particular attention will be dedicated to personalized medicine through gene therapy by genome editing. The Doctorate offers PhD fellowships open to international recruitment of highly motivated and talented students. These students will be trained to carry out research in these fields over a three years program with up-to-date facilities and in a stimulating scientific environment. At the end, they will be able to plan and develop competitive research proposals. The training program presents opportunities in genetics, immunology, infectious diseases, haematology, nephrology, developmental defects, metabolic diseases/encephalopathy, dermatology and gastroenterology. The program responsible is the Pr. Alessandra Renieri. The Faculty Board is composed by 72 members from 15 academic and non-academic research centres from 6 countries. |
| 311 | ITCC fellowship program |
| 313 | GCP training |
| 332 | There is a lot of it at research active centres; as a Glasgow employee, I have to go to those courses and I wouldnt be able to go to another course. |
| | 1. Dedicated study coordinator, biostatistics and metodologist |
| | 2. A biobank facility |
| 344 | 3. Lab researcher |
| | Animal research training course (RRR) and systematic review course |
| 347 | (Syrclle) |
| 363 | the Coordinating Centre for Clinical trials in University Hospital |
| | Not sure to understand the question |
| 402 | We have in france obligatory training to conduct reasearch projet, with updates every 2 years |
| 435 | Clinical trial center and Biobank organization and SOAPS training for researchnurses |
| | GCP |
| | BROK |
| 449 | statistical courses |
| 451 | PNDS (protocole National de Diagnostic et Soins) sum up about a rare disease medical important information for diagnostic and management for a general practitioner, based on a wide bibliography review. |
| 512 | Survivorship Passport tool use |
| 531 | FCRIN structure that accompanies physicians for designing clinical trials, and also help to improve in general the quality of clinical research, for exemple, onsite visists of clinical research units. |
| | Nurse coaching and training for caregivers for severely disabled patients both kids and adults |
| 538 | Respiratory care and management for kids and adults and specifically there is good skill in NIV launching and secretion management |
| 563 | expertise related to multidisciplinary clinical approach on rare disorders particularly in some specific populations and age groupsand on natural history of very early age at onset disorders |
| 585 | We have a research hub in the UMCG which provides information and templates |

| | |
|-----|---|
| | GCP training is at the national level. |
| 590 | Clinical trial center that conducts all profit trials and some non profit trials for the institution |
| 595 | Long term follow up of CLP patients, surgically, speech and growth |
| 598 | Standardized dosage of serum hepcidin, exosomal ferritin, LPI (labile plasma iron) |
| | Multiorgan iron quantification. Validated and standardized procedure. |
| 631 | The quantification of iron in heart, liver and pancreas has permitted to tailor the chelation therapy and has improved the prognosis. |
| 639 | Clinical trial network |
| 648 | stem cell transplantation facility |
| | Master Degree on Rare Diseases (University of Torino) |
| 658 | Member of Undiagnosed Diseases Network |
| | BROK |
| | GCP |
| 672 | research quality monitor of department |
| 683 | graduate school offers courses of 3ec at postgraduate level |
| | BROK |
| | GMP |
| | GCP |
| | Statistics |
| 694 | ethics |
| 710 | GCP training wide available in UK |
| | Multidisciplinary, psychological and physiotherapeutic continence training in patients with incontinence due to congenital malformations treated surgically. |
| | This can serve as a model to improve function and quality of life in primarily surgically treated patients, and realise the "multidisciplinary team" requirement of the ERNs in a more meaningful way than by just bringing together the different physician's disciplines. |
| 727 | |
| 733 | The German Academy for Rare Neurological Diseases - best practice and workshop based training with focus on rare brain diseases reaching from NGS to imaging technologies and symptomatology |
| 746 | We are working in the biggest academic hospital of the country (Erasmus Medical Center in Rotterdam, The Netherlands) that has many educational possibilities that are also accessible to foreigners. They already make use of that. Everybody is welcome. |
| 750 | as specified in ERN PaedCan |
| 751 | GCP, guidelines from central committee concerning clinical trials with humans, local protocol to conduct research |
| 755 | CIBERER (Spanish NetWare for rare disease disorders) |
| 767 | Pediatric multidisciplinary therapies and surgery in rare disease. experience in more of 40 years |
| 786 | NIHR-funded research training; on-line GCP; increasing embedding of research skills in clinical training programmes for all medical trainees |
| 790 | Animal models of solid and haematopoietic stem cell transplantation |

Field summary for F2

F2. What, in your view, are the most important research support needs to help ERN researchers achieve the goals of the EJP-RD goals (max 3):

| Answer | Count | Percentage |
|--|-------|------------|
| Training stays of research fellows in other ERN research units (1) | 168 | 59,79% |
| Facilitate sharing of data and biosamples (2) | 187 | 66,55% |
| Engage in relevant grant and other funding opportunities (3) | 230 | 81,85% |
| Promote active engagement with the Research Community (4) | 94 | 33,45% |
| Other | 4 | 1,42% |

| ID | Response |
|-----|---|
| 379 | provide the HCPs within the ERNs with some financial support |
| 723 | financial support to hire additional staff for clinical trials |
| 786 | on line navigation resource bringing all regulatory and governance processes and associated documents into one place, regularly updated, with explanatory videos/webinars |
| 791 | Funding, funding and funding |

Field summary for F3

F3. What, in your view, are the most important research skills training domains that need to be addressed to help ERN HCPs raise the level of their research? Please pick your top 3!

| Answer | Count | Percentage |
|--|-------|------------|
| Scientific thinking (1) | 86 | 30,60% |
| Scientific tools and methodologies (2) | 189 | 67,26% |
| Ethics, quality and risk management (3) | 93 | 33,10% |
| Study & Site(s) management (4) | 108 | 38,43% |
| Concrete research skills such as data/database management, laboratory techniques, and clinical research operations (5) | 184 | 65,48% |
| Interactions with public/participants (6) | 31 | 11,03% |
| Other | 8 | 2,85% |

| ID | Response |
|-----|---|
| 301 | funding ! |
| 379 | financial support |
| 390 | Possibility to obtain adequate funding. |
| 527 | funding |
| 587 | help for administrative issues |
| 645 | funding |

| | |
|-----|--|
| 683 | al qualified ERN HCP should have these capabilities, as they are part of the selection procedure |
| 791 | This suggests you have a very low level of expectation of the seem to have a very low opinion of the scientific skills and existing expertise already present with research-active arms of the ERNs. |

Field summary for F4

F4. Which of the following types of training measures would address these domains most efficiently for your group (choose top 3)?

| Answer | Count | Percentage |
|---|-------|------------|
| Physical visits of junior researchers in other laboratories/research groups (1) | 183 | 65,12% |
| Training Workshops/Seminars (2) | 190 | 67,62% |
| Webinars (3) | 76 | 27,05% |
| e-learning (4) | 66 | 23,49% |
| Combination of campus-teaching and e-learning (Blended Learning) (5) | 125 | 44,48% |
| Other | 4 | 1,42% |

| ID | Response |
|-----|---|
| 280 | personal advice |
| 301 | a platform already trained to coordinate trials, take care of regulatory issues, etc, on the European level |
| 379 | money |
| 645 | fundings |

Field summary for F4a

Physical visits: What would be the main target group for this format in your group?

| Answer | Count | Percentage |
|--|-------|------------|
| (Principal) investigators, either clinical or non-clinical researchers (1) | 134 | 47,69% |
| PhD students (2) | 137 | 48,75% |
| IT staff (3) | 48 | 17,08% |
| Laboratory scientist (4) | 91 | 32,38% |
| Trial manager/project coordinator (5) | 87 | 30,96% |
| Community engagement staff (6) | 22 | 7,83% |
| Other, please specify (7) | 8 | 2,85% |
| Other | 9 | 3,20% |
| Not displayed | 98 | 34,88% |

| ID | Response |
|-----|-----------------------|
| 226 | Specialist chirurgici |
| 295 | post-DOCs, physicians |
| 331 | nurses |
| 334 | Research nurse |

| | |
|-----|---|
| 504 | residents/ fellows |
| 653 | nurses |
| 727 | The multidisciplinary team, including physicians, physiotherapists, psychologists, and the nursing professions. |
| 729 | hospital management |
| 784 | Resident fellows |

Field summary for F4b

Physical visits: What would be the main expertise level of users of this format:

| Answer | Count | Percentage |
|---------------|-------|------------|
| basic (1) | 48 | 17,08% |
| junior (2) | 138 | 49,11% |
| senior (3) | 115 | 40,93% |
| expert (4) | 62 | 22,06% |
| Not displayed | 98 | 34,88% |

Field summary for F4a2

Training Workshops/Seminars: What would be the main target group for this format in your group?

| Answer | Count | Percentage |
|--|-------|------------|
| (Principal) investigators, either clinical or non-clinical researchers (1) | 153 | 54,45% |
| PhD students (2) | 138 | 49,11% |
| IT staff (3) | 51 | 18,15% |
| Laboratory scientist (4) | 84 | 29,89% |
| Trial manager/project coordinator (5) | 98 | 34,88% |
| Community engagement staff (6) | 17 | 6,05% |
| Other | 5 | 1,78% |
| Not displayed | 91 | 32,38% |

| ID | Response |
|-----|---|
| 334 | Research nurse |
| 653 | nurses |
| 727 | surgeons, physiotherapists, psychologists, and the nursing professions. |
| 784 | Resident fellows |
| 789 | Administrative staff |

Field summary for F4b2

Training workshops/seminars: What would be the main expertise level of users of this format:

| Answer | Count | Percentage |
|---------------|-------|------------|
| basic (1) | 53 | 18,86% |
| junior (2) | 139 | 49,47% |
| senior (3) | 137 | 48,75% |
| expert (4) | 67 | 23,84% |
| Not displayed | 91 | 32,38% |

Field summary for F4c2

| Training Workshops/Seminars: What would be the most efficient size of training workshops? | | | |
|---|-------|------------|--|
| Answer | Count | Percentage | |
| < 20 participants (1) | 113 | 40,21% | |
| 20-50 participants (2) | 61 | 21,71% | |
| 50 -100 participants (3) | 7 | 2,49% | |
| > 100 participants (4) | 0 | 0,00% | |
| Other | 2 | 0,71% | |
| No answer | 7 | 2,49% | |
| Not displayed | 91 | 32,38% | |

| ID | Response |
|-----|----------------------------|
| 226 | Tavolo di discussione |
| 727 | one to two teams at a time |

Field summary for F4a3

| Webinars: What would be the main target group for this format in your group? | | | |
|--|-------|------------|--|
| Answer | Count | Percentage | |
| (Principal) investigators, either clinical or non-clinical researchers (1) | 62 | 22,06% | |
| PhD students (2) | 47 | 16,73% | |
| IT staff (3) | 26 | 9,25% | |
| Laboratory scientist (4) | 32 | 11,39% | |
| Trial manager/project coordinator (5) | 45 | 16,01% | |
| Community engagement staff (6) | 17 | 6,05% | |
| Other | 1 | 0,36% | |
| Not displayed | 205 | 72,95% | |

| ID | Response |
|-----|----------------------|
| 789 | Administrative staff |

Field summary for F4b3

| Webinars: What would be the main expertise level of users of this format: | | | |
|---|-------|------------|--|
| Answer | Count | Percentage | |
| basic (1) | 26 | 9,25% | |
| junior (2) | 54 | 19,22% | |
| senior (3) | 54 | 19,22% | |
| expert (4) | 35 | 12,46% | |
| Not displayed | 205 | 72,95% | |

Field summary for F4a4

| e-Learning: What would be the main target group for this format in your group? | | | |
|--|-------|------------|--|
| Answer | Count | Percentage | |
| (Principal) investigators, either clinical or non-clinical researchers (1) | 51 | 18,15% | |
| PhD students (2) | 42 | 14,95% | |
| IT staff (3) | 24 | 8,54% | |

| | | |
|---------------------------------------|-----|--------|
| Laboratory scientist (4) | 27 | 9,61% |
| Trial manager/project coordinator (5) | 37 | 13,17% |
| Community engagement staff (6) | 18 | 6,41% |
| Other | 0 | 0,00% |
| Not displayed | 215 | 76,51% |

| ID | Response |
|----|----------|
|----|----------|

Field summary for F4b4

e-Learning: What would be the main expertise level of users of this format:

| Answer | Count | Percentage |
|---------------|-------|------------|
| basic (1) | 24 | 8,54% |
| junior (2) | 45 | 16,01% |
| senior (3) | 42 | 14,95% |
| expert (4) | 29 | 10,32% |
| Not displayed | 215 | 76,51% |

Field summary for F4d4

e-Learning: Please describe briefly the focus and topics addressed with regard to existing or under development e-learning Focus: Research/Medical practice/Other Topics addressed

| Answer | Count | Percentage |
|---------------|-------|------------|
| Answer | 13 | 4,63% |
| No answer | 53 | 18,86% |
| Not displayed | 215 | 76,51% |

| ID | Response |
|-----|--|
| 314 | Clinical trial design and conductance, design of companion diagnostic trials, statistical design of biomarker trials, |
| 444 | ILDs |
| 578 | standard methods for intra-patient comparisons; sharing the collaborators for the project's call |
| 643 | E-learning platform are available/under development focused on medical practice (Case reports) and laboratory supports (sweat test). Data mining Study design |
| 650 | Study coordination |
| 660 | Research/ Medicine practice |
| 707 | Functioning of websites |
| 746 | Public access to specialized information |
| 753 | Research and medical practice in cystic fibrosis Translational research |
| 761 | Topic: rare renal disease and kidney transplant |
| 776 | Ontology, registries, omics and multi-omics data processingrare Research and Medical practice |
| 780 | All rare diseases of our network -OMICs: variants fliktering and annotation |
| 785 | |

FAIR Data

Codification

Field summary for F4a5

Blended Learning: What would be the main target group for this format in your group?

| Answer | Count | Percentage |
|--|-------|------------|
| (Principal) investigators, either clinical or non-clinical researchers (1) | 88 | 31,32% |
| PhD students (2) | 78 | 27,76% |
| IT staff (3) | 35 | 12,46% |
| Laboratory scientist (4) | 50 | 17,79% |
| Trial manager/project coordinator (5) | 73 | 25,98% |
| Community engagement staff (6) | 16 | 5,69% |
| Other | 3 | 1,07% |
| Not displayed | 156 | 55,52% |

| ID | Response |
|----|----------|
|----|----------|

| | |
|-----|-----------|
| 727 | see above |
|-----|-----------|

| | |
|-----|----|
| 780 | nd |
|-----|----|

| | |
|-----|-----------------|
| 786 | research nurses |
|-----|-----------------|

Field summary for F4b5

Blended Learning: What would be the main expertise level of users of this format:

| Answer | Count | Percentage |
|---------------|-------|------------|
| basic (1) | 37 | 13,17% |
| junior (2) | 89 | 31,67% |
| senior (3) | 82 | 29,18% |
| expert (4) | 45 | 16,01% |
| Not displayed | 156 | 55,52% |

Field summary for F4c5

Blended Learning: What would be the most efficient size of training workshops?

| Answer | Count | Percentage |
|--------------------------|-------|------------|
| < 20 participants (1) | 67 | 23,84% |
| 20-50 participants (2) | 38 | 13,52% |
| 50 -100 participants (3) | 9 | 3,20% |
| > 100 participants (4) | 0 | 0,00% |
| Other | 1 | 0,36% |
| No answer | 10 | 3,56% |
| Not displayed | 156 | 55,52% |

| ID | Response |
|----|----------|
|----|----------|

| | |
|-----|-----------------------|
| 226 | Tavolo di discussione |
|-----|-----------------------|

Field summary for F5

F5. In addition to more general training and support needs mentioned above: does your group have any disease group-specific: training needs? research support needs?

| | | |
|---------------|-----|--------|
| Answer | 48 | 17,08% |
| No answer | 233 | 82,92% |
| Not displayed | 0 | 0,00% |

| ID | Response |
|-----|---|
| 202 | yes: idiopathic nephrotic syndromes |
| 226 | Supporto alla ricerca: personale dedicato e investimenti database and registry management, synchronisation |
| 227 | clinical visits to synchronize surgical skills Funding for exploratory trials |
| 232 | Workshops for defining disease outcome surrogate markers relevant for EMA and FDA |
| 252 | no |
| 254 | no |
| 287 | Research support needs |
| 289 | transcriptomic data interpretation |
| 295 | Portal Hypertension, vascular disorders of the liver |
| 314 | Funding, training courses in clinical trial design and statistics |
| 328 | difficult to make compatible with clinical work demands |
| 334 | - |
| 379 | no |
| 387 | Pulmonary Fibrosis |
| 403 | no |
| | Training needs - adult patients, transition, neonatal age |
| | Research support needs - additional training of junior researchers, IT support, data storage and handling, bio samples storage and handling, secretarial and management support |
| 412 | secretarial and management support |
| 444 | I am interested in pragmatic clinical trials design |
| 458 | no |
| 460 | Statistical support training: introducing novel techniques, database management |
| 492 | research support: logistics and administration, funding |
| 578 | none |
| 585 | Contact with other groups working on translational research in vasculitis |
| 618 | no |
| 633 | Research support needs |
| 643 | Training needs funding for salary of post-doc |
| | proposition of collaborative sites into ERN for a academic clinical trial (one principal investigator, other sites co-investigators) |
| 645 | with a safety of the intellectual property by ERN |
| 653 | none |
| 658 | Medical doctors in training |
| 660 | research support needs muscular dystrophies |
| 675 | |

| | |
|-----|--|
| | amyotrophic lateral sclerosis |
| 707 | We would need someone to manage database and to be trained to do this. |
| 727 | none |
| | Help creating awareness, knowledge and interest from the hospital management |
| 729 | Difficult to increase the possibilities of clinical trials in our center |
| | Legislative stipulation to allow (clinical) data sharing in the global context |
| | Validation of therapeutic biomarkers |
| | use of artificial intelligence for genomic (diagnostic) research |
| 733 | Training for research nurses |
| 741 | Statistic support for trial design and data analysis |
| 746 | no |
| 750 | exchange of expertise by offering fellowships |
| 753 | Economic support to attend training sessions |
| 767 | Research Support |
| 776 | Rare eye diseases program |
| 780 | yes |
| 782 | Funding and resources |
| 784 | Funding |
| 786 | Developing innovative study designs to use small patient numbers most effectively |
| | - No other training needs than those above mentioned |
| | - Support could be useful in order to perform genetic analysis, provision of non-refundable drugs and to increase the number of active investigators |
| 787 | research support need. |
| 789 | research support need. |
| 796 | access to efficient genetic testing |
| 808 | The available funding is very competitive. There are many good groups working on relevant problems. If novel solutions for rare diseases are truly wanted, there should be an increase in funding! |

Field summary for F6

F6. In what way(s) are patients/patient representatives currently involved as members in your research practice?

| Answer | Count | Percentage |
|---|-------|------------|
| as partners in creating awareness among patient communities (1) | 182 | 64,77% |
| by being invited at conferences (2) | 182 | 64,77% |
| by participating in research boards (3) | 102 | 36,30% |
| Other | 10 | 3,56% |

| ID | Response |
|-----|---|
| 402 | only in human and social sciences projects |
| 403 | we do not have a patient support group for chILD in Germany |

| | |
|-----|--|
| 405 | not involved |
| 538 | stakeholders at our site |
| 596 | by being involved in guidelines |
| 727 | by critically accompanying our work |
| 767 | Parents of patients |
| 786 | by being consulted as to their priorities for research at the outset, rather than afterwards |
| 789 | Not much |
| 791 | Research grant Cols |

Field summary for F7

F7. What opportunities and barriers do you see to promote patient involvement in the near future? Major opportunities: Please describe briefly (max 2) Major barriers: Please describe briefly (max 2) Please elaborate on how opportunities could be strengthened and barriers overcome:

| | | |
|---------------|-----|--------|
| Answer | 87 | 30,96% |
| No answer | 194 | 69,04% |
| Not displayed | 0 | 0,00% |

| ID | Response |
|-----|--|
| | major opportunities: patients want to be involved in their illness and want to be part of the reflection regarding diagnostic and therapeutic strategies, as well as to know the progress of the research |
| 202 | Major barriers: nothing Opportunities: patient engagement, study patient topics. |
| 212 | Barriers: language, travel costs |
| 225 | organization problems -delineare i veri bisogni , -impegnare il Loro tempo |
| 226 | |
| 231 | None Qualified patients do not have the time, voluntaries are often either rather biased and not so easy personalities, and many patient organizations are funded by industry, and run by professionals rather than patients. |
| 232 | |
| 236 | Barriers: funds/ organization/ time |
| 246 | Doctors tend to exclude the patients from the knowledge on their disease Opportunities: European networking in the field of rare diseases |
| | Barriers: Language barriers/ divisions among different organizations |
| 252 | |
| 254 | Very few patients in VRT groups ... |
| 258 | barriers: language and fundings |
| 280 | They feel not capable to participate in any part of research, despite all attempts to get them engaged. They also say they lack the time. barrier - language barrier for majority of people |
| 289 | |

| | |
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| | opportunity - numerous. |
| | Excellent collaboration with the National Alliance - www.vzacna-onemocneni.cz |
| 291 | Lack of funding Opp: a better knowledge of the daily-life patient needs |
| 300 | Barr: not always the needs perceived as priority are relevant to the cure of the disease Major opportunities: willing of parents/patients Major barriers: MD who don't wish it |
| 311 | Parents/patients participation to the groups opportunities: personal experience led to design of exploratory studies |
| 313 | barriers: acceptance by medical community |
| 328 | language barriers as all activities require English proficiency |
| 331 | Collaboration patient organisations Opportunities: increasing engagement of individual patients and patients associations in research |
| 334 | Barriers: regulations, lack of habit One of the most important problem in clinical trials is the placebo arm. Rare cancer patients require therapy and placebo is usually associated at patient's discomfort For traslational and pre clinical research the problem is the sharing of the experimental results or cell lines However the research both clinical and preclinical in rare cancer is a very open field reach of opportunity |
| 344 | opportunity; ePAG system |
| 347 | barrier; Cost of work loss reimbursement for abcense and travel costs of patient representatives |
| 352 | Lack of time! |
| 363 | the opportunity: to share the information in patient associations opportunity to collaborate within EndoERN |
| 379 | barrier: no funding, even not to go to meetings; so they give up |
| 382 | Increasing awareness so that patients come fireard Major opportunities: patient perspective and needs. |
| 387 | Major barriers: patient bias -direction of research -development of better care through proms barrier |
| 394 | funding opportunities: to develop broad support in targeted patient population for this type of research |
| 395 | Barriers: ? I do not see any barriers MO: To adaot reaserch to patients needs and wishes |
| 402 | |

| | |
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| | MB: Difficulties to understand our world |
| | To overcome: promote expert patients, trained to understand the mode of thinking of doctors and researchers |
| 403 | Patient support group needed |
| | Opportunities: |
| | ePAGs, yPAGs - professional coaching, inclusion in research boards |
| 407 | Barriers: lack of time for serious involvement, narrow focus on own history while representing broad spectrum of conditions |
| | There are a lot of opportunities, but lack of wish to participate, to influence |
| 409 | Promoting such initiatives as Song |
| | major opportunities - new treatments; life-saving medications |
| | major barriers - lack of time at a busy clinical setting; less confidence in official medicine tools and success |
| 412 | Measures - using media methods to raise confidence and attract attention; reimburse time of clinicians for research work; use the Network channels for quicker and timely information about trials/new medications |
| | Major opportunities: Patients become more e-educated and trained, CPMS and international collaboration of patient groups stimulates future research involvement. |
| | Major barriers: rarity of cases and patient organisations in small countries |
| 434 | Rare disease networking promotes collaboration and patient oriented activities. |
| | Opp: Recruitment |
| 436 | Barriers: Recruiting enough patient representatives |
| | Opportunities: see real need if the patients/Create confidence |
| 444 | Barriers: Lack of knowledge how research works |
| | - difficult to engage patients in basic research with long term outcome |
| | - the profile/focus of the science group mismatches with current clinical patients' needs |
| | - difficult to find patients that represent a heterogeneous patient group |
| 449 | |
| 451 | Major barrier: foreign language especially if English native do not pay attention the way they speak. |
| | major opportunity: development of more clinically relevant studies, with earlier benefit for the patient (PCOM) |
| 458 | major barrier: legal representation of patient: not as a patient, but as a lay men expert. Vulnerability of the patient |
| | Advantages. Patient-related Outcome measurements, identification of Problems not yet addressed in Research |
| 487 | Barriers: Ethical considerations; delay of projects |
| | opportunities: data and sample collection - patient centered research outcomes |
| 492 | |

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| | barriers: rare disorders - patients from all over the world should be able to connect efficiently |
| 527 | Barriers: knowledge of patients, applicability of an individual patients interests |
| | Major opportunities: Lay/patient address issues in a different perspective that could eventually enrich the scientific approach to the problem |
| 547 | Major barriers: patients associations most times focused on therapies in a short term results and sometimes the most promising research it is not always the shortest road |
| | Major opportunities : design of trial (endpoint - parameters) |
| 551 | Major barriers : possibility to be very proactive (travel, and problem of language) |
| | Opportunity: patients has the most information about the phenotype, existing PAGs are supporting the research |
| | Barriers. they are frequently not able to describe the detailed clinical problems, the have not enough health literacy, many of the rare diseases do not have PAG |
| 574 | More citizen health education has to be performed, PAGs should be established in more disease group |
| | - patient's understanding of the importance of adequate monitoring |
| 578 | - more personnel for an adequate patient's monitoring |
| 585 | Joun us at meetings |
| | opp: Understanding their needs/adapt our vision |
| | Bar: discrepancies in culture of sciences for many |
| 606 | Working more with the patients' group |
| | Research following the needs of patients |
| | QoL and PROM's |
| 626 | Less interest in causes of disease |
| | Opportunities: patient awareness, patient compliance. These opportunities could be strengthened organizing specific events for improving patients' awareness and compliance. |
| 631 | Barriers: patients associations not unified; need to establish a regulation for patients associations in rare diseases. Funding; need to obtain funding from National and European Institutions. |
| | Psychological discomfort when to visit participating physician from both sides-when to be a doctor-patient,when to be a partner. |
| | Time schedule for meetings. |
| 633 | Patients scare of "right decision" making. |
| | Major opportunity: society willing |
| 637 | Major barrier: MD resistance |
| | Major opportunities: involvement in patient reported outcomes; ethics of research |
| 639 | • Major barriers: multiple clinical trials for a limited number of patients; |

| | |
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| | unequal access to health care over Europe |
| | Major opportunities: the active engagement of patients may impact on care decision and identify urgent medical options for clinical trials programs. Major barriers: patients decisions may diverge from research opportunities. N-of-1 trials could be to promote for engagement of more patients in the near future. |
| 643 | |
| 645 | major opportunities: find patients, knowledge of the diseases by the patients, and fundings by associations Engagement in all clinical research phases is increasing and patients' associations involved in many actions. Involve patients in clinical trial design and guidelines development Major barriers patients' associations still lacking for some "common" rare diseases and for many very rare diseases. |
| 649 | Finding resources for running activities still a major problem Major opportunities: identifying clinically relevant endpoints and critical revision / interpretation of the results |
| 650 | Major barriers: ethical issues Major opportunities: relatively high numbers of patients, accesibility. Major barriers: small patients associations with not much experience. I think both health providers and patient associations should work for a more prominent role of patients in research. |
| 651 | It would be interesting to increase number of research calls in which participation of both researchers and patients were required. Opportunity: Sharing the design of research projects and clinical trials |
| 652 | Barrier: Scattering of involved people Opportunities: participation in conferences and workshops Barriers: insufficient level of knowledge on the biological nature and evolution of their disease We can strengthen opportunities and overcome barriers by actively educating our patient communities on the latest developments basic and applied science has to offer to them. |
| 653 | |
| 658 | None involvement is essential |
| 661 | barriers: time and training Patient organisations need time and money to be involved. patients could be more involved in clinical practice research |

| | |
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| | patients could be more involved in defining study end-points |
| | MO: find cases, drive patients into the ERN network-make people understand that ERN centers are chosen to e in ERN as they provide more resources for patients |
| 707 | MB: missing information about ERN-share information about ERN major opportunities: to involve patients associations |
| 711 | |
| 715 | The patients that are involved in general funding, are often patients from large patients organisations, not rare diseases |
| 722 | Major barrier: we do not have a patient organization including all the disease managed by our subgroup in the ERN |
| | Major opportunities: that the ERNs require patient involvement from the clinicians , and that also national authorities have to accept their pivotal role via the ERNs. |
| | Major barriers: in the field of rare congenital malformations needing surgical treatment, the vast majority of paediatric surgeons strongly opposes the necessary centralisation of patient care, which is called for by the patient representatives. So they try to keep them out. |
| 727 | The patients should receive a secured seat and vote in the ERNs (presently they are well-tolerated, but not a formal partner, as you can see in the organograms of the different ERNs), with the opportunity to send teams to the ERN clinics to check the degree of fulfilment of the operational criteria, and report directly to the national and European authorities. |
| 729 | Limited information in Portugueses |
| | Opportunities: - Development of PROMs for clinical trials |
| 733 | Barriers: - Appropriate funding for involvement of patients; - identification of fitting / appropriately qualified patients |
| | Major opportunities: Education: to give patients all the important decisions about their cure path in a simple manner. To strenght-->to organize ad hoc multidisciplinary meetings answering their questions and listening their needs |
| 741 | Major barriers: Different languages could represent a barrier and not always the cultural mediator is available. To overcome-->to make aware the health system of this problem |
| 746 | to make the patient owner of his data using a personal health environment |
| 750 | The paediatric cancer community works in close collaboration with Childhood Cancer International - European Branch. This community is strongly involved in a broad range of activities in fostering research, participating in local and high-stakeholder discussions |
| 753 | cross infections among cystic fibrosi patients |
| 761 | look at ERKnet Registry |
| | Internet /Mail / w-up |
| 767 | Financial |
| 776 | There are no such traditions in our state, but we have started to work with patients, and helped to establish patient parent's organizations. We have early vision rehabilitation program for blind and low vision children. |
| | Opportunities: A better dissemination of the information concerning research programs and a better understanding of what are the most important outcomes for the patients |
| 780 | Barriers: sometimes an absence of communication between the patients |

| | |
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| | representatives of the different countries |
| 782 | Major barriers: resources and financial support to include patients in the different phases Major Opportunities: Adherence of Patients to trials and therapies Major barriers: geographical distances and psychological involvement |
| 784 | Improve inter-centre collaboration and data sharing. Development of a protocol clearly understandable by the patients. When needed, psychological support Opportunities: ePAGs - embedded in ERNs; national societies federating to European umbrella group to increase visibility and voice Barriers: very rare conditions - hard to get sufficient members to sustain momentum. Lack of consultation by industry, in part reflecting the rules protecting patients from direct marketing. |
| 786 | there needs to be a more effective communication of patients needs to both medical professionals and industry, with opportunity for dialogue around "difficult issues". |
| 787 | - Patients should be informed about all the aspects of the disease, and some disease-specific informative booklets will soon be available for this purpose. The creation of patient's groups and associations, also independent from the hospital setting, can improve patient's involvement and quality of life - Logistic problems and communication barriers (linguistic and technological): they can partially be removed by improving active follow up and patient's disease specific groups and organizations. |
| 788 | Major opportunity; promotion and better understanding of the study |
| 789 | Not so many barriers. Patients are willing to get involved. the barriers lie on the difficulties of the HCP to provide the infrastructure to start clinical trials and support enrollment by already burdened staff. It could be useful to share information on which trials are open and where so experts can send patients in different centers of the network (newsletters, website...) The opportunities can be enormous: in many areas there are several new drugs or treatment approaches (gene therapy and gene editing). |
| 796 | Major opportunities: increasing number of patients, increased level of knowledge about the disease Major barriers: time to make the genetic diagnosis, short available time from patients to participate |
| 808 | The involvement of PAGs in the ERNs is very helpful and helps to establish patient-researcher-physician collaborations. It's also inspiring and helpful to meet patient representatives at conferences. |

Field summary for F8

F8. What, in your view, are the most important opportunity and barrier to equal access to research for countries less or not yet represented in your ERN that can be addressed by research training measures?
Major opportunity: Major barrier:

| | | |
|---------------|-----|--------|
| Answer | 71 | 25,27% |
| No answer | 210 | 74,73% |
| Not displayed | 0 | 0,00% |

| ID | Response |
|-----|--|
| | Major opportunity: developing clinical and translational researches |
| 202 | Major barriers: financial support To obtain experience. |
| 212 | Barrier: costs |
| 225 | lack of resources |
| 226 | Finanziamenti Major opportunity: european networking. ERN |
| 252 | Major barrier: lack of facilities, organization, resources in some countries opportunities: additional cohorts of the patients, practical experiences, fresh ideas |
| 265 | barriers: undernourished staff and equipment, historical lack of research practice |
| 280 | They don't even have the opportunity to provide the surgical care at the required level, so research is not first on their mind |
| 289 | barrier - language, traveling, lack of recognition that the standard of care in pediatric oncology should be participation in academic clinical trials. opportunity - numerous Learning from experts |
| 291 | Establishment of ERN resources Opp: improve access of patients to innovative therapies |
| 300 | Barr: low income countries have urgent needs not covered |
| 311 | Major barrier: money |
| 328 | Research needs funding Opportunity: increasing synergies |
| 334 | Barrier: differences in resources, non-eligibility of certain countries for some European calls, different funding from national agencies creating inequalities among countries |
| 344 | I think in some ERN areas there are not the same facilities present in other countries Major barriers; Endorsement as Affiliated Partners by National Ministry of Health. |
| 347 | Reimbursement for participation costs. the major opportunity to reach research with European partners |
| 363 | the main barrier is the lack of financial support |
| 387 | Major opportunity: common protocols |
| 394 | management of research/cohorts |
| 395 | opportunity: ERN network |

| | |
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| | barrier: countries may not have research infrastructure and therefore may not have access to .. |
| | Major opportunity: Improvement of skills by sharing knowledge |
| 407 | Major barrier: Brain drain - Researchers moving permanently to host countries, thereby aggravating lack of resources in their countries |
| | Awareness |
| 409 | Lack of research group |
| | major opportunity - research seminars/workshops; blended learning |
| 412 | major barrier - lack of financial resources to the Network; unclear financial involvement of the Member States |
| 434 | Major opportunity is the strive to international collaboration and the barrier-lack of resources and experience in basic, -omic research of clinical specialists. |
| 436 | Not sure |
| | MAjor opportunity: a better understanding of the disease (more global) |
| 444 | Major barrier: the different levels of expertise. |
| | major opportunity: to decrease the existing inequalities in research needs |
| | major barrier: legal issues, patient emancipation, implementation and periodical control of GCP and GDPR |
| 458 | opportunity: greater study Population; |
| 487 | barrier: Language and regulatory aspects |
| | Major opportunity: coming to sites to see as fellows with specific grants |
| 538 | major barrier: implementation of SoC |
| | Major opportunity: Sharing experience within ERN |
| 547 | Major barrier: HCP interconnection within ERN is just begging, so results will take time to appear. |
| | opportunity of widening expertise and becoming interested in sharing data |
| 563 | barrier time expenditure |
| | - sharing multi-language collaborators |
| 578 | - too much different HCP organization, no time and language barrier |
| | Differences in knowledge and funding possibilities |
| 585 | Different in legislation |
| | opportunities: Transborders case discussions |
| | migrating courses and elearning |
| | Bar: Language |
| 606 | initiative of juniors to participate in such courses and finally budgets |
| | more numbers; more different views to research |
| 626 | language and resources |

| | |
|-----|--|
| | Major opportunity: training, workshops, e-learning, campus |
| 631 | Major barrier: funding |
| 633 | I have no experience on it. Barrier: Clinical trials from pharma companies for rare diseases usually do not involve Romania, however this seems to change over the last years. Academic research is hampered by funding as well as background of the researchers that is usually less than that of other Universities in Europe. Again this is changing in the last years. |
| 636 | Major opportunity: Romania has a relatively large population of almost 20million and many patients. Geneticists have increasing skills in research as technologies become more available. |
| 637 | Major barrier: money major opportunity: data sharing,; skills sharing |
| 639 | major barrier: unequal access to health care over Europe Major opportunities : e-learning platforms including training programs have to be implemented to equal access those countries that are not represented in ERN LUNG |
| 643 | I Don't know: we firstly need to work |
| 645 | maybe post-doctoral exchanges Important to extend studies and reach less represented / advanced countries. This can increase recruitment in clinical trials and improve standard of care for patients. |
| 649 | Major barriers: funding, paucity of local resources. Major opportunity: networking, availability of new drugs for rare conditions |
| 650 | Major barrier: local restriction in data sharing I do not really know. I do not have enough information of countries not included in our ERN to tell. Our ERN covers most of main countries in Europe. Those not included are countries from which I do not know much about their research in the topic. |
| 651 | Opportunity: Teleconsultation and samples exchange |
| 652 | Barrier: Costs Opportunity - to get them involved |
| 653 | Barrier - the different local "successful business prerequisites" in terms of scientific funding, legal and ethical practices etc. Major opportunity: networking |
| 658 | Major barrier: heterogeneity in HC systems |
| 661 | involvement with ERN spread collaboration and involvement |
| 675 | find a common language Major barrier: money, time, and skills training |
| 683 | major opportunity: expand patient database |

| | |
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| | MO: understand how advanced countries manage rare disease and cooperate with small series of patients that would not be helpful for develop own research |
| 707 | MB: no way to share cases and to maintain privileges on cases or participate in authorship, no instrument to encourage sharing cases support researchers |
| | systematical data collection |
| 711 | access to online systems of sharing data |
| | Major opportunity: most of the colleagues are eager to close up to the standard in research and treatment the richer and bigger countries have. |
| 727 | Major barrier: budget cuts in the bigger and richer countries make the physicians and researchers their work at their limits, and sharing with colleagues on top of it is only possible if additional funding is provided by the politicians - no political will to do so is recognisable at the moment |
| | major opportunity - help these countries and HCPs to create a proper infrastructure |
| 729 | major barrier - lack of human resources and funding |
| | Opportunities: - joint projects to raise level of quality of research / training - can be addressed through better equipped fellow exchanges (including consumables) limited to those countries |
| 733 | Barriers: - Identification of research groups that can use the training to sustainable raise research capacity |
| | Major opportunity: to avoid health migration |
| 741 | Major barrier: easily these countries are weaker (less number of patients, less human resources, less money) |
| 746 | information and knowledge should be shared by educating each other and welcome each other |
| | opportunity: twinning programmes to foster research are very much needed; |
| 750 | barrier: limitations in local resources (personnel limitations in clinics as well as in research) |
| 753 | Financial support as major barrier |
| 761 | look at ERKnet Registry |
| 764 | training fellowships |
| | Internet |
| 767 | Financial |
| 776 | Funding of special activities in less research intensive countries |
| | To develop a better communication and valorization of the ERN- networks by the member states (with or without HCPs) and the EC |
| | An harmonization and simplification of the administrative rules (for instance for the development of new therapeutic protocols with the companies (or not) |
| 780 | Financial support |
| | Opportunity: involvement of EU population in trials |
| 782 | Barriers: different legislations |

| | |
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| | Major opportunity: bring together data from different groups allows a faster reaching of statistic significance of results |
| | Major barriers: differences in funds availability in the different countries; lack of exchange of ideas and projects. |
| 784 | Opportunity: blended eLearning allows remote access that can be supplemented with face-to-face activity - less costly in both money and time, allows progress intermittently |
| 786 | Barrier: underpinning capacity and local research infrastructure |
| 787 | The lack of technological infrastructures and of dedicated researchers, the lack of specific goals for the reaserch and a fair organization and program. Improving them, in my opinion, the number of involved HCP will gradually rise. |
| 789 | The major barriers are lack of knowledge on grant writing in certain countries and centers, lack of information on upcoming calls beforehand |
| 796 | Major opportunities: increasing number of patients, increased level of knowledge about the disease |
| 808 | Major barriers: financial support, short available time from patients to participate |
| | I'm not sure research training measures are the right solution for this problem. First, the ERNs need to be firmly established and sufficiently financed. At the moment it hinges on the willingness of expert leaders in a field to make time for the collaborative effort, often in their free time. Adding more to this already very full work schedule would not necessarily help. It needs funding to pay for personnel, that does the research, does the training, does the patient care. |

Field summary for F9

F9. Do you think that any form of research skills training and/or research support could help to create this opportunity and overcome this barrier? Please briefly clarify why & how.

| | | |
|---------------|-----|--------|
| Answer | 60 | 21,35% |
| No answer | 221 | 78,65% |
| Not displayed | 0 | 0,00% |

| ID | Response |
|-----|--|
| 202 | webinars are suitable |
| 212 | Training will help in choosing the best topic to be studied given the limited resources |
| 222 | yes |
| 225 | more GCP and clinical trials training |
| 226 | arrange courses |
| 231 | Investire per creare nuove opportunità di ricerca che si possa autofinanziare |
| 265 | yes: the inclusion in the multinational trials, the financial support and guidance in management of the trials the education of the young researches in the experienced research centers (but unfortunately they get good offers and they do not come back to the |

| | |
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| | poorer home country...) |
| 280 | not at this time |
| 289 | the best way are personal visits and later on joint projects with leadership of more experienced centers |
| 300 | Invest in grants that include always low income countries |
| 301 | what is needed is a common platform to coordinate all aspects of clinical trials. I am afraid any other "minor" support will not make any difference |
| 328 | Funding |
| 334 | Yes but just partly, I see the major barriers the fact that not all European countries are eligible in all European calls as well as the fact that some countries allow for applying for personnel and certina costs and some others do not. This is a major problem and source of inequalities. |
| 344 | Yes i do. I think that the access to some facilities or trianing can help to overcome these barriers |
| 347 | Financially supported combined research programs for ERN Members and Affiliated Partners. |
| 363 | yes, any form will help |
| | Yes |
| 387 | Could be the opportunity of homogenate protocols and research methods. |
| 395 | Training certainly helps to improve the awareness. |
| | - Short term visits |
| | - Guaranteed domestic career plan after return to home country |
| 407 | Making research attractive for young people |
| 409 | Better training in the university acquiring research knowledge and scientific thinking |
| 412 | Yes, if young and more senior researchers are given the opportunity for training at advanced settings. This will overcome differences in a quicker and structured way. |
| 434 | I think it could based on exmples from our country, when research initiatives coupled with gained skills created efficient research groups and competence centres. |
| 436 | Not sure |
| 444 | Yes, i think that webinar and e-learning methods could improve that. |
| 458 | yes, the physical visits, the inetractive workshops, and the blended learning |
| 547 | The clinical interconnection is already moving but a special effort should be done for research support, with a special program of R&D programs for ERN |
| 563 | yes all |
| 578 | Yes, but it is very difficult. Several Italian HCPs are not so organized and/or prompt to share research with other ERN-EYE of the rest of Europe; an official EU document addressed to HCP general manager (also translate in Italian language) about the ERN-EYE Center missions for the sharing of clinical data and research could be very useful |
| 585 | Yes but it should be also discussed at another level. |
| 626 | yes, involving more countries and patients itself gives more insight in needs and different views. |
| 631 | Yes. See point F8 |
| 633 | Definitely help and improve skills for research,training. |
| | Funding |
| 637 | Fellowships |
| 639 | this will allow to build a research network on specific area of research all over europe |
| 643 | Blended learning and e-learning platforms may support research and |

| | |
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| | clinical training implementing distance education with important consequences on shared fast learning over the countries. |
| | fundings for post-doctorants |
| 645 | post-doctoral exchanges |
| | Yes I think so. Formation is a major tool for improving research and creating collaborations and connections. |
| | Any step forward in the ERN functioning requires funding (there are no funds but for coordination, funding definitely insufficient to guarantee survival of ERNs) |
| 649 | international networking of referent centres |
| 650 | shared IT platforms for data and samples sharing |
| | Yes. I do think ERN structure is a fantastic opportunity to address ambitious research projects. |
| 651 | Needs are essentially, staff, time and funding. Also training in particular tools and platforms, but the budget should include provision for research staff. |
| 653 | Yes. The forms of basic research training were listed above and all are feasible - namely researcher visits, workshops, seminars and webinars. That is why all of them should be tried. |
| 658 | ERNs are meant to connect centres with different expertise within a some focus, with the final aim of harmonise clinical care in EU via networking and filling clinical and legislative gaps |
| 675 | because can overcome theoretical and practical obstacles by disseminating shared experience and knowledge |
| 683 | training is important, but is only sensible if followed by implementation (and thus investment) |
| | Anonimous database and authorship involmente even if you can provide few cases and low scientific support. |
| 707 | This would encourage small/less advanced centers to cooperate. |
| 727 | Of course, doing a little bit is far better than nothing. |
| | Not only medics and health professionnels need training, also the management staff to understand the needs and allow things to happen and multidisciplinary teams to work. |
| 729 | Initiatives targeting the hospital management staff also would be important |
| 733 | better equipped fellow exchanges (including consumables) limited to those countries |
| 741 | We don't think so because some problems are not affordable (e.g. minor economic resources) and nevertheless we think the skills and the highess expertise should be centered in few very specialised networked centres |
| 746 | open access to knowledge and information, professionals with expertise and tools |
| 750 | yes, training and research opportunities will foster the level of care and improve childhood cancer outcomes in widening countries and in particular in those with low health expenditure rates |
| 753 | Yes. In favour of sharing experiences |
| 767 | Come in expert Centre and ti See how manage rare disease , learn on Site ; collaboration with expert Centers |
| 776 | Yes, if there is funding for networking |
| 780 | Financial support would be very helpful and communication |
| 784 | Yes, skill training for young scientist and funds to bring back to the country of origin skills and methods learnt in other countries. Opportunities of personalized tenure tracks in order to not loose expertise. |
| 786 | Yes, but it will take time to build that capacity - probably 5-10 years |
| 787 | Creating a solid research community, with trained scientists, appropriate means of communication and supplies, and with defined research projects |

will lead to an improvement of research quality and the enhancement of each HCP

789 Yes; it would be usefull to have short training on upcoming research grants and calls, newsletters, alerts...

796 Yes, it could help us simplify procedures

808 See F8.