Key Actions Developed by the ERN Coordinators Group and the Networks

Franz Schaefer

Heidelberg, Germany

Chair, ERN Coordinators Group

Coordinator, European Reference Network for Rare Kidney Diseases (ERKNet)

I have no disclosures to make
with respect to the contents of this presentation.

Harmonizing disease management:
Adoption and development of guidelines and pathways

Monitoring performance and outcomes:
Patient registries

Promoting **research and innovation**

Online disease information

Virtual Consultation:
Clinical Patient Management System

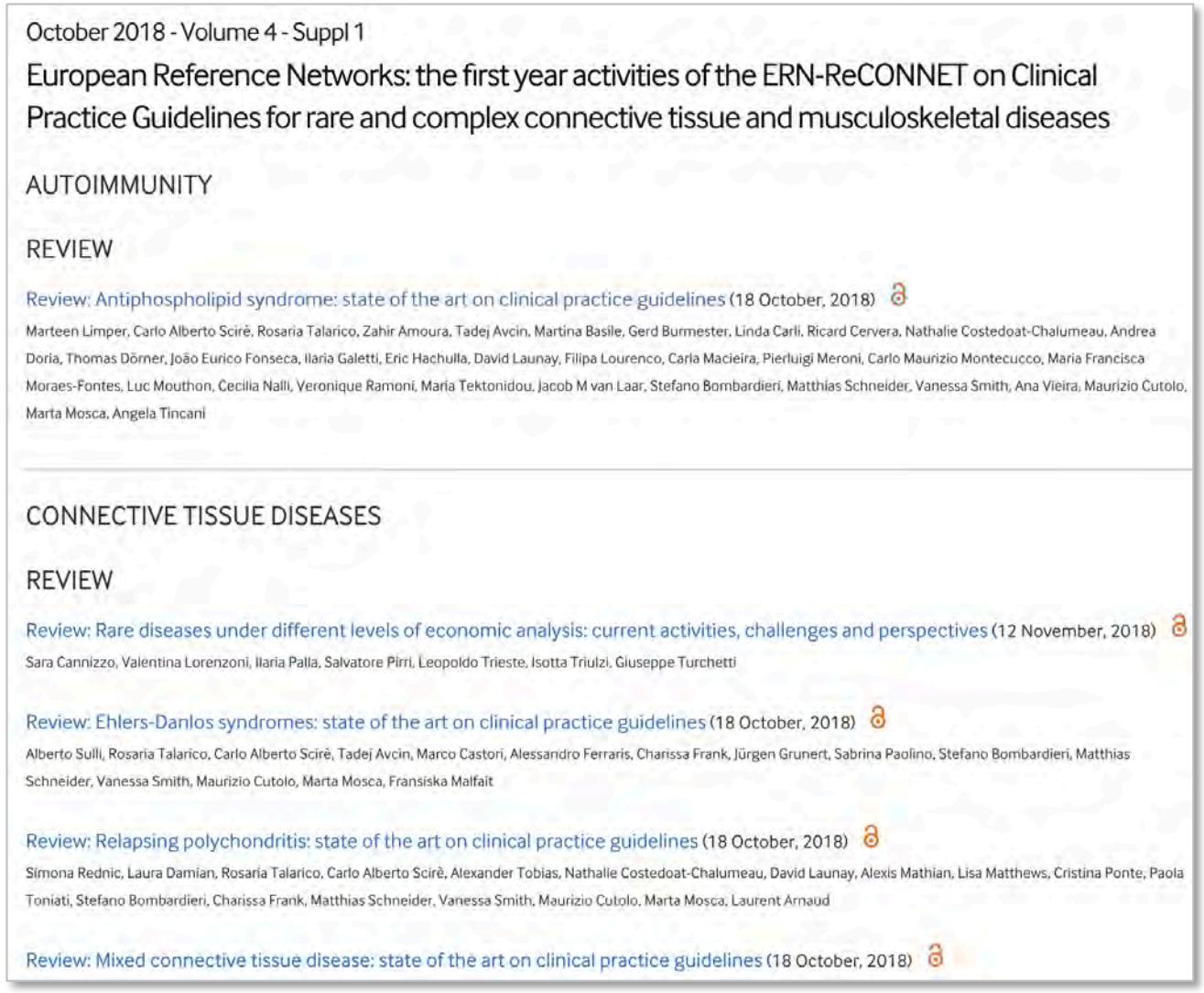
Training:
CMEs,
Webinars, eLearning

Clinical Guideline Activities

European Reference Networks

Review of Existing Evidence Base

e.g. ReConnet



October 2018 - Volume 4 - Suppl 1

European Reference Networks: the first year activities of the ERN-ReCONNET on Clinical Practice Guidelines for rare and complex connective tissue and musculoskeletal diseases

AUTOIMMUNITY

REVIEW

Review: Antiphospholipid syndrome: state of the art on clinical practice guidelines (18 October, 2018)

Marteen Limper, Carlo Alberto Scirè, Rosaria Talarico, Zahir Amoura, Tadej Avcin, Martina Basile, Gerd Burmester, Linda Carli, Ricard Cervera, Nathalie Costedoat-Chalumeau, Andrea Doria, Thomas Dörner, João Eurico Fonseca, Ilaria Galetti, Eric Hachulla, David Launay, Filipa Lourenco, Carla Macieira, Pierluigi Meroni, Carlo Maurizio Montecucco, Maria Francisca Moraes-Fontes, Luc Mouthon, Cecilia Nalli, Veronique Ramoni, Maria Tektonidou, Jacob M van Laar, Stefano Bombardieri, Matthias Schneider, Vanessa Smith, Ana Vieira, Maurizio Cutolo, Marta Mosca, Angela Tincani

CONNECTIVE TISSUE DISEASES

REVIEW

Review: Rare diseases under different levels of economic analysis: current activities, challenges and perspectives (12 November, 2018)

Sara Cannizzo, Valentina Lorenzoni, Ilaria Palla, Salvatore Pirri, Leopoldo Trieste, Isotta Triulzi, Giuseppe Turchetti

Review: Ehlers-Danlos syndromes: state of the art on clinical practice guidelines (18 October, 2018)

Alberto Sulli, Rosaria Talarico, Carlo Alberto Scirè, Tadej Avcin, Marco Castori, Alessandro Ferraris, Charissa Frank, Jürgen Grunert, Sabrina Paolino, Stefano Bombardieri, Matthias Schneider, Vanessa Smith, Maurizio Cutolo, Marta Mosca, Fransiska Malfait

Review: Relapsing polychondritis: state of the art on clinical practice guidelines (18 October, 2018)

Simona Rednic, Laura Damian, Rosaria Talarico, Carlo Alberto Scirè, Alexander Tobias, Nathalie Costedoat-Chalumeau, David Launay, Alexis Mathian, Lisa Matthews, Cristina Ponte, Paola Toniati, Stefano Bombardieri, Charissa Frank, Matthias Schneider, Vanessa Smith, Maurizio Cutolo, Marta Mosca, Laurent Arnaud

Review: Mixed connective tissue disease: state of the art on clinical practice guidelines (18 October, 2018)

Clinical Guideline Activities

European Reference Networks

Review of Existing Evidence Base

e.g. ReConnet

Evaluation and endorsement of existing guidelines

e.g. ERKNet

Thematic Area: Alport Syndrome

The following guidance documents have been adopted based on standardized reviews and are followed in all ERKNet centers:

1) **Expert Guidelines for the Management of Alport Syndrome and Thin Basement Membrane Nephropathy.**
J Am Soc Nephrol 2013; 24:364-75

Core Recommendations:

1. Genetic testing is the gold standard for the diagnosis of Alport syndrome and the demonstration of its mode of inheritance.
2. All affected members of a family with X-linked Alport syndrome, including most mothers of affected males, should be identified and followed.
3. Males with X-linked Alport syndrome and individuals with autosomal recessive disease should be treated with renin-angiotensin system blockade.
4. Affected mothers of males with X-linked Alport syndrome should be discouraged from renal donation because of their own risk of kidney failure.
5. Genetic testing should be considered in some individuals with thin basement membrane nephropathy to exclude X-linked Alport syndrome.

Comments by evaluators:

- Useful and comprehensive guidelines.
- The recommendations are described as expert opinions (Level D) / opinions of respected authorities (Level III).
- The health questions are specifically described and further categorised by sub types- Alport syndrome (X-Linked, autosomal recessive) and thin basement membrane nephropathy.
- The term autosomal dominant Alport syndrome is not discussed in these guidelines.

2) **Clinical practice recommendations for the treatment of Alport syndrome: A statement of the Alport Syndrome Research Collaborative.**
Pediatr Nephrol 2013; 28:5-11

Clinical Guideline Activities

European Reference Networks

Review of Existing Evidence Base

e.g. ReConnet...

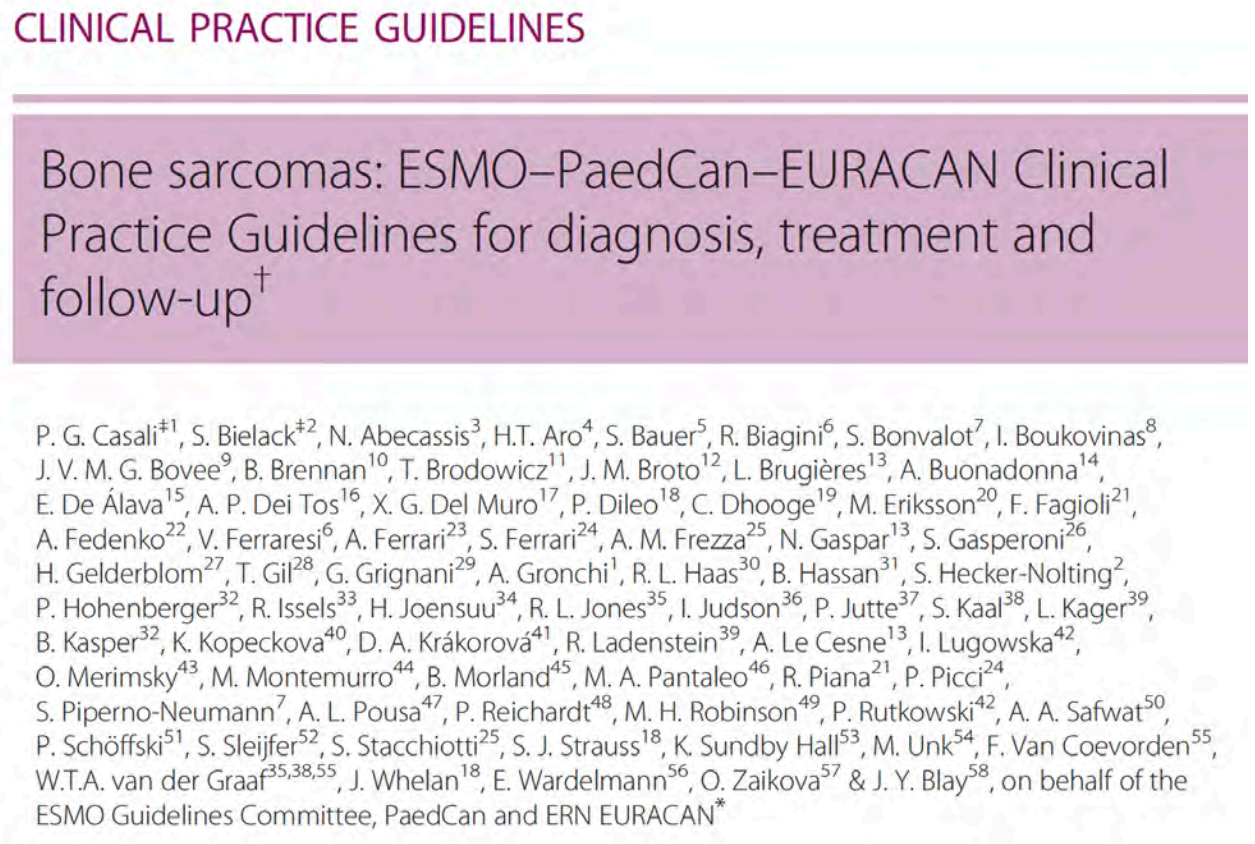
Evaluation and endorsement of existing guidelines

e.g. ERKNet...

New Guideline Development

e.g. Euracan/PaedCan, ERN-Ithaca...

- Collaboration with professional societies
- Collaborations between ERNs
- ERN Taxonomy Project



CLINICAL PRACTICE GUIDELINES

Bone sarcomas: ESMO–PaedCan–EURACAN Clinical Practice Guidelines for diagnosis, treatment and follow-up†

P. G. Casali[‡1], S. Bielack[‡2], N. Abecassis[3], H.T. Aro[4], S. Bauer[5], R. Biagini[6], S. Bonvalot[7], I. Boukovinas[8], J. V. M. G. Bovee[9], B. Brennan[10], T. Brodowicz[11], J. M. Broto[12], L. Brugières[13], A. Buonadonna[14], E. De Álava[15], A. P. Dei Tos[16], X. G. Del Muro[17], P. Dileo[18], C. Dhooge[19], M. Eriksson[20], F. Fagioli[21], A. Fedenko[22], V. Ferraresi[6], A. Ferrari[23], S. Ferrari[24], A. M. Frezza[25], N. Gaspar[13], S. Gasperoni[26], H. Gelderblom[27], T. Gil[28], G. Grignani[29], A. Gronchi[1], R. L. Haas[30], B. Hassan[31], S. Hecker-Nolting[2], P. Hohenberger[32], R. Issels[33], H. Joensuu[34], R. L. Jones[35], I. Judson[36], P. Jutte[37], S. Kaal[38], L. Kager[39], B. Kasper[32], K. Kopeckova[40], D. A. Krákorová[41], R. Ladenstein[39], A. Le Cesne[13], I. Lugowska[42], O. Merimsky[43], M. Montemurro[44], B. Morland[45], M. A. Pantaleo[46], R. Piana[21], P. Picci[24], S. Piperno-Neumann[7], A. L. Pousa[47], P. Reichardt[48], M. H. Robinson[49], P. Rutkowski[42], A. A. Safwat[50], P. Schöffski[51], S. Sleijfer[52], S. Stacchiotti[25], S. J. Strauss[18], K. Sundby Hall[53], M. Unk[54], F. Van Coevorden[55], W.T.A. van der Graaf[35,38,55], J. Whelan[18], E. Wardelmann[56], O. Zaikova[57] & J. Y. Blay[58], on behalf of the ESMO Guidelines Committee, PaedCan and ERN EURACAN*

Clinical Guideline Activities

European Reference Networks

Review of Existing Evidence Base

e.g. ReConnet...

Evaluation and endorsement of existing guidelines

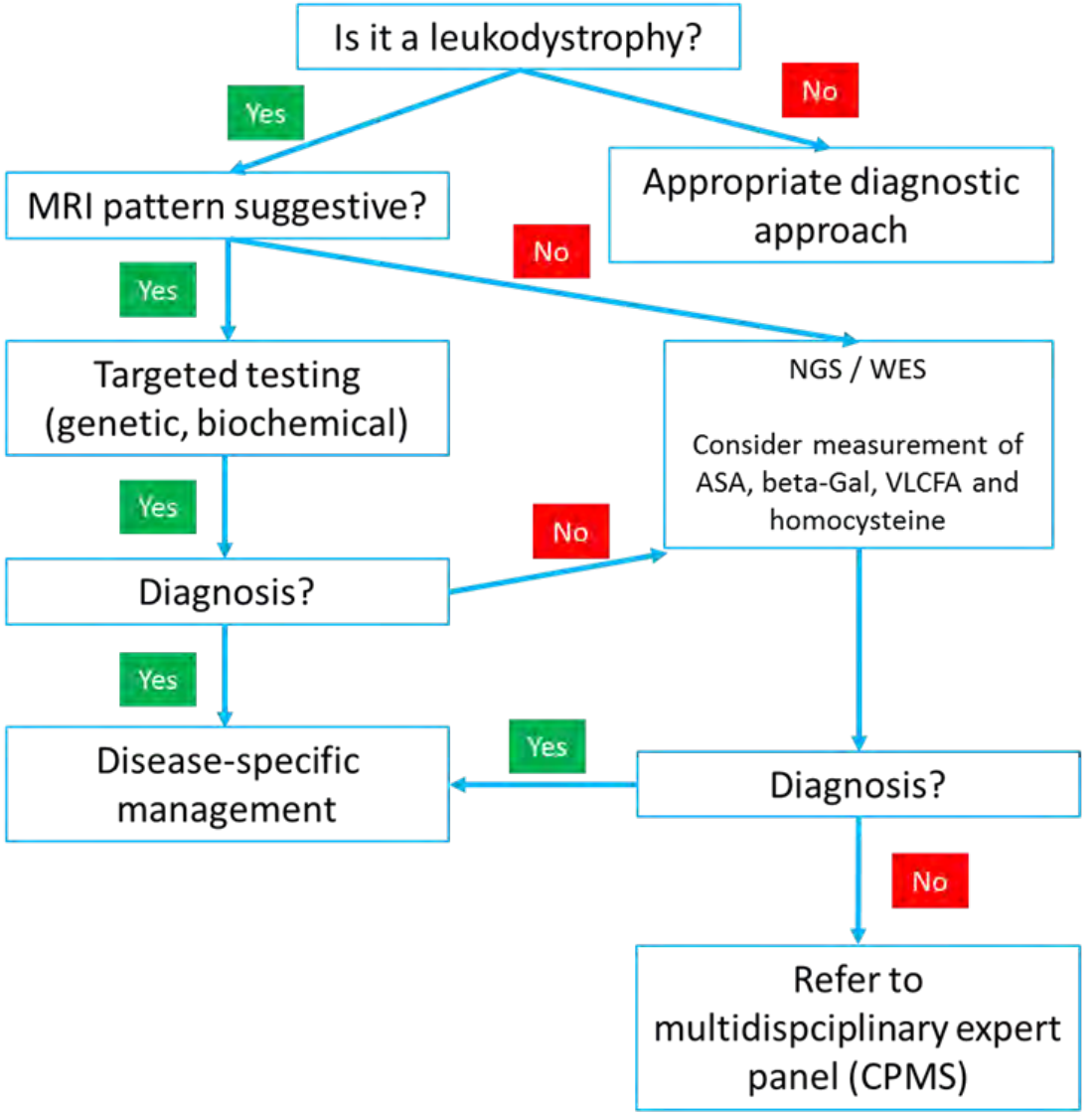
e.g. ERKNet...

New Guideline Development

e.g. Euracan/PaedCan, ERN-Ithaca...

Diagnostic Flowcharts, Clinical Rating Scales

e.g. ERN-RND...

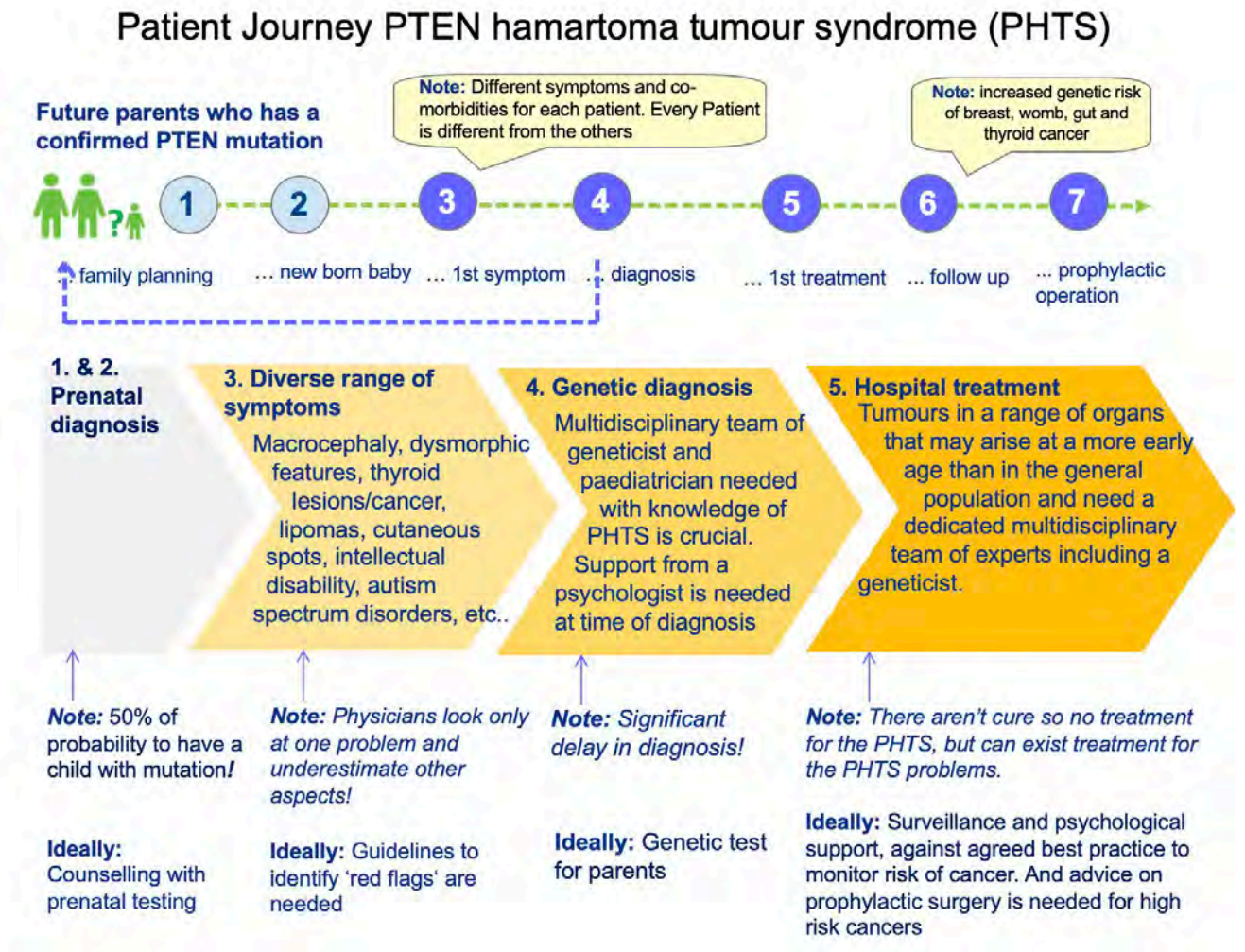


Clinical Guideline Activities

ERN Genturis: “Patient Journeys“

- Developed by patients
- Medical details approved by ERN professionals
- Contents:

  Typical patient pathway
  Typical challenges and pitfalls
  Medical issues where specialists can help
  Unmet patient needs at various disease stages



Education and Training

All ERNs organizing CME Courses, summer/winter schools
- good collaborations with professional specialist societies

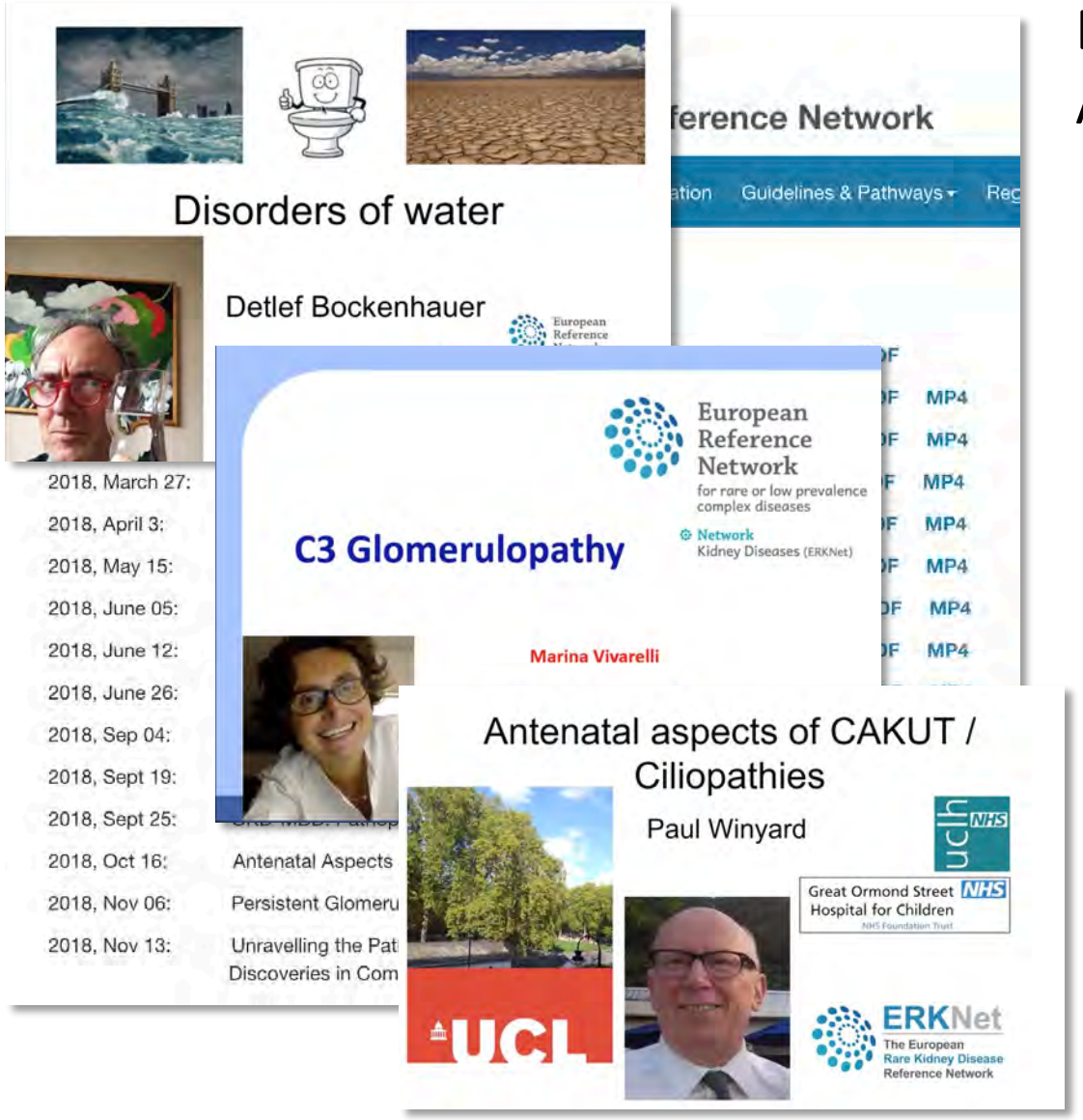
Webinars programs starting (e.g. Ithaca, ERKNet...)

CEF grant 2019/2020: **eLearning** platforms

ERN-RND, ERN-Eye: Clinical fellowship exchange grants
-> Funding for regular **clinical exchange training programs needed**

Rare Kidney Disease Webinar Curriculum

ERKNet

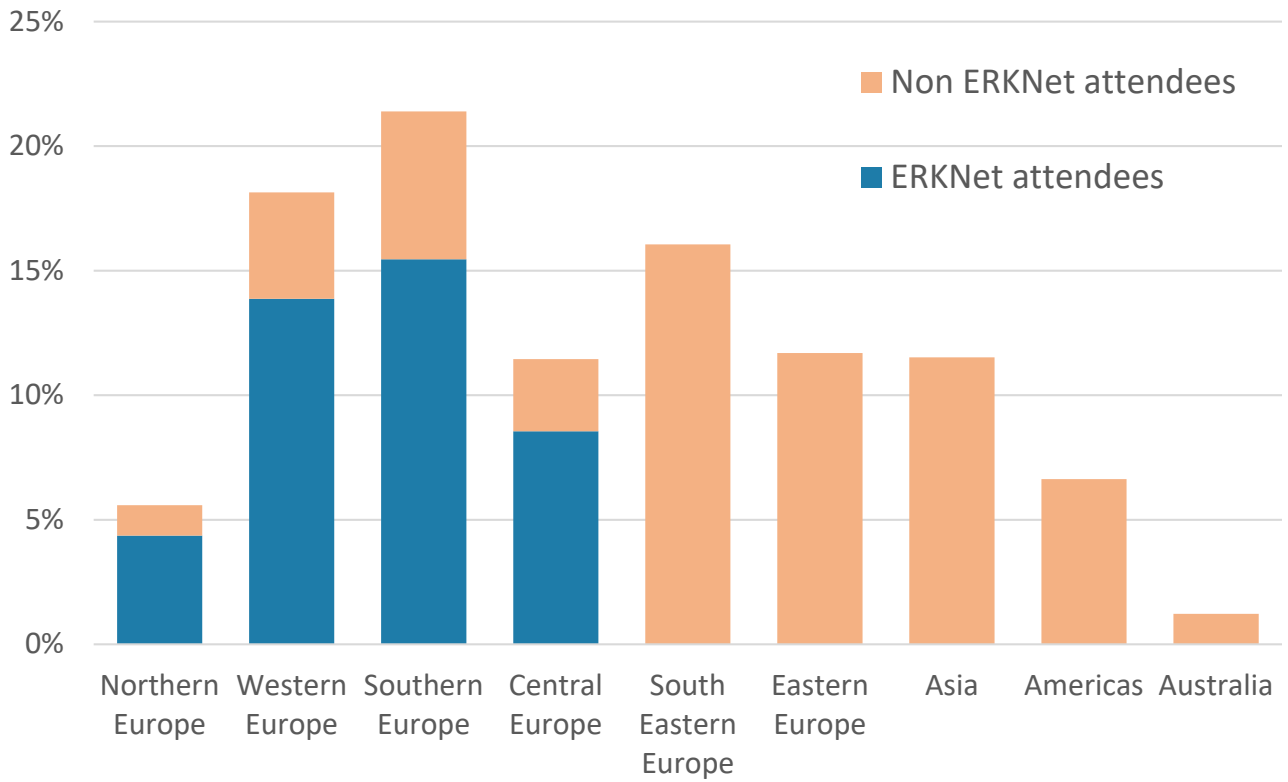


Biweekly live streams

Announced via ERN & professional society newsletters

100-150 registrations per Webinar

50-100 live attendees, 150-200 post-event downloads

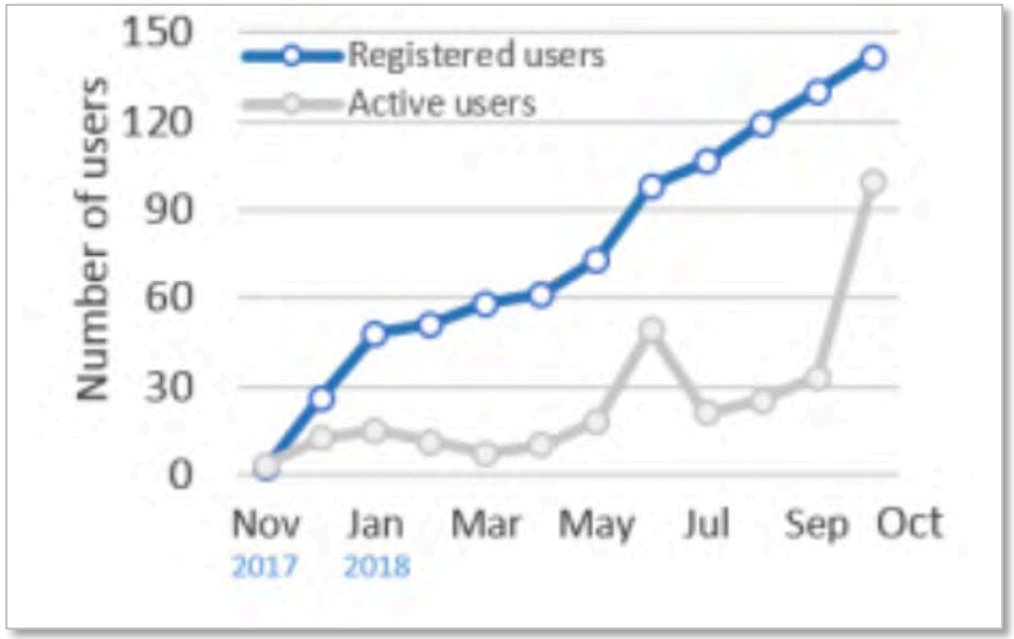


Virtual Consultation: The CPMS

European Reference Networks

Early experience:

- eConsulting platform with unique features:
  Advanced image/histopathology slide viewing functionalities
- Potentially cost-effective tool by sparing physical cross-border patient referral
- Time-consuming data entry,
  difficulty to arrange multi-expert VCs
- Operational help desks being implemented
- Data protection issues by GDPR
- Re-think target groups / access concept :
  „Experts rarely need expert advice“



Virtual Consultation: The CPMS

European Reference Networks

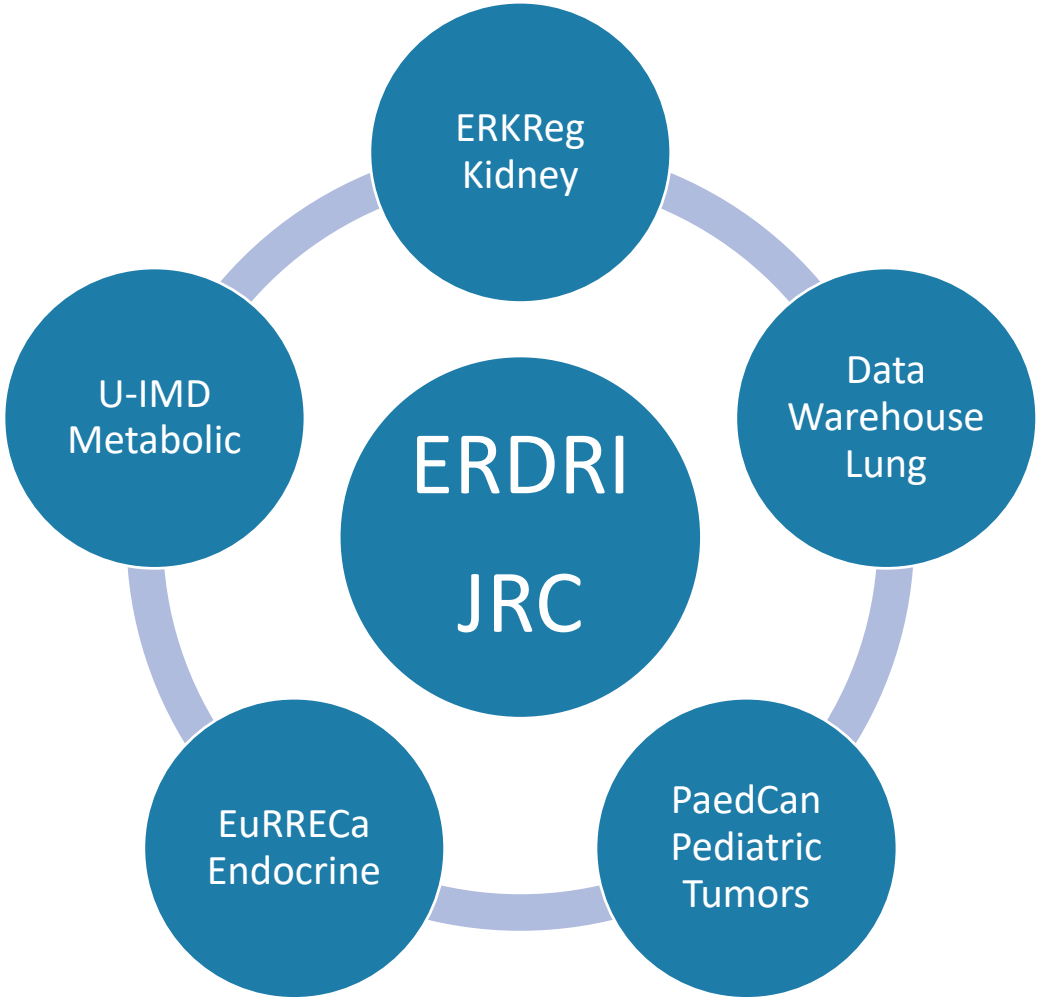
Keys to success:

- Definition of use indications and (national) access pathways
- Awareness of medical community
- Streamlined / customized data entry
- In the long run: Reimbursement system for expert services

ERN Patient Registries

European Reference Networks

- High priority for most ERNs
- Health Programme Research Call:
  Registries for European Reference Networks
- 5 ERNs funded to develop core registries
- Collaboration with JRC
  European RD Registration Platform
- Further EU support envisioned
- Local center support will be key to success



Performance and Outcome Monitoring

European Reference Networks

WG Montoring and Quality Indicators

Phase 1: (2019) Implementation of **ERN monitoring system** (e.g. patients processed in CPMS, CMEs, Webinars, guidelines adopted/developed, dissemination indicators)

Phase 2: (2020) Monitoring of **patient management & outcomes** within ERN centers

Research and Innovation

ERN Research Working Group:
- **Mapping** ERN research capacities ongoing
- **Strategy Workshop** Jan 24 2019

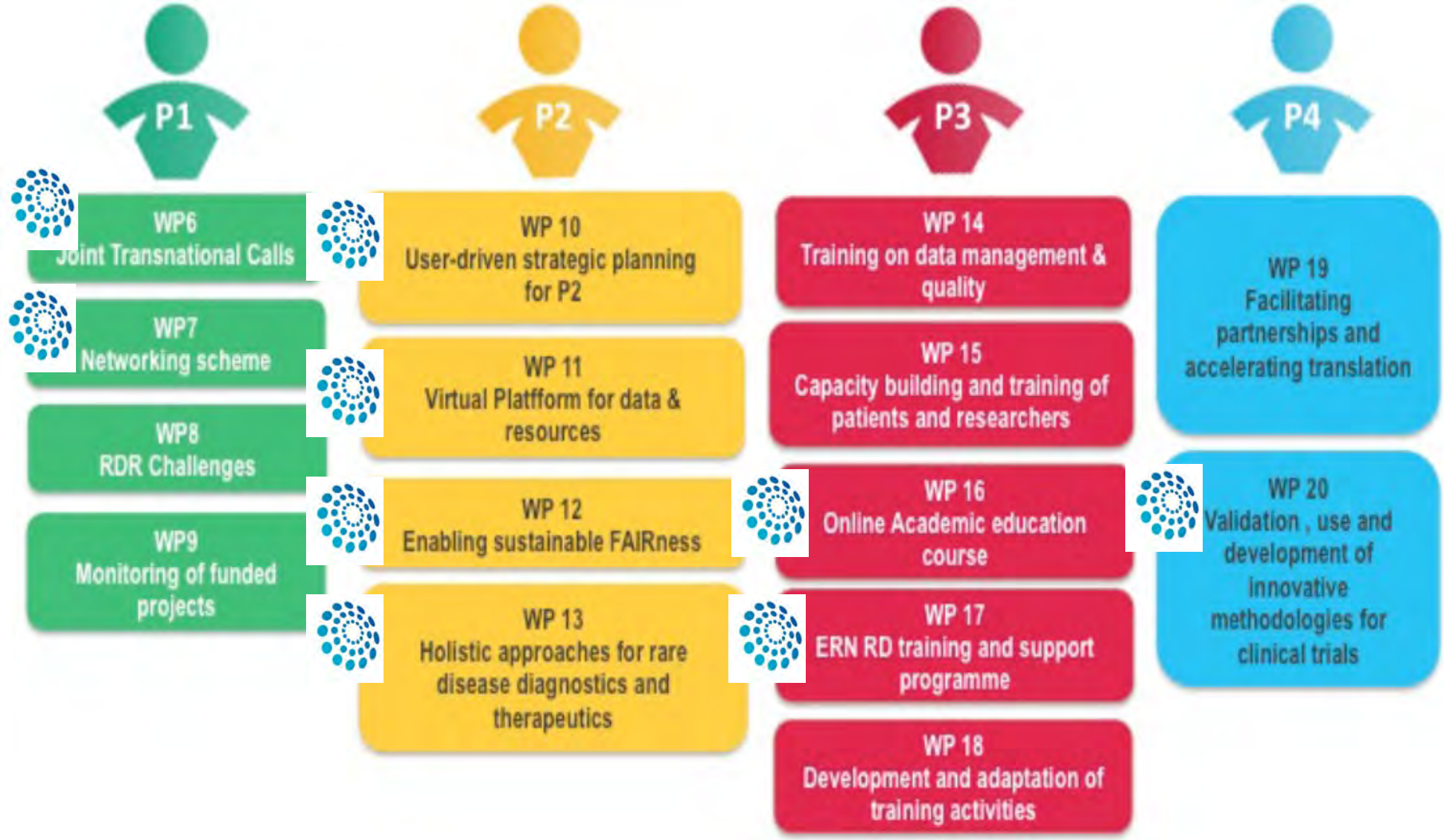
Pediatric clinical research:
Involvement in **conect4children** Paneuropean Clinical Research Network

Transveral COST proposal:
EaRN-Gen for promotion of genetic diagnostics

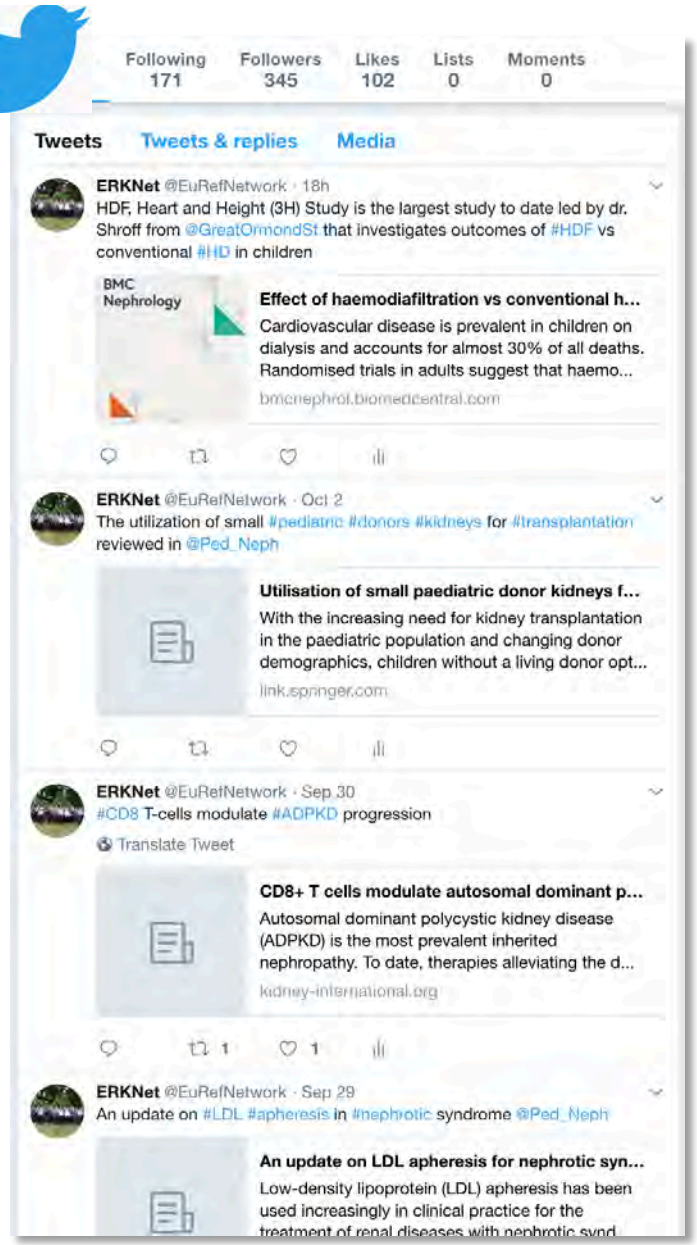
European Joint Programme for Rare Disease Research (**EJP-RD**):
Multi-level involvement of ERNs

Research and Innovation: EJP-RD

European Reference Networks



Dissemination Activities



Expected ERN Expansion: The Coordinators‘ View

European Reference Networks

Geographic expansion and **closure of expertise gaps** will be vital to materialize full potential of ERNs

Full membership and Affiliated Partnerships are equally valid models of cooperation

While new partners are welcome, **careful selection** according to competency criteria and ERN needs will be essential to
- **maintain quality** and
- **avoid operational challenges**
  (in view of unchanged coordination budgets)