



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Holistic approach to paediatric research

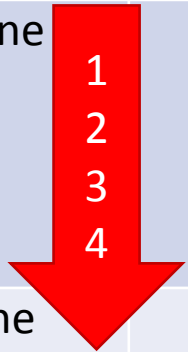
EnprEMA Workshop June 7th 2018

Mark Turner

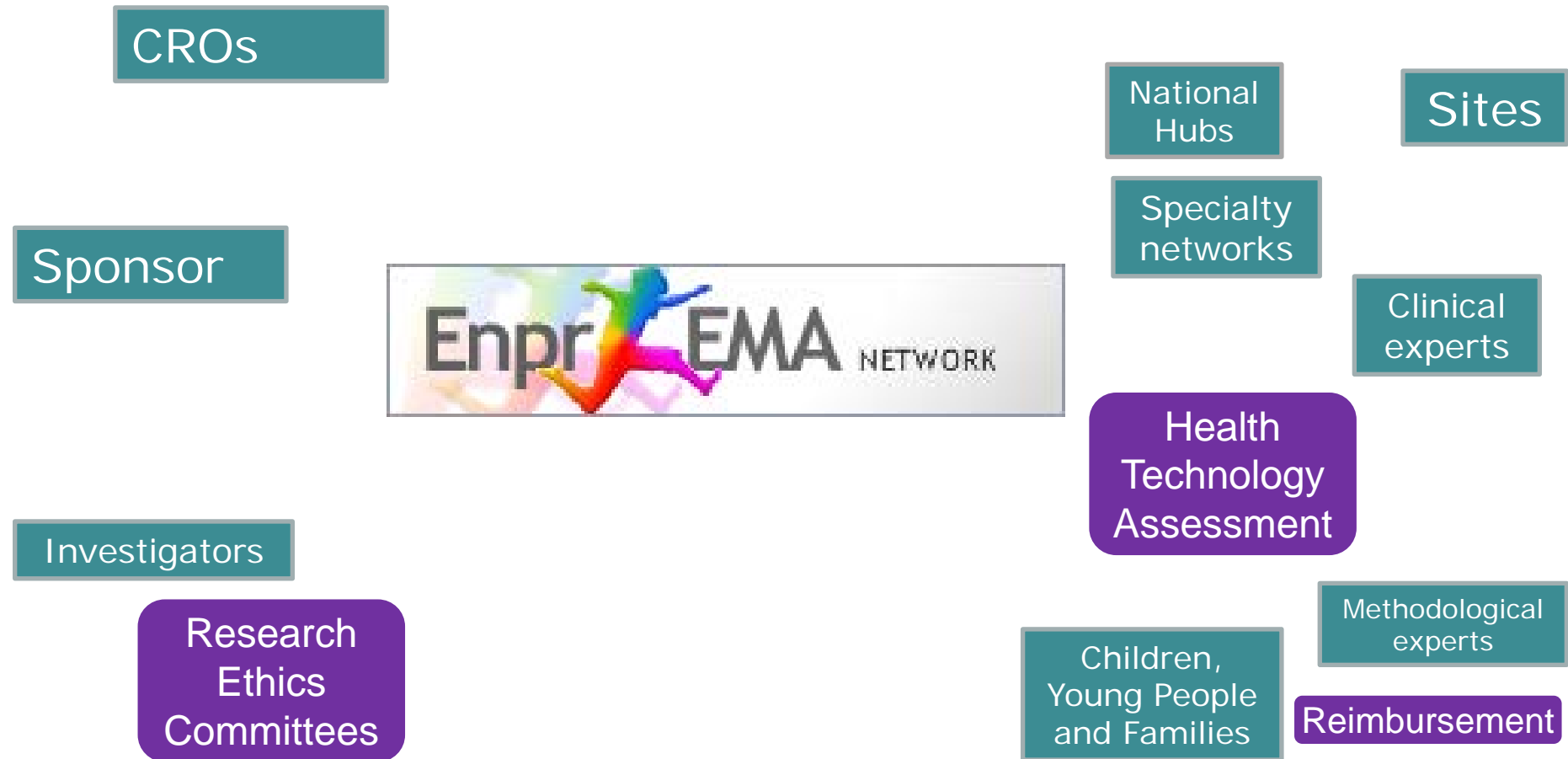


Step in the evaluation of medicines			
Identification of therapeutic need			
Identification of new, or repurposed, medicine			
Preparation of medicine for administration to infants, children and young people			
Pre-clinical testing of medicine			
Clinical testing of medicine			
Defining costs of medicine			
Specification of when to use the medicine			

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Step in the evaluation of medicines	Resources needed	Skills needed	Groups most likely to be helpful
Identification of therapeutic need	Understanding of effects of condition	Elicitation of actionable insights	Learned societies, advocates, ERNs
Identification of new, or repurposed, medicine	Candidate molecules	Assessment of whether candidate molecules may be useful	Sponsor Academics
Preparation of medicine for administration to infants, children and young people		Formulation science, manufacturing	Sponsor CYP
Pre-clinical testing of medicine		Relevant models and expertise	Sponsor
Clinical testing of medicine	Information about potential study participants Access to study participants	Study design, conduct and compliance with regulatory requirements Managing trials	Sponsor Research networks Advocates
Defining costs of medicine	Information about costs and comparators	Health economics	Health Technology Assessment agencies
Specification of when to use the medicine	Evidence about the effects of the medicine in real-world settings	Guideline development (evidence review, consultation and consensus)	Learned societies, advocates, ERNs



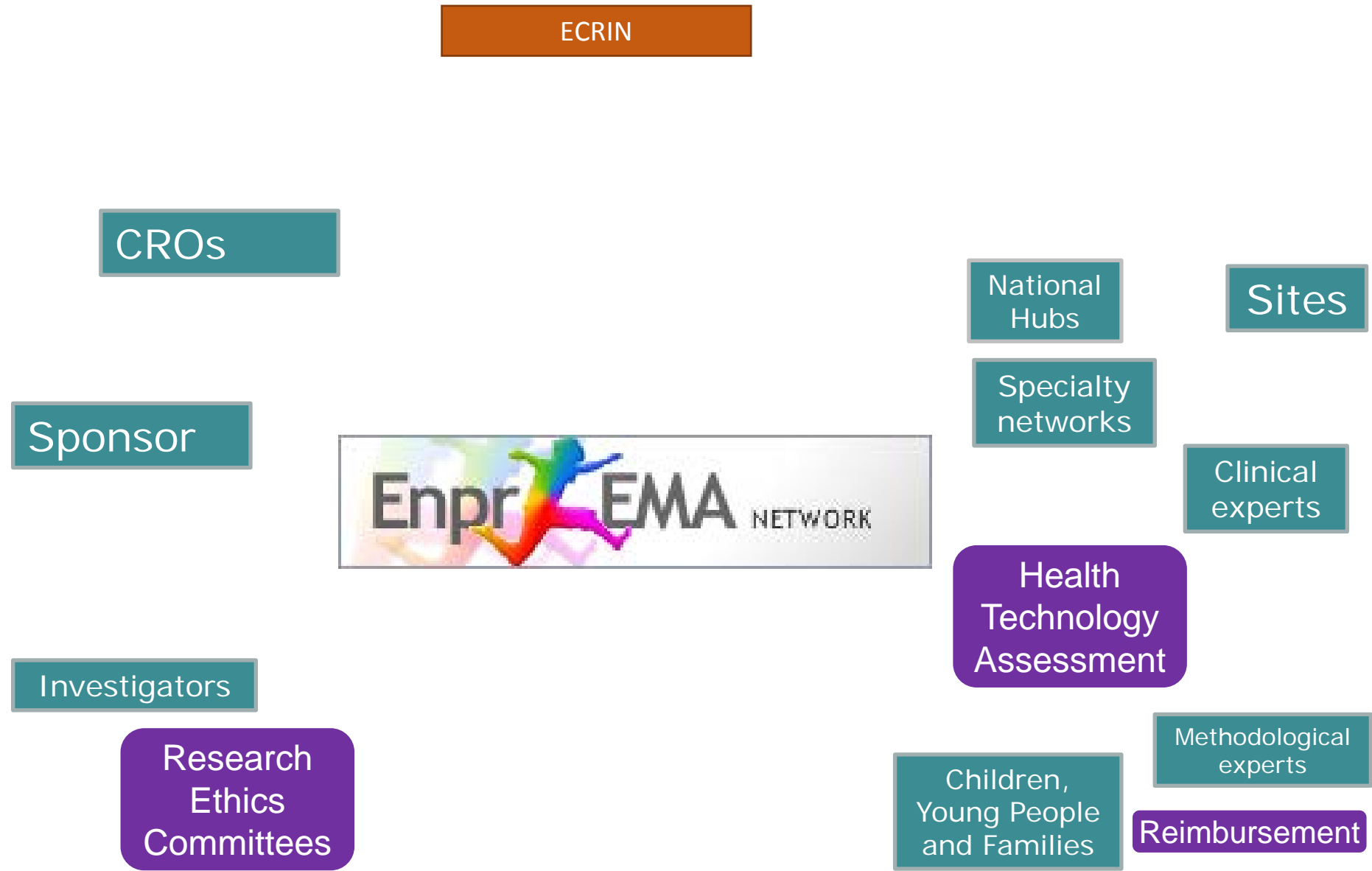
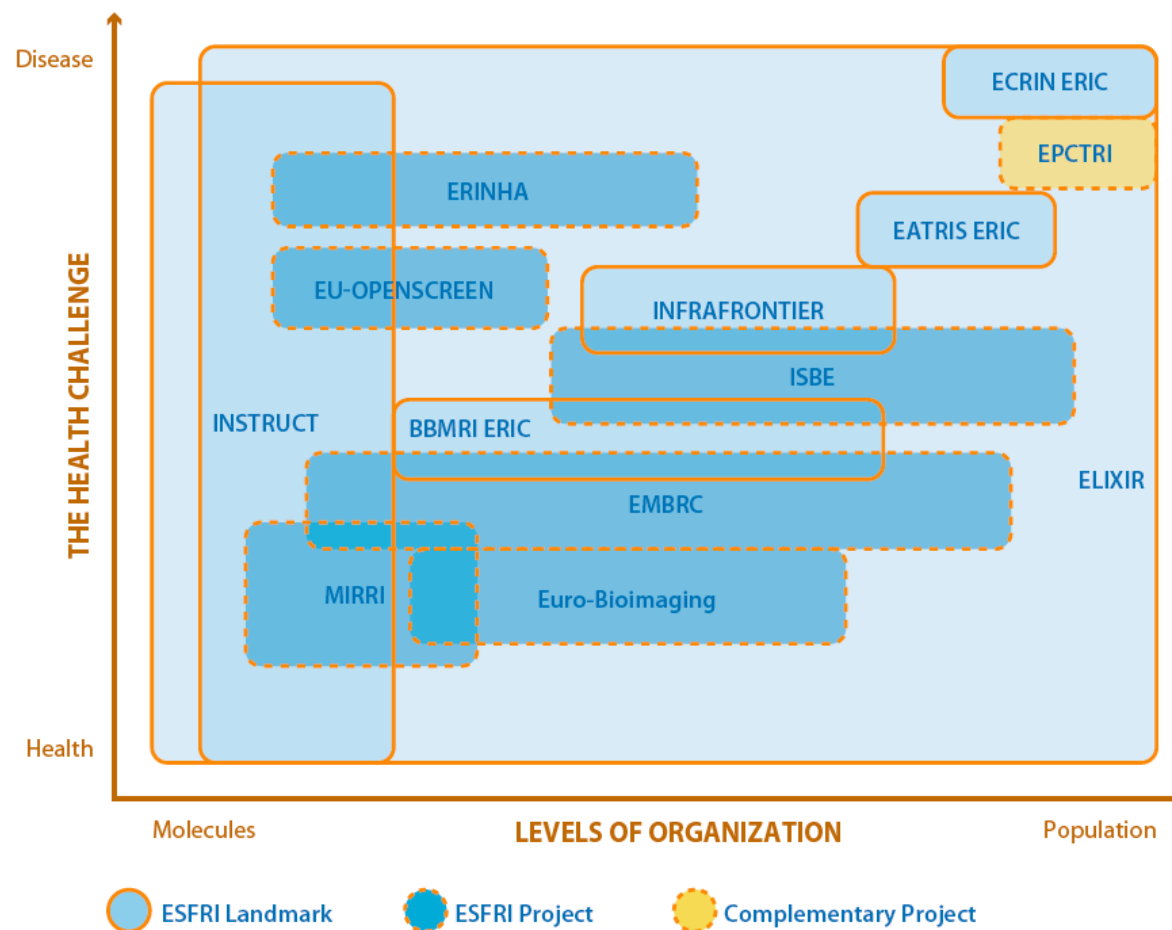
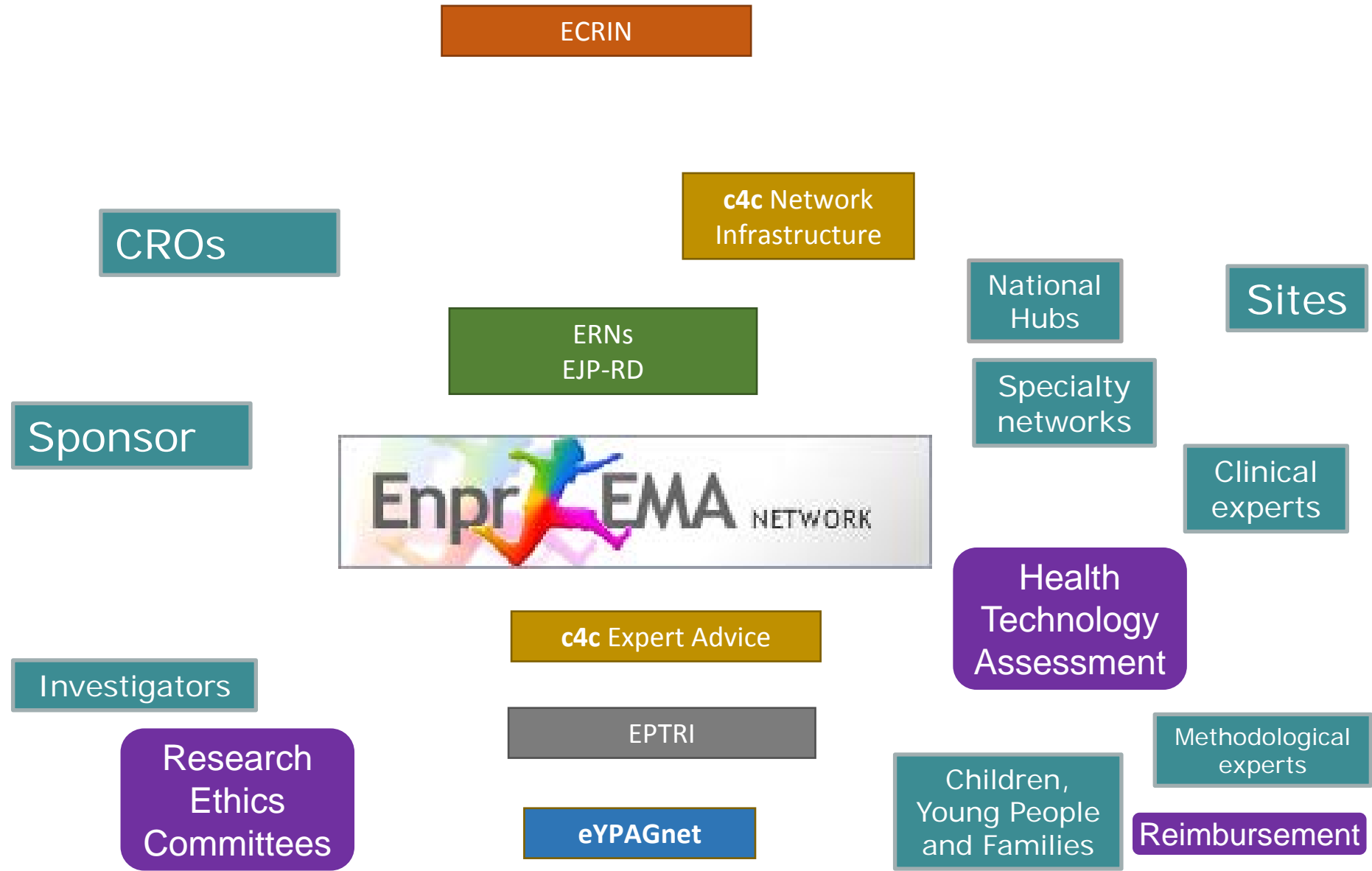
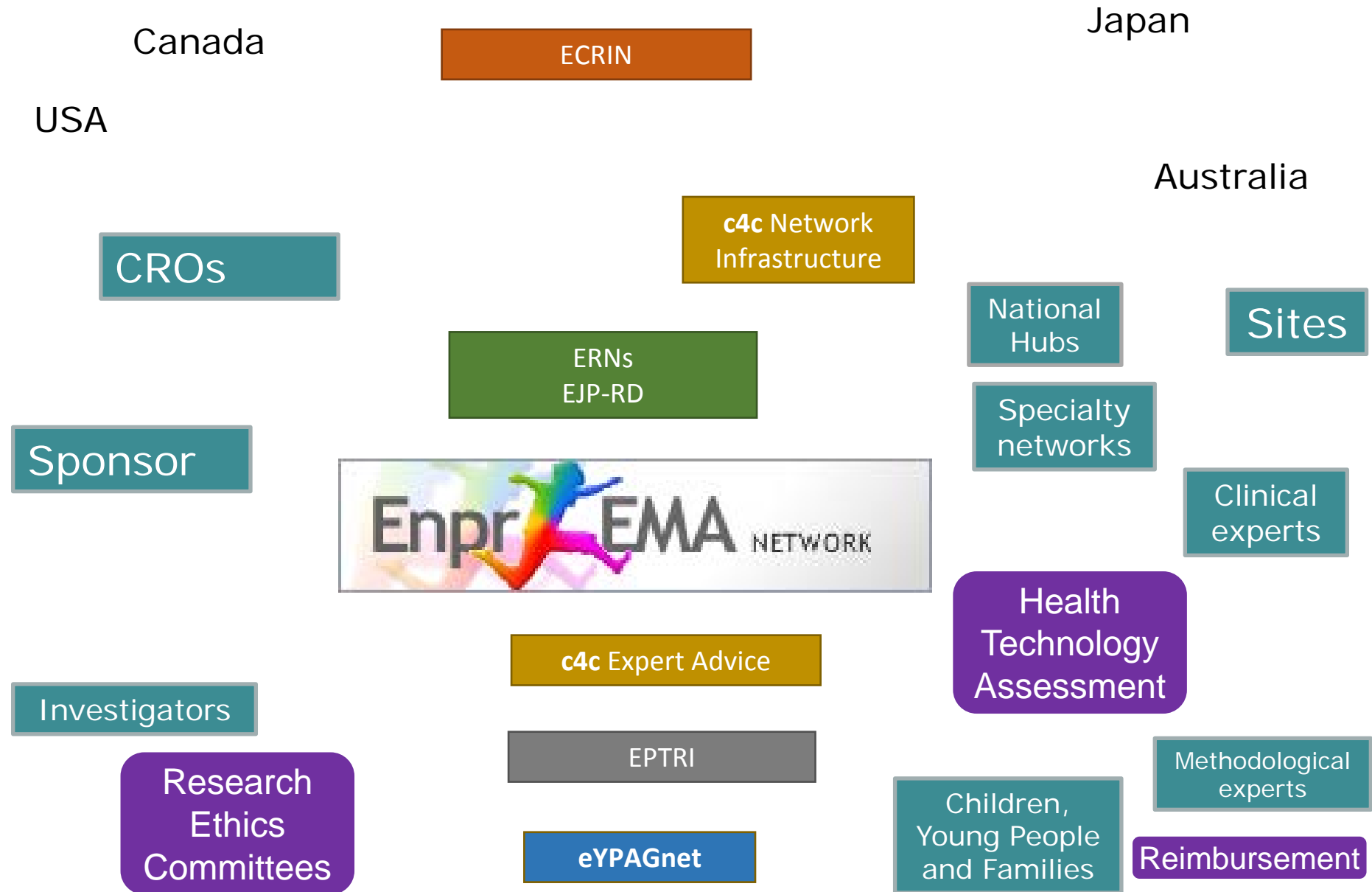


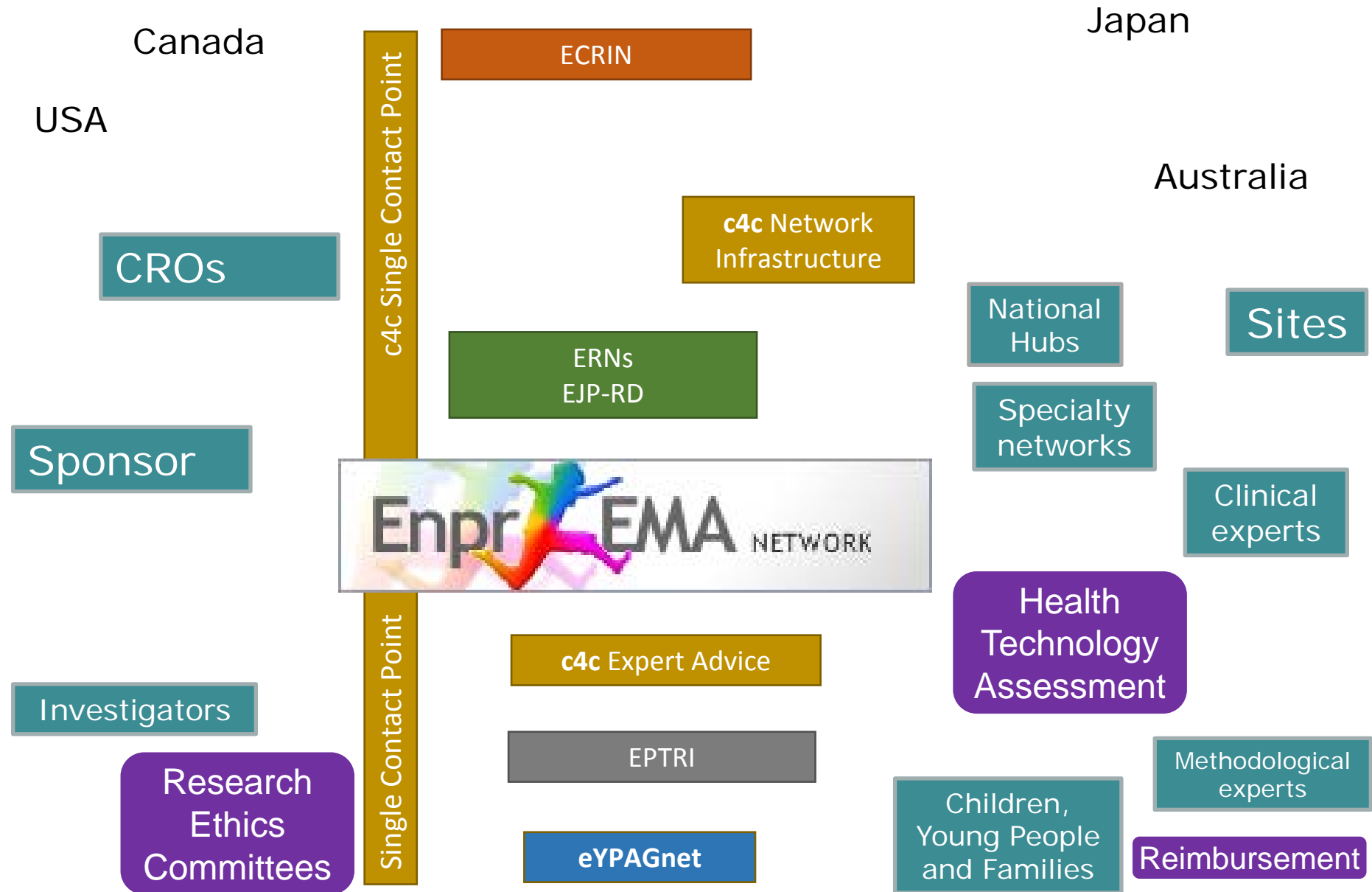
Figure 3: the indicative position of ESFRI RIs relative to the different levels of organisation in the Health domain.



Existing infrastructures such as the ESFRI Landmarks ECRIN, BBMRI, EATRIS and others could connect in a "meta-structure" aiming at providing a full pipeline for drug development.







How to develop a holistic view

1. Describe each stakeholder
 - a) What do stakeholders do?
 - b) What could be done with better communication between stakeholders?
2. Develop ways to link stakeholders
 - a) ? EnprEMA Working Group
3. Test and refine links
 - a) Real-world problems when links are working

Step 1: EnprEMA June 7th

Five minute presentations

- Describe what each stakeholder
 - Does
 - Could do with good communication with other stakeholders
- Suggestions for the future