



European Network of Paediatric Research at the European Medicines Agency



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Report from the coordinating group (CG) Update on Enpr-EMA activities, achievements and challenges

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Topics for discussion

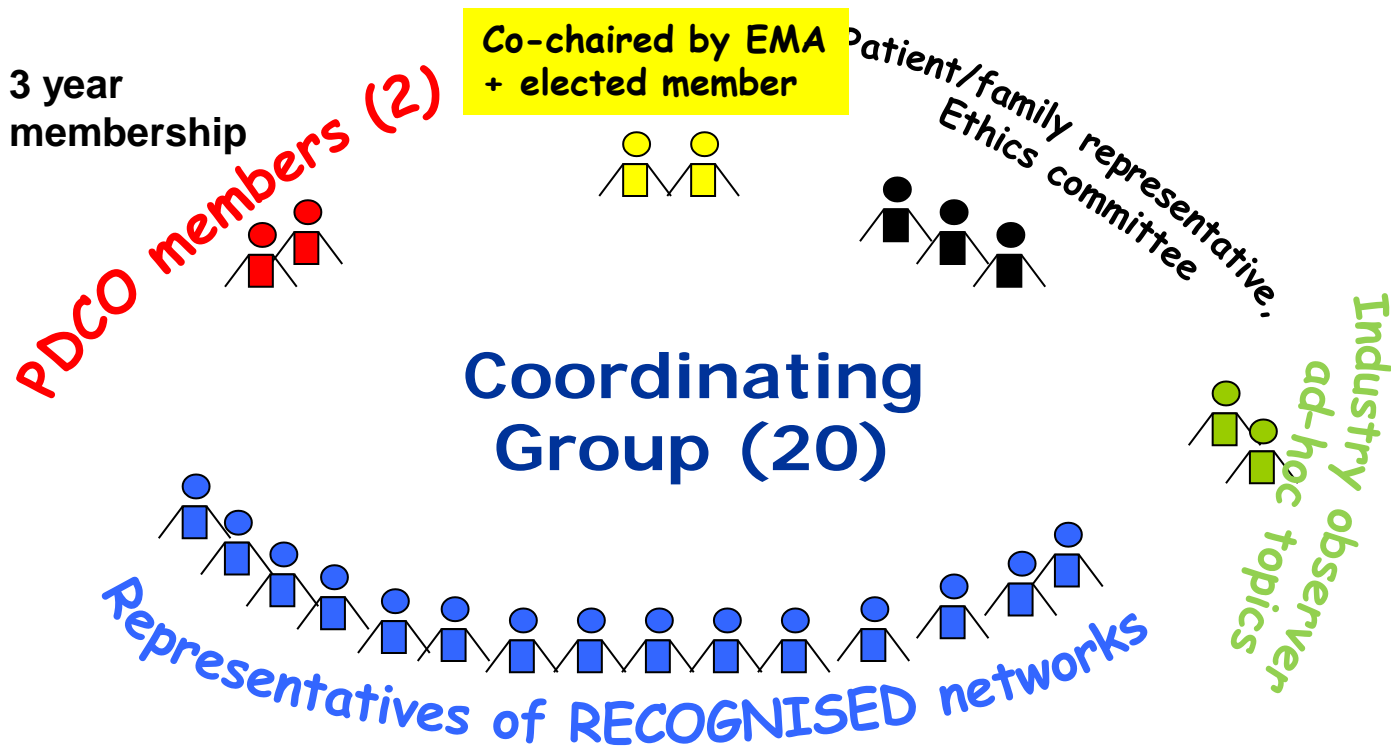
Overview of EnprEMA

Selected achievements

Enpr-EMA represents European networks in Europe and globally

- An umbrella network of existing networks
 - enables collaboration and learning of individual networks from each other
 - versus the different role of the clinical trial networks which actually conduct and perform the clinical trials.
- The roles of Enpr-EMA include:
 - identify problems and challenges for individual networks
 - act as an European voice to raise awareness on these challenges and difficulties on an European level
 - propose solutions – as some of the ad-hoc Enpr-EMA working groups have done.

Structure - Coordinating Group



What Enpr-EMA does not do

- **fund** studies
- act as a **CRO** and **manage** studies
- **decide** on research priorities which remain the responsibility of
 - the Member States
 - the Commission through the Community programmes
 - each individual network



Achievements

- Expansion
- New Subgroups proposed
 - Infections
 - Paediatric Oncology

Achievements

- WG “Dialogue and interaction with Ethics Committees”:
- Paper
- Submission of comments on proposed changes to the U.S. Common Rule. The comments focused on two points relating to paediatric research:
 - the value of taking an international perspective when revising the Common Rule
 - Informed Consent

Achievements

- WGs “How to establish communication between Enpr-EMA, networks and industry” and “Sharing good practices within Enpr-EMA and with industry” decided to merge.
- Paper
- Develop a model to put on the EMA website showing how industry can engage with Enpr-EMA/networks and how this could benefit them

Achievements

- “Framework for networks to interact with industry and regulators when implementation/conduct of clinical trials agreed in PIPs is no longer possible”..
- Draft SOP

**Joint EFGCP / DIA / EMA
Better Medicines for Children Conference 2016 on**

Optimisation of drug development for the benefit of children



**10 & 11 October
2016**

**EMA, London,
United Kingdom**

Problems that are identified but not addressed by EnprEMA

The contrast between:

- Volume of work
 - more than 1000 PIPs have been approved leading to the recruitment of > 500,000 children over the next 5 years)

Table 13 - Paediatric clinical trials by year of authorisation (or, if not available, by year of protocol upload into EudraCT).

| | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 |
|--|------|------|------|------|------|------|------|------|------|------|
| Paediatric ¹ trials | 340 | 362 | 342 | 407 | 391 | 372 | 401 | 344 | 434 | 763 |
| Total number of trials (adults and / or children) | 4272 | 4855 | 4640 | 4555 | 4134 | 3971 | 3865 | 3576 | 3588 | 4242 |
| <i>Proportion of paediatric trials of all trials (%)</i> | 8.0 | 7.5 | 7.4 | 8.9 | 9.5 | 9.4 | 10.4 | 9.6 | 12.1 | 18 |
| Exclusively ² paediatric trials | 196 | 188 | 185 | 241 | 230 | 218 | 257 | 211 | 284 | 473 |

Source: EudraCT Data.

¹ A paediatric trial is a trial that includes at least one participant < 18 years of age

² An exclusively paediatric trial is a trial that includes only participants < 18 years of age

Problems that are identified but not addressed by EnprEMA

The contrast between:

- Volume of work
 - more than 1000 PIPs have been approved leading to the recruitment of > 500,000 children over the next 5 years)
- Lack of site capacity
 - Individual studies are relatively small; studies in each specialty are relatively rare.
 - Lack of critical mass at site and national level.

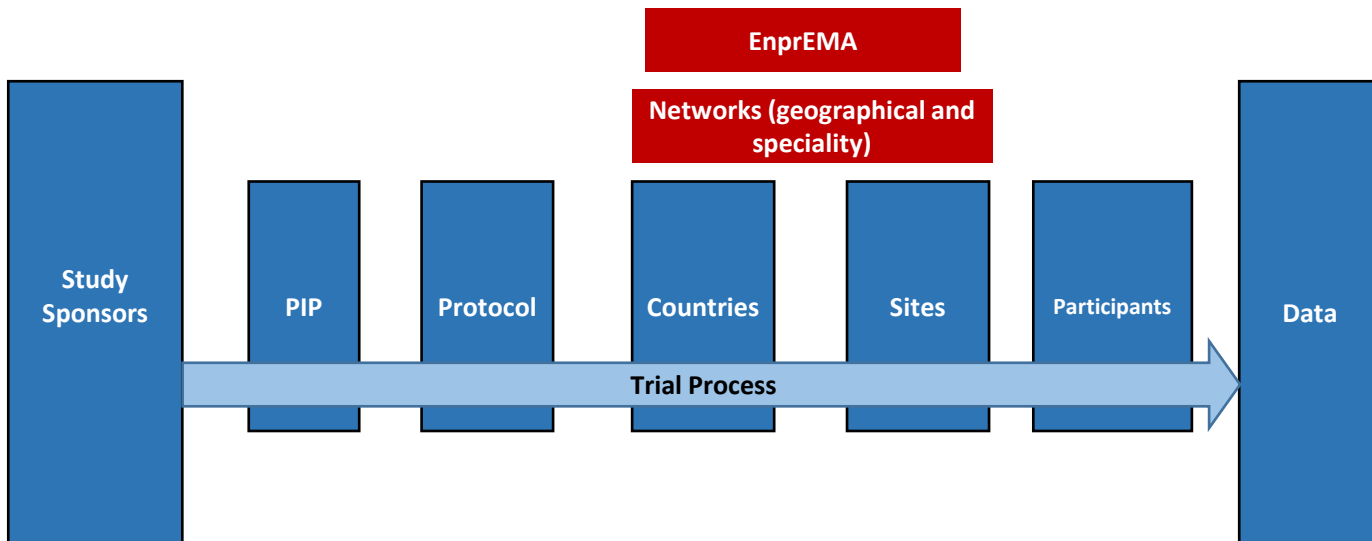
Lessons learnt about how to build a sustainable paediatric clinical trials network

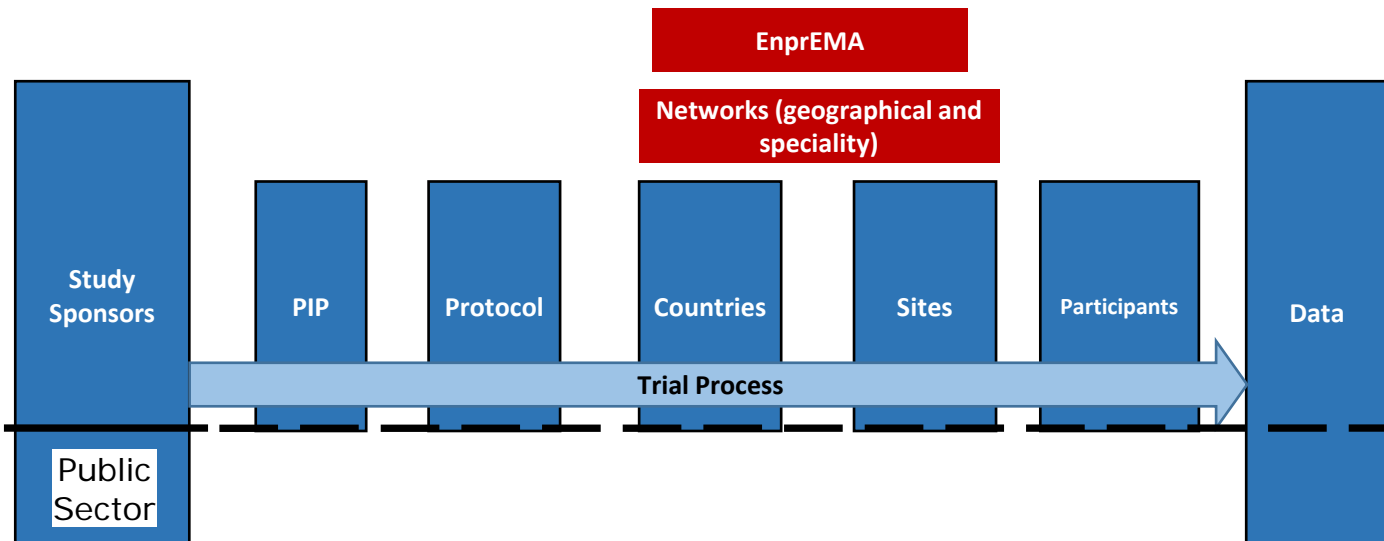
- A number of funding streams to support networks are in place
- Funding needs to match national health care systems

Table 4. Existing sources of funding for paediatric research infrastructure

| Source of funding | Current Example |
|--|--|
| In-kind support from health care systems (hospitals etc. – sustainability challenge is ring-fencing) | All over (usually without ring-fencing so difficult to manage) |
| Cash payments to network to spend on dedicated staff | |
| Universities | Brussels |
| Health Care systems | Finland, |
| Governments | UK, Switzerland |
| Industry | |
| Subscriptions from members | Proposed model for PTC |
| Fee for service | UK, Finland |

Austria

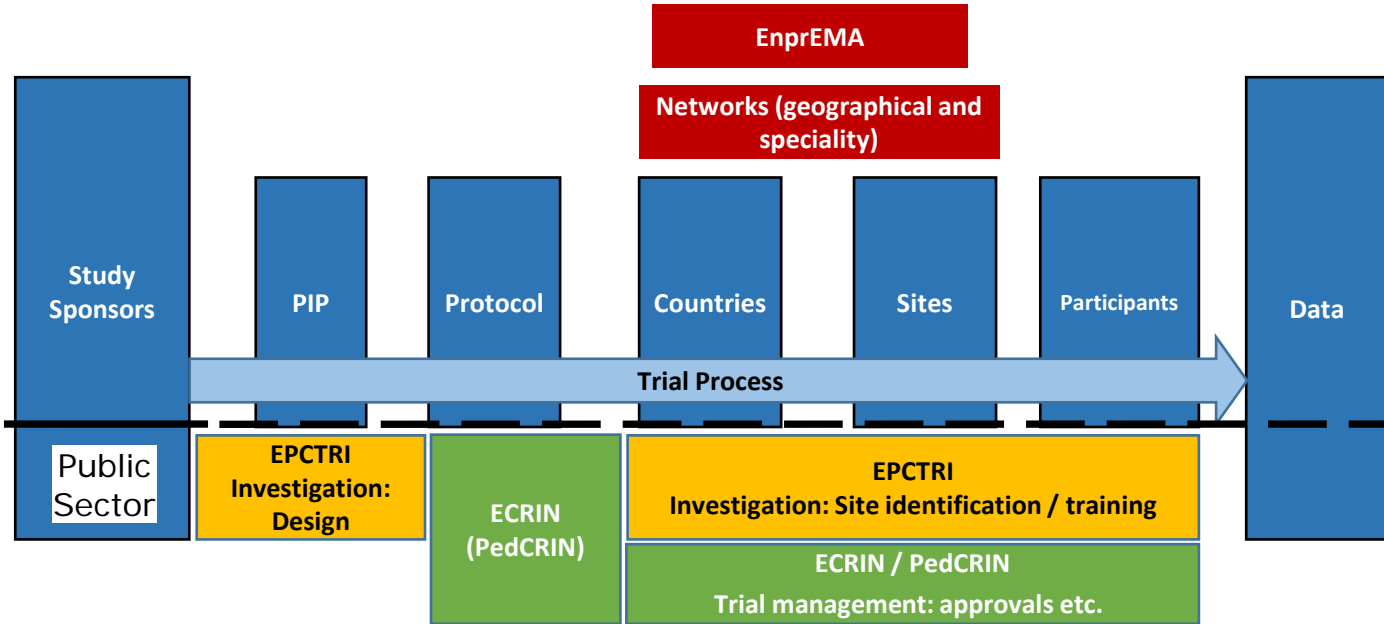


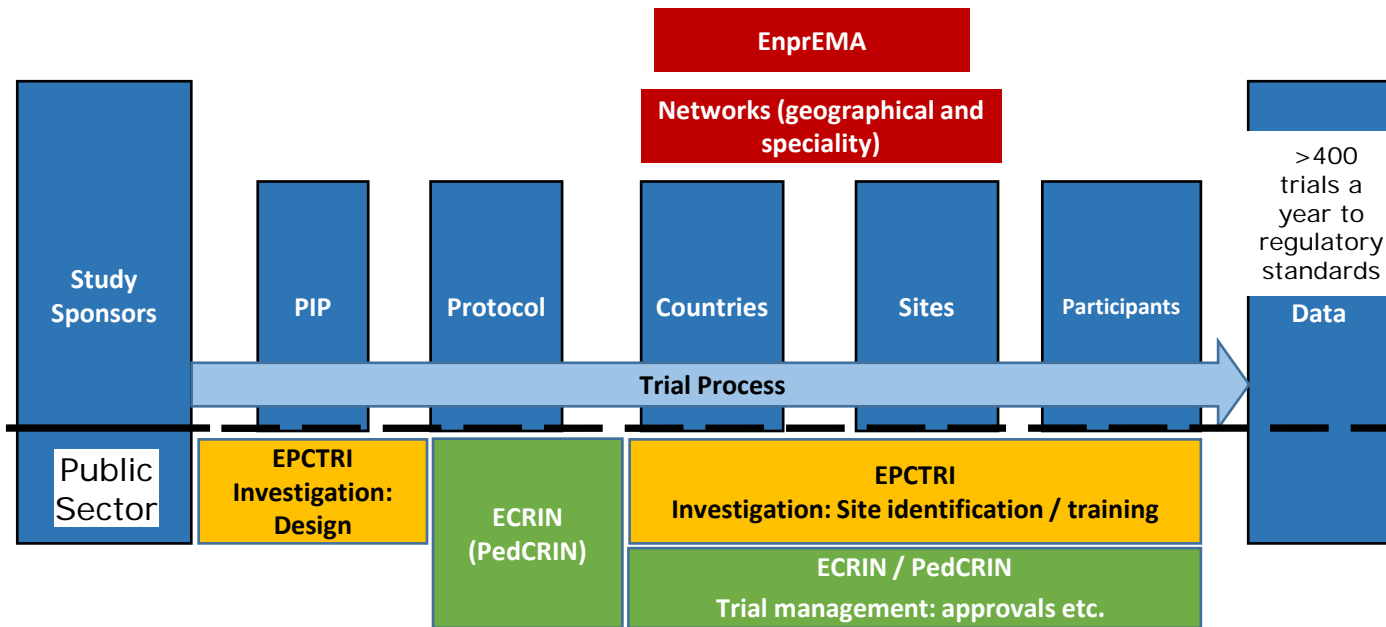


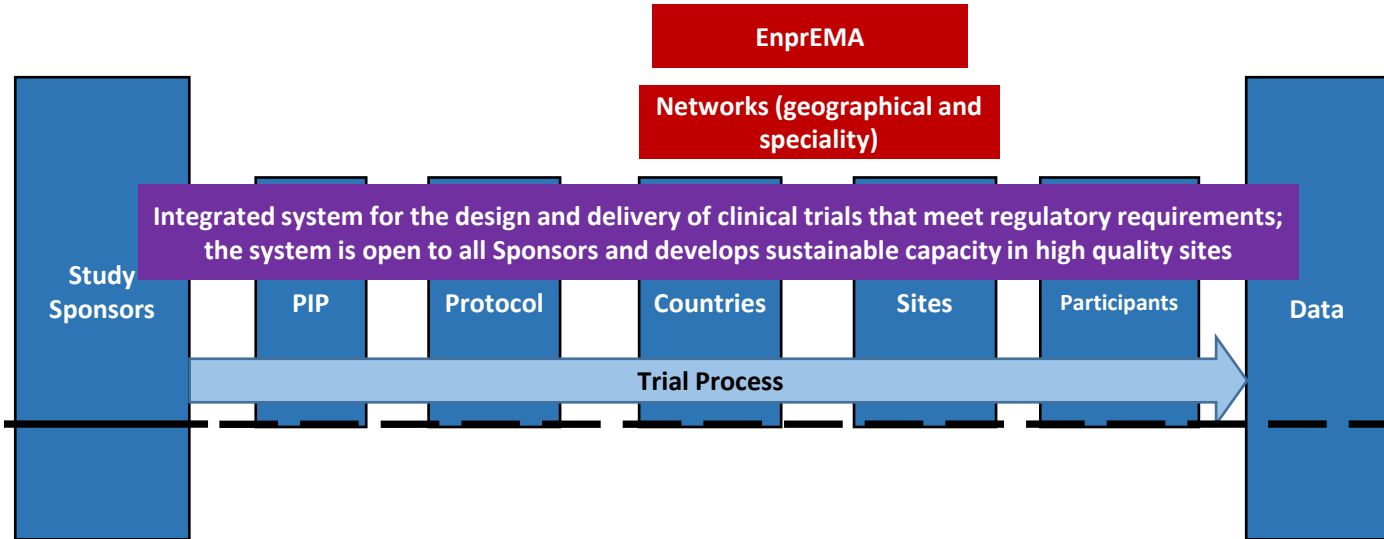
Achievements

Adoption of European Paediatric Clinical Trials Research Infrastructure (EPCTRI) on the 2016 Road Map of the European Strategic Forum of Research Infrastructure

- Eligible for INFRA-DEV003 to upgrade existing infrastructures
- Eligible for INFRA-DEV001 to scope out new infrastructures







Global Context

Common needs but

- Patchwork of purposes
- Patchwork of funding

North America

Europe

Japan

South Korea

Australia

Global Context

Common needs but

- Patchwork of purposes
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North America

| | | |
|-----|-----|--------------|
| NIH | PTN | PTC/ iACT |
|-----|-----|--------------|

Europe

| | | | |
|-------------|------|-------|--------|
| Enpr EMA | IMI2 | ECRIN | EPCTRI |
|-------------|------|-------|--------|

Japan
South Korea

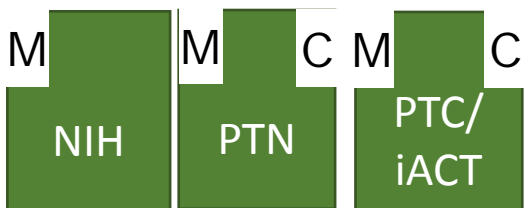
Australia

Global Context

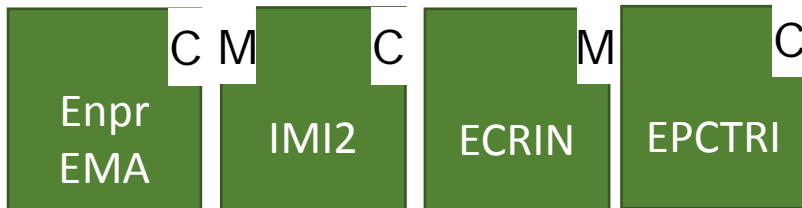
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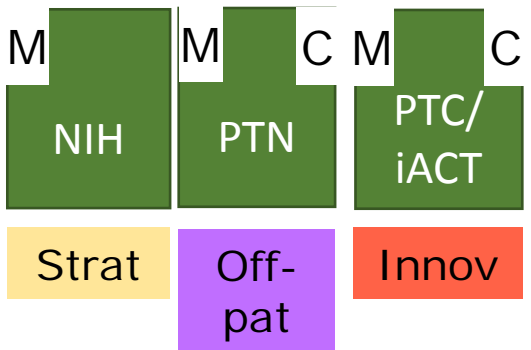
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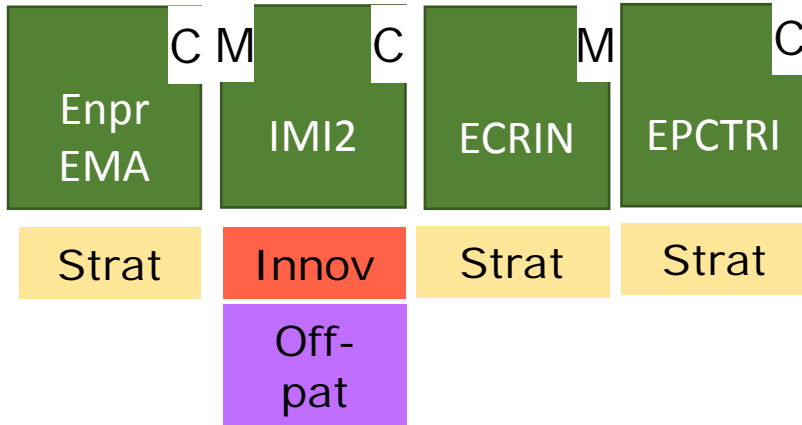
M: Management of Clinical Trials
 C: Coordination of a portfolio of Clinical Trials

- I: Strategic trials: funded publically
 Studies to adapt off-patent medicines to children
 Studies of Innovative medicines

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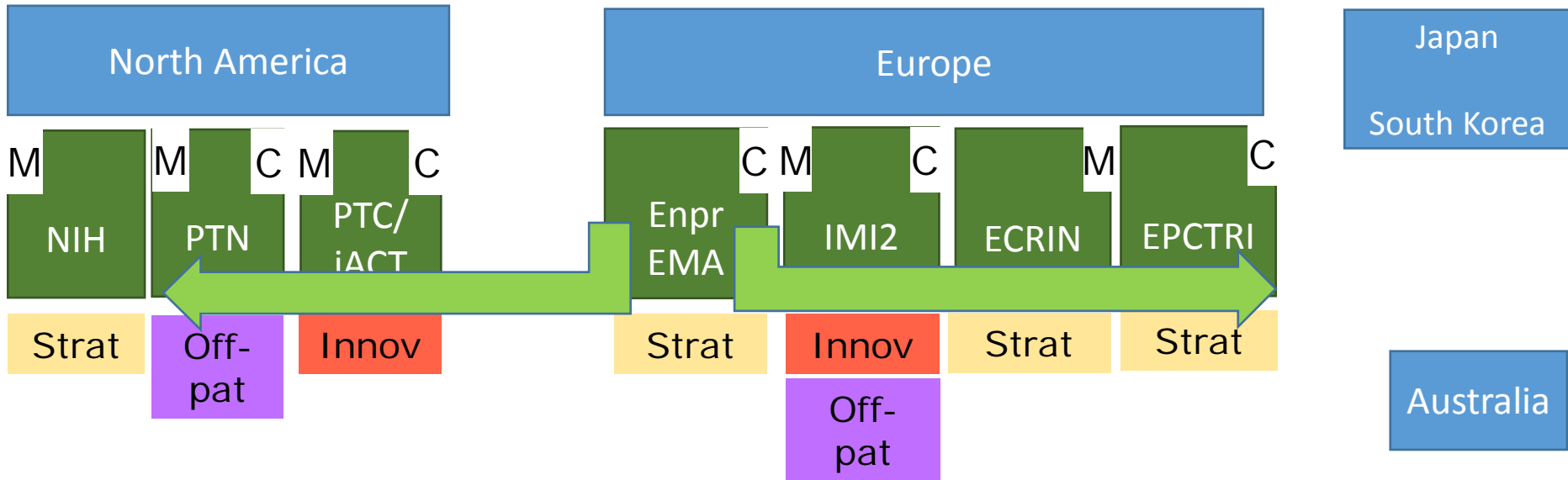
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Conclusions

EnprEMA is a thriving forum for interactions between networks, Pharma, CROs, regulators and children, young people and families

EnprEMA promotes shared practice within Europe and with other parts of the world