PARENT –Joint Action
cross-border PAtient REgistries iNiTiative

Patient Registries & HTA

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The need… The challenge….

- **HTA analysis** of medicinal products and medical devices is more and more **in need of 'real-world' data** in order to address aspects of **clinical effectiveness** and **economic evaluation**.

- A valuable **candidate source** for such data is **patient registries.**

- The use of registry data for HTA purposes has been limited and faced with **data quality**, as well as **procedural barriers**.

- The Cross-border Patient Registries Initiative (PARENT) Joint Action has initiated a dialogue and collaboration with the European HTA community in order to improve the present situation.

PARENT Summary

Aim: Provide MS with guidelines and recommendations on improving registry interoperability and use of data for secondary purposes (indicators, research, etc.) in a cross-border setting

05/2012 - 05/2015 (36 months)
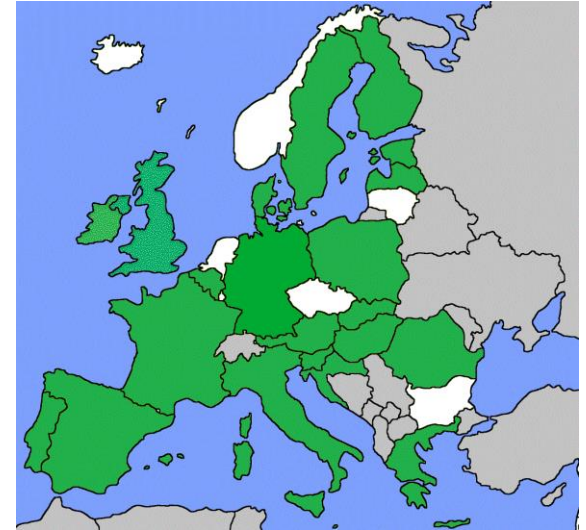Budget: 3.4 Mio € (60% EC)
11 Associated partners
12+ Collaborating partners

PARENT Associated Projects Group:
EAR EFORT, EPIRARE (&RD-CONNECT), EUReMS, EUCERD JA, EUBIROD, EUROCISS, EPAAC, EuraHS, eHGI, epSOS, EUnetHTA, SHN, EHR4CR (&EMIF)
Additional partners and stakeholders:
NICE UK, CPME, ESIP, EFPIA, EBE, EUREGHA, HIQA Ireland and many others

PARENT envisions a Community of Patient Registry holders **sharing common interests.** This to be achieved via **issuing guidelines** and **producing supporting tools** targeted at improving the quality of registry data, as well as registry maintenance and operations.

Patient registries are...

- "an **organized system** that uses **observational study methods** to collect **uniform data** (clinical or other) to evaluate specified **outcomes** for a **population** defined by a particular **disease, condition, or exposure**, and that serves a predetermined scientific, clinical, or policy **purpose(s)**"

AHRQ — Agency for Healthcare Research and Quality
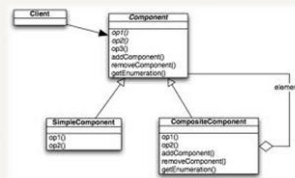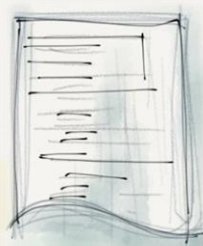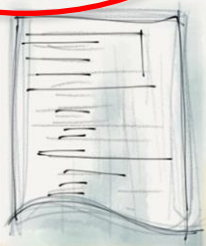Advancing Excellence in Health Care

PARENT Framework: **the tools**

PARENT-RoR.eu

Pilot **Registry of Registries (www.parent-ror.eu)** released in November 2013
Mapping patient registries across the EU

PARENT Methodological guidelines

Contribution of ≈ 40 authors

Steps in the process:

- **12/2013** – Workshop
Review and discussion of draft structure

- **05/2014** structure presented at eHGI representative meeting

- **06/2014** First draft of the Guidelines - workshop on open issues

- **Ongoing**: Final editing

- **12/2014**: Final version

- Definitions and typology of patient registries
- Diversity in use
- Overview of European Registries

Policies and strategies towards patient registries
Legal and ethical aspect

Process model
- Process definition
- Definition of results
- Process landscape
- Definition of roles
- Process critical point definition

Data sources of registries
- Primary and secondary data sources
- Interoperability consideration
- Evaluation od data sources

Planning a registry
- First steps
- Team establishment
- Action plan
- Planning resources
- Funding strategy
- Risks

Registry content design
- Research questions and hypotheses
- Study design
- Target population
- Patient selection
- Size of registry

Registry data model
- Definitions, standards, methodologies, tools
- Interoperability
- Data modeling
- Reference sets, ontologies

Registry set-up and running a registry
- Collecting data
- Data entry, cleaning
- Controlling and storing
- Analysis, dissemination
- Other overarching processes

Data elements of registry
- Types of registry data
- Developing data definitions and variables
- Classifications and Value sets

- **Changing, improving a registry**
- **Stopping the registry**

May 2015: Guidelines to be presented for endorsement by eHealth Network

Two key use cases will be supported.

Use case 1:

- Help existing and new registries to be more interoperable (*Guidelines, best practices, CDS, SW*)

- Assess Interoperability of these registries/data sources (*Assessment Tool*)

Use case 2:

- Ability to identify registries/data sources (*PARENT Registry of Registries*)

- Provide search, retrieval and maintenance of (RoR) metadata from these registries

- Support exchange of data across registries (Query broker)

Who benefits?

HTA authorities (MS and EC)

EC

ERN

JRC

Research organizations

Reducing barriers for data re-use
Access to data

Access to data

Quality of data

Pharma &Device Industry

MS

Guidelines, Recommendations, Methodologies

Best practices

Information models, ontologies, vocabularies

Services, SW tools repository

Registry of Registries, Assessment Tool

Knowledge Management Platform

PARENT Framework

Guidelines
Comparison
IT tools

Health Auth.

Registry holders

xxxxx

Insurers

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HTA & patient registries: **the collaboration**

- PARENT WP6 WORKSHOP: The policy and implementation context for cross-border patient registries, Brussels 27.03.2014

- PARENT, ESC, EUnetHTA JA2 meeting Brussels, 15.04.2014

- Review of the ESC CRF/registry model for Atrial Fibrillation -   fitness for HTA of Novel Oral Anticoagulants (NOAC)

Emerging themes from HTA - feedback points

So far collaboration has pointed out:

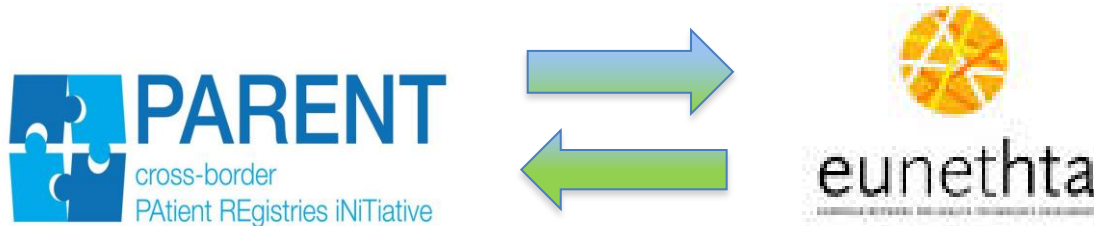- **Registries** could add to regular HTA methods:
  - Real life safety and clinical effectiveness
  - Rare events
  - Long term data
  - help in describing the population of interest
  - help in collecting data for later assessements

- An absolute necessity for registry data used in HTA is that registries **publish studies** based on the use of their data - > Guidelines, RoR

HTA & patient registries I

- **HTA related questions**
  - Do relevant registries exist already? -> Need for data resource identification tool
  - Do we need to alter/edit/change registries to facilitate HTA re-assesments?
  - Should we always contact registries to inform them of new technolgies?

- **HTA could use registries**
  - Early detection of new methods: collaboration in deciding which data to collect
  - Access to real life data when evaluating or monitoring the method over time (re-assessments)

HTA & patient registries II

- **Time-critical element in data acquisition** – particularly for rapid assessments. Use of HTA methods vs. the lifetime of technology (e.g. - early phases of the technology, registry data not yet available.)

- **Meaningful data** – **patient outcomes.** In a HTA perspective, it is important that we choose the most relevant outcomes, especially patient centered outcomes. → Methodology guidelines are needed about the outcome data (PARENT WP5 – RoR impact)

Joint Synergies (long term)

In order to address the above issues:

- Need to **investigate and enhance the linkage between registries and planned HTA work**.

- Establish a **process of notification** of registries with regard to **emerging/new technologies**. Such a process could be a means of closing the HTA/registry loop (e.g. by ensuring that the necessary codes are in use by the registries).

- ….etc.

Joint Synergies (short term)

Based on the potential ability of PARENT on promoting and supporting of registry-based HTA, PARENT and EUnetHTA JA (& EMA), joint synergies may include:

- PARENT's Guidelines for Patient Registries to incorporate relevant issues from:
    - The HTA Core Model
    - Methodological Guidelines for Rapid REA of Pharmaceuticals
    - Glossary of HTA Adaptation Terms
- Contribution to PARENT guidelines (HTA section co-authorship, review)
- PARENT's **generic registry data** set to be integrated to the extend possible with the **HTA Core Model**

The advantage of PARENT is that it can link the HTA needs with the registry holders and thus improve data quality and limit procedural barriers.

Next Collaborative Event

ISPOR 17th Annual European Congress

8-12 November 2014
Amsterdam RAI
Amsterdam, The Netherlands

Tuesday, 11 November 2014

| 9:00 - 10:00 | WORKSHOPS - SESSION II |

USE OF REAL WORLD DATA

W8: PATIENT REGISTRIES AS HTA TOOLS IN ECONOMIC OUTCOMES RESEARCH: REQUIREMENTS, BARRIERS, AND THE WAY FORWARD

Workshop participants will be provided with an overview of the analysis and results achieved through the collaborative work (PARENT JA & EUnetHTA JA2) accomplished thus far. In addition, participants will have the opportunity to get hands-on experience with the tools developed by PARENT JA.

Thank you for your attention

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