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SCIENCE MEDICINES HEALTH

## Update on Real World Evidence Data Collection

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An agency of the European Union 



## What is Real-world evidence?

“**Big data**” = umbrella term describing large data sets from any source.

“**Real-World data**” is a term used to describe healthcare related data that is collected outside of randomised clinical trials.

We focus on “**Real-World Evidence**” (RWE) meaning evidence coming from registries, electronic health records (EHRs), and insurance data.....

*.....where studies may be required by regulators through scientific advice, CHMP or PRAC and the subsequent results are used to inform regulatory and potentially HTA decision-making.*



## What is Real World Evidence?

*.....data that are collected outside the constraints of conventional randomised clinical trials.*

**Digital phenotypes**

**Electronic Health  
Records**

**Registries**

**Hospital data**

**Health Insurance  
Data**

**Patient and  
physician surveys**

**Biobanks**

**Genomes**

## Where is Use of RWE relevant to EU network/Agency work?

**Effectiveness**

**Benefit-risk**

**Pharmacovigilance**

**Innovative medicines**

**Adaptive pathway**

**Impact of regulatory measures**

**Utilisation in clinical practice**

**HTA Decision making**



## Place of Real-World Evidence and potential for the future

### RWE is already in routine use in the EU

- Particularly true for marketed products and for safety monitoring and drug utilisation.
- Increasing interest in the use of RWE for efficacy, outcomes for HTA, and for rapid cycle evaluation of medicines.
- There is major potential to increase the use of RWE to support lifecycle product development and monitoring and to improve decision-making for regulation and HTA.



## Potential of Real World Evidence to support Safe and Timely Access to Medicines for Patients (STAMP)

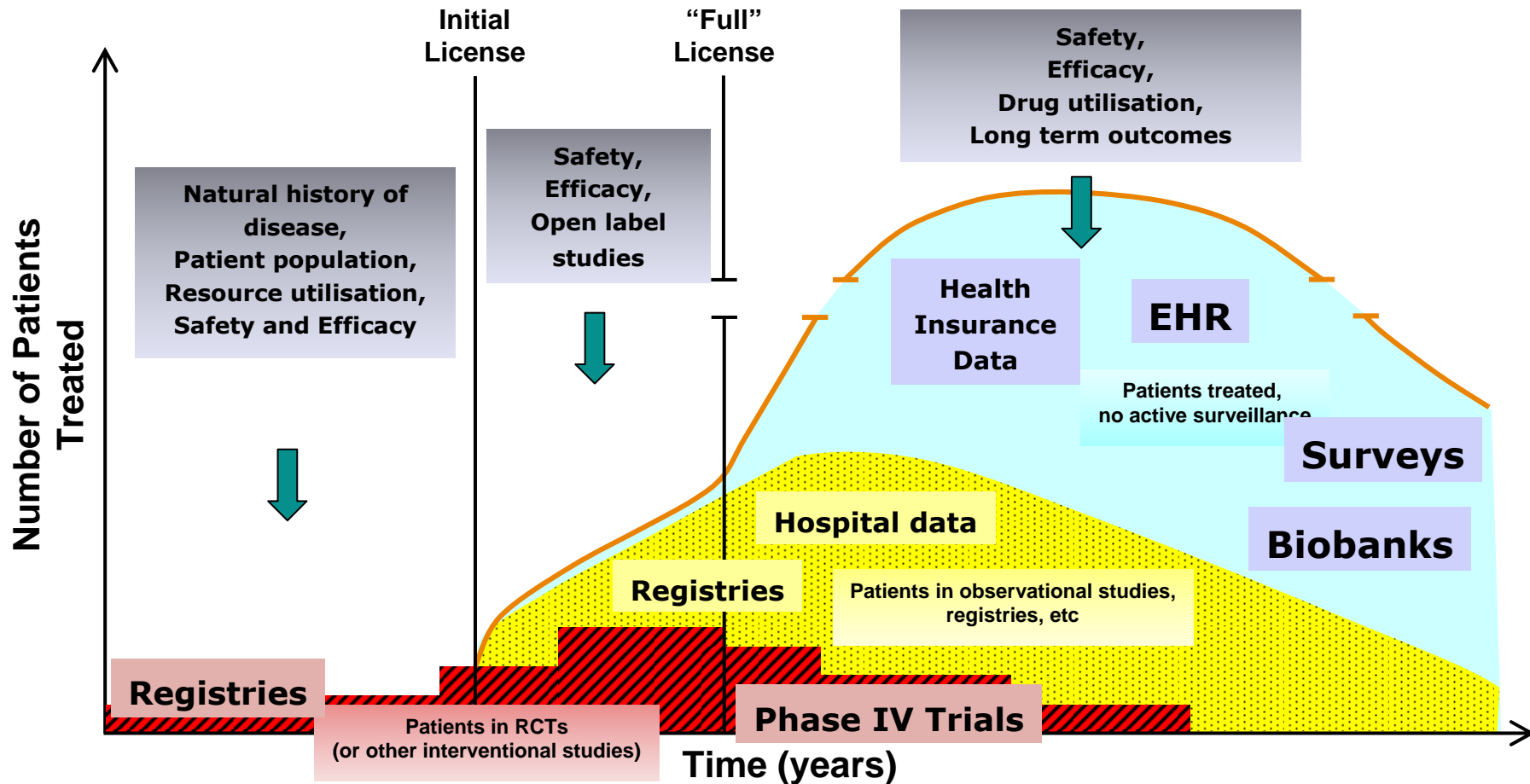
- Products in development: clinical trials the most important source of knowledge.
- However, RWE can inform development for example providing information on existing therapies and on the profile of patients needing treatment.
- Early product entry in niche indications will most likely use registries to collect effectiveness, safety and HTA information.
- Electronic Health Records and insurance data will become major sources of knowledge once the use of a product is more established.

*While the greatest potential of RWE is for authorised products, there is an important role in supporting innovative products and adaptive pathways.*

# RWE through the lifecycle



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## Challenges to realising the potential of Real World Evidence to support STAMP

There are challenges to realising the full potential for RWE and these *include*:

- incomplete access to electronic healthcare data from different MSs and a lack of hospital in-patient data;
- variable data quality and a lack of harmonisation;
- the need to develop methods for efficacy and HTA outcomes;
- delays to start studies;
- fragmentation of EU efforts to harness the potential.

# What is the current European landscape?



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## **Problem**

Increasingly number of individual product registries established to address post-licensing commitments rather than utilising existing disease registries.

This results in duplication of effort, a likely slower resolution of the initial concern and multiple, relatively inflexible registries with limited application in the future.

## **Aims of the Initiative – pilot phase launched September 2015**

To facilitate discussions at an early stage in the authorisation procedure between industry and registry owners to increase use of existing disease registries.

Where no suitable existing registry exists, to support the creation of new registries based on standard methodological approaches to ensure wider downstream applicability.

Understand better the challenges faced by registries and industry alike in collaborating.

Map ongoing initiatives at national and international level.



## Real-World Evidence: looking forward

There are already many national and EU initiatives ongoing to strengthen RWE.

There is a need for:

- Planning of RWE collection and analysis throughout product development:
  - delivered through scientific advice for products in development
  - Also delivered through benefit risk management planning at initial and post-authorisation
- Coordination between existing initiatives, leveraging outputs and identifying gaps;
- Cross-stakeholder collaborative approach to fill the gaps for RWE access and analysis.

In this way, the challenges can be addressed the full potential of RWE realised in supporting product development, monitoring and decision-making.