

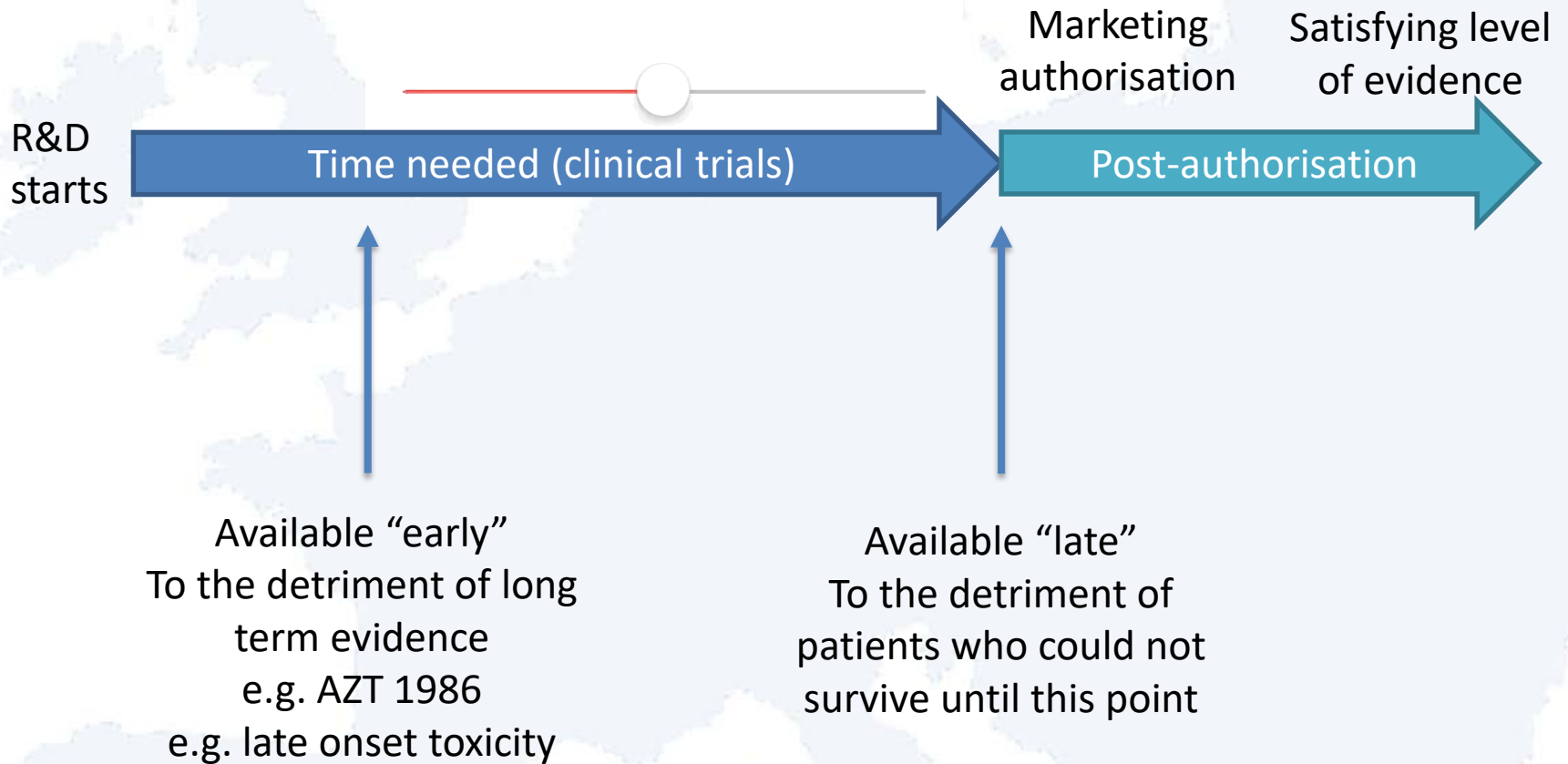


Real world data contribution to regulatory and coverage decisions

François Houyez, Director of Treatment Information and Access
European Parliament, 17 March 2017



Too early ? To soon?



Registers vs Patients: aims of a register

Register reality

1. Epidemiological research
2. Clinical research
3. Natural history of the disease
4. Disease surveillance
5. Treatment evaluation (efficacy/safety)
6. Genetic mutations database
7. Healthcare/Social Services planning

Patient expectations

1. Healthcare/Social Services planning
2. Treatment evaluation (efficacy/safety)
3. Natural history of the disease
4. Epidemiological research
5. Clinical research
6. Disease surveillance
7. Genetic mutations database

EPIRARE project 2013. 3,437 questionnaires analysed covering 500 rare diseases

Data collection: 1/07/2012 until 1/02/2013

http://www.epirare.eu/download/D1.2_EPIRARE_PatientSurvey_FINAL.pdf

Major Highlights of Patient Preferences

- Patients see data sharing as an imperative as long as:
 - Consent is obtained
 - Privacy and confidentiality are protected
 - Results are communicated to them
 - Patients are part of the governance
 - Access to data is transparent – especially cross border

RWE needed as (inter alias)

- Disease registries, more than product registries
- RWE patients different from CT patients
 - Comorbidities, Co-medications
- RWE data of interest for regulators and HTA
 - e.g. spontaneous haemorrhages in haemophiliacs using HIV protease inhibitors (compassionate use data) 1996
 - Measuring the efficacy of risk minimisation measures for cidofovir pre-MA (renal toxicity)
 - Celgene register for lenalidomide pay-for-performance agreement
- For gene therapy: single intervention – long-term outcome

RWE to measure if medicines work as expected

- Pay for performance agreements
 - Treatment paid to the MAH only for patients who respond
 - i.e. 100 patients treated, drug costs 10 000€/patient
 - Total cost: 1 000 000 €
 - In fact 60 patients responded as expected (registry data)
 - Paid to the MAH: 600 000€
 - Can only work if the MAH can verify whether 60 and not 100 of patients responded
 - Access to data? Data accessed/analysed by trusted third party? Who?
- Can we trust RWE data as RCTs (GCP, inspections)?

Importance of measuring Health outcomes via registries

- to ensure that patients are fully instructed in the proper use of medicines (dosage, compliance, etc.), so that, they have as great an impact on the disease as possible
- If inventor firm would support efforts by generic producers to copy its medicines, as such copying would further increase the number of users and hence the measure of the invention's impact on the disease burden (Patent Pool Initiative WHO)
- This would align the interests of inventor firms with those of patients and the generic drug producers — interests that currently are diametrically opposed