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Use cases for DARWIN EU

Payers Perspective

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Survey on the need of payers for RWD (Questions)

- To what extent are payers willing to use RWD for decision making on pricing & reimbursement?
- What types of data sources do they prefer for decision making on pricing & reimbursement?
- What are reasons for which payers would need RWE? For instance, assessing budget impact, changes in use over time, effectiveness in practice etc
- What additional circumstances would increase or change the acceptance and/or use RWE?
- In which (challenging) situations (e.g. for orphan medicinal products - including any specific products), are RWE needed the most?
- In which of these specific situations would international collaboration in collecting RWD be most beneficial?
- For which of these situations is RWD mostly urgently needed; and which of these situations could provide a suitable use-case for DARWIN?

Collected results from payers

- Most payers are still very cautious regarding RWD, prefer RCT data when possible
- Some of the payer & reimbursement organisations only have a remit for an initial assessment and therefore do not expect that RWD will become important for their decisions
- If RWD is useful, it is often perceived as additional to the RCT data:
  - Long-term effectiveness (is not available in trials)
  - Pharmacoeconomic assessments/budget impact
  - Comparing patient population in practice to patient population in the trials
  - Comparing actual use to the dose-regime that has been used in the trials
  - To assess price-volume agreements that are based on prescription data
  - In reassessment/re-evaluation of reimbursement decisions.

Most preferred use cases from the payer perspective

- (ultra)- orphan drugs and advanced therapy medicinal products (maybe oncology (agnostic))
  - Maybe also combination therapies (oncology)

- International approach to treatments for small patients groups
  - Limited information on these products after market authorisation
  - High uncertainty, high prices, high heterogeneity
  - Information on historic controls is often necessary/essential

- Direct collaboration between countries may be difficult for different reasons

- DARWIN EU may provide a structure to collect relevant data on an European level

Specific examples for use cases mentioned by payers (1)

- Haemophilia (gene therapies)
- Zynteglo (beta-thalassemia)
- Zolgensma (spinal muscular atrophy (SMA))
- Libmeldy (metachromatic leukodystrophy (MLD))
- Encetrinib, Larotrectinib (oncology, agnostic treatments)

Specific examples for use cases mentioned by payers (2)

- Current treatment of SMA, multiple myeloma (MM) and haemophilia
  - Type and duration of treatment
  - Combinations (sequential, consecutive) of treatments
  - Patient characteristics
  - Endpoints (survival, motoric function, ventilation (SMA), OS (MM), number of bleedings)

- Tumour agnostic drugs
  - Use of those drugs in Europe
    - Specified for tumour location/type
  - Long-term outcomes considering PFS and OS

- Use of COVID antiviral drugs
  - Use of different drugs in Europe
  - Effectiveness in practice

Final suggestions from payers

- Maybe select use cases that relate to the first data partners that are selected for DARWIN-EU
  - Example 1: GLP1 agonists, diabetes and obesity (for example IPCI, CPRD (GP))
  - Example 2: agnostic drugs using cancer registry (for example IKNL)
  - Example 3: bisphosphonate and teriparatide treatment of osteoporosis, fracture risk (for example IPCI, CPRD (GP) and Bordeaux hospital data)

- Important to know what type of information is available with data partners in order to understand which questions are realistic:
  - What type of information is available (drugs, patient outcomes and/or combinations)?
  - How recent are the available data and what is the follow-up of patients?
  - Can we really collect information that payers want (clinical outcomes, PROMS etc)?

- Use cases mentioned by payers for DARWIN EU are mostly in OMPs, ATMPs and oncology
  - these use cases are also of interest for HTA & Regulators