



Using RWE. Pilots from DARWIN-EU

Examples with pilots from DARWIN-EU. Is the data/evidence ready for use by payers? First insights

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Perspectives on use of RWD/RWE by 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

- International approach to treatments for small patient 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 a European level
- (ultra)- orphan drugs and advanced therapy medicinal products (maybe oncology (agnostic)), maybe also combination therapies (oncology)
- 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)

Early experiences on multiple myeloma case study

- 6 databases from 5 countries were selected (DE, ES, EE, FR, NL)
- Different types of data available (hospital databases, GP databases, population databases)
- Different sizes of databases (national vs local)
- Completeness of data differs across databases
- Information on treatment patterns is limited (only 1 database)

Take home messages

- DARWIN-EU may provide interesting opportunities for payers to receive RWD/RWE on medicines that may be used as part of their decision-making
- Current DARWIN-EU pilots for payers/HTA are in oncology
- Different databases can be used but not easy to accumulate and compare data
 - More data sources from more countries will be needed
 - Do we need more data curation in advance?
 - Are qualification procedures necessary?
- More pilots are needed to get a better insight into the usefulness of data collected through DARWIN-EU for payers
- Fundamental is also more investment in data analytics and methodology
- Discussions on whether a federated data approach provides sufficient detailed information i.e for rare diseases.

