EVENT REPORT

RWE4Decisions
REAL WORLD EVIDENCE
PUBLIC WEBINAR SERIES

Supporting HTA/Payer decision-making: Health data initiatives in Germany

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MODERATED BY

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SPEAKERS

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Dr Martin Danner
Managing Director, Federal Association of Self-Help Organisations for People with Disabilities and Chronic Diseases and their Relatives (BAG Selbsthilfe)

Dr Barthold Deiters
Head of Pharmaceuticals, Society for Efficiency and Quality in Health Insurance Companies (GWQ ServicePlus AG)

Dr Alexander Natz
Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)

The second RWE4Decisions webinar in the 2022 series focused on the new German process for routine practice data collection (AbD). Early engagement between industry, clinicians, patients and decision-makers, coordination between HTA assessors and the European Medicines Agency, and patient involvement in the process were all identified as important elements to optimize the use of RWD to inform Payer benefits assessments. However, there is still concern that the process is burdensome and may not deliver reliable evidence.

Dr Antje Behring outlined that under the German reimbursement system, there is free pricing at launch, and only after one year following the benefit assessment a price is negotiated. RCTs remain the preferred approach for the early benefit assessment and single-arm trials are in general not accepted, apart from orphan drugs and very few individual cases.

In terms of Real-World Data (RWD), the clinical registries in Germany do not capture all the relevant outcomes needed, particularly in relation to confounders, and may contain incomplete and low-quality data. Furthermore, due to the lack of a unique patient identifier in Germany, electronic health records are difficult to use. Germany has legislated for the development of centralised data collection structures by the Research Data Centre (FDZ) and the Federal Cancer Registry, which could address some of the shortcomings. While still under development, these centres will enable access to a wider range of medical data (e.g. medical billings) and support collection of more comprehensive data to suit Payers' needs.

To tackle these drawbacks, a new law enables the G-BA to request routine practice data collection (AbD) to inform the benefit assessment when major uncertainties exist that could be resolved by data collection within a specified timeframe. This process is only used sparingly – for orphan medicines or those authorised conditionally or under exceptional circumstances, no relevant ongoing studies, no comparator evidence and patient-centred or longer-term outcomes are missing. Then there is an assessment of necessity and feasibility of data collection as the G-BA may conclude that collecting the missing data would render the process too lengthy and therefore unfeasible.

These considerations are explored by the Institute for Quality and Efficiency in Health Care (IQWiG) and consultation is undertaken with experts. If AbD is deemed appropriate, especially in cases where there are good existing clinical registries, the G-BA specifies the research question which generally relates to the condition, not just the individual treatment. The health technology developer must then create a protocol and statistical analysis plan for approval by the G-BA and the duration of data collection is agreed. Monitoring of data collection is then expected at 18-month intervals.

Onasemnogene abeparvovec (compared to nusinersen) in spinal muscular atrophy (SMA) was the first product to undergo the AbD. The protocol is publicly available, and the interim reports will be published. Two other products have been approved for AbD (risdiplam for SMA and brexucabtagen autoleucel for mantle cell lymphoma) and three more are under consideration for myelofibrosis and haemophilia.

To ensure additional data collection can start at market entry, early engagement is needed with the registry holder, health technology developer, regulator and payer; but currently this early engagement is challenging before the marketing authorisation is granted. The protocol also needs to be drafted as soon as the IQWiG concept is received so that expert hearings can be focused on implementation issues. IQWiG and the G-BA are learning from the implementation challenges, but early experience shows that their implementation is burdensome and careful thought should be given as to whether a RCT would be preferable to a registry-based study.

Panellists then gave their views on the AbD process.
Dr Martin Danner underlined the importance of involving patients in all aspects of the design and implementation of the AbD, including determination of patient-relevant outcomes. The role of 'Self-Help Organisations' in Germany is fundamental to maintain communication throughout the process. Currently, patients in Germany have good access to medicines launched in the market. Patients are focused on the pricing of new drugs, following the benefit assessment. Endpoints in the assessment involve patient-relevant outcomes, such as quality of life. German patient organisations are well integrated in the early consultations within the G-BA, as well as in the discussion of study designs and the significance of study results. This ensures G-BA decisions are reached by consensus between all interested parties. This approach could serve as blueprint for patient organisations' role in benefit assessments across the European Union.

Dr Barthold Deiters highlighted the need for sickness funds to better use RWD to optimise the use of medicines, by for example addressing questions on populations to be treated and the role of comparators. Publications and engaging with physicians ensure continual and transparent dialogue between interested parties. In 2020, a data pool was introduced in collaboration with scientific institutions to analyse how drugs and their interventions are assessed. The findings are then shared between insurers and physicians. Challenges may arise due to RWD's volatility in time, however the use of RWD should not be underestimated.

Representing industry, **Dr Alexander Natz** called for solutions to fill data gaps in the overall process. In particular, opportunities for Member States to agree on data requirements and/or registries should be taken into consideration. The AbD procedure

follows a strong and robust methodology which grants the appropriate timespan for companies to generate additional data. Whilst the AbD procedure is burdensome, industry welcomes the increased use of RWD and suggests that an international registry, designed to meet the needs of systems like the AbD, might alleviate that burden. Early communication, across Europe, prior to setting up registries would avoid overloading companies in the data collection requirements. This should entail clear questions to developers on what is needed in terms of data collection in order to clarify their usefulness.

The following questions were raised by the audience and panellists:

- How could Germany use, within its system, data that is being collected in other countries?
- What impact does the GDPR have in Germany in the context of secondary use of data, and what opportunity does the European Health Data Space (EHDS) offer?
- Is AbD a Real-World Evidence generator or does it rather fall in the classical clinical research category?
- In practical terms, how does the G-BA select the companies that have to undergo the AbD?

In conclusion, panellists agreed that learnings from the German AbD system highlight the need for earlier collaboration among decision-makers, health technology developers, clinicians, patients and registry holders. This would help ensure that an appropriate data collection plan is agreed when a medicine enters the market, and that evidence of sufficient quality would be generated to inform the benefit assessment within a reasonable timeframe. Collaboration across jurisdictions is also needed to ensure data collection plans and reports are published, and to discuss implementation of EU health data governance regulations to enable appropriate data sharing.

RWE4Decisions REAL WORLD EVIDENCE

RWE4Decisions is a multi-stakeholder group, which has developed **stakeholder actions** that will better enable the use of real-world evidence in HTA/payer decisions about highly innovative technologies. The work has been commissioned by the Belgian National Institute of Health and Disability Insurance (INAMI-RIZIV) and contributors include HTA bodies and payers, regulatory agencies, patient groups, clinicians, industry and academic experts/researchers.

For further information and to watch the recording of the webinar, visit our website at **https://rwe4decisions.com/event/public-webinar-series-supporting-hta-payer-decision-making-health-data-initiatives-in-germany/**

We want to hear what you are doing to progress learnings on the use of RWE! Contact us at **secretariat@rwe4decisions.com** if you would like to join the RWE4Decisions Learning Network.

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