HTA319

# 3-year Report Card: RWE Collection in Germany

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## Introduction

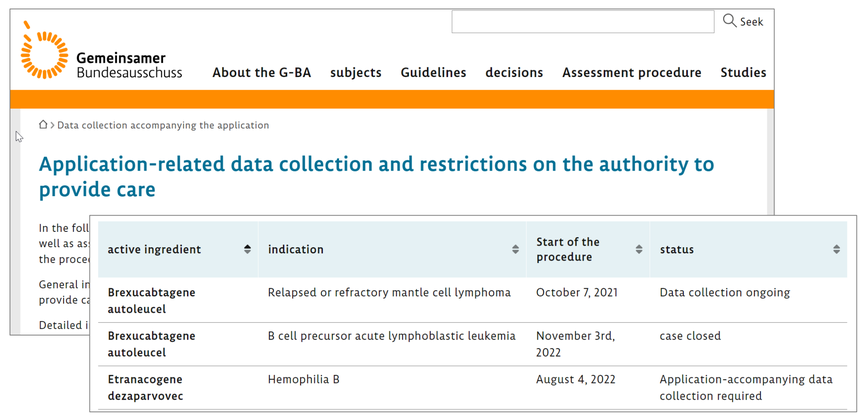
In 2020, legislation was passed in Germany that allows the G-BA to request real-world evidence (RWE) be collected to support a future renewed benefit assessment.

This is particularly relevant for products that receive expedited EC approval (e.g., orphan medicines, conditional approvals, approval under exceptional circumstances, and advanced therapy medicinal products [ATMPs]), because evidence available for the benefit assessment at launch is immature or insufficient for payers to derive meaningful conclusions around value.

This research compares characteristics and outcomes of therapies assessed to date and summarises key learnings for future therapies.

## Methods

Publicly available G-BA decisions around application-related data collection were identified from www.g-ba.de and key information was extracted (June 2023).




| active ingredient | indication | Start of the procedure | status |
|---|---|---|---|
| **Brexucabtagene autoleucel** | Relapsed or refractory mantle cell lymphoma | October 7, 2021 | Data collection ongoing |
| **Brexucabtagene autoleucel** | B cell precursor acute lymphoblastic leukemia | November 3rd, 2022 | case closed |
| **Etranacogene dezaparvovec** | Hemophilia B | August 4, 2022 | Application-accompanying data collection required |

## Results

**Table 1:** Products that have begun the G-BA assessment procedure for application-accompanying data collection, as of June 2023.

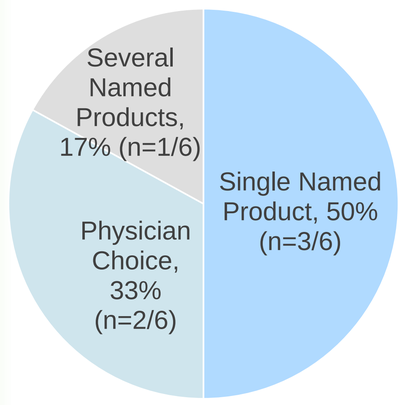
| Product | ATMP | Pivotal Trial Design | Outcome |
|---|---|---|---|
| *Onasemnogene abeparvovec* | ✓ | Single-arm | Data Collection Ongoing |
| *Risdiplam* | ✗ | Single-arm | Data Collection Required |
| *Brexucabtagene autoleucel (in MCL)* | ✓ | Single-arm | Data Collection Required |
| *Valoctocogene roxaparvovec* | ✓ | Single-arm | Data Collection Required |
| *Etranacogene dezaparvovec* | ✓ | Single-arm | Data Collection Required |
| *Fedratinib* | ✗ | Single-arm | Data Collection Required, but Not Carried Out* |
| *Brexucabtagene autoleucel (in ALL)* | ✓ | Single-arm | N/A |
| *Exagamglogene autotemcel* | ✓ | Single-arm | N/A |

Assessment Ongoing | Assessment Complete

Six products have completed the assessment of requirement for data collection, with two more currently being assessed. Data collection itself has begun for just one of the products – Zolgensma (onasemnogene abeparvovec).

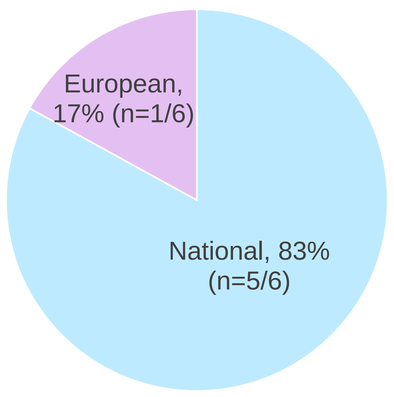
All eight products are EMA approved orphan medicines and six are ATMPs. All products launched with single-arm pivotal trials and so are lacking direct comparative data.

**Chart 1:** Comparators Requested



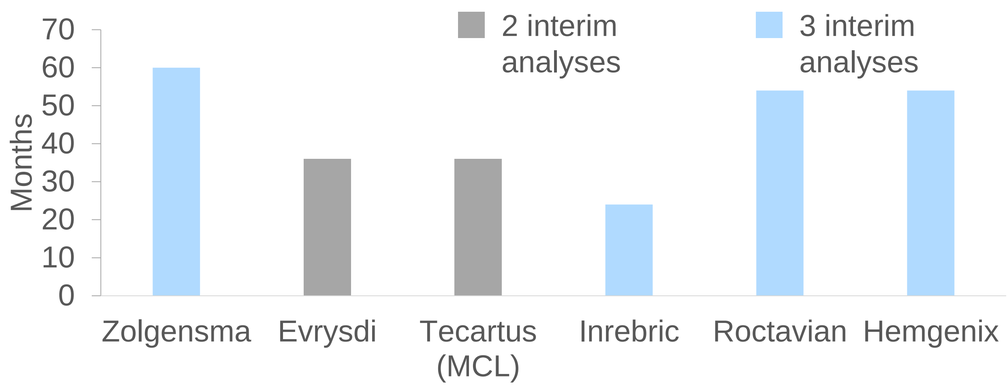

All six data requests specify that a non-randomised comparison with the current standard of care should be carried out; 67% of products require a specific comparator whilst the other 33% use patient-specific comparators (e.g., physician's choice).

**Chart 2:** Primary Registry Type Requested




The G-BA named their preferred primary data source for each product and requested data to be collected in a study-specific register. The majority were German national disease registers.

**Chart 3:** Duration of Follow-up Requested




The duration of requested data collection ranges from 24 to 60 months and, depending on study duration, two or three interim analyses were requested.

## Conclusions

Manufacturers with orphan medicines eligible for benefit assessment using single-arm pivotal studies are expected to be mandated for further data collection from the G-BA. This will likely include a request for non-randomised comparison to current SoC, with data collection likely via a national disease register and up to 5 years of monitoring required. This potential barrier also presents the opportunity to demonstrate additional benefits through post-launch RWE collection. Further research and monitoring is needed to establish if this data collection can be utilised to secure better HTA outcomes and enable price-renegotiation in Germany.

*The manufacturer of Inrebric (fedratinib) did not fulfil its obligation to create a statistical analysis plan and study protocol before carrying out the data collection during the application, therefore data collection will not be carried out.

**Abbreviations: RWE:** Real-world evidence; **G-BA:** Gemeinsamer Bundesausschuss (Federal Joint Committee); **ALL:** Acute lymphoblastic leukaemia; **MCL:** Mantle cell lymphoma; **ATMP:** Advanced therapeutic medicinal product; **EC:** European Commission; **EMA**: European Medicines Agency; **SoC:** Standard of care; **HTA**: Health technology assessment, **N/A**: Not applicable.

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