Scope of vignette:
- authorised products (with marketing authorisation)
- decision process about routine use (and not individual requests for reimbursement)
- submissions for P&R made by manufacturers

Green = related to/any special considerations for OMPs/UOMPs

Germany	**Standard process (non-orphan drugs)** **Benefit assessment by IQWIG, standard appraisal by G-BA**	**Special process (orphan drugs)** **Benefit assessment by G-BA, appraisal by G-BA**
Overview of health system and P&R/HTA process	Social insurance based health system [1] Statutory health insurance reimburses all licensed medicines following market access (provided the medicine is in principle eligible for reimbursement) based on a temporary price. The Federal Joint Committee (G-BA) and Institute for Quality and Efficiency in Health Care (IQWiG) are responsible for conducting benefit assessments of these newly authorised pharmaceuticals. The final resolution by G-BA forms the basis of negotiations on the final prices of statutory health insurances pay. Two distinct processes for non-orphan and orphan drugs exist. [2]	
Differentiation of rare disease treatments in the P&R system	EMA orphan designation	
Eligible medicines	All new drugs with marketing authorisation (non-orphan). Excluded are, for example, life-style indications.	- All new drugs with marketing authorisation and EMA orphan designation. The special process applies as long as the company revenues from the statutory health insurance (at pharmacy retail prices, including VAT) do not exceed 50 million euros over the past 12 months - The company may irrevocably notify the G-BA, if they want a standard (non-orphan) benefit assessment to be carried out for the new authorized drug with an orphan designation
Process	- The MAH submits a dossier to the G-BA with all authorisation documentation and studies carried out on the pharmaceutical. All parts of submission dossier template need to be completed. It aims to prove the added benefit of the drug over the appropriate comparator, specified by the G-BA [2]. The G-BA delegates the assessment to IQWiG	- Benefit assessment by G-BA: No comparison to appropriate comparator, but by results of their pivotal marketing authorization studies / based on the data submitted for marketing authorisation - Similar to standard process, with the exception that only selected parts of dossier template (Module 1-5) need to be completed (simplified requirements)

Germany	Standard process (non-orphan drugs) Benefit assessment by IQWIG, standard appraisal by G-BA	Special process (orphan drugs) Benefit assessment by G-BA, appraisal by G-BA
	- Benefit assessment done by IQWIG: Comparison to appropriate comparator as set by the G-BA (full assessment including recommendations regarding extent and plausibility of additional benefit) - The result of the benefit assessment is published on the internet, and pharmaceutical companies, federations, and experts are given the opportunity to submit written and verbal statements on the result - 3 months later after accounting for input received, the G-BA passes a resolution on the benefit assessment (extent of benefit, patient groups eligible, requirements for quality-assured administration, cost of treatment) - Within 6 months, the Central Federal Association of Health Insurance Funds (GSK-SV) and the MAH negotiate the price, e.g. rebate on retail price. If no agreement, arbitration board to determine price, considering EU price levels [2]	
Disease specific expert input (e.g. clinicians or patients in any stage of the process)	- (General and/or disease specific) Patient representatives participate in all sessions, but can only put topics on agenda, not vote.	- (General and/or disease specific) Patient representatives participate in all sessions, but can only put topics on agenda, not vote.
Key domains in assessment	Clinical benefit assessment (comparison to appropriate comparator)	Clinical benefit assessment
Evidentiary requirements	- Preference for RCTs, which can be complemented (but in general not replaced by) by non-RCT data [2]	- Preference for RCTs, but non-RCT data is more likely to be accepted (greater understanding for the challenges related to OMPs). [2]; - G-BA has no formal lower requirement for data collection, evaluation or validity for medicines with orphan designation; however, because the procedure requires G-BA to accept the pivotal studies submitted for initial MA, the acceptance of non-randomised or non-comparative data is greater for drugs with orphan designation - For OMPs, G-BA may request from the company to submit, within a reasonable

Germany	Standard process (non-orphan drugs) Benefit assessment by IQWIG, standard appraisal by G-BA	Special process (orphan drugs) Benefit assessment by G-BA, appraisal by G-BA
		time, an application-related data collection and evaluation for the purpose of benefit assessment (applies also to non-orphan Drugs authorized under "conditional approval" or „under exceptional circumstances")
PROMs	- Generic (preferably SF-36) and disease specific PROMs. Must be validated - EQ-5D not suitable, except visual analogue scale section	- Generic (preferably SF-36) and disease specific PROMs. Should be validated - EQ-5D not suitable, except visual analogue scale section
Appraisal framework	Appraisal by G-BA - levels of benefit: • Non-quantifiable, minor, considerable or major additional benefit • No additional benefit proved • Less benefit than the comparative therapy	Appraisal by G-BA: additional benefit guaranteed, levels of benefit: • Non-quantifiable, minor, considerable or major additional benefit • There is no general rule or criteria, but the rarity of the disease or a special target population (e.g. children) are taken into account also for assessment of new medicines without orphan designation
Reimbursement decision		
Pricing process	Price negotiation with statutory health insurance umbrella organisation (GKV-SV): If no additional benefit proven, reference pricing or agreement of price that must not exceed the comparator's price.	Price negotiation with statutory health insurance umbrella organisation (GKV-SV): the guaranteed additional benefit ensures a strong negotiation position and reasonable reimbursement.
Managed entry agreements	None	
Main challenges in appraising medicines for rare diseases (tick all that apply)	☐ Lack of good quality clinical data ☐ Lack of real world data ☐ Introducing value for money ☐ Monitoring treatment efficacy ☐ Managing budget impact ☐ Lack of criteria/transparency of OMP P&R processes ☐ Making arrangements to work for all stakeholders ☐ Lack of long-term meaningful outcomes	

Germany	Standard process (non-orphan drugs) Benefit assessment by IQWIG, standard appraisal by G-BA	Special process (orphan drugs) Benefit assessment by G-BA, appraisal by G-BA
	☐ Other, please specify	
Impact of special processes	The guaranteed additional benefit for orphan drugs ensures a strong negotiation position or strengthens the negotiation position of the company when negotiating the reimbursement price paid by the statutory health insurance funds. Consequently, it ensures a reasonable reimbursement for medicines for rare diseases, taking into account the possible limitations that medicines for rare diseases might face in the development and marketing process.	
Proposed policy change	None	
Joint initiatives		
SOURCES		
1	https://www.eu-patienten.de/en/behandlung_deutschland/deutsches_gesundheitssystem/gesundheitssystem_deutschland.jsp	
2	http://www.english.g-ba.de/benefitassessment/information/faq/#4	

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This vignette was compiled based on information provided by country experts and desk research. The information provided may be incomplete or contain inaccuracies. If you have any comments or updates, please email us at the following email addresses:

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