Securing Access to Medicines

Three Reform Areas for AMNOG in Global Competition

vfa. Die forschenden Pharma-Unternehmen

Why AMNOG needs to be realigned now

AMNOG was a key reason why patients in Germany gained early access to new medicines. It combined rapid availability with benefit-based pricing through negotiated agreements between payers and pharmaceutical companies. In doing so, it established an internationally recognized regulatory framework designed to strike a balance between patient access, affordability, and incentives for innovation. Global challenges and interventions introduced through the Statutory Health Insurance Contribution Rate Stabilization Act (BStabG) that are extraneous to the AMNOG framework have now thrown this balance off course.

The geopolitical landscape has fundamentally changed. Germany is no longer competing solely within Europe to secure a high-quality supply of medicines. Competition for research, development, early market launches, and investment is global. At the same time, German reimbursement prices are acquiring new global significance as international price referencing becomes increasingly widespread and as the US adopts most-favored-nation (MFN) drug pricing. In addition to existing international price-referencing systems, a low price in Germany may now also be used as a reference for a US price, thereby driving that price down. Given the outstanding importance of the US market, this increases the risk that innovative medicines will be launched later in Germany or may no longer be launched there at all.

Recent interventions through the BStabG have significantly weakened the core principle of AMNOG. Benefit-based price negotiations were intended to ensure that the therapeutic value of a medicine is reflected in its reimbursement amount, thereby maintaining the intended statutory balance between affordability, incentives for innovation, and giving patients access to high-quality medicines. Elements extraneous to AMNOG – such as across-the-board discounts, price-driven rather than benefit-based rebate agreements for patented medicines, and more restrictive price-volume mechanisms – undermine this principle. As a result, the recognition and rewarding of additional benefit in price negotiations is increasingly being replaced by a predetermined price. This intervention, including its application to agreements that are already in place, undermines predictability and reliability, two key prerequisites for launching new and innovative medicines and for positive investment and location decisions in Germany.

Medical progress is creating new challenges for AMNOG. Targeted therapies, small patient populations, novel therapeutic approaches in earlier lines of treatment or for chronic conditions are increasingly difficult to accommodate within existing assessment and pricing models. A system designed to meet the needs of future healthcare must reflect these developments and be capable of appropriately capturing the value of innovation without jeopardizing early access to innovative medicines.

Now more than ever, we have to ask ourselves: does AMNOG still have a future under these conditions – can it continue to embrace innovation and ensure a reliable supply of medicines? The answer is yes – but only if it returns to its core principles while adapting to the new realities. Action is urgently needed in **three key areas of reform**:

1. Actively address the risks of US pharmaceutical pricing policy for patient access to medicines

Global developments can no longer be ignored. They must be reflected in national policy frameworks. Drug prices cannot be viewed in isolation, country by country; they must always be considered in the context of international price referencing. This will require, in particular, **taking the US price level into account; allowing 12 months of unrestricted pricing; providing a more workable confidentiality option**; and **using reference criteria for pricing that reflect the value of innovative medicines**.

2. Return to a benefit- and evidence-based AMNOG

AMNOG's core principle must be restored and strengthened: reimbursement must reflect the proven additional benefit and therapeutic value of an innovation. The distortions introduced by the BStabG – including additional across-the-board discounts, non-benefit-based rebate agreements, and more restrictive price-volume mechanisms – have increasingly overridden this principle and are undermining the reliability of the system. **These interventions must be reversed.** Only then can AMNOG once again provide the predictable, benefit- and evidence-based framework needed to support the timely launch of medicines. In addition, an **inflation adjustment** should be introduced to prevent innovations from losing value over time.

3. Future-proof AMNOG

If AMNOG is to continue delivering innovative access to medicines, it must **evolve in step with medical progress**. This includes **recognizing additional benefit in special therapeutic situations**, establishing a **reliable assessment framework**, developing **innovative reimbursement models**, and **taking relevant healthcare needs** into account in price negotiations. AMNOG must also be better aligned with **European assessment processes**.

Geopolitical challenges
Competition for innovation

Benefit-based negotiation has been undermined
BStabG interventions extraneous to AMNOG

Medical progress
New challenges for benefit assessment

The way forward

1. Actively address the risks of US pharmaceutical pricing policy for patient access to medicines

- ☑ Take US price levels into account
- ☑ Strengthen confidentiality and free pricing
- ☑ Use reference benchmarks that reflect the value of innovation

2. Return to a benefit- and evidence-based AMNOG

- ☑ Correct BStabG distortions
  - mandatory discounts
  - prescribing-steering rebate agreements for patent-protected medicines
  - price-volume mechanisms
- ☑ Introduce an inflation adjustment

3. Future-proof AMNOG

- ☑ Recognize special therapeutic situations
- ☑ Align better with EU HTA
- ☑ Make zVT (appropriate comparator therapy) decisions reliable
- ☑ Take relevant healthcare needs into account
- ☑ Enable innovative reimbursement models

Goal: Securing Access to Medicines
rapid access to innovation | reliable framework conditions | a strong pharmaceutical hub

Reform area

1

Actively address the risks of US pharmaceutical pricing policy for patient access to medicines

The risks posed by current US pharmaceutical pricing policy threaten the future of innovative patient care. Policymakers must tackle these risks head-on to preserve Germany's attractiveness as an innovation hub and ensure that patients can continue to count on rapid and broad access to innovative medicines.

Recommendation

Leverage AMNOG as a key driver of patient access and Germany's competitiveness as a pharmaceutical hub

Ensure innovation reaches patients

Germany's declining attractiveness as a hub for innovation is disconnecting the country from medical progress. As it is, one in three medicines approved in the US over the past ten years is not approved in Germany. This means physicians lack access to 175 innovative medicines, including 51 with Breakthrough Therapy designation and the associated high medical potential. At the same time, Europe is losing ground as a destination for early approvals, including compared with China (CRA 2026).

Against the backdrop of new national legislation and US pharmaceutical pricing policy, one thing is clear: Germany's innovation gap is not primarily a regulatory problem. It is closely tied to the attractiveness and reliability of the German market. This underlines the urgency of making the AMNOG process predictable, ensuring that AMNOG continues to recognize the value of innovation, and creating stronger incentives for rapid market access. New statutory requirements driven solely by short-term cost-saving goals are not the answer.

In the global competition for innovation, AMNOG needs to be leveraged as a strategic factor in Germany's competitiveness as a pharmaceutical hub. Cost containment cannot be the sole benchmark, and it must not come at the expense of patients' access to innovative therapies. More than anything else, AMNOG must ensure that innovation reaches patients.

Address global challenges head-on

AMNOG pricing can no longer be viewed through a purely national lens. Through international price referencing under US drug pricing policies, German reimbursement prices feed directly into the world's largest pharmaceutical market. This puts significant downward pressure on prices in the US, putting the ability to recoup investment in research and development at risk. National price-setting decisions therefore have global implications for investment decisions and market launch strategies and may ultimately jeopardize the availability of innovative medicines in Germany. The BStabG interventions in AMNOG pricing significantly exacerbate these risks. The principle of negotiation under AMNOG must therefore be reinstated to ensure sufficient flexibility for individual agreements.

The risks posed by US pharmaceutical pricing policy must be addressed head-on to safeguard access to innovative medicines in Germany. These significant international repercussions must be mitigated by taking US price levels into account in reimbursement negotiations.

To keep Germany attractive for new market launches, free pricing should apply for the first twelve months after launch. This would make it more attractive for companies to seek European approval promptly for new medicines despite the effects of international price referencing, and to launch them in Germany as well.

The existing option to keep negotiated reimbursement amounts off public price lists (the ”confidentiality option”) must also be made permanent, expanded, and made genuinely workable. This would help limit the negative impact on other countries and ensure that potential European solutions are not undermined. To this end, the additional 9 % discount and the restriction to first-time market launch must be abolished. The rules must ensure that confidentiality is both effective and practical in real-world use. This should also include removing the requirement for pharmaceutical companies to compensate for distributors’ trade margins. The industry should not be required to bear costs it did not cause, an approach that the German Federal Constitutional Court (BVerfG) has likewise considered inappropriate.

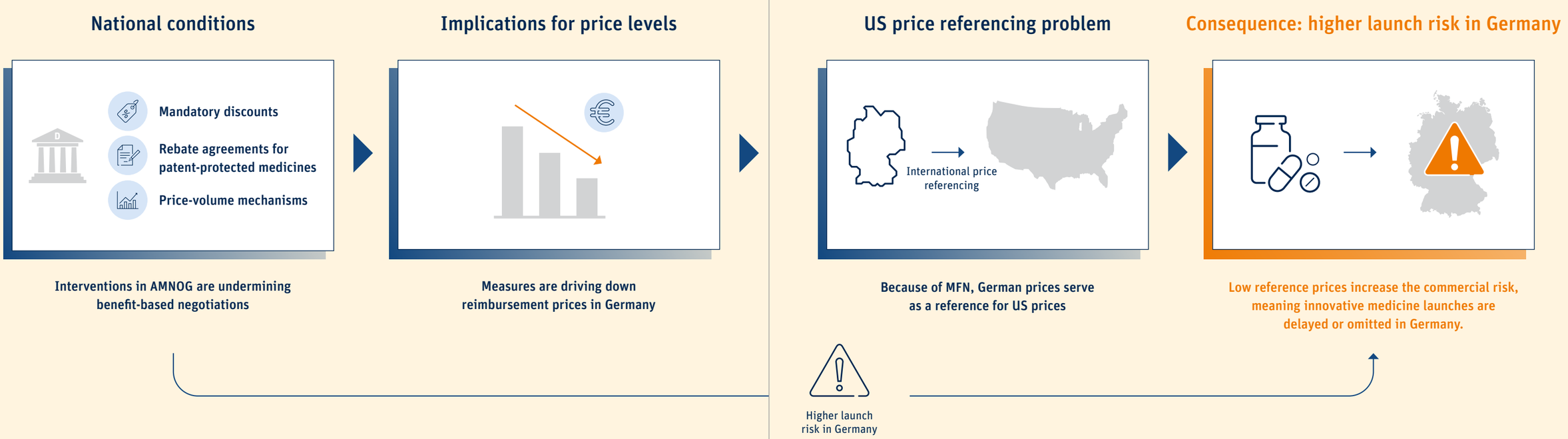
Price negotiations should also draw on innovation-appropriate reference benchmarks (”innovation anchors”). Generic prices reflect competition after patent protection expires. This is why they are not an appropriate benchmark for the value of a new therapy. The relevant benchmarks should instead be medical progress and comparable patented treatment options. This would give patients faster access to incremental innovations while strengthening incentives for research and development.

The challenges posed by MFN pricing call for new approaches and sustainable solutions. The measures outlined or currently under discussion are unlikely to be enough to address the associated risks effectively or with the necessary determination. Even so, they are a necessary first step and should be implemented consistently.

vfa proposal:

Pricing must evolve to reflect global developments and better recognize the value of innovation. Initial measures include:

- taking US price levels into account,
- allowing twelve months of free pricing,
- strengthening the confidentiality option, including by removing the additional discount and the requirement to compensate for distributors’ trade margins; and
- using innovation-appropriate reference benchmarks in price negotiations.



Reform area

2

Return to a benefit- and evidence-based AMNOG

Recent legislative interventions are putting the future of access to innovative medicines at risk. These policy missteps must be urgently corrected. Reliable, innovation-friendly framework conditions are essential to ensure rapid access to innovation and a strong pharmaceutical sector.

Recommendation

Create an environment that encourages innovation

Correct policy distortions

The core principle governing the pricing of innovative medicines under AMNOG has always been clear: The reimbursement amount is negotiated between the pharmaceutical company and the National Association of Statutory Health Insurance Funds (GKV-Spitzenverband) on the basis of the Federal Joint Committee's (G-BA) additional-benefit decision, and the medicine is reimbursed at the negotiated amount. This internationally recognized, benefit-based approach to pricing has made it possible to reflect the value of innovation appropriately while also rewarding pharmaceutical companies for generating and submitting the most comprehensive evidence possible. The BStabG has largely undermined this core principle by introducing measures that are extraneous to the AMNOG framework:

- An additional mandatory discount of 8.5 % on already negotiated reimbursement amounts intensifies price pressure regardless of a medicine's therapeutic value.
- Rebate agreements that steer prescribing of patented medicines abandon AMNOG's evidence-based principle and allow statutory health insurers to drive patient care on the basis of price alone.
- Restrictive price-volume mechanisms significantly reduce reimbursement amounts that were negotiated on the basis of benefit.

Taken together, these instruments run directly counter to AMNOG's principle of benefit-based pricing. The proven additional benefit and therapeutic value of an innovation are increasingly being overridden by across-the-board discounts, volume-based mechanisms, and subsequent rebate arrangements. In the future, through tendering procedures, statutory health insurers will have a decisive say in which specific therapy a patient receives, rather than the doctor treating the patient. In other words, prescribing will be driven by the lowest possible price rather than the most appropriate treatment. These measures go beyond deliberate government intervention in existing benefit-based reimbursement agreements. More than that, they mark a fundamental shift away from a system focused on benefit and patient care toward one in which the lowest price increasingly determines which medicines are prescribed.

Stop the erosion of innovation

The value of innovation is also being eroded by the complete absence of an inflation adjustment for reimbursement amounts. There is a tendency to overlook how pharmaceutical innovation is financed. The revenues generated by medicines available today do not merely recoup past investments. In fact, they are the sole economic foundation for current and future research and development. Clinical trials, highly qualified personnel, laboratory materials, and the continued development of already approved therapies are all facing substantial inflation-driven cost increases. When these real cost increases collide with reimbursement amounts that remain rigidly frozen for years, the financial resources available for new research projects shrink dramatically. This does not even factor in soaring energy, raw material, and production costs, which put additional pressure on security of supply. An AMNOG that seeks to strengthen Germany as a pharmaceutical hub must therefore provide for an inflation-adjustment mechanism to safeguard the funding needed for tomorrow's medical breakthroughs.

Patient access and Germany's pharmaceutical competitiveness at risk

Overall, price pressure has now been intensified to a degree that pharmaceutical companies can neither reliably anticipate nor accept. This compromises the future launch of new medicines and their continued availability. This development is also highly problematic for Germany as a location for innovation and pharmaceutical manufacturing. It weakens Germany in the global competition for research and investment. Investment and location decisions are neither automatic nor guaranteed. Reliable, innovation-friendly conditions for pharmaceutical investment require these harmful interventions to be reversed and benefit- and negotiation-based pricing to be restored.

vfa proposal:

- Innovation-hostile measures such as the additional manufacturer discount, rebate agreements for patented medicines, and restrictive price-volume mechanisms must be abolished.
- An inflation-adjustment mechanism for medicines subject to negotiated reimbursement amounts must be established.

Return to AMNOG principles

Benefit- and evidence-based AMNOG

Additional-benefit decision

Negotiation between pharmaceutical companies and payers

Reimbursement amount reflects the value of the innovation

Reliability and predictability

Rapid access to innovation

A strong pharmaceutical hub

Reform area

3

Future-proof AMNOG

New European assessment processes and advances in medicine are placing additional demands on AMNOG. These developments must be addressed to ensure that benefit assessment remains reliable, aligned with European processes, and open to innovation. Key priorities include recognizing special therapeutic situations; better aligning with the European HTA process; establishing greater reliability in determining the appropriate comparator therapy; taking relevant healthcare needs into account; and enabling innovative reimbursement models. However, further developing AMNOG will only achieve its intended goal if it is freed from the extraneous interventions described above, as a means of restoring Germany's international competitiveness.

Recommendation

Recognize additional benefit in special therapeutic situations

Research means change

In recent years, advances in medicine have increasingly enabled targeted therapies for well-defined (often smaller) patient populations, as well as earlier treatment approaches. These advances are creating new challenges for the assessment of medicines.

Regulatory authorities have been adapting to this shift for years. They assess case by case whether randomized controlled trials (RCTs) are appropriate, or whether alternative study approaches and endpoints need to be chosen to demonstrate benefit. This evaluation balances the timely availability of effective and safe medicines against the need to achieve the highest possible certainty in the study results. Key factors weighed up in this decision-making process include the nature, severity, and rarity of the disease and the level of unmet medical need. Where there is early evidence that patients are likely to derive particular benefit from the new treatment, or where it is clear that assessment of the long-term treatment effects would take too much time, prompt access to the treatment is needed. This access should be based on the evidence appropriate to the specific situation, including non-randomized studies as well as endpoints and risk factors relevant to the therapeutic context.

Structural flaw in the AMNOG process

The AMNOG process has yet to adapt its approach to non-randomized studies and to endpoints appropriate to the specific therapeutic context. However, the legal framework of benefit assessment recognizes in the Medicinal Product Benefit Assessment Ordinance (AM-NutzenV) that there are therapeutic settings in which it is ”impossible or inappropriate to conduct or demand studies at the highest level of evidence.” In such cases, ”best available evidence must be submitted.”

Furthermore, the AM-NutzenV stipulates that assessments should be conducted ”based on the available evidence” if ”valid data on patient-relevant endpoints is not yet available.”

However, these provisions have little practical effect in the assessment process because the appropriateness of requiring clinical studies at the highest level of evidence (i.e. RCTs) and data on patient-relevant endpoints is not reviewed. Submitted evidence from lower evidence levels and the available study results are routinely deemed unsuitable. As a result, the unique features of particular treatment situations are not adequately addressed. This has been a structural flaw in AMNOG since its introduction in 2011. The consequences are becoming increasingly clear. Additional therapeutic benefit is not given appropriate weight in special therapeutic situations. This shortcoming negatively impacts the availability and use of new treatments in patient care.

Establish whether a special therapeutic situation exists

At the request of the pharmaceutical company, the G-BA must determine whether a special therapeutic situation exists. This applies in particular to specific types of marketing authorization, such as conditional marketing authorization or marketing authorization under exceptional circumstances. A special therapeutic situation should also be deemed to exist where this is established on the basis of criteria such as the severity or prevalence of the disease and the availability of adequate treatment alternatives, i.e., an unmet medical need. The feasibility and appropriateness of conducting clinical studies and collecting relevant endpoints must also be taken into account. This applies in particular to assessing long-term treatment effects in early lines of treatment and in chronic or infectious diseases. The considerations of the regulatory authorities, as well as the

treatment objectives set out in relevant clinical guidelines and reflected in routine clinical practice, should also be taken into account.

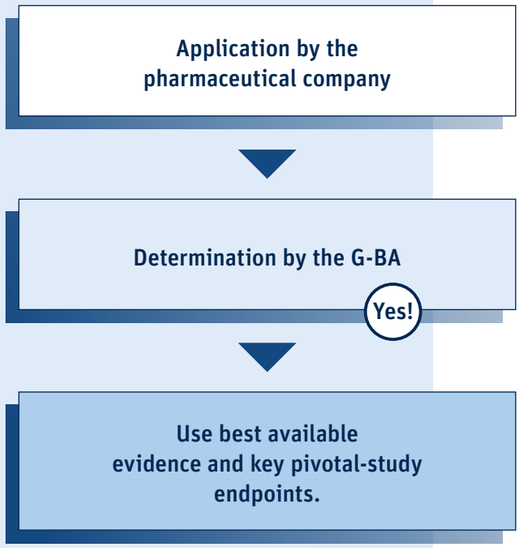
Use the best available evidence

Where a special therapeutic situation exists, the best available evidence and the key endpoints from the pivotal studies supporting the marketing authorization should be decisive for the assessment of additional benefit and used as the basis for the assessment. The level of certainty achieved with the best available evidence in the specific special therapeutic situation must be taken into account when assessing additional benefit.

vfa proposal:

- The G-BA determines whether a special therapeutic situation exists.
- The best available evidence and the key endpoints from the pivotal studies supporting the marketing authorization must be used to demonstrate additional benefit.

Does a special therapeutic situation exist?



Recommendation

Better align European HTA and national benefit assessment

Strengthen Europe's attractiveness as a location for innovation through European collaboration

Joint clinical assessment of new medicines at the European level offers an opportunity to strengthen Europe's position in the global competition for innovation while making benefit assessment in Germany more efficient. If implemented effectively, EU HTA (Health Technology Assessment) can eliminate duplication, streamline national processes, reduce bureaucracy, and improve market access. At the same time, it offers a historic opportunity to harmonize the fragmented clinical assessment landscape across Europe without limiting national responsibility for reimbursement decisions. To ensure that this potential translates into better patient care and greater European competitiveness, AMNOG must be systematically aligned with the European process.

Use European assessments efficiently

EU HTA can only deliver on its promise to reduce bureaucracy if the European assessment results are used consistently within AMNOG. However, the current rules leave too much scope for parallel national assessments based on the same European evidence. This risks turning European cooperation into another layer of assessment rather than simplifying national processes. The AMNOG process therefore needs a clear requirement to take European assessment results into account, thereby providing a more solid basis for national assessments. Any divergence from the European assessment should be based solely on new additional analyses submitted by the pharmaceutical company.

At the same time, the European and national requirements for analyses and evaluations must be more closely harmonized. German requirements can currently necessitate extensive additional national analyses. National requirements should therefore be aligned with the European framework wherever possible, with deviations limited to justified exceptional cases. Conversely, where they go beyond existing national requirements, European analyses should also be eligible for consideration in the German benefit assessment in appropriate areas, such as endpoints.

Take endpoints recognized at European level into account

For EU HTA to realize its full potential, national assessment standards must be more closely aligned with the European framework. Germany-only approaches to recognizing endpoints add to the fragmentation and make it harder to design clinical trials that meet both regulatory approval and benefit-assessment requirements. This increases development costs and weakens Europe as a location for research and innovation.

Endpoints are a key interface between regulatory approval and benefit assessment. Where requirements continue to diverge, additional evidence requirements arise without necessarily generating greater insight. AMNOG therefore needs greater flexibility to allow health-related endpoints used in EU HTA and key endpoints from European regulatory studies to be taken into account as a matter of principle.

Align the timing of European HTA and AMNOG

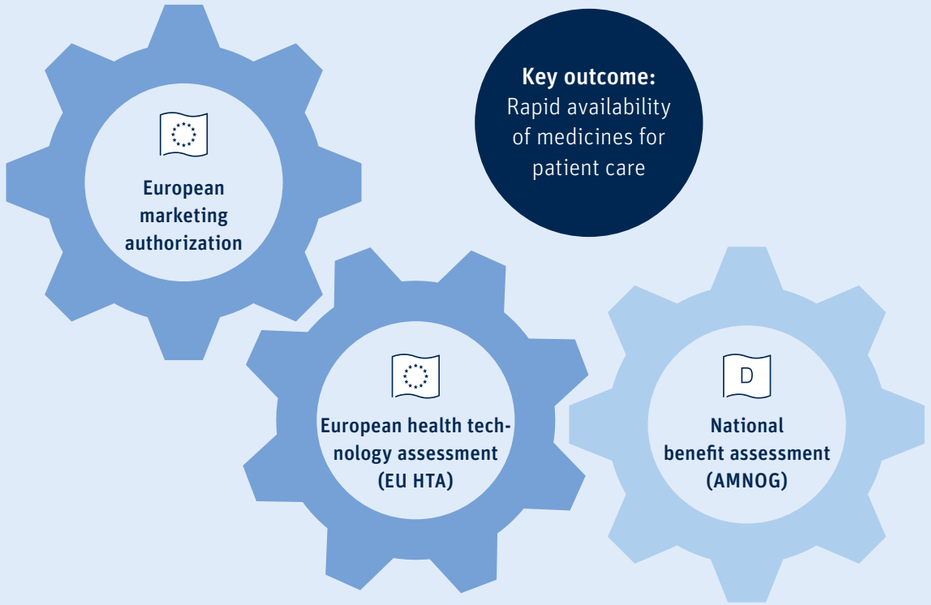
EU HTA can only provide an efficient basis for benefit assessment if the timing of the national process is better aligned with the publication of the European assessment. The desired alignment is not yet guaranteed, as AMNOG procedures can begin before the European assessment results are available. In such cases, the European results can only be incorporated later, during the formal response process. Parallel national assessments are produced that duplicate substantial portions of work carried out at the European level. So, rather than reducing duplication, EU HTA can end up creating more work for HTA bodies.

National procedural requirements should therefore be made more flexible to ensure that the European report always forms the basis for the benefit assessment, provided it is published within the first four weeks after the AMNOG procedure begins. Alternatively, the G-BA could be given the option to temporarily suspend the procedure until the European report is published. Both approaches would enable rapid market access and immediate use of the European assessment.

vfa proposal:

- Take endpoints recognized at European level into account.
- Align the timelines of European HTA and AMNOG procedures.
- Reduce fragmentation and make efficient use of European assessments.

Align
European and national processes



Seamless incorporation of EU results into the national process, avoiding duplication of effort and procedural obstacles

Recommendation

Improve reliability around the appropriate comparator therapy

The choice of appropriate comparator therapy (zVT) is central to the AMNOG process, as the additional benefit of new drugs must be demonstrated against this comparator. The zVT must continue to reflect the current state of medical knowledge without diminishing the value of high-quality evidence. Despite the existing option of consulting the G-BA for advice, the zVT requirement adds an element of uncertainty for the companies concerned. That is because the chosen zVT can be changed at any time by the G-BA, even after the relevant comparator studies have been initiated or completed. Such changes are particularly problematic when they undermine clinical studies conducted by pharmaceutical companies for the purpose of benefit assessment or cause them to be disregarded altogether, especially when those studies were conducted in accordance with G-BA advice. In such cases, formal requirements can result in additional benefit being deemed unproven despite high-quality scientific evidence. As a result, the value of the innovation in patient care is no longer clear to prescribers either.

More predictability is urgently needed. Where a clinical study has used an appropriate comparator therapy (zVT) in line with the G-BA's advice, additional benefit should also be demonstrable against that therapy. To improve transparency and predictability, the G-BA should also (as was standard practice for many years) proactively inform pharmaceutical companies of any changes to the appropriate comparator therapy as early as possible following a consultation.

vfa proposal:

- Allow additional benefit to be demonstrated versus a G-BA-advised zVT.

Recommendation

Take relevant healthcare needs into account

Indispensable in real-world healthcare

Pharmaceutical research continuously delivers new therapeutic approaches. Some medicines help patients through new mechanisms of action where existing treatment options are inadequate. Other new drugs may be the very first approved treatment option targeted to meet the needs of patients with specific conditions. Additionally, pharmaceutical innovations, whether used in outpatient or inpatient settings, can significantly ease the burden on the healthcare system – for instance, by helping preserve patients' ability to work and substantially reducing follow-up and long-term care needs, thereby also reducing costs. For all these reasons, they can rapidly become the standard of care and an indispensable part of patient care.

Care perspective overlooked

Yet under AMNOG, the therapeutic importance and system-wide benefits of such innovations are often not adequately recognized because their additional benefit is rejected on formal grounds. This has been the case in 38 % of all procedures to date. The consequences for the subsequent price negotiations can be serious. Where additional benefit has not been recognized, the reimbursement amount is intended to be set so that annual treatment costs do not exceed those of the zVT. A rigid negotiation framework of this kind cannot adequately reflect the actual therapeutic value of new medicines. The MFN approach to US price referencing (described above) further compounds the problem, as rigid price caps can make early market launches less attractive. The failure to take the patient care perspective into account therefore has a negative impact on the availability of new therapies and also compromises the ability to meet future healthcare needs.

Identify healthcare needs within AMNOG

The therapeutic significance of medicines with new active ingredients must be better reflected in benefit assessments. Where the formal additional benefit of a new drug cannot be demonstrated, greater consideration of its relevance to patient care is essential. In its decisions, the G-BA must explicitly recognize when a relevant healthcare need exists and the medicine contributes to meeting it, even where additional benefit has not formally been demonstrated. In making this determination, the expertise of clinicians and the patient perspective must be given due weight. Input from medical societies, from experts with hands-on treatment experience, and from patient representatives via the consultation process could help achieve this. This approach would bridge potential gaps between benefit assessment outcomes on the one hand, and expert medical opinion and real-world prescribing practices on the other.

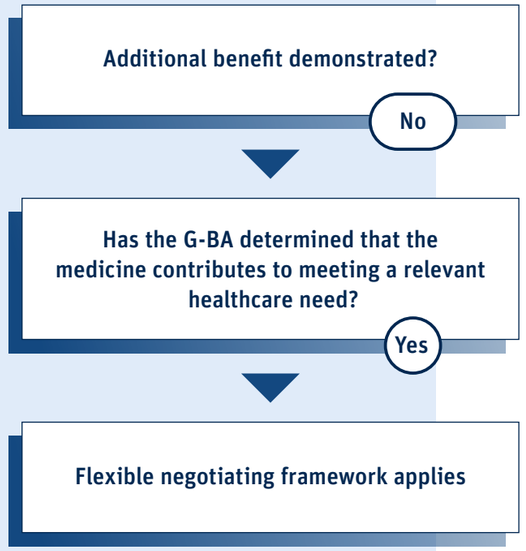
Enable flexibility in negotiations

If early benefit assessment decisions conclude that a relevant healthcare need exists and a medicine contributes to meeting it, the necessary flexibility must be provided for negotiating a reimbursement amount as per section 130b SGB V (the German Social Security Code, Book 5). This would give both negotiating parties – the National Association of Statutory Health Insurance Funds (representing the payers) and the pharmaceutical company – the leeway to jointly recognize the value of significant patient care-related pharmaceutical innovations during price negotiations. In such cases, it would be permissible to agree on a reimbursement amount that exceeds the applicable price limits for the specific scenario.

This would help ensure that patients continue to have access to essential medicines. At the same time, the principle that the reimbursement amount is primarily based on the outcome of benefit assessment is not undermined.

vfa proposal:

- Review whether a relevant healthcare need exists and whether the medicine contributes to meeting it.
- Create a flexible framework for reimbursement agreements.



Recommendation

Enable innovative reimbursement models

Lack of implementation

Innovative reimbursement models are currently difficult to implement within the framework of centralized AMNOG price negotiations with the National Association of Statutory Health Insurance Funds. The few reimbursement models concluded at the collective-contract level capture only a fraction of the opportunities to tailor agreements to individual cases. Often, prerequisites such as a suitable evidentiary basis or appropriate integration of such models into the morbidity-based risk adjustment scheme (“Morbi-RSA”) are lacking. So far, such models are most commonly found in individual contracts between certain health insurance providers and pharmaceutical manufacturers, primarily covering the first year after launch. However, once a centrally agreed reimbursement amount has been established, there is little incentive to adhere to existing (or establish new) decentralized agreements.

Existing challenges

The AMNOG pricing and reimbursement system is not tailored to suit therapies with justifiably limited evidence or one-time treatments with long-lasting effects. There are no provisions to ensure pricing levels commensurate with their clinical benefit. Gene and cell therapies, many of which are designed for one-time administration, are expected to provide a lasting response, but clinical data on the actual durability of response may still be limited at the time of market entry. Generally, therapeutic success can vary from case to case, whether for long-term treatments or one-time therapies. If necessary, ongoing treatment can be adjusted or discontinued, in which case the medicine itself generates no further costs. This is not possible with one-time treatments.

For this reason, pay-for-performance models are currently being discussed primarily for one-time treatments. However, certain criteria for implementation would apply, such as measurable outcome criteria and reasonable administrative effort.

Evolution of the legal framework

The legal framework should explicitly safeguard the broad range of options for different contractual models. Section 130b of the German Social Code, Book 5 should be amended to allow an alternative option of linking reimbursement to measurable treatment outcomes. In addition to refund models, the framework should also allow pharmaceutical companies and the National Association of Statutory Health Insurance Funds to agree on installment-based and multi-year adaptive payment models.

Make outcome measurement flexible and administratively manageable

Agreeing on suitable outcome criteria is central to pay-for-performance models. The contracting parties should be able to agree on a case-by-case basis on the data sources, outcome criteria, level of measurement, data analysis, data sharing, responsibilities, and deadlines for data provision. Outcome measurement could, for example, be conducted retrospectively using existing data or prospectively through targeted data collection and could include multiple assessment times.

Relevant outcome criteria might be clinical endpoints, appropriate surrogate endpoints, patient-reported measures, and healthcare-related measures. These could include avoided hospital admissions, a reduced need for subsequent treat-

ments, or other criteria that can be derived from routine data. The key is to ensure that the measures are both valid and administratively feasible. A legal definition of success criteria does not seem expedient as their suitability would need to be scrutinized case by case.

A degree of flexibility is required on the measurement side, too. Depending on the therapeutic situation, outcomes may be measured at the individual patient level, for a defined subpopulation, or across the entire treated population.

Necessary framework conditions

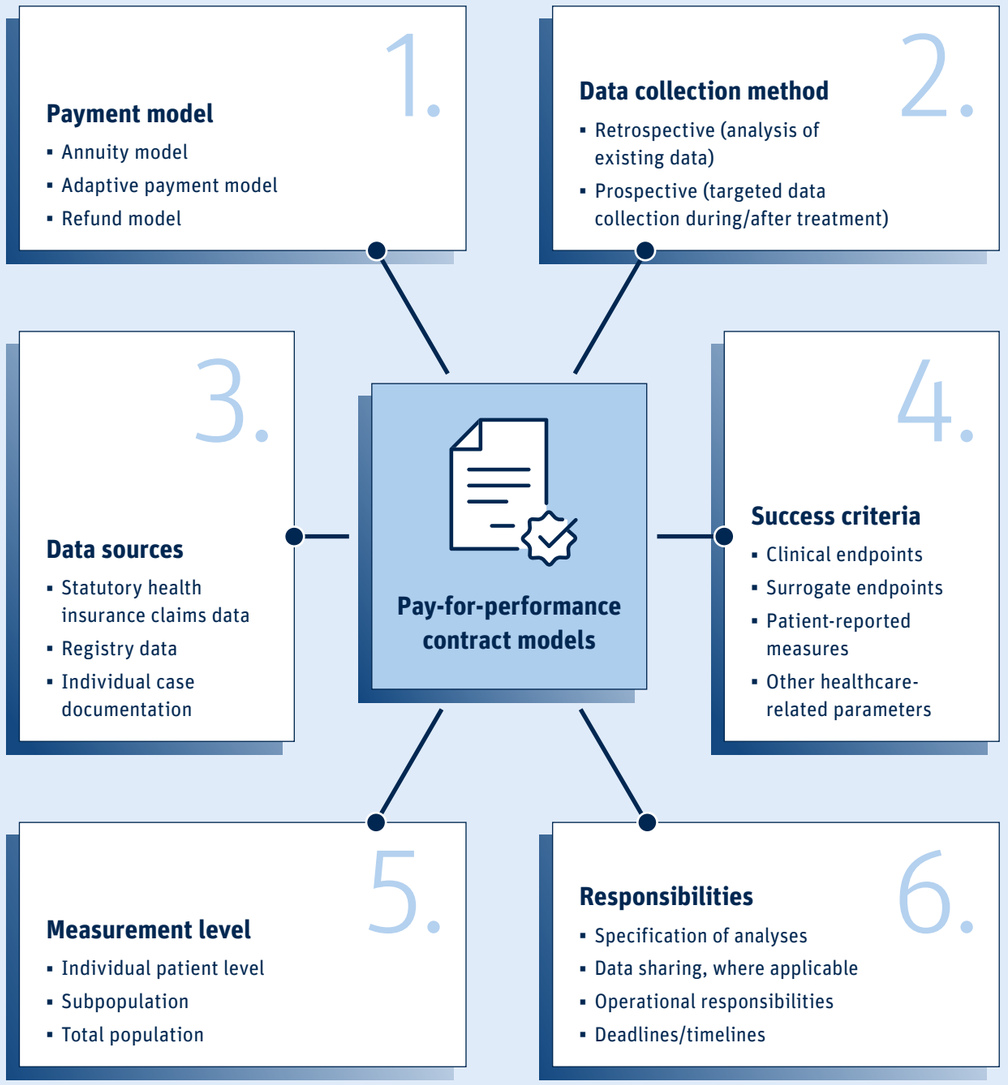
The current design of the risk pool within the risk adjustment scheme creates unequal treatment of different payment models. Refund models may be financially more attractive to statutory health insurers, while annuity models are placed at a disadvantage. The risk pool threshold, above which a portion of expenditure is reimbursed based on actual costs, is applied once for refund models but multiple times for annuity models.

As early as 2022, the Federal Office for Social Security (BAS) proposed a specific P4P reimbursement mechanism to address this issue. Under the proposal, an insured person receiving a medicine under a pay-for-performance contract would be assigned a case ID. This would allow the expenditure associated with each case to be tracked over multiple years, even in cases where the insured person switches health insurers. On this basis, both additional reimbursement amounts and any repayments to the Health Fund could be calculated, putting refund and annuity models on an equal footing. This proposal should be implemented without further delay. Implementation will in particular require amendments to section 268 SGB V and the Risk Adjustment Scheme Ordinance (Risikostrukturausgleichs-Verordnung).

vfa proposal:

- Innovative reimbursement models can be negotiated jointly.
- The legal framework must ensure sufficient flexibility for the design of reimbursement models.

Key aspects of performance-based contract models



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