ALAN
Acute Leukaemia Advocates Network

Involvement of German Patients in Health Technology Assessment and Reimbursement of Medicines at European and National Levels

Involvement of German Patients in Health Technology Assessments and Reimbursement of Medicines at European and National Levels

When new cancer medicines[1] are developed, pharmaceutical companies must apply for marketing authorization from the European Medicines Agency (EMA). This process has been in place since 1995 and covers all 27 European Union (EU countries), plus Iceland, Liechtenstein, and Norway—collectively referred to as "Europe" or “European countries” in this factsheet, prepared by the Acute Leukemia Advocates Network (ALAN).

The EMA reviews all the clinical trial data to decide whether the benefits of the medicine outweigh its risks. This is called the ‘benefit-risk assessment’. Only if the answer is yes, is the medicine granted a marketing authorization for use across Europe.

1. A European Joint Health Technology Assessment Since 2025

EMA approval alone does not guarantee patient access to a new medicine. For a medicine to be reimbursed through public healthcare systems, it must also undergo a Health Technology Assessment (HTA).

HTA is defined as “*A multidisciplinary process that summarizes information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. Its aim is to inform the formulation of safe, effective, health policies that are patient focused, and seek to achieve best value*" (EUnetHTA, 2007).

An HTA evaluates whether a treatment offers added benefits over existing options—and whether those benefits justify additional costs. It supports decision-making in the reimbursement process[2] and typically involves two main steps:

- **Assessment**: a scientific review of the evidence (e.g., how strong are the trial results?).
- **Appraisal**: a decision-making step that looks at context (e.g. is it for a rare disease? Is it affordable?).

Until 2024, each country did both steps on its own (see Figure 1).

European collaboration has extended beyond marketing authorization (AMM) to include the HTA assessment phase. This expanded cooperation follows a joint process governed by the European HTA Regulation (HTAR) (see Figure 2).

The goal is to help patients get quicker access to effective medicines by avoiding repetition of work between countries. It also helps ensure greater transparency in decision-making.

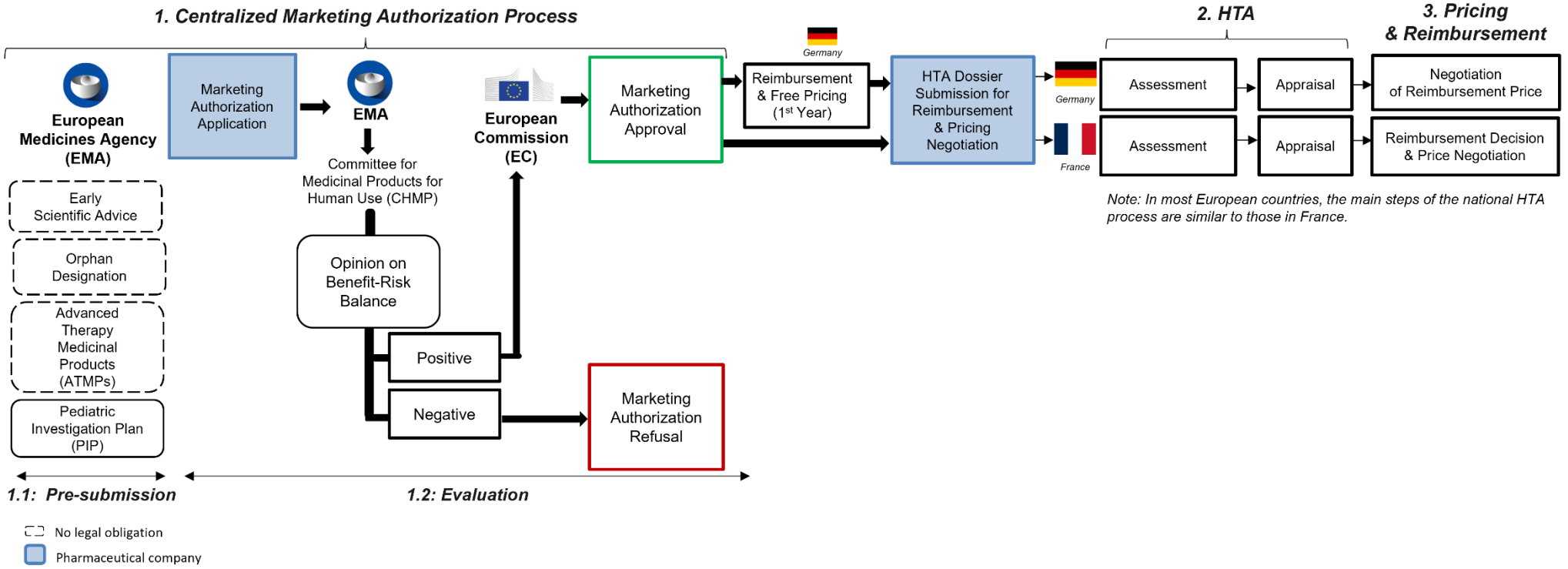
The EU HTA process currently applies to cancer medicines, including leukemia medicines, and advanced therapy medicinal products (ATMPs). It will expand to rare disease medicines (orphan drugs) in 2028, and to all centrally authorized medicines by 2030. Selected medical devices will also be included starting in 2026[3].

[1] In the EU, Iceland, Norway and Liechtenstein, most new and innovative medicines are approved through a centralized procedure: they are reviewed once by the EMA and, if approved, authorized for use in all of these countries. This process is required for medicines that treat serious conditions like cancer, HIV/AIDS, diabetes, and rare diseases, or that use advanced technologies like gene or cell therapy. For other medicines, it’s optional, but often chosen.

[2] For more information on what is HTA and how it differs to reimbursement, see our companion factsheet: *“Health Technology Assessment and Reimbursement: What is the Difference?”*

[3] Class III implantable medical devices (MDs); Class IIb active device intended to administer and/or remove medicinal product(s) (ARMP) MDs; Class D in-vitro diagnostics (IVD)

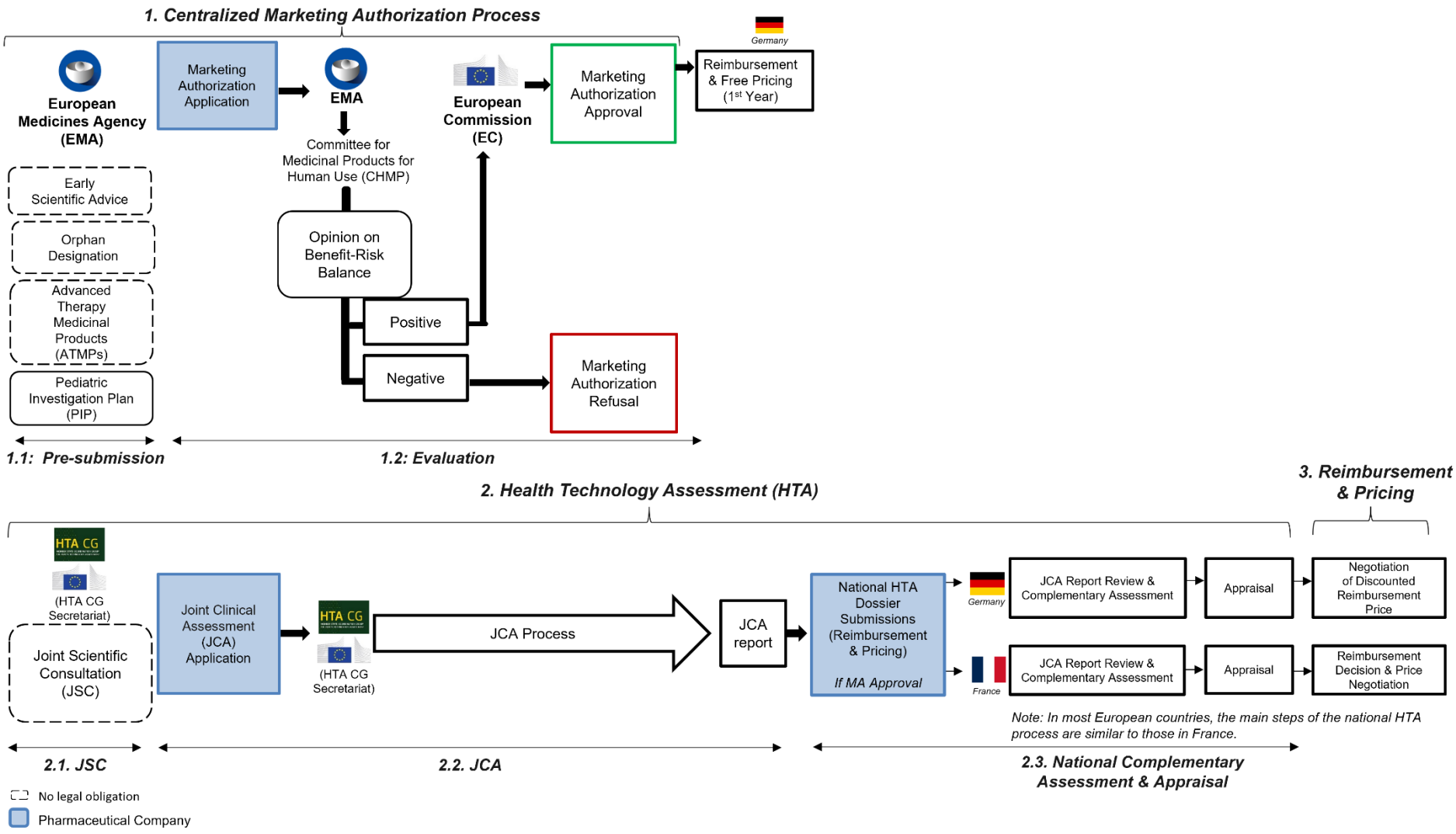
Figure 1. Marketing Authorization and HTA Processes of Cancer Medicines Until 2024



Acute Leukemia Advocates Network (April 2026). Adapted from French National Authority for Health (*Haute Autorité de santé*, HAS).

In the EU, Iceland, Norway and Liechtenstein, most new and innovative medicines are approved through a centralized procedure: they are reviewed once by the EMA and, if approved, authorized for use in all of these countries. This process is required for medicines that treat serious conditions like cancer, HIV/AIDS, diabetes, and rare diseases, or that use advanced technologies like gene or cell therapy. For other medicines, it's optional, but often chosen.

Figure 2. Marketing Authorization and HTA Processes of Cancer Medicines Since 2025



Acute Leukemia Advocates Network (April 2026). Adapted from French National Authority for Health (*Haute Autorité de santé*, HAS).

For more information on the marketing authorization process, read our companion factsheet "How Medicines Are Evaluated in Europe?"

2. How Patients Are Involved in European HTA

Since 2025, the European HTA Regulation has introduced two key processes at the European level: Joint Scientific Consultation and Joint Clinical Assessment. Their purpose and timeline are in Table 1.

Table 1. Objectives and Timeline of European HTA Processes

Step	Purpose	Timeline
Joint Scientific Consultation (JSC)	Provide early advice to help pharmaceutical companies design the right clinical studies	Early input before trials begin.
Joint Clinical Assessment (JCA)	Produce a shared scientific report on how a new medicine works compared to existing treatments	Starts at the time of EMA submission and runs in parallel.

These processes are jointly led by national authorities, the HTA Coordination Group (HTA CG), with support from the European Commission's HTA Secretariat and input from patients and other experts through the HTA Stakeholder Network.

Germany is represented in the HTA CG by the Federal Ministry of Health (*Bundesministerium für Gesundheit*, BMG), the Federal Joint Committee (*Gemeinsamer Bundesausschuss*, G-BA), and the Institute for Quality and Efficiency in Health Care (*Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen*, IQWiG).

For more information on European HTA governance and process, read our companion factsheet *"*How Medicines are Evaluated in Europe?*"*.

2.1. Joint Scientific Consultations (JSCs)

The JSCs of the HTA CG share a similar approach with the EMA's scientific advice (*scientific advice*), but their objectives differ. EMA scientific advice guides pharmaceutical companies on the design of their clinical trials before initiation, with a view to obtaining a marketing authorization. It primarily assesses the medicine's safety and efficacy. The JSCs, on the other hand, help pharmaceutical companies improve the design of their clinical trials before initiation, in order to generate the necessary evidence for future JCAs.

The contribution of patients, clinicians, and other relevant experts is a fundamental component of JSCs, as defined in the HTAR. They do so as individual experts. Patient contribution is not based on scientific or technical expertise but on their knowledge and lived experience of the disease, knowledge of existing treatments, and understanding of patient expectations regarding therapeutic innovations.

Patient organisations may also be invited through the HTA Stakeholder Network, which includes groups representing patients, healthcare professionals, and scientific societies.

Per the HTAR, the involvement of patients and clinicians in JSCs is mandatory— unlike EMA's scientific advice, where patient participation is optional. In practice, however, constraints (such as availability or conflicts of interest) may exceptionally limit participation, though measures are implemented to avoid such situations. All experts, whether patients or clinicians, must declare any conflicts of interest.

2.1.1 How Patients are Selected for a JSC

The HTA Secretariat is responsible for identifying experts (patient and clinician) and compiling a list of experts who have fulfilled the public declaration of interest requirements.

To build this list, the HTA Secretariat may request suggestions from:

- The HTA Stakeholder Network
- European Reference Networks (ERNs)
- The Orphanet portal for rare diseases and orphan drugs
- National contact points designated under Article 83(1) of Regulation (EU) No 536/2014 of the European Parliament and of the Council
- The EMA's pool of patient experts

If no suitable expert is identified through these channels, the HTA Secretariat may also consult:

- Other databases beyond those previously mentioned,
- Members of the Coordination Group (CG) and its subgroups,
- International and European organisations and agencies.

Any shared information must comply with the General Data Protection Regulation (GDPR)—for example, the expert must have given explicit consent for their details to be shared.

Final selection is made by the JSC Subgroup (the dedicated group within the HTA CG for joint scientific consultations). All selected experts must sign a confidentiality agreement.

The HTA Secretariat then relies on the Brussels Centre for Collaboration in Health (BCCH) to manage contractual and logistical arrangements with the experts.

2.1.2 What Patients Do in a JSC

Expert patients and clinicians:

- Receive the draft briefing package submitted by the company,
- Provide written or oral contributions (interviews) using a structured template (see Table 2).
- Review key documents, such as the list of issues and the outcome document,
- Are invited to participate in a discussion meeting with the JSC subgroup of the HTA CG and the pharmaceutical company (see Figure 3).

In addition to individual contributions, patient organisations may be consulted to provide broader perspectives on the disease or therapeutic options.

Table 2. Structured Form for JSC Patient Input

Section Number	Section Title	Description
1	Scope and objectives	Explains the purpose of the questionnaire.
2	How to complete this questionnaire	Provides guidance on how to fill it out.
3	Background information of the individual patient	Collects basic details about you as the contributing patient
4	Input by patient contributors (or representatives, proxies, carers, etc.)	Your experience with the disease
4.1	Impact of the disease/condition	How the condition affects your daily life and that of carers
4.2	Experience with currently available therapies/health technologies	How current treatments work (or don't work)
4.3	Expectations for new therapies/health technologies	What you hope a new treatment could improve or change;
4.4	Clinical development plan	Your thoughts on the proposed study design
4.5	Additional information	Any other points you would like to raise
4.6	Summary and key messages	A short summary of your most important points

The template described in Table 2 is valid until April 2026, after which the JSC subgroup will update it.

Clinicians use a different format than patients to provide their input. They use the PICO framework (Population, Intervention, Comparators, Outcomes) when advising on study design during JSCs (see Table 3).

Table 3. PICO Framework

Elements	Meaning
P – Population	Who is the treatment for?
I – Intervention	How does it work?
C – Comparators	What is it being compared to?
O – Outcomes	What outcomes matter most to patients?

All contributions to JSCs are submitted via the HTA IT Platform, which is managed by the HTA Secretariat,

For further details on the procedural requirements for JSCs on medicinal products, refer to Implementing Regulation 2024/3169 and the applicable Procedural Guidance for JSCs on Medicinal Products.

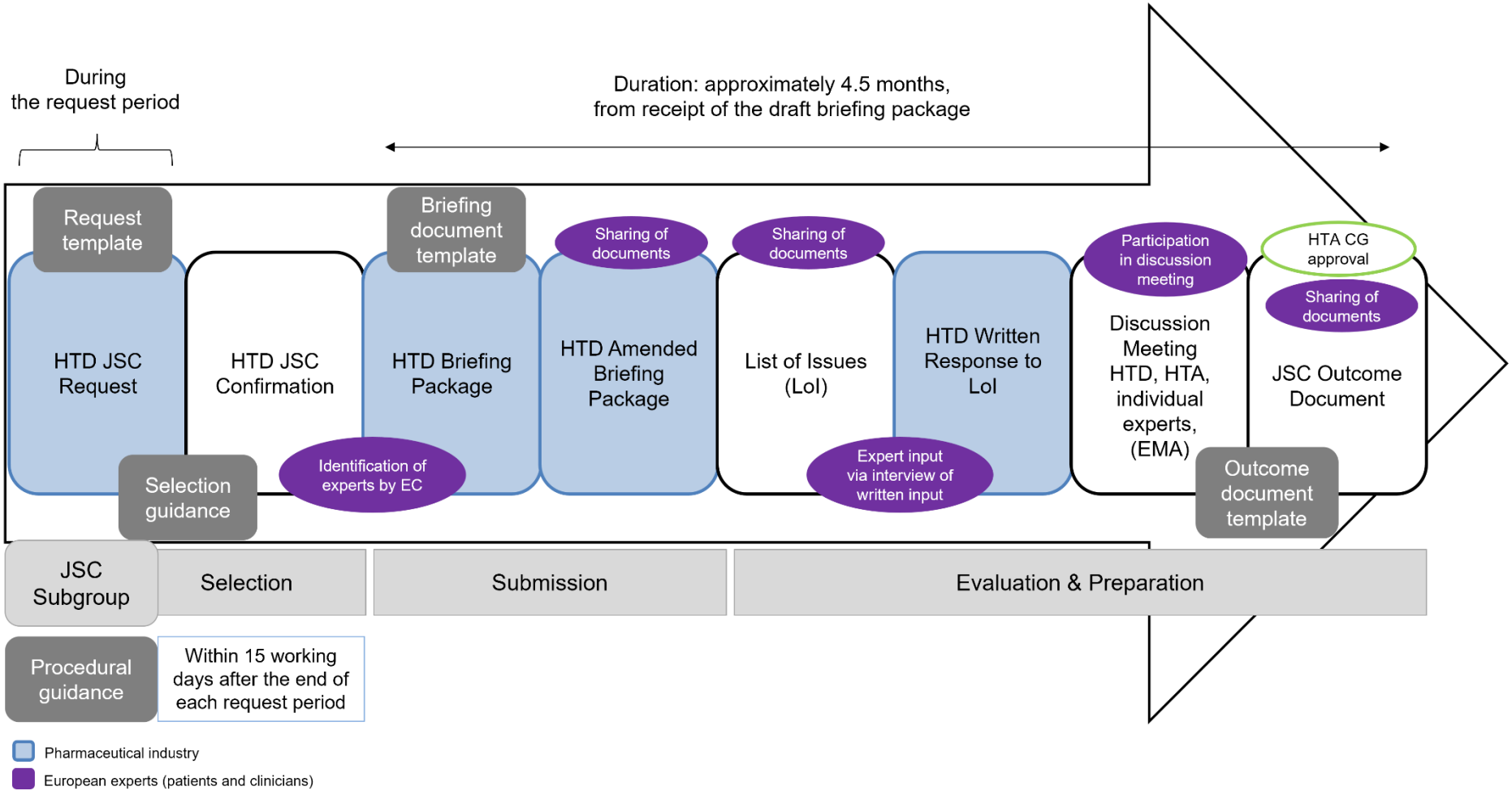
If you cannot find the answers to your questions, you may also contact SANTE-HTA-JSC@ec.europa.eu.

2.2. Joint Clinical Assessments (JCAs)

A JCA report is a shared scientific report drafted by two European HTA bodies (referred to as assessor and co-assessor). It is the outcome of the assessment phase, during which the new medicine is compared to existing treatments. Each country will subsequently use this report to inform its appraisal phase, which involves deciding on the medicine's funding (reimbursement, access conditions, etc.).

The JCA process begins when a company notifies the HTA Secretariat of the medicine's claimed indication (i.e., the proposed therapeutic use) at the same time it submits a marketing authorization application (MAA) to the EMA. At this stage, no clinical trial data are submitted.

Figure 3. Involvement of European Patients in Joint Scientific Consultations (JSCs)



Acute Leukemia Advocates Network (April 2026). Adapted from HTA CG "Health technology assessment: Webinar for patients and clinical experts". February 6, 2026.

The assessor and co-assessor develop an initial draft assessment scope using the PICO framework. It is also called draft PICO proposal, it is shared with all European countries via a PICO survey to gather national needs and priorities.

To ensure patient perspectives are reflected, patient experts may be invited to contribute, either directly by the HTA Secretariat or indirectly through national HTA agencies (see Table 3 & Figure 4).

2.2.1. How Patients are Selected

German patients may contribute to a JCA in two ways:

- As individual experts with European-wide experience, selected by the HTA Secretariat (see Section 2.1.1),
- As national experts, consulted by the G-BA, the German agency responsible for health technology assessments, via its Coordination Committee for Patient Representation (*Koordinierungsausschuss Patientenvertretung*).

While participation at the national level is optional, it is mandatory at the European level. In practice, constraints (such as availability or conflicts of interest) may exceptionally limit this participation, though measures are implemented to avoid such situations.

Expert patients must fill a declaration of interest each time they are selected to contribute, based on the specific medicine being assessed.

2.2.2. What Patients Do in a JCA?

As individual experts with European-wide experience, selected by the HTA Secretariat

The HTA Secretariat may invite European-level experts (patients and clinicians) to provide input on the draft PICO proposal. This step is optional.

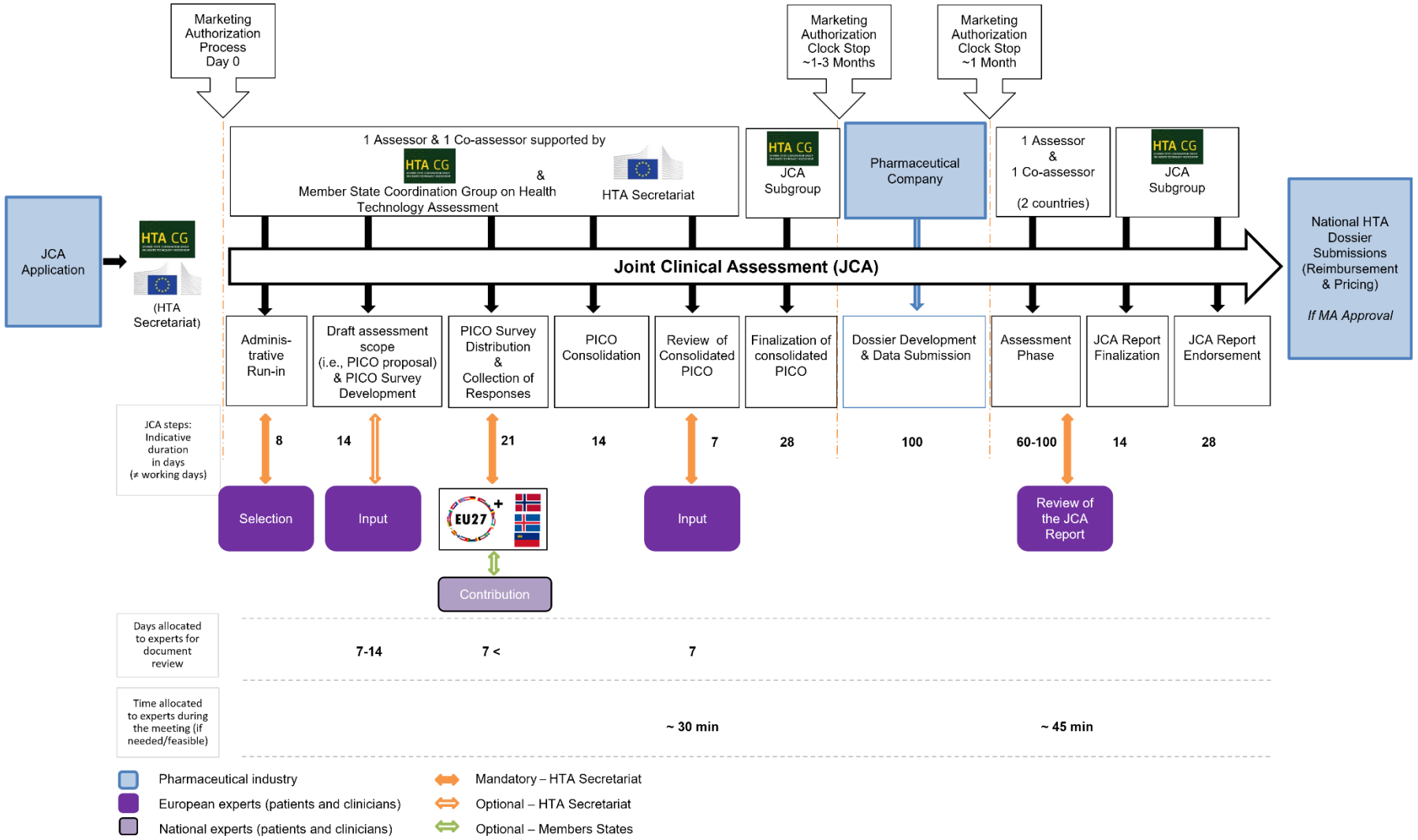
Once the assessing and co-assessing countries have reviewed and consolidated the comments on the proposed PICO(s) [4], the HTA Secretariat shares the consolidated version of the PICO with European experts, allowing them to provide further input. This step is mandatory.

After finalization, the consolidated PICO is sent to the manufacturer, which must then submit a dossier containing the data and evidence addressing the PICO requirements.

Based on the data provided by the manufacturer, the assessing and co-assessing countries conduct their evaluation in light of the consolidated PICO and draft a JCA report. European experts (patients and clinicians) are then invited to review the draft JCA report before its validation, in accordance with Article 15 of the JCA Implementing Regulation (see Table 3 & Figure 4).

[4] Each country can submit one or more PICOs that reflect its national needs. These remain in the JCA process unless the country later chooses to remove them. If several PICOs are similar, they may be merged to simplify the process — but only if the relevant countries agree.

Figure 4. Involvement of European Patients in Joint Clinical Assessments (JCAs)



MA : Marketing authorization; HTA CG : Member State Coordination Group on Health Technology Assessment; PICO : Patient, Intervention, Comparators, Outcomes

Acute Leukemia Advocates Network (April 2026). Adapted from French National Authority for Health (*Haute Autorité de santé*, HAS). And HTA CG .

As national experts, consulted by the G-BA

As the PICO survey is circulated to Member States for their input, they have the opportunity to engage national patient experts to contribute to their response to the proposed PICO(s). Germany is one of the few of Member States —such as Cyprus and France —that engage experts to comment on the PICO proposal, as this step is optional.

As soon as the selected patient expert has signed the confidentiality agreement, the G-BA Coordination Committee for Patient Representation provides them with the available information (i.e., the indication and, if the consultation timeline allows, the proposed PICO(s)), so that they can submit their comments using the PICO criteria.

The timeline for submitting PICO input is tight—only 21 days— and includes the selection, approval and contribution of expert patients.

All JCA contributions are submitted via the HTA IT Platform.

2.2.3. Information and Questions

For more information on the selection and role of patient and clinician experts in JCAs, visit the HTA Secretariat website and its dedicated FAQ page. You can also refer to the procedural requirements for JCAs, including Implementing Regulation 2024/1381 and the applicable Procedural Guidance for JCAs on Medicinal Products**.**

If you cannot find answers to your questions, you may contact: SANTE-HTA-JCA@ec.europa.eu.

For technical issues related to the HTA IT Platform, send your inquiries to: SANTE-HTA-IT-SUPPORT@ec.europa.eu.

3. How German Patients are Involved in HTA and Reimbursement at National Level

After a JCA is completed for a new medicine, the pharmaceutical company must apply for national reimbursement in each country. Each country then decides:

- Whether the medicine should be reimbursed
- Which patients should have access
- Under what conditions (e.g. hospital use only)
- At what price

In Germany, access to new medicines is fast: reimbursement begins automatically when the medicine receives EMA marketing authorization, and manufacturers are free to set the price for the first 12 months. This means that patients may have access even before the JCA report is finalised.

The JCA dossier does not replace the need for a national benefit assessment known as AMNOG (*Arzneimittelmarktneuordnungsgesetz*). Instead, the contents of the JCA are integrated into the AMNOG dossier, with adaptations to meet German methodological requirements. Although IQWiG and the G-BA cannot request data already submitted during the JCA, they may ask for supplementary information. Pharmaceutical companies, for their part, are prohibited from resubmitting at the national level any data previously submitted at the European level.

This benefit assessment is referred to as an "early benefit assessment" (*Frühe Nutzenbewertung*), because it is conducted immediately after the medicine enters the German market.

G-BA's Patient Participation Department (*Stabsstelle Patientenbeteiligung*), which works closely with the Coordination Committee for Patient Representation.

3.1. What the G-BA and IQWiG Do in Health Technology Assessment

The G-BA is the ultimate decision maker and relies on assessments by IQWiG, which evaluates the added benefit (*Zusatznutzen)* of the medicine. The G-BA then issues the final decision on the extent of that added benefit. The key questions guiding IQWiG's and G-BA's assessments and decisions are whether a new treatment

- Prolongs life
- Alleviates symptoms or complications
- Improves quality of life.

To demonstrate added benefit compared with existing therapies, the pharmaceutical company submits a dossier to the G-BA immediately after marketing authorization. The G-BA forwards it to IQWiG for scientific assessment. In this dossier, the company must show the patient-relevant added benefit of the new medicine versus an appropriate comparator therapy (ACT).

This assessment determines the extent of additional therapeutic value the new product provides for patients and the certainty of the supporting evidence. IQWiG follows transparent scientific principles set out in its General Methods (Version 8.0), which describe how evidence from published and unpublished clinical studies is evaluated.

Based on IQWiG's assessment —including input from patients via a standardized questionnaire— and oral input from clinical experts, manufacturers, and patient organisations during the G-BA's oral hearings (*mündliche Anhörung*), the G-BA conducts its appraisal. Within three months, it issues a binding decision on the extent of the added benefit, eligible patient populations, and requirements for use.

The extent of added benefit and the certainty (probability) of added benefit are graded in ascending order, as shown below in Table 4 and Table 5 respectively.

Table 4. Categories of Added Benefit

Added benefit	Basis
Less benefit / Harm	The benefit of the medicine is smaller than that of the appropriate comparator therapies (ACT).
No added benefit	No added benefit has been demonstrated
Non-quantifiable added benefit	Evidence suggests added benefit, but the data do not allow quantification.
Minor added benefit	A moderate, meaningful improvement in therapy-relevant outcomes compared with the ACT.
Considerable added benefit	A significant improvement in patient-relevant outcomes (e.g. survival, morbidity, quality of life).
Major added benefit	A sustained, substantial improvement in therapy-relevant outcomes previously unattained with the ACT.

Table 5. Probability of Added Benefit

Added benefit	Basis
Hint	Weakest certainty of conclusions
Indication	Medium certainty of conclusions
Proof	Highest certainty of conclusions

3.2. 'Involvement of Affected Individuals' at IQWiG and G-BA

Patient involvement in HTA can vary across countries and depends on terminology and institutional frameworks. In Germany, IQWiG and the G-BA distinguish between:

- Patient participation (*Patientenbeteiligung* or *Einbeziehung von Patienten*)
- Patient representation (*Patientenvertretung*), the latter being a less active form of participation

IQWiG uses the term "affected individuals" (*Betroffene*), which includes

- Patients, and their relatives
- Healthy target groups of specific health interventions
- Self-help groups and patient organisations (whose representatives may or may not be affected by a disease)
- Patient advocates.

Since December 2025, IQWiG's updated *General Methods (Version 8.0)* includes a standalone chapter on integrating patient perspectives (*"Einbindung von Betroffenenperspektiven"*). This elevates patient involvement from an ad-hoc practice to a structured, methodological standard, replacing previously scattered references.

3.3. How German Patients Provide Input to National HTAs

3.3.1. Patient Involvement in Early Advice and Early Access Programs

The G-BA provides early advice to pharmaceutical companies upon request. Patient organisations are actively involved in these procedures, particularly in defining the appropriate comparator or study design. IQWiG does not participate in the G-BA's early advice process.

Germany does not operate a separate early access programme, as reimbursement begins automatically once marketing authorization is granted. Instead, patient involvement focuses on the early benefit assessment.

3.3.2. Patient Involvement in Early Benefit Assessment

IQWiG's General Methods (Version 8.0) explicitly integrate affected individuals and patient organisations into early benefit assessments of medicines, except for orphan medicines, which are evaluated solely by the G-BA.

Who Can Submit Input?

When the G-BA Pharmaceuticals Subcommittee (*Unterausschuss Arzneimittel*) commissions IQWiG with an early benefit assesment, IQWiG initiates the involvement of affected individuals by consulting the spokesperson of G-BA's Coordination Committee for Patient Representation and the head of G-BA's Patient Participation Department (*Stabsstelle Patientenbeteiligung*). These stakeholders act as gatekeepers, identifying relevant patient organisations and inviting them to contribute. Individual patients are not directly involved in the early benefit assesment

process. Members of the Coordination Committee for Patient Representation are nominated by Germany's four major federal patient and self-help organisations:

- German Disability Council (*Deutsche Behindertenrat*, DBR)
- National Association of Patient Advisory Centres (*BundesArbeitsGemeinschaft der PatientInnenstellen*, BAGP)
- German Association of Self-Help Groups (*Arbeitsgemeinschaft Selbsthilfegruppen e.V.*)
- Federation of German Consumer Organisations (*Verbraucherzentrale Bundesverband e. V*.)

The Coordination Committee for Patient Representation act as a gatekeeper, identifying relevant patient organisations and inviting them to contribute and/or nominate affected individuals.

Unlike patient organisations in France, Canada, or Scotland, German patient groups cannot currently sign up for email alerts, follow social media updates from the G-BA or IQWiG, or consult a public list of medicines under review and submission deadlines.

Direct Involvement with Personal Contact

The spokesperson of the Coordination Committee for Patient Representation sends IQWiG's standardised questionnaire to identified relevant patient organisations representing affected individuals, inviting them to contribute to the early benefit assessment.

The primary reason for using a standardized questionnaire as a direct method of patient involvement is to meet the tight deadlines of the assessment process.

This standardised questionnaire covers:

- Disease-related information
  - Experience of affected individuals
  - Needs of specific patient groups
- Treatment-related information
  - Experience with currently therapies
  - Expectations of new treatments
- Additional information if relevant

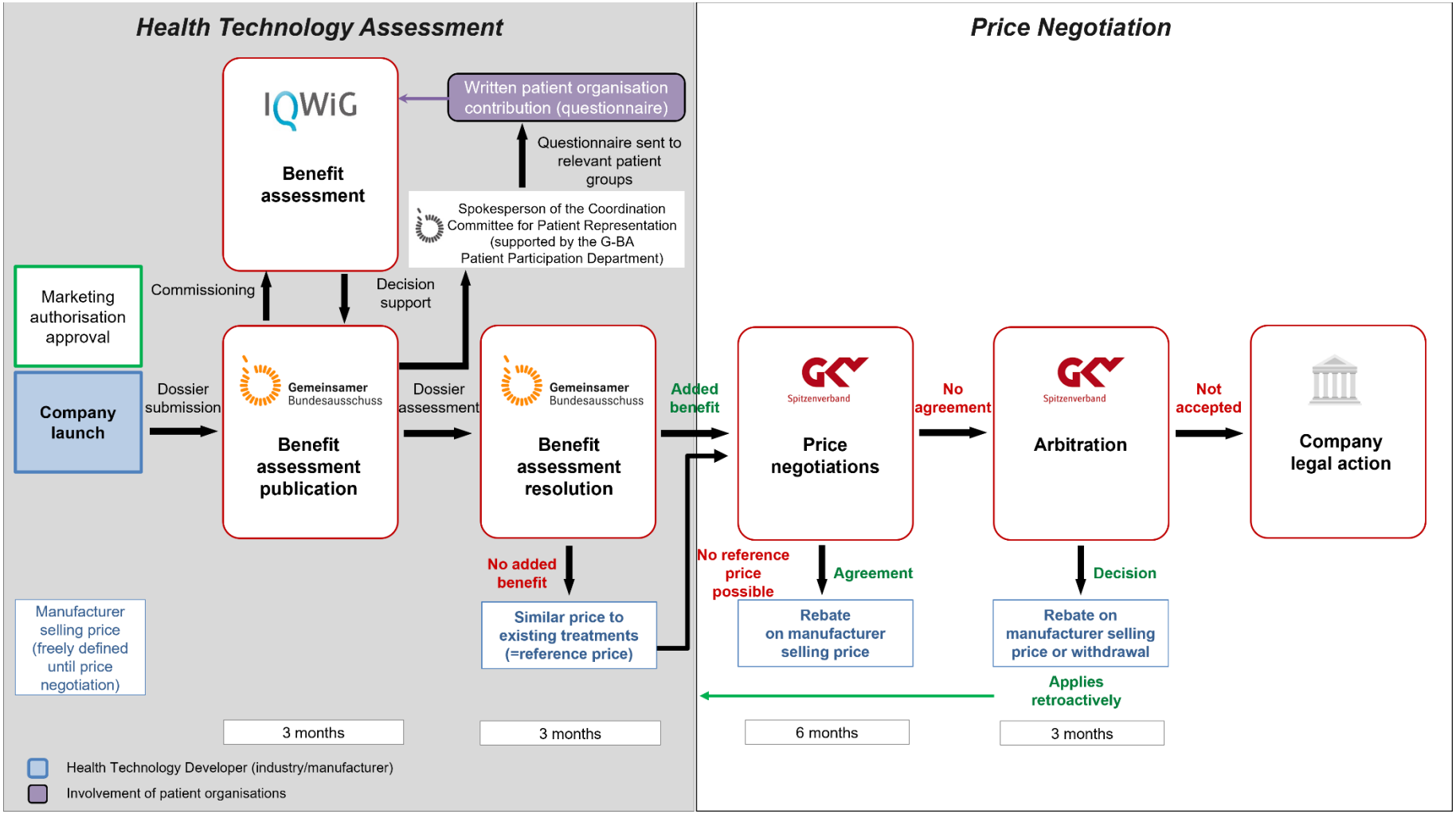
Affected individuals' written contributions help contextualize patient-relevant outcomes:

- Mortality – Does the treatment prolong survival?
- Morbidity – Does the treatment reduce symptoms and complications?
- Side effects – What are the adverse effects of the treatment?
- Health-related quality of life – Does the patient feel better?

The questionnaire is also available on IQWiG's website. Participants facing difficulties can contact the Institute for assistance. Patient groups have 15 working days (starting with IQWiG's contact to the spokesperson of the G-BA's Coordination Committee for Patient Representation inviting for contribution) to submit written answers to the questionnaire.

See Figure 5 for an overview of when and where written patient contributions are included in the early benefit assessment process (i.e., German HTA).

Figure 5. Involvement of German Patients in Early Benefit Assessment (National Complementary Assessment & Appraisal)



Gemeinsamer Bundesausschuss, G-BA: Federal Joint Committee; *Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen, IQWiG*: the Institute for Quality and Efficiency in Health Care; Benefit assessment report (*Nutzenbewertungsbericht);* Spokesperson of the Coordination Committee for Patient Representation (*Sprecher des Koordinierungsausschusses der Patientenvertretung*), Patient Participation Department (*Stabsstelle Patientenbeteiligung*), Benefit assessment resolution (*Beschluss zur Nutzenbewertung)*; GKV-SV: Federal Association of Statutory Health Insurance Funds (*Gesetzliche Krankenversicherung Spitzenverb*and, *GKV-SV*)

Chart adapted from SKC Beratungsgesellschaft mbH and PHARMA Deutschland

Indirect Involvement without Personal Contact

Indirect forms to integrate the perspectives of affected individuals may include IQWiG-produced outputs if already available at the time of the early benefit assessment:

- Real-life stories[5] (*Erfahrungsberichte*)
- Patient pathways (*Patientenwege*).

Direct and indirect methods are not alternatives but mutually reinforcing.

Public Comments and Appeals

While IQWiG (via the G-BA) invites patient organisations to provide input at the start of the HTA process, they cannot comment on draft benefit assessment reports.

IQWiG only opens public or patient commenting on "long" assessments of medical devices, procedures, or drug classes (*Nutzenbewertung von Arzneimitteln und von nichtmedikamentösen Verfahren*). This option is not available for the early benefit assessment of single medicines.

3.4. What Happens after the G-BA Resolution?

Although patients at the Coordination Committee for Patient Representation are involved throughout the process, they do not have voting rights when the G-BA adopts its resolution.

After the G-BA adopts its resolution, price negotiations begin between the pharmaceutical company and the Federal Association of Statutory Health Insurance Funds (*Gesetzliche Krankenversicherung Spitzenverband*, GKV-SV).

If no added benefit is confirmed, the medicine may remain on the market, but it will only be reimbursed at the same level as existing treatments—the so-called *reference price*—or at a price no higher than the standard therapy.

If the G-BA confirms that the new medicine offers an added benefit—for example, improved survival, symptom control, or quality of life—this added value becomes the basis for negotiating a higher reimbursement price.

The G-BA's resolution is binding and determines the extent to which the medicine's cost will be covered by the GKV-SV. If the manufacturer and GKV-SV cannot reach an agreement, an independent arbitration board —on which patient organisations have a consultative role— intervenes to determine the final price.

The negotiated reimbursement price is applied retroactively, usually from the seventh month after the medicine first entered the market. Medicines may later be reassessed if new clinical evidence becomes available.

[5] Translation per IQWiG´s General Methods. Version 7.0. of 19 September 2023

Want to Learn More?

Please read our companion factsheets:

- Health Technology Assessment and Reimbursement: What is the Difference?
- How Medicines Are Evaluated in Europe?
- Involvement of French Patients in Health Technology Assessment and Reimbursement of Medicines at European and National Levels

With sincere thanks to the staff of the Haute Autorité de Santé (HAS) for their valuable input and careful review of sections 1 and 2, and to the staff of the G-BA and IQWiG for their constructive contributions to the entire factsheet.

Written by Anne-Pierre Pickaert, advisor in health advocacy and communication (anne-pierre@care4access.fr), on behalf of the Acute Leukemia Advocates Network (April 2026).

REFERENCES

Arcà, E.; Barlassina, A.; Eze, A.; Strammiello, V. Enhancing Patient Engagement in HTA: Using Consensus Research to Overcome PICO Scoping Challenges Under the EU HTAR. J. Mark. Access Health Policy 2025, 13, 27. https://doi.org/10.3390/jmahp13020027

EUnetHTA (2007). EUNetHTA comments on the Discussion Document: "Health in Europe: A Strategic Approach". https://ec.europa.eu/health/ph_overview/strategy/docs/R-032.pdf. Accessed on on July 31, 2025.

European Commission. IMPLEMENTING THE EU HEALTH TECHNOLOGY ASSESSMENT REGULATION. 2023. https://health.ec.europa.eu/latest-updates/factsheet-implementing-eu-health-technology-assessment-regulation-2023-05-16_en Accessed on October 16, 2025.

European Union. Regulation (EU) 2021/2282 of the European Parliament and of the Council of 15 December 2021 on health technology assessment and amending Directive 2011/24/EU (Text with EEA relevance). PE/80/2021/INIT. https://eur-lex.europa.eu/eli/reg/2021/2282/oj

Commission Implementing Regulation (EU) 2024/1381 of 23 May 2024 laying down, pursuant to Regulation (EU) 2021/2282 on health technology assessment, procedural rules for the interaction during, exchange of information on, and participation in, the preparation and update of joint clinical assessments of medicinal products for human use at Union level, as well as templates for those joint clinical assessments. http://data.europa.eu/eli/reg_impl/2024/1381/oj

European Federation of Statisticians in the Pharmaceutical Industry (2025) German Benefit Assessment - White Paper LATEST METHODOLOGICAL REQUIREMENTS IN THE GERMAN BENEFIT ASSESSMENT CROSS-COMPANY COLLABORATION. https://www.efspi.org/wp-content/uploads/2025/05/GermanHTA_WhitePaper_2025.pdf Accessed on October 16, 2025.

G-BA. The benefit assessment of medicinal products in accordance with the German Social Code, Book Five (SGB V), section 35a. https://www.g-ba.de/english/benefitassessment/ Accessed on October 16, 2025.

IQWiG (2023) Beteiligung an der frühen Nutzenbewertung des IQWiG. Fragebogen zur Beschreibung einer Erkrankung und deren Behandlung für Betroffene https://www.iqwig.de/sich-einbringen/betroffenensicht-einbringen/ Accessed on October 16, 2025.

IQWiG (2025). Allgemeine Methoden (Version 8.0). Uploaded on December 19, 2025. https://www.iqwig.de/ueber-uns/methoden/methodenpapier/ Accessed on January 23, 2026

IQWiG Contributing the perspective of patients https://www.iqwig.de/en/participation/contributing-the-patients-perspective/ Accessed on October 16, 2025.

IQWiG Drug approval and early benefit assessment in Germany https://www.iqwig.de/en/presse/in-the-focus/new-drugs-approval-benefit-assessment-coverage/1-drug-approval-and-early-benefit-assessment-in-germany/ Accessed on October 16, 2025.

IQWiG. Contributing the perspective of patients https://www.iqwig.de/en/participation/contributing-the-patients-perspective/ Accessed on October 16, 2025.

IQWiG. The AMNOG procedure https://www.iqwig.de/en/presse/media-centre/figures-and-graphs/das-amnog-verfahren/ Accessed on October 16, 2025.

IQWiG Responsibilities and objectives of IQWiG https://www.iqwig.de/en/about-us/responsibilities-and-objectives-of-iqwig/ Accessed on October 16, 2025.

IQWiG. Patient-relevant endpoints – the essence of benefit assessments https://www.iqwig.de/en/presse/media-centre/figures-and-graphs/infographic-patient-relevant-endpoints/ Accessed on October 16, 2025.

Member State Coordination Group on Health Technology Assessment (HTA CG). EU HTA Regulation for Patients and Clinical Experts. Webinar May 16, 2025.

Member State Coordination Group on Health Technology Assessment (HTA CG). Health technology assessment: Webinar for patients and clinical experts". February 6, 2026.

PHARMA Deutschland. Das AMNOG-Verfahren. https://www.pharmadeutschland.de/unsere-themen/preise-und-regularien/das-amnog-verfahren/ Accessed on October 16, 2025.

ALAN
Acute Leukaemia Advocates Network