ALAN
Acute Leukaemia Advocates Network





How Medicines Are Evaluated in Europe

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How Medicines Are Evaluated in Europe

When new cancer medicines[1] are developed, pharmaceutical companies must apply for marketing authorisation from the European Medicines Agency (EMA). This process has been in place since 1995 and covers all 27 European Union (EU countries), plus Iceland 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 authorisation 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 (seeFigure 1).

European collaboration has extended beyond marketing authorisation (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.

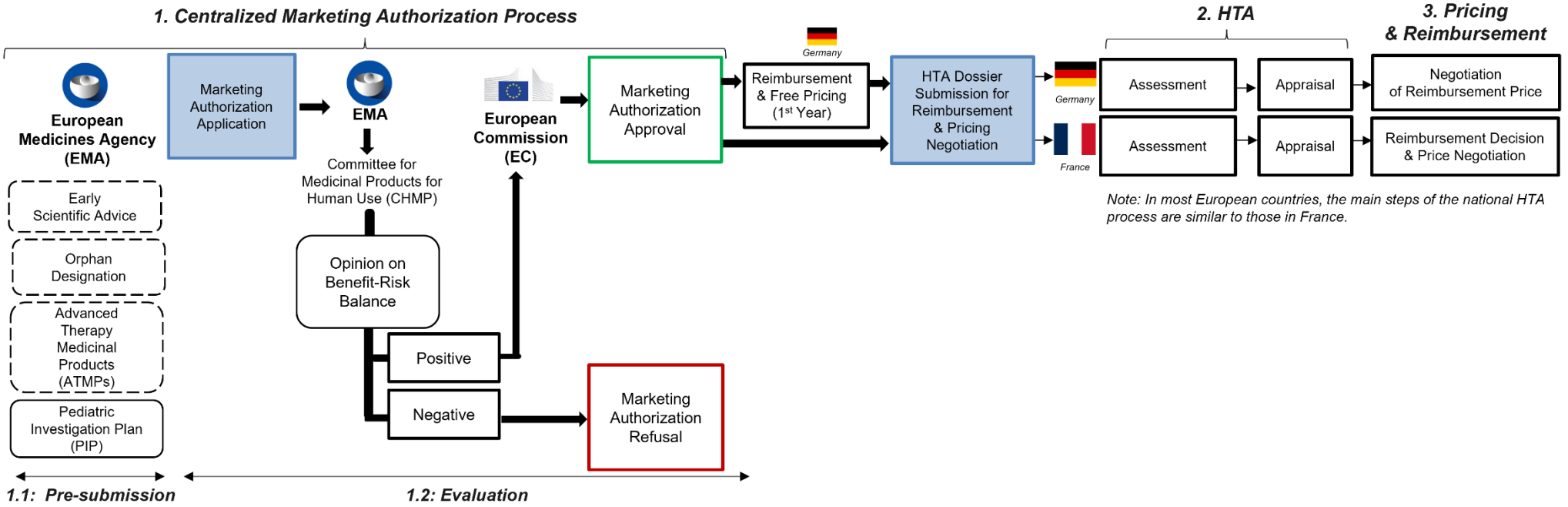
The EU HTA process currently applies to cancer medicines, including leukemia medicines, and advanced therapy medicinal products (ATMPs). It will expand to rare disease medicines (orphan drugs) in 2028, and to all centrally authorized medicines by 2030. Selected medical devices will also be included starting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

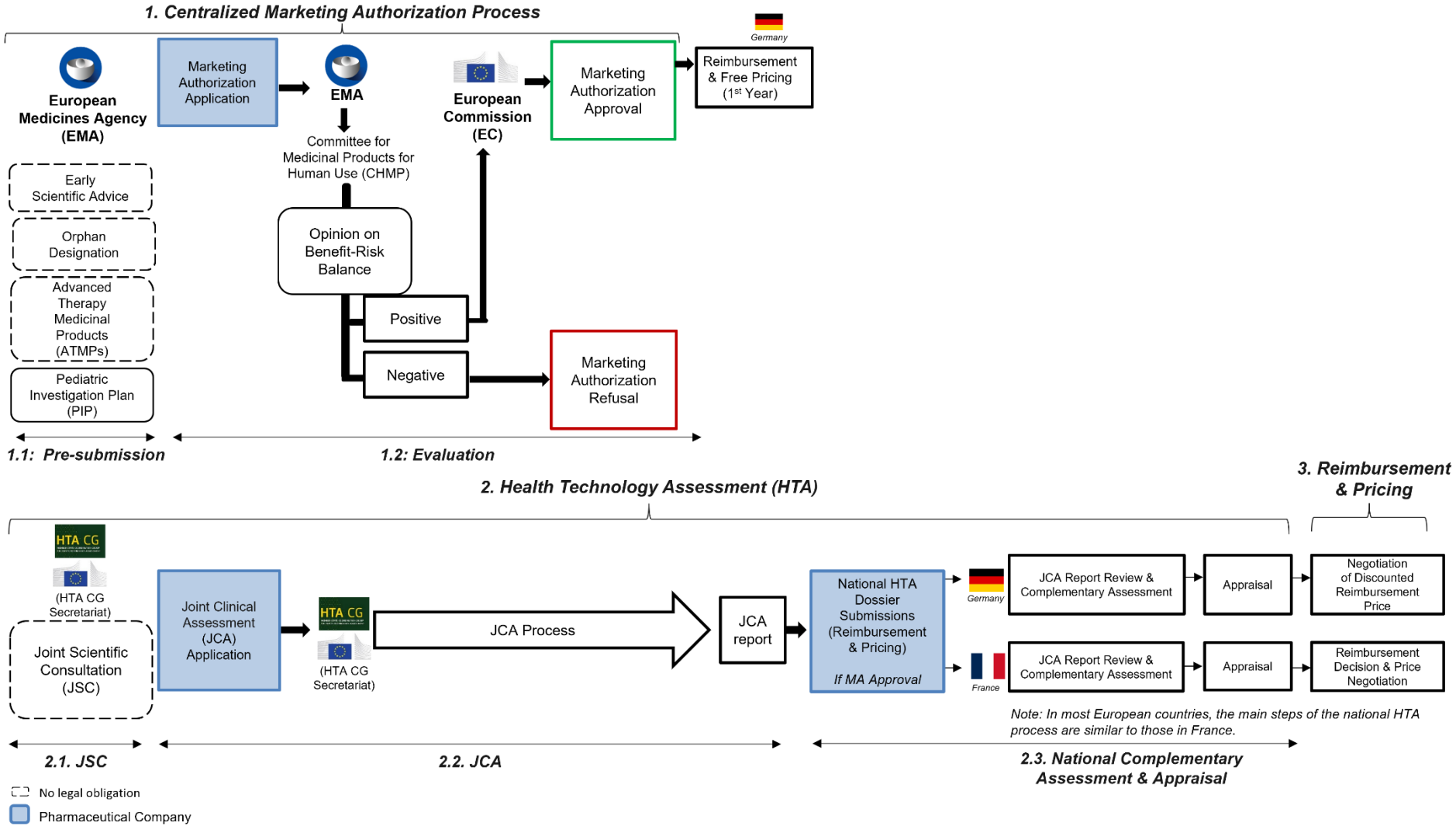
Figure 1. Marketing Authorisation and HTA Processes of Cancer Medicines Until 2024



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

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Figure 2. Marketing Authorisation 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).

2. How Medicines Get Authorized in Europe

2.1. Pre-submission

Before applying for marketing authorisation, companies prepare by:

- Requesting 'scientific advice' from the EMA—if they wish—on how to best design their clinical trials.
- Applying for special status—if applicable—such as orphan designation for rare diseases and Advanced Therapy Medicinal Product (ATMP) classification for gene or cell therapies
- Submitting a Pediatric Investigation Plan (PIP) if the medicine might be used in children (this is mandatory).

2.2. Regulatory Evaluation

When ready, the company submits its marketing authorisation application to the EMA. The EMA's Committee for Medicinal Products for Human Use (CHMP) reviews the evidence and decides whether to recommend approval. If approved, the European Commission grants a marketing authorisation. If not, the medicine cannot be used in Europe.

Before a medicine is approved in Europe, the EMA reviews the evidence to decide if its benefits outweigh the risks. Companies prepare by seeking early advice and meeting specific regulatory requirements.

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3. How Medicines Are Evaluated Through European and National HTAs

3.1. Two European-Level Steps in the HTA Process

Since 2025, two HTA steps have been carried out at the European level: Joint Scientific Consultation and Joint Clinical Assessment. Their purpose and timeline are in Table 1.

Table 1. European-Level Steps in the HTA Process

Step	Purpose	Timeline
Joint Scientific Consultation (JSC)	Early advice to help companies design the right clinical studies	Early input before trials begin
Joint Clinical Assessment (JCA)	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 steps are led by the HTA Coordination Group (HTACG), which includes representatives from national HTA bodies. The HTACG includes four subgroups (see Figure 3)*:*

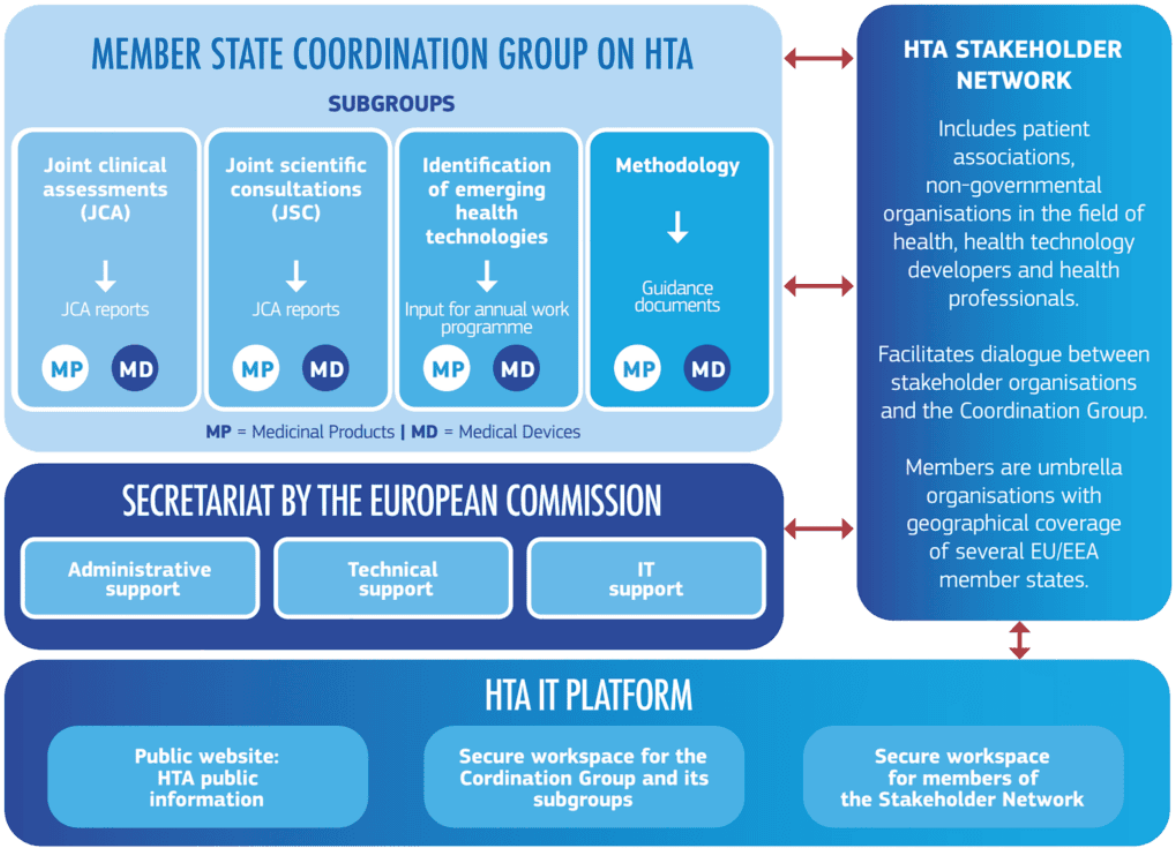
- One for JSCs
- One for JCAs
- One to identify new and emerging technologies
- One to develop shared methods and procedures

The HTACG is supported by a secretariat hosted by the European Commission known as the HTACG Secretariat. It coordinates tasks and ensures that timelines are met.
To include real-world perspectives, the HTACG works with the HTA Stakeholder Network, which brings together patients, healthcare professionals, researchers, and industry. This network helps identify experts, including patients, who can contribute to JSCs and JCAs.

In brief:

- The HTACG Subgroups conduct the work
- The HTACG Secretariat (European Commission) supports coordination
- The HTA Stakeholder Network brings in expert input, including patients and clinicians.

Figure 3. Member State Coordination Group on HTA (HTACG)



Since 2025, two health technology assessment (HTA) steps—Joint Scientific Consultation (JSC) and Joint Clinical Assessment (JCA)—have been carried out jointly by European countries to improve how medicines are evaluated for the purpose of reimbursement. This process is led by national bodies through the HTA Coordination Group (HTACG) with its subgroups, the support from the HTACG Secretariat (the secretariat by the European Commission), and input from patients and other experts through the HTA Stakeholder Network.

3.1.1. Joint Scientific Consultation (JSC)

A JSC helps companies improve their trial design before studies begin, ensuring they generate the kind of evidence needed for future HTA. While similar to the scientific advice provided by the EMA, the purpose differs: EMA advice focuses on designing safe and effective trials for marketing authorisation, whereas JSCs aim to support future HTA decisions (see Table 2).

Table 2. Similarities and Differences Between Early Advice and JSC

	Scientific Advice (Regulatory)	Joint Scientific Consultation (HTA)
Purpose	Help design safe and effective trials for marketing authorisation	Help improve trial design to support HTA decisions
Focus	Efficacy, safety and quality of pharmaceuticals	Added benefits compared to existing treatments, relevance for health systems.
Who leads it	EMA (via its Committee for Human Medicinal Products, CHMP)	HTA Coordination Group (HTACG)
When it happens	Early in clinical development	In parallel with EMA scientific advice

Patients, clinicians, and other experts such as statisticians and health economists contribute to ensure planned studies address the most relevant questions.

Clinicians use the PICO framework—Patient, Intervention, Comparator, and Outcomes (see Table 3)—both when advising on study design during Joint Scientific Consultations (JSCs) and when reviewing medicines during Joint Clinical Assessments (JCAs). Patients, on the other hand, use a structured template to provide input during JSCs, and contribute using the PICO framework only during JCAs.

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?

A JSC gives companies early advice on how to plan their clinical trials. It helps make sure the studies collect the right information for future HTA evaluations.

3.1.2. Joint Clinical Assessment (JCA)

A JCA is a joint scientific report that compares a new medicine to current treatments:

- It's written by two designated HTA bodies (the assessor and co-assessor) and serves as a shared scientific base for all countries.
- The process begins when a company applies to the EMA for marketing authorisation.
- At that point, the company submits the intended use of the medicine (its claimed indication) to the HTACG Secretariat—but not yet the clinical data.

Next, the assessor and co-assessor draft a proposed assessment scope using the PICO framework. This draft is circulated to all European countries through a "PICO survey" to gather

national needs and priorities. To ensure that patient perspectives are considered, both European-level and national patient experts may be invited to contribute.

Once the final PICOs are agreed, the company submits a dossier of evidence that addresses each PICO element. This dossier forms the basis for the scientific assessment by the assessor and co-assessor HTA bodies. Table 4 summarizes what a JCA —and what it is not.

Table 4. What JCA Is and Isn't

What a JCA is	What a JCA is NOT
A scientific report written jointly by national HTA bodies	A report that includes conclusions and recommendations
A scientific review of how strong and reliable the data is	A score or ranking of the medicine
A report focusing on answering each country's PICO elements	A country-specific report.
An assessment of how a new medicine compares to current treatments	An appraisal, which includes pricing, budgets, and national context.

A JCA compares a medicine to existing treatments but does not decide on reimbursement—that is the responsibility of each country through its national appraisal process.

For more information on how patient involvement in HTA takes place at European level, see our two companion factsheets 'Involvement of French/German Patients in Health Technology Assessments at European and National Levels'.

3.2. National Assessments and Appraisals

Once the JCA is complete, each country's HTA body uses the report as a common scientific base and adds its own assessment and appraisal according to national priorities and practices.

In some countries, such as Italy and Spain for example, HTA processes also take place at regional or local level—and in some cases even at hospital level—to determine how and where the medicine will be used.

A positive national HTA outcome therefore does not automatically guarantee that new medicine will be included in regional or hospital formularies. Additional approval steps are required. These steps are usually carried out by national, regional, or local health authorities—often distinct from HTA bodies. They decide:

- Whether the medicine will be covered by the public healthcare system
- Which patients will have access
- How it will be used (e.g. in hospitals only)
- At what price

These decisions are based on:

- National or regional or local health priorities
- The medicine's impact on national, regional or local healthcare budgets
- Whether the treatment is cost-effective

Since 2025, European countries have started working together on the comparative scientific evaluation of new medicines (assessment). But each country still makes its own decisions about funding and patient access (appraisal).

Want to Learn More?

Read our companion factsheets:

- Health Technology Assessment and Reimbursement: What is the Difference?
- Involvement of French Patients in Health Technology Assessment
- Involvement of German Patients in Health Technology Assessment

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