EU HTA and other legislative initiatives: Impact on small & mid-sized companies

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EUCOPE members' economic footprint

Impact of geopolitical uncertainty on the pharmaceutical industry

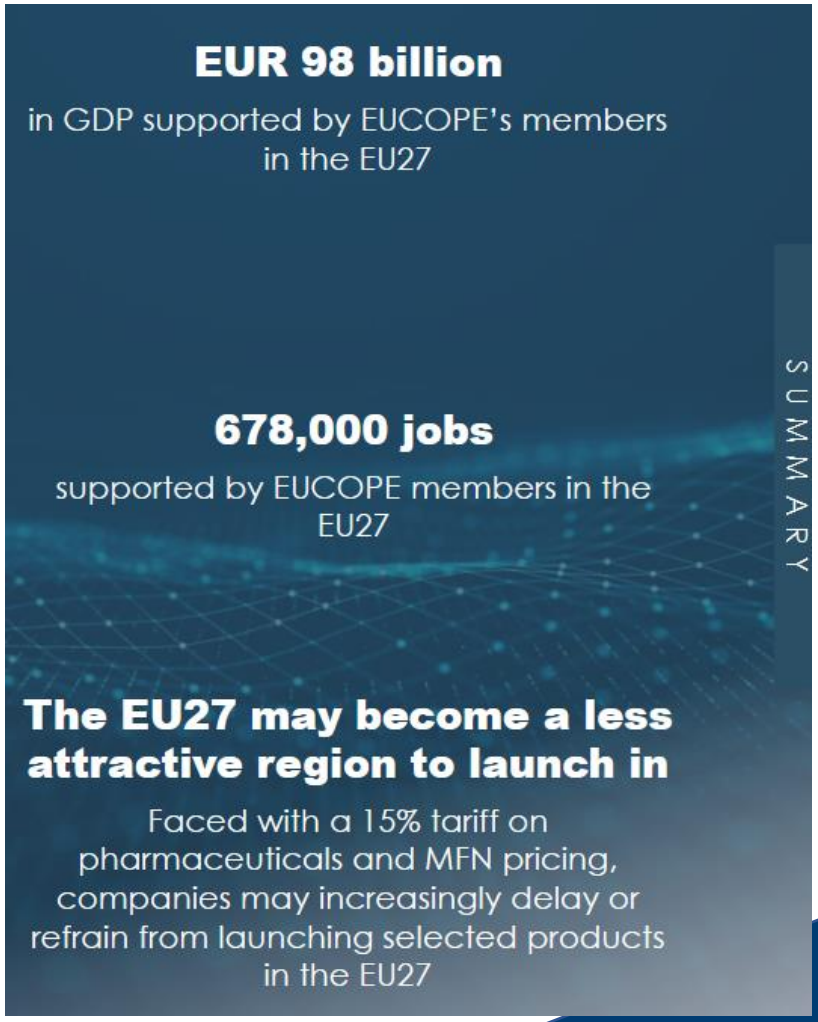
- Copenhagen Economics study (2026) on **EUCOPE members' economic contribution and trade implications** following U.S. policy measures

- From **early-stage innovators** with none or a few products on EU market, to **established firms** with broad product portfolios

    - 38% of member companies do not yet have a centrally approved product on the market in the EU

    - **Significant contribution to the EU economy**, supporting historic €195 bn. trade surplus in pharmaceutical products (2024)

- Reactions to MFN pricing may be further amplified by **additional P&R/market access pressures**



JCA footprint of small & mid-sized companies

- 13 out of 25 forecast JCAs were started in 2025. 1 JCA has started so far in 2026
  - 7/13 products have orphan designation
  - 3/13 products are ATMPs
- 8/13 products are being developed or launched by small or mid-sized companies
  - Many developed by small biotech companies with less exposure to European market

International non-proprietary name (INN) / Common Name	Indication - Summary	Substance type (classification)
Autologous melanoma-derived tumor infiltrating lymphocytes, ex vivo-expanded	Treatment of melanoma	ATMP
Tovorafenib	Treatment of paediatric low-grade glioma (LGG)	Chemicals
Discontinued JCA / MAA withdrawn		
Sasanlimab	Treatment of bladder cancer	Biologicals
Onasemnogene abeparvovec	Treatment of 5q spinal muscular atrophy (SMA)	ATMP
Lurbinectedin	Maintenance treatment of adult patients with extensive-stage small cell lung cancer (ES-SCLC)	Chemicals
Camizestrant	Treatment of adults with locally advanced or metastatic breast cancer	Chemicals
Tarlatamab	Treatment of extensive-stage small cell lung cancer	Biologicals
Catequentinib	Treatment of synovial sarcoma or leiomyosarcoma	Chemicals
Senaparib	Maintenance treatment of advanced epithelial high-grade ovarian, fallopian tube or primary peritoneal cancer	Chemicals
Relacorilant	Treatment of adult patients with platinum-resistant epithelial ovarian, fallopian tube, or primary peritoneal cancer	Chemicals
Ensartinib	The treatment of adult patients with anaplastic lymphoma kinase (ALK)-positive advanced non-small cell lung cancer (NSCLC).	Chemicals
Zopapogene imadenovec	Treatment of respiratory papillomatosis in adults	ATMP
Sintilimab	Treatment of non-squamous non-small cell lung cancer in adults	Biologicals
Sonrotoclax	Treatment of adult patients with relapsed or refractory mantle cell lymphoma (MCL)	Chemicals

The proposal for an EU ‘Biotech Act’

Clinical Trials Regulation (CTR) Changes:

Welcome shorter timelines, improved role of Reporting Member State, and measures to support innovation

Supplementary Protection Certificate (SPC):

Welcome albeit very limited SPC extension

Regulatory Provisions:

The simplification, support and clarification could particularly benefit less established companies

New Structures / Bodies:

should help developers navigate complex (regulatory and financing) frameworks and prepare systems for novel technologies

Strategic Projects and Financing Measures:

accelerators for biotech lifecycle with administrative & financial support; mostly beneficial for start-ups, SMEs and pre-commercial developers

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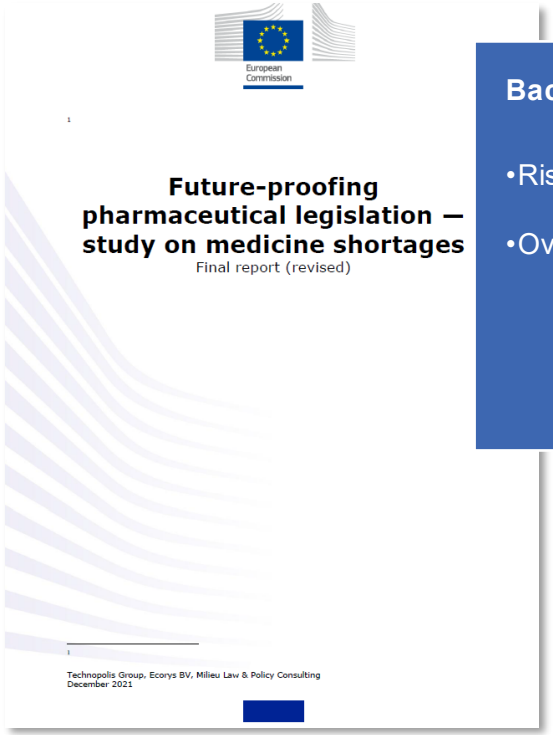
What additional proposals should be considered?

- Introduce a **definition of mid-caps** tailored to the biopharmaceutical sector
- Reframe **biopharmaceutical spending as an investment**
- Strengthen **opportunities for dialogue** in JSC & JCA

The proposal for a Critical Medicines Act (CMA)


European Commission

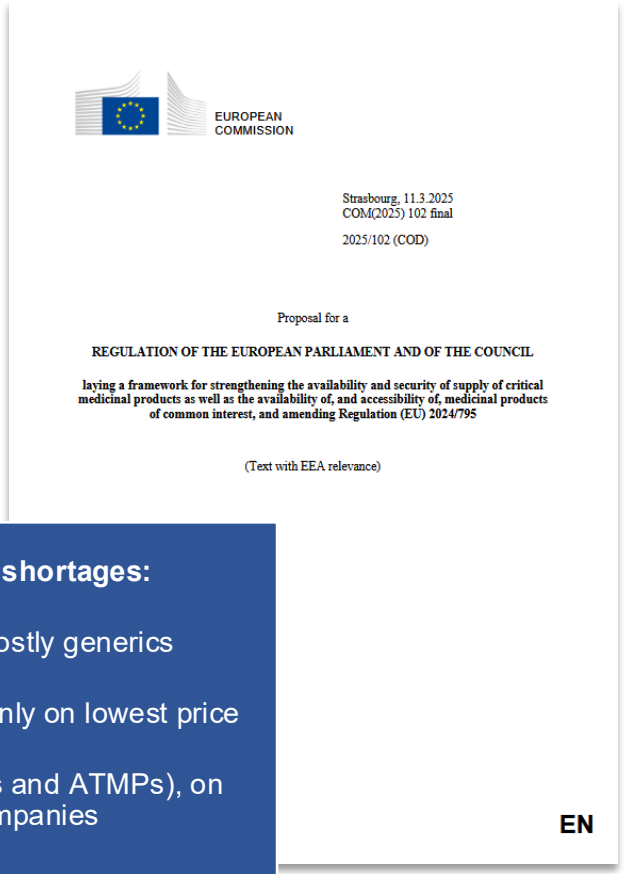
Future-proofing pharmaceutical legislation — study on medicine shortages

Final report (revised)

Technopolis Group, Ecorys BV, Milieu Law & Policy Consulting
December 2021

Background for the CMA:

- Rising shortages of critical medicines pose risks to patient care
- Over-reliance on non-EU supply chains increases vulnerability in crises


EUROPEAN COMMISSION

Strasbourg, 11.3.2025
COM(2025) 102 final

2025/102 (COD)

Proposal for a

REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

laying a framework for strengthening the availability and security of supply of critical medicinal products as well as the availability of, and accessibility of, medicinal products of common interest, and amending Regulation (EU) 2024/795

(Text with EEA relevance)

EN

The CMA should focus on addressing the drugs most frequently impacted by shortages:

- The Act should focus mainly on supply of APIs in the EU Critical Medicines List, mostly generics
- Public Procurement criteria for critical medicines should be improved, not relying only on lowest price
- Proposals on joint procurement of medicines of common interest (especially OMPs and ATMPs), on the basis of a JCA are premature and could increase uncertainty for innovative companies

First experiences with JCA – perspective of small and mid-sized companies

Some initial experiences with the JCA

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- Companies receive requests for underlying data or amendments to their dossier, **with short turnaround times**

- **This uncertainty for companies risk avoidable access delays**, especially for rare diseases where evidence generation is inherently constrained by small patient populations and heterogenous clinical practice across Member States

- Additional opportunities for interaction help identify potential issues earlier in the process and **ensure a high-quality JCA dossier submission**

    - **A pre-submission meeting with JCA Assessor & Co-Assessor** around 3-months prior to EMA submission would help ensure company readiness and provide valuable input for the preparation of the JCA

- **The EMA SME office is a best-practice example**, of offering advice, guidance and assistance including briefing meetings and early dialogue opportunities for smaller companies

Points to consider from an industry perspective

- **Remain flexible in the first years of JCA** and introduce tweaks based on practical experiences

- Continue to **provide additional clarity** for companies (e.g. in the form of Q&A documents and case examples):

  - Interpretation of the Conflict of Interest rules
  - Application of the methodological guidance
  - Scope of products subject to JCA
  - Information considered as commercially confidential
  - How confidential data shared for horizon scanning is protected and used

- **Make use of existing points of interaction in JCA** (e.g. HTD input on JCA scope, scope explanation meeting) to address potential uncertainties as early as possible

It remains difficult to access early advice

- The number of JCA forecast in 2026 is double that of 2025 (50 JCA vs 25 JCA), however the number of JSC slots has not followed suit
  - **The number of JSC slots has been increased from 5-7 (2025) to 8-12 (2026)** and the number of request periods has increased from two (2025) to four (2026)
  - **EUCOPE members report not being accepted for a JSC**, and relying on national advice meetings, that cannot give consolidated advice for JCA
- A recent EUCOPE workshop with members stressed the importance of early advice and highlighted the following challenges companies face when applying for a JSC:
  - The frequency of request periods and number of JSC slots is limited
  - There are limited resources available to pursue early advice
  - The perceived ability to receive meaningful input in JSC
- The JCA scope will be expanded to include all OMPs from 13 January 2028. **The number and frequency of JSC slots must be urgently increased** to meet the high demand for early advice