Reunião Anual do
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ASSUNTOS REGULAMENTARES

Nova legislação farmacêutica e HTA: exercício de equilíbrio inovação versus competitividade

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CEAR 25 anos

Tópicos

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- Acesso e Sustentabilidade
- Microeconomia e macroeconomia
- Avaliação de tecnologias de saúde
- Inovação e competitividade
- Semaglutido, que lições retirar!

Nova legislação farmacêutica

- Regulamento 2021/2282- relativo à avaliação das tecnologias da saúde (12Jan2025)
- Proposta de Diretiva - Código da União relativo aos medicamentos para uso humano
- Proposta de Regulamento - Procedimentos da União para a autorização e a supervisão de medicamentos para uso humano e que estabelece regras que regem a Agência Europeia de Medicamentos

Objetivos

- Garantir que todos os doentes em toda a UE tenham **acesso** atempado e equitativo a medicamentos seguros, eficazes e a **preços comportáveis**
- Reforçar a segurança do abastecimento e garantir que os doentes, independentemente do local onde residam na UE, tenham sempre acesso aos medicamentos
- Proporcionar um ambiente atrativo e favorável à **inovação** e à **competitividade** para a investigação, o desenvolvimento e a produção de medicamentos na Europa
- Tornar os medicamentos mais sustentáveis do ponto de vista **ambiental**

Acesso aos medicamentos em países europeus

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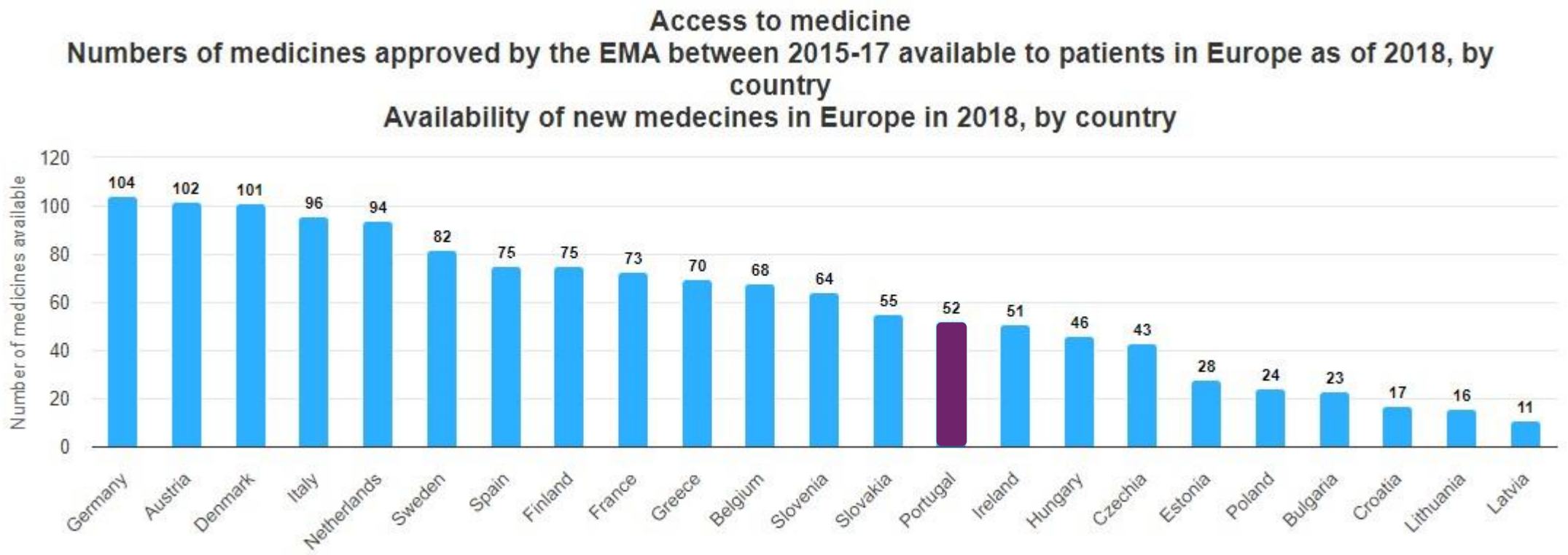

Fonte: https://commission.europa.eu/strategy-and-policy/priorities-2019-2024/promoting-our-european-way-life/european-health-union/reform-eu-pharmaceutical-legislation_en

Tempo até disponibilidade (2018-2021)

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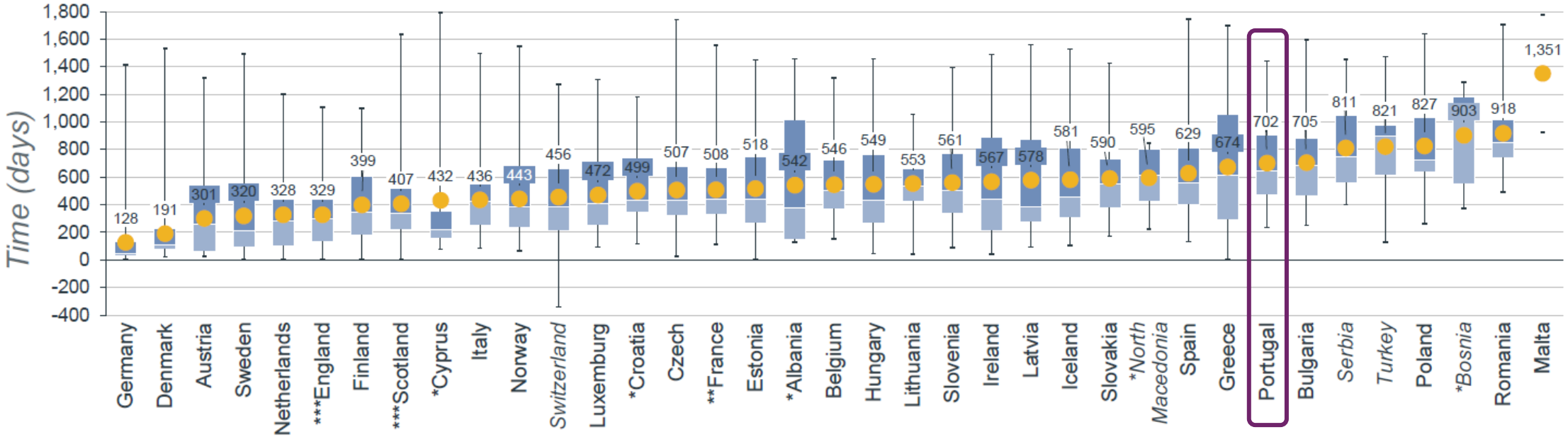
~45%

EU average rate of availability in 2022 vs 47% in the previous study

517 days

Average time for a new medicine to be available in European market is 6 days longer than the previous study

Access gap between the highest and lowest country is **83%** in the 4-year cohort, and **80%** for longer timelines



Fonte: https://www.efpia.eu/media/s4qf1eqo/efpia_patient_wait_indicator_final_report.pdf

Regulamento HTA

FRAMEWORK FOR JOINT HTA COOPERATION

- Joint clinical assessments (JCAs).
- Joint scientific consultations (JSCs).
- Identification of emerging health technologies.
- Common procedures and methodologies across the EU.

KEY PRINCIPLES OF THE HTA REGULATION

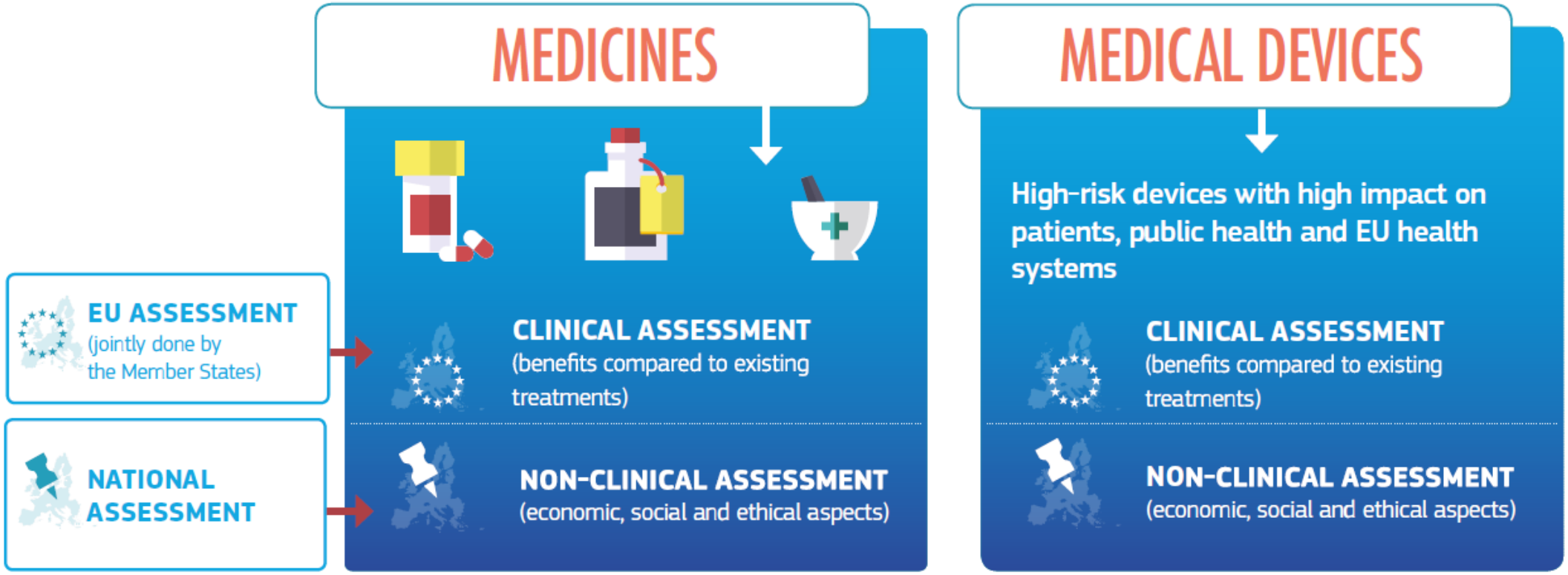
- Only on clinical domains of the assessment: No economic assessment or any conclusion on pricing and reimbursement.
- Driven by EU HTA bodies who remain responsible for drawing conclusions on added value for their health systems.
- High quality, timeliness and transparency.
- Use of joint work in national HTA processes.
- Input from independent experts.
- Stakeholder engagement and inclusiveness.
- Progressive implementation.

TIMELINE FOR MEDICINES

- 12 January 2025: New oncology medicines and advanced therapy medicinal products will be assessed at EU level.
- 13 January 2028: Orphan medicinal products to be added to the joint work.
- 13 January 2030: All new medicines will come under the scope of the regulation.

Fonte: https://health.ec.europa.eu/system/files/2023-09/hta_regulation-implementation_factsheet_en.pdf

Regulamento HTA



Fonte: https://health.ec.europa.eu/system/files/2023-09/hta_regulation-implementation_factsheet_en.pdf

HTA EU

- Os Estados-Membros deverão poder efetuar as análises complementares de âmbito clínico, a grupos de doentes, comparadores ou resultados em termos de saúde diferentes, necessários ao processo ATS nacional
- Os Estados-Membros continuam a ser a única entidade responsável pelos processos nacionais de ATS, pelas conclusões sobre o valor de uma tecnologia da saúde e pelas decisões resultantes das ATS
- Os Estados-Membros deverão continuar a ser responsáveis por tirar conclusões a nível nacional sobre o valor acrescentado clínico de uma tecnologia da saúde
- Redução dos encargos administrativos para os Estados-Membros e os criadores de tecnologias da saúde, facilitando o acesso ao mercado de produtos novos e inovadores e reduzindo os custos

Regulamento HTA: avaliação conjunta clínica/HTA

EU joint clinical/HTA assessment	Implications	Challenges	Opportunities
Information, data, analyses and other evidence	• Submitted only once at Union level • Directly comparative clinical studies and not excluding observational studies	• How to contribute and influence the comprehensive EU dossier • Head-to-head RCT vs indirect comparisons • Very challenging to present observational data on innovative medicines before marketing authorization	• Get involved sooner in the HTA evidence generation • Real world evidence studies
Methodology and Joint clinical assessment report	• Highest quality in a timely manner • Comprehensive evidence release	• Keeping up to date	• Be able to contribute at EU level
EU PICO & Relative effectiveness	• Broad PICO context as lower likelihood of subgroup analysis, as many times seen in Portugal	• To navigate into the more broad context and fine tuning it to the Portuguese reality	• Early market value assessment available to all stakeholders

Regulamento HTA: tomada de decisão

Decision making	Implications	Challenges	Opportunities
Complementary clinical analyses by Member states	• Exceptions and deviance from the EU framework	• Difficulties in justifying the need for PT uniqueness	• Be able to deviate from EU context in exceptional cases
Member States sovereignty in therapeutic value judgement (TVJ)	• Similar to the current situation in Portugal	• INFARMED full matching TVJ with EU evidence pack	• Push the likelihood of added therapeutic value in Portugal
Economic value	• Similar to the current situation in Portugal	• INFARMED full alignment with comparators in the EU PICO, if not reimbursed in Portugal	• Avoid off-label comparators sometimes used by INFARMED
Pricing and public financing	• At the discretion of Member States	• How to accelerate pricing and reimbursement in Portugal relative to other EU countries	• Pressure to benchmark across EU countries in the long run
Progressiveness	• Start with a small number of joint clinical assessments. Progressive expansion three years after 2025/2028/2030	• Transition period between 2025 and 2028 where EU HTA and PT HTA can happen at the same time for different products of the same pharmaceutical company	• All stakeholder will have time to adjust

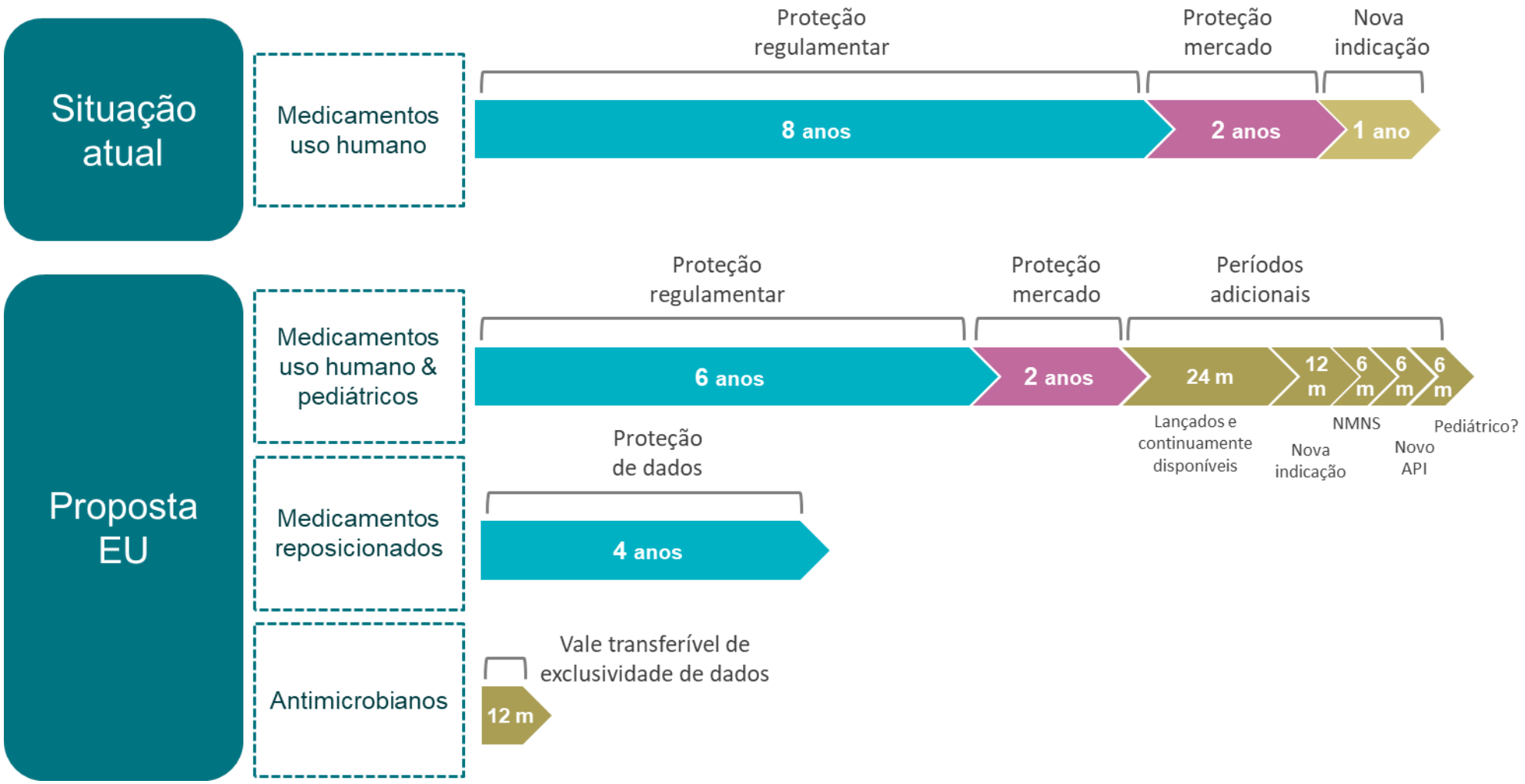
Regulamento HTA: transparência e acesso à inovação

Decision making	Implications	Challenges	Opportunities
Transparency	• **Health technology developer** can signal technical or factual inaccuracies and confidential and commercially sensitive information • **Patients representatives**. Opportunity to provide inputs during the preparation of the draft joint scientific consultation outcome document	• How to adequately reflect Health technology developer expectations and concerns • Incorporate and value patients inputs objectively • How to involve PT patients representative	• PT to understand in advance the results and conclusions of the EU assessment • Use EU inputs for local advocacy
Access	• Shorter time from MA to P&R if no assessment duplication • Earlier European focus on technologies with major impact on patients, public health or healthcare systems, and hence better access for patients	• Avoid clinical value assessment redundancies • Avoid different speeds for EU and PT driven HTA	• Foster time to reimbursement • Align with the best EU practices on access to innovation

HTA EU - equilíbrio inovação versus competitividade

- Inovação
  - Pode facilitar o acesso à inovação na perspetiva dos doentes
  - Por si só, impacto reduzido no estimulo à I&D e inovação, eventualmente no longo termo ambiente mais competitivo e favorável à inovação
- Competitividade
  - Em termos teóricos, a competitividade estimula a inovação, e a inovação promove maior bem-estar e crescimento económico
  - Atrasos e condicionantes do acesso aos medicamentos ocorrem predominantemente na fase de preço e comparticipação nos Estados Membros

Períodos de proteção regulamentar dos dados e do mercado



Inovação

- Inovação é o motor mais poderoso para a competitividade na indústria farmacêutica
- Beneficia os consumidores através do desenvolvimento de novos e melhorados bens, serviços e processos
- Ajuda a impulsionar o crescimento económico e o grau de aumento do nível de vida
- Aumenta a prosperidade e melhora a qualidade de vida das pessoas
- Tanto a concorrência como a política de patentes podem promover a inovação, mas cada uma requer um equilíbrio adequado para que tal aconteça

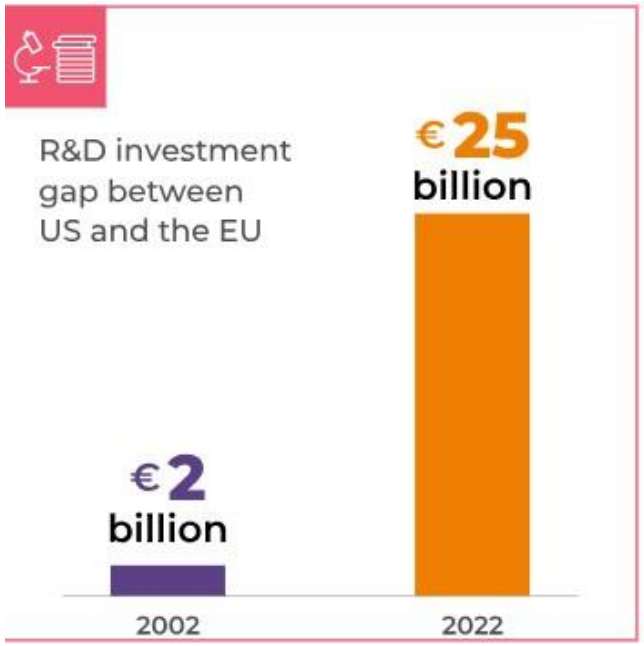
Efeitos práticos

- Redução expectável do período de proteção/exclusividade
- Dependência de mecanismos de exceção para obtenção de períodos mais longos de exclusividade
- Efeito imprevisível na entrada de 2ªs e 3ªs opções no mercado
- Aumento dos custos de I&D
- Politicas de preços mais agressivas

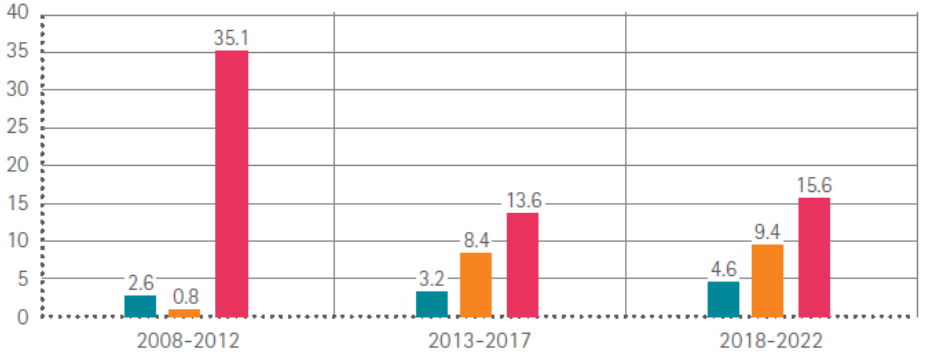
Competitividade

- Europa a perder competitividade a um ritmo acelerado
- Declínio de 25% no investimento europeu em I&D
- Quota de 19,3 % da atividade global de ensaios clínicos em 2020, uma diminuição de 6,3 %, em comparação com uma média de 25,6 % nos últimos dez anos

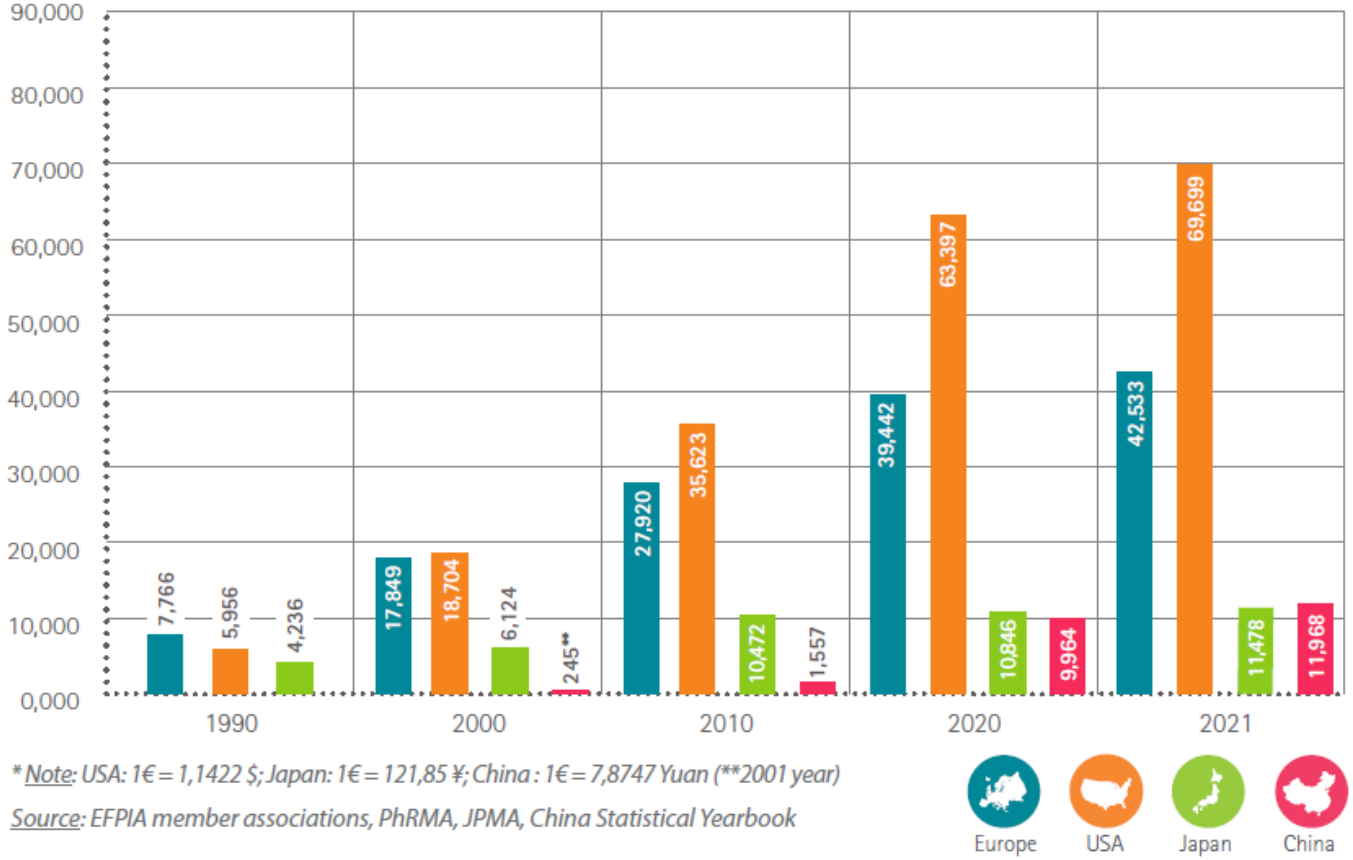
Europa: défice de investimento em I&D



PHARMACEUTICAL R&D EXPENDITURE ANNUAL GROWTH RATE (%)



PHARMACEUTICAL R&D EXPENDITURE IN EUROPE, USA, JAPAN AND CHINA
(€ MILLION, 2020 CONSTANT EXCHANGE RATE*), 1990–2021

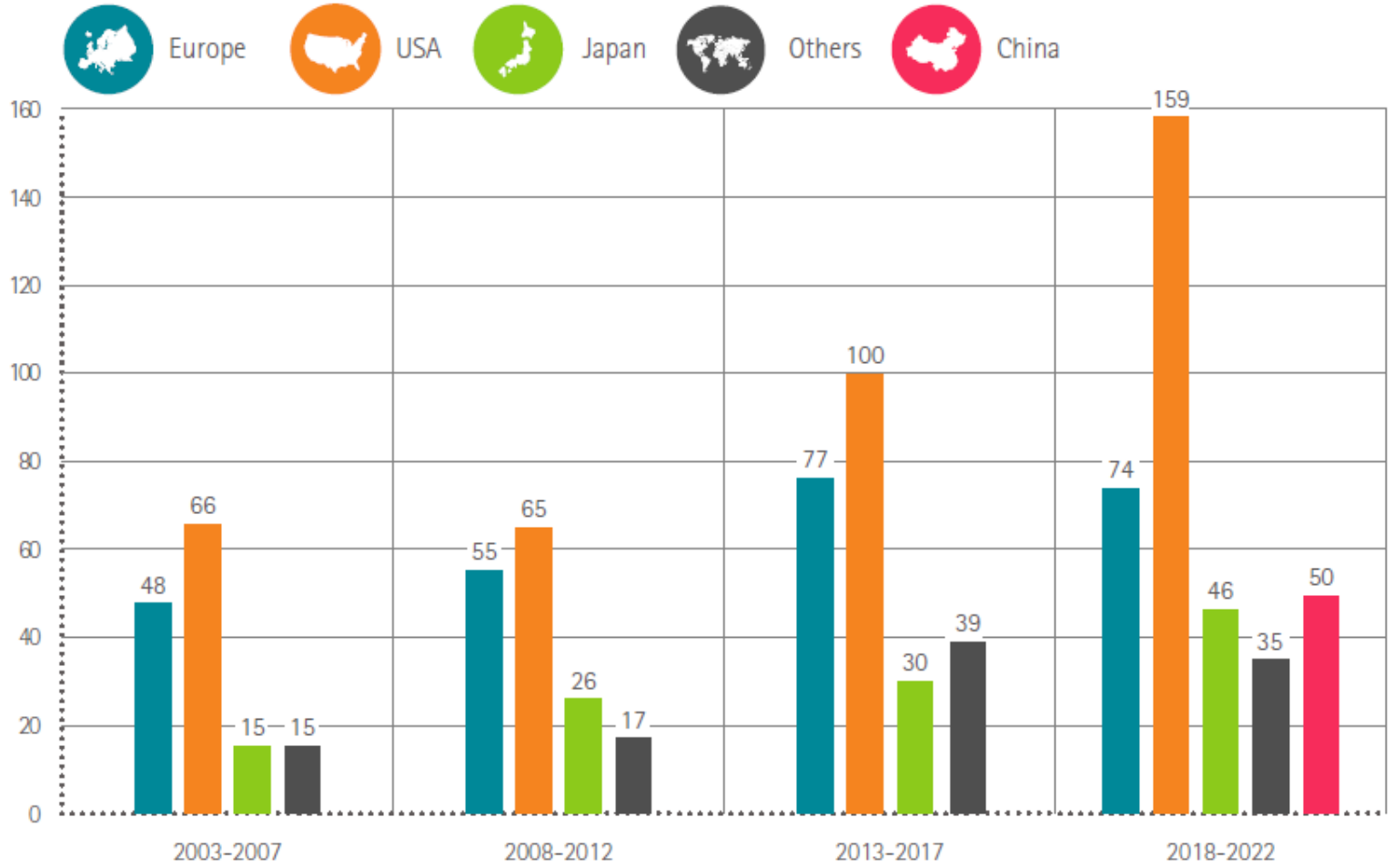


*Note: USA: 1€ = 1,1422 $; Japan: 1€ = 121,85 ¥; China : 1€ = 7,8747 Yuan (**2001 year)

Source: EFPIA member associations, PhRMA, JPMA, China Statistical Yearbook

Fonte: https://www.efpia.eu/media/rm4kzdlx/the-pharmaceutical-industry-in-figures-2023.pdf

Inovação: novas entidades quimicas ou biológicas



Fonte: https://www.efpia.eu/media/rm4kzdlx/the-pharmaceutical-industry-in-figures-2023.pdf

Atratividade e dimensão do mercado

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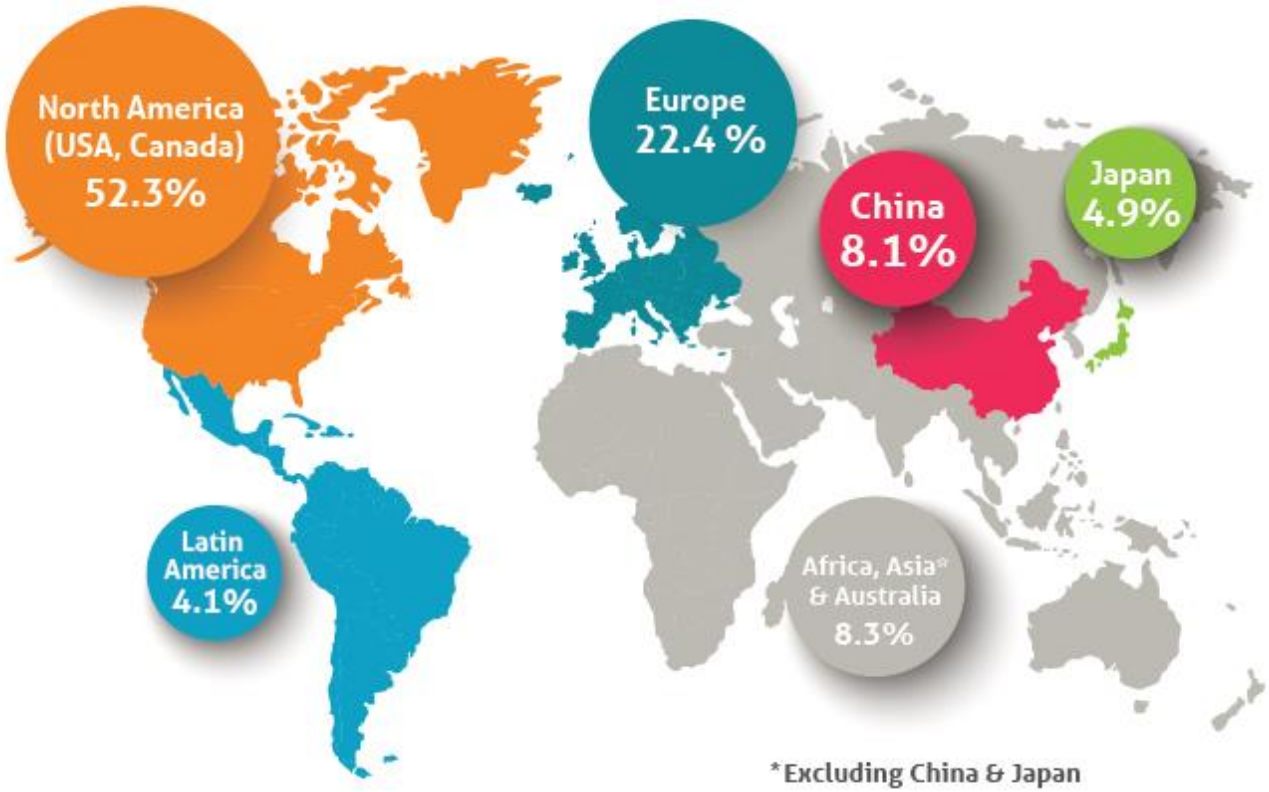
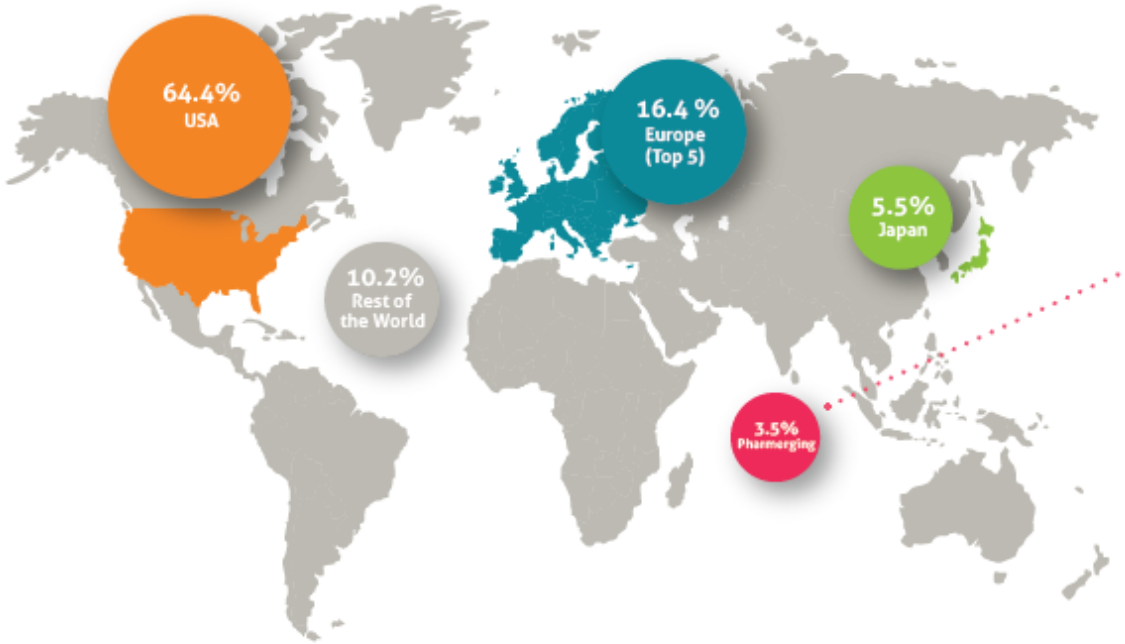
BREAKDOWN OF THE WORLD PHARMACEUTICAL MARKET – 2022 SALES

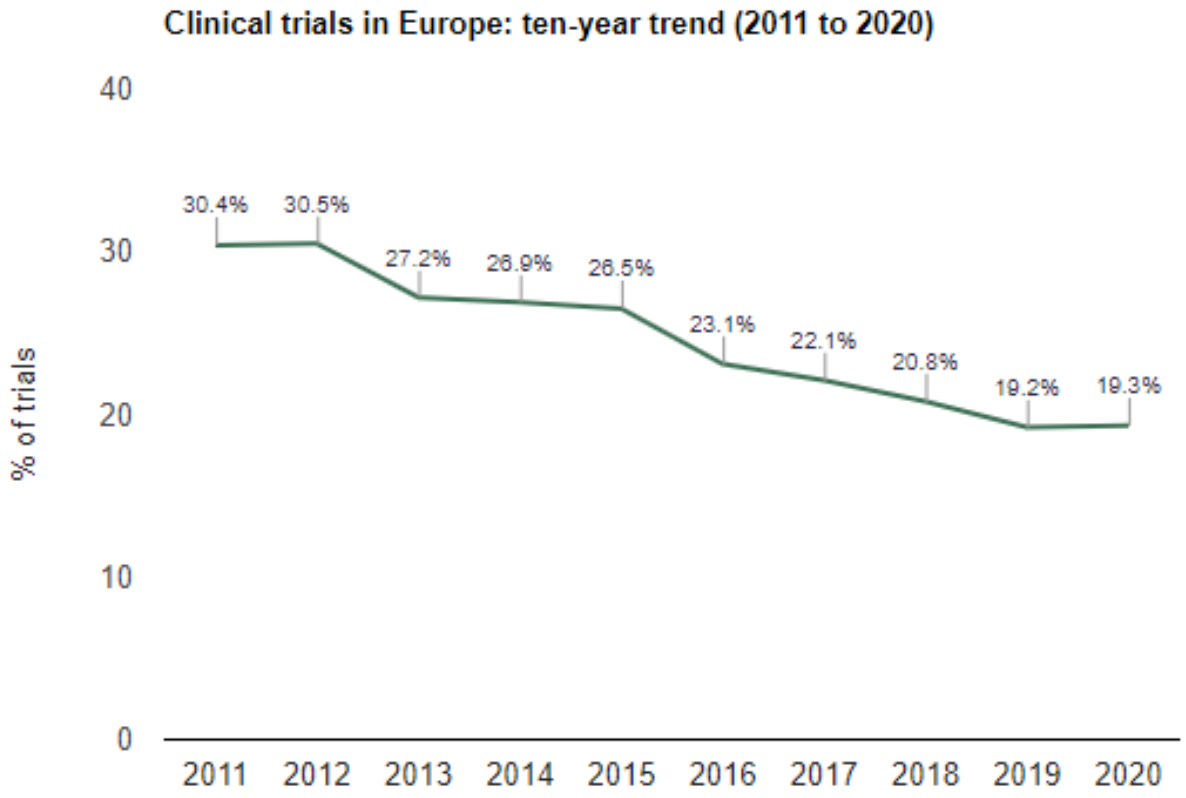


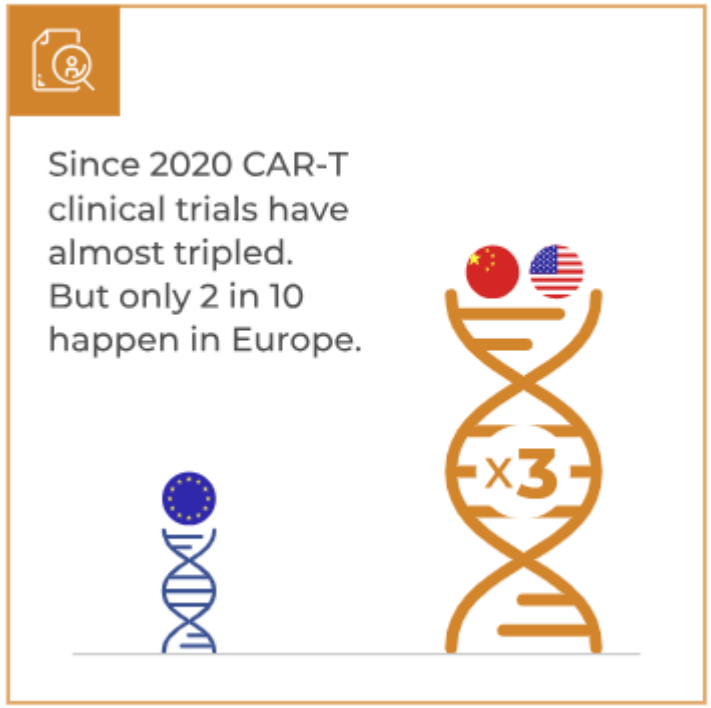
GEOGRAPHICAL BREAKDOWN (BY MAIN MARKETS) OF SALES OF NEW MEDICINES LAUNCHED DURING THE PERIOD 2017-2022



Fonte: https://www.efpia.eu/media/rm4kzdlx/the-pharmaceutical-industry-in-figures-2023.pdf

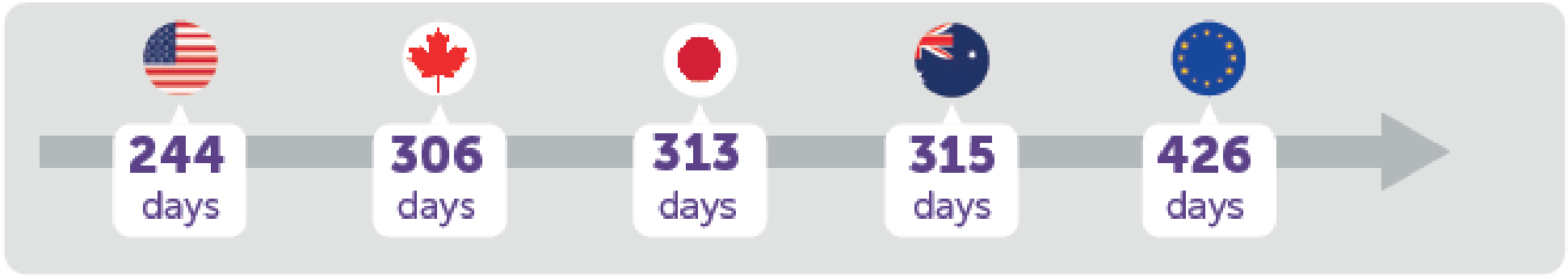
Europa: perda de competitividade na investigação clínica





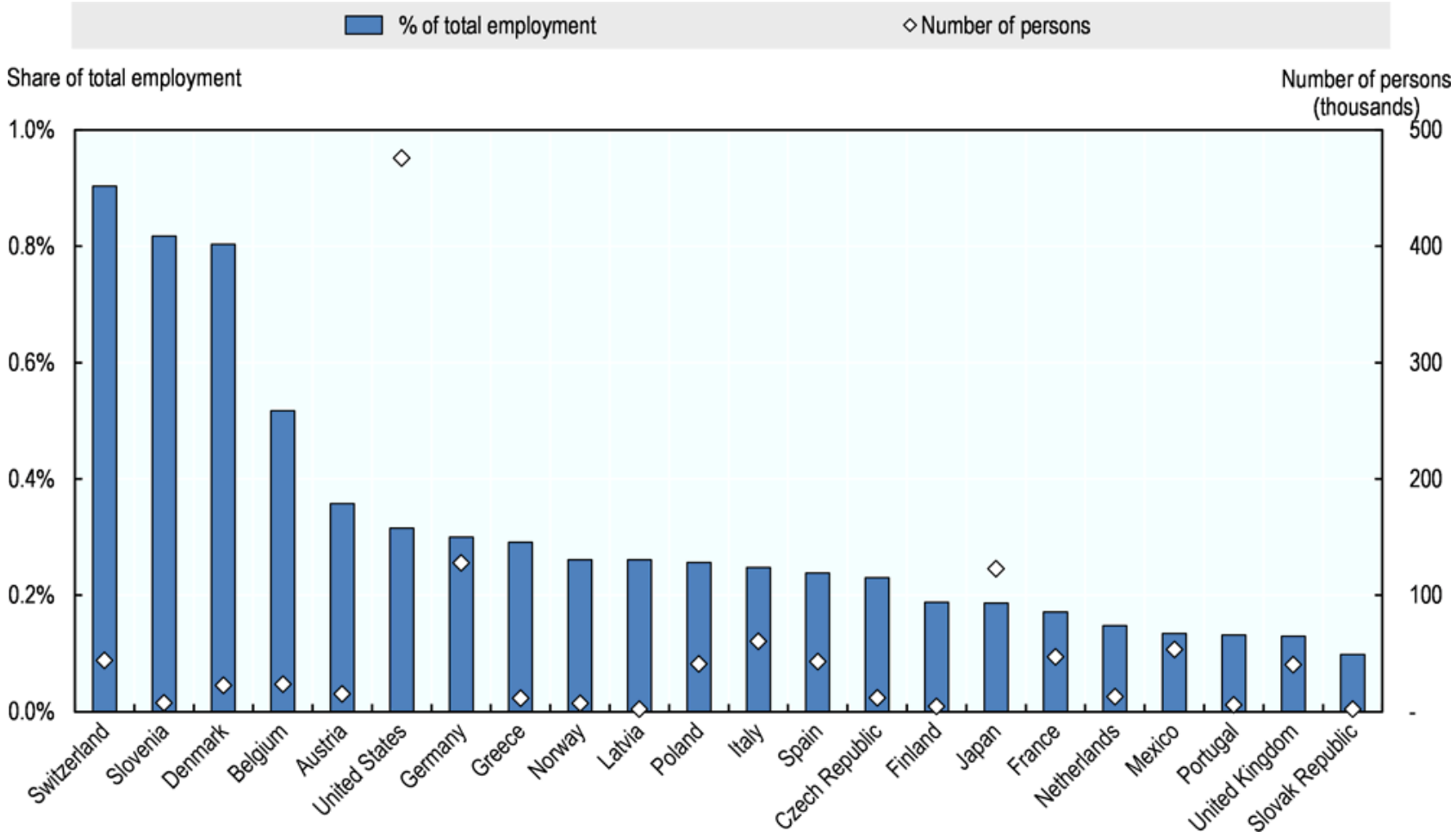
Fonte: https://www.clinicaltrialsarena.com/clinical-trials-analysis/europe-accounts-for-19-3-share-of-global-clinical-trial-activity-in-2020/?cf-view&cf-closed; https://www.efpia.eu/pharmaceutical-legislation/

Europa: maior lentidão na aprovação de novos medicamentos



Fonte: https://www.efpia.eu/media/gy5j1nkt/efpia-recommendations-on-the-revision-of-the-pharmaceutical-package.pdf

OCDE: emprego na industria farmacêutica (2014 ou 2015)



Fonte: https://www.oecd-ilibrary.org/social-issues-migration-health/pharmaceutical-innovation-and-access-to-medicines_9789264307391-en

Sector farmacêutico: atividade impar na Europa

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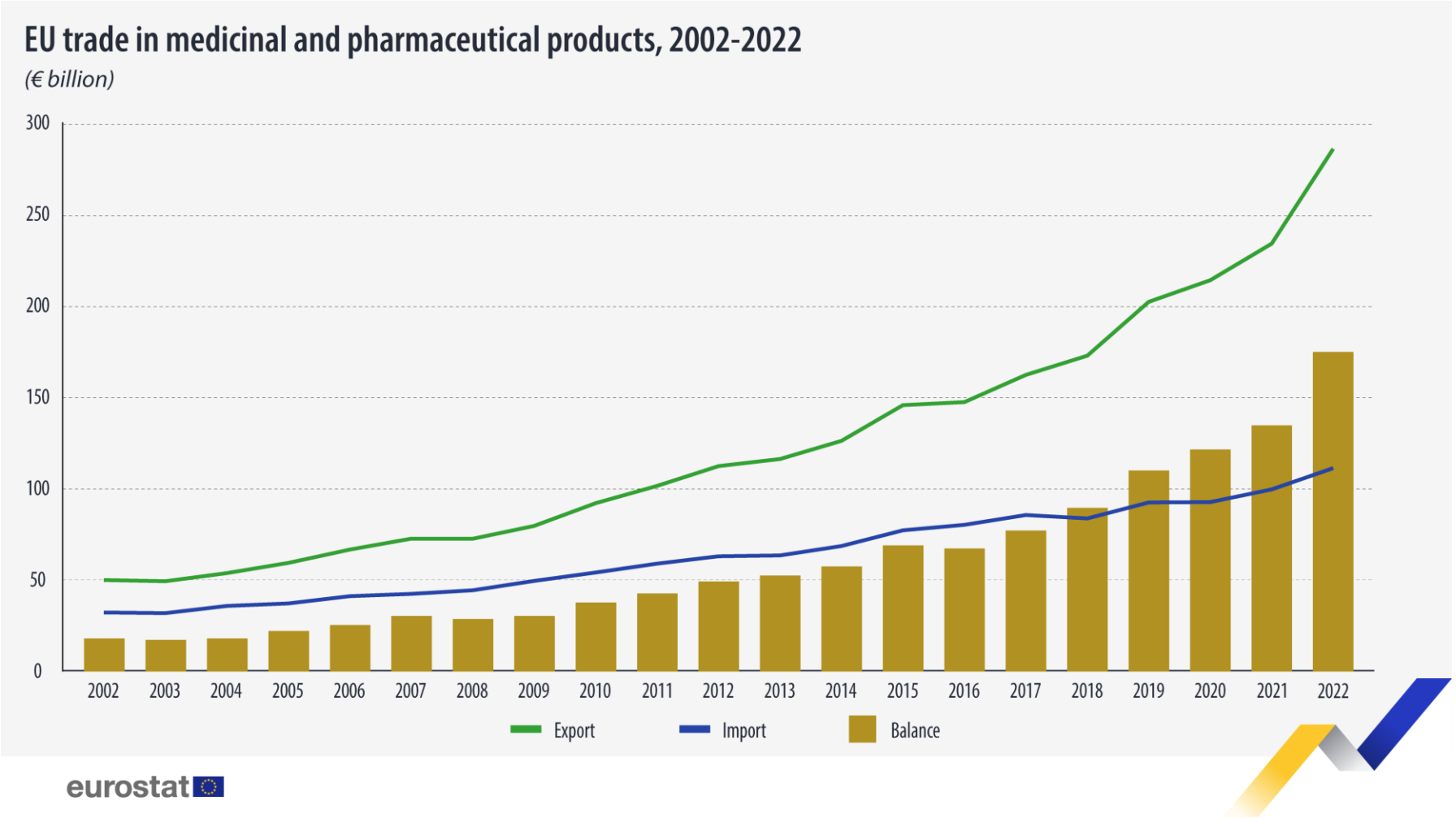


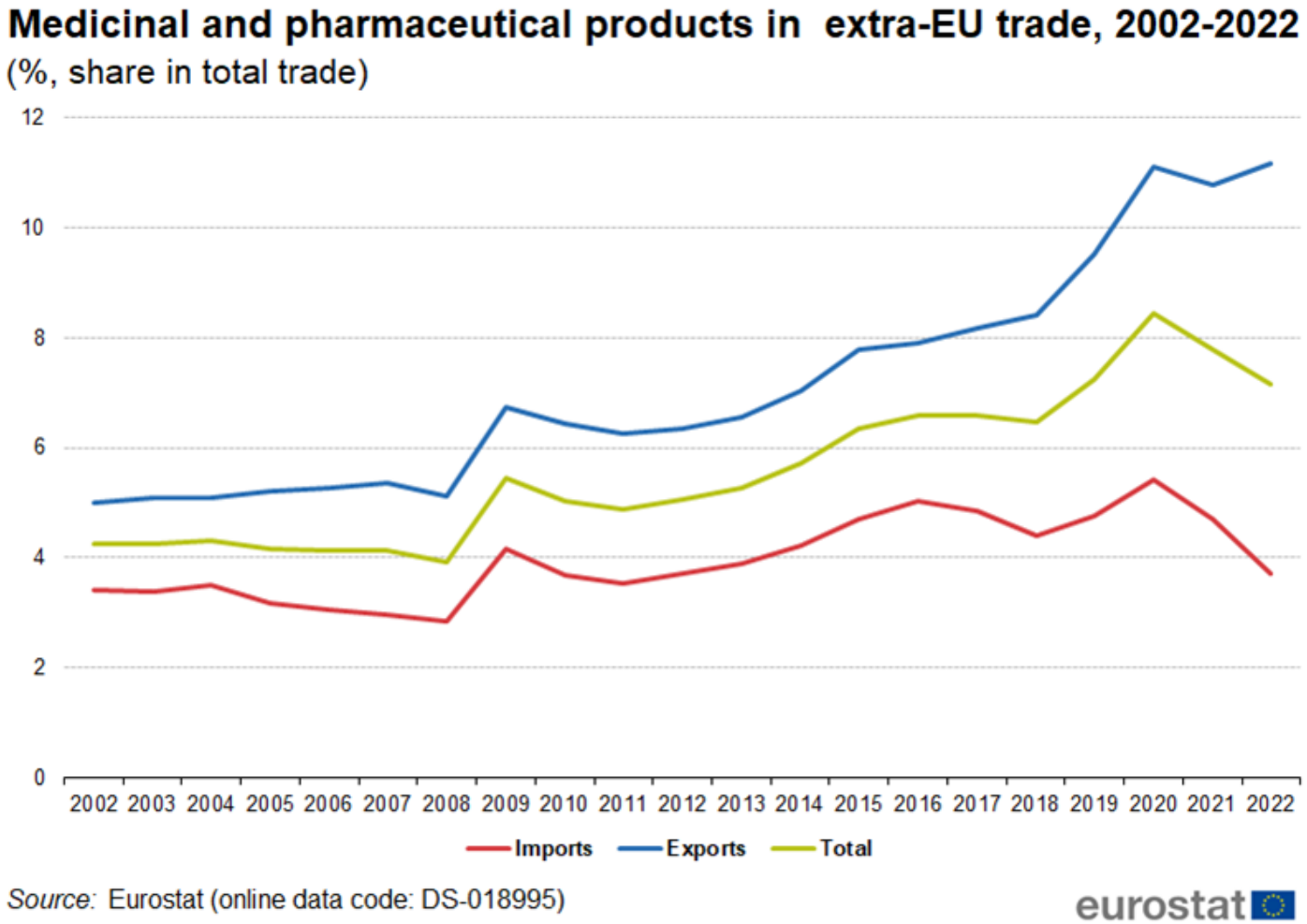
In 2022 compared with 2021, imports (+12 %) and especially exports (+22 %) grew strongly, mainly because of increasing prices.

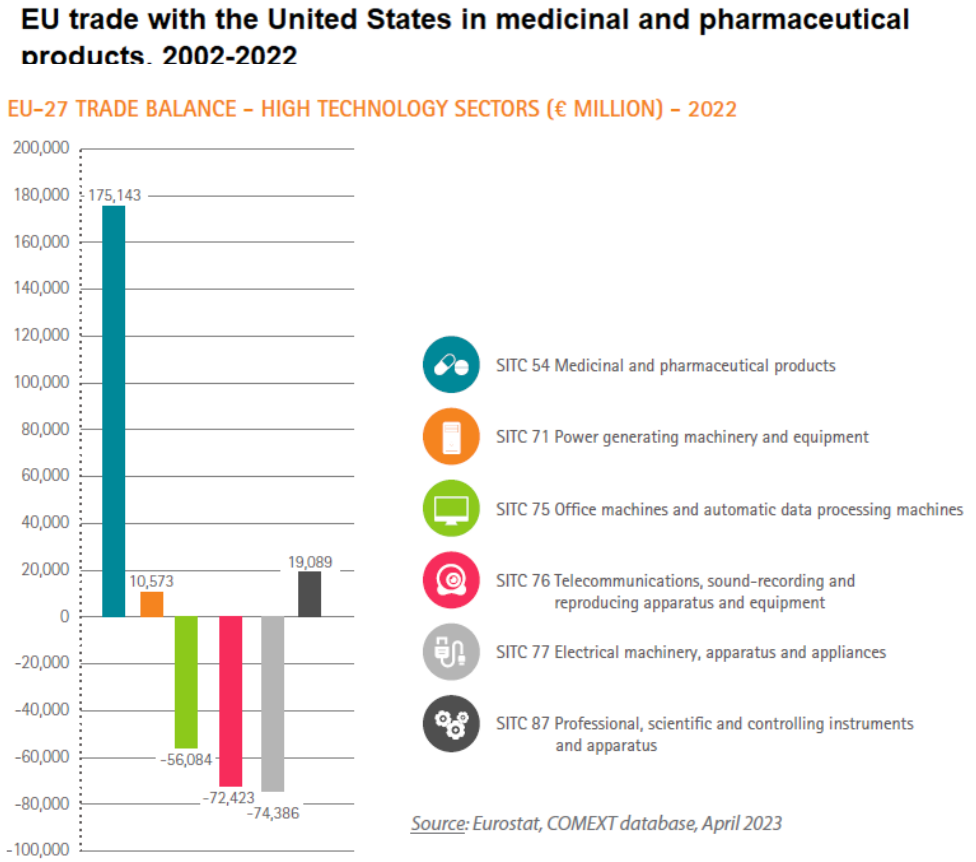
The United States and Switzerland were the EU's main trading partners for medicinal and pharmaceutical products in 2022.

Among the EU Member States, Germany was the largest exporter of medicinal and pharmaceutical products in 2022.

Fonte: https://ec.europa.eu/eurostat/en/web/products-eurostat-news/w/ddn-20230424-1

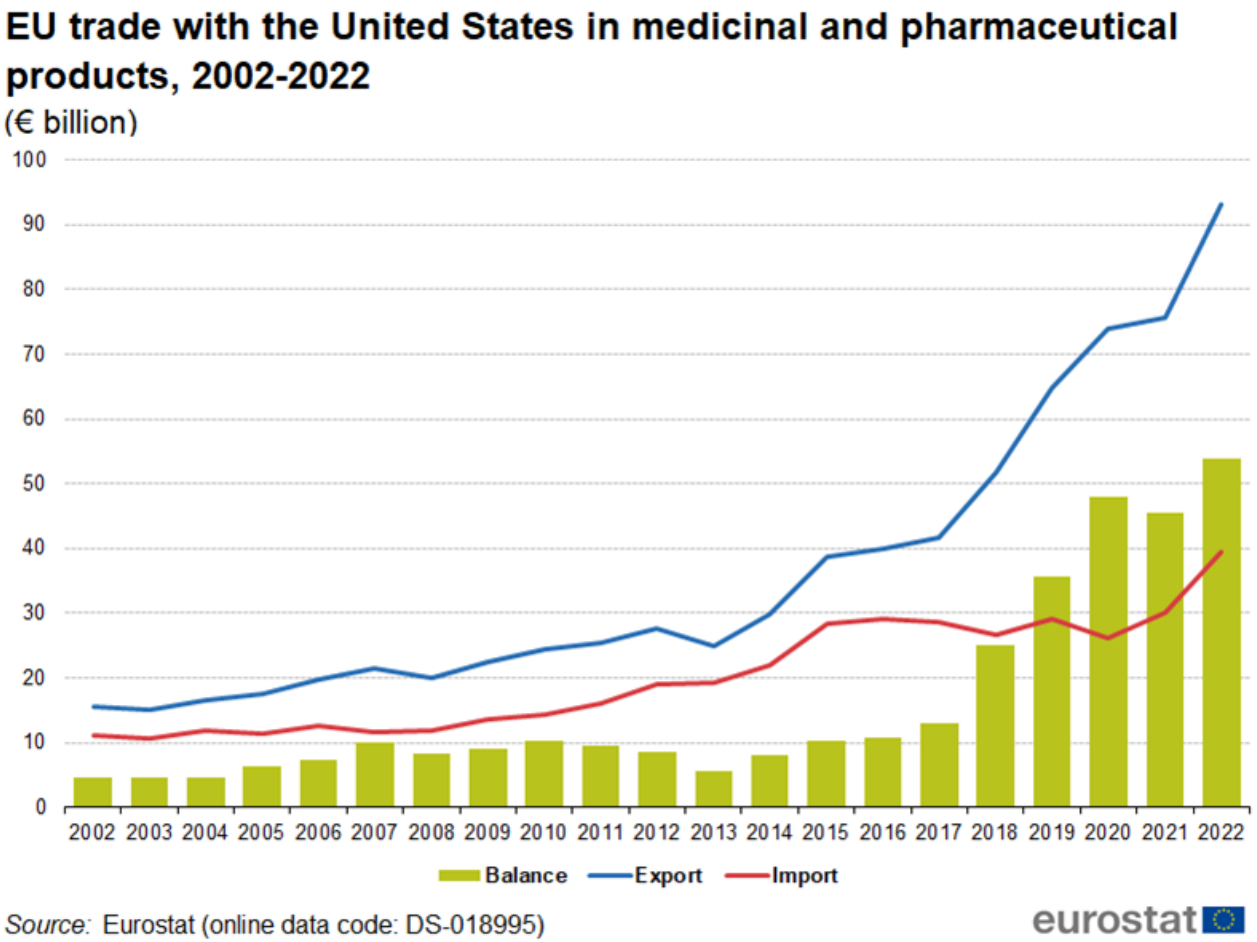
Sector farmacêutico: atividade impar na Europa

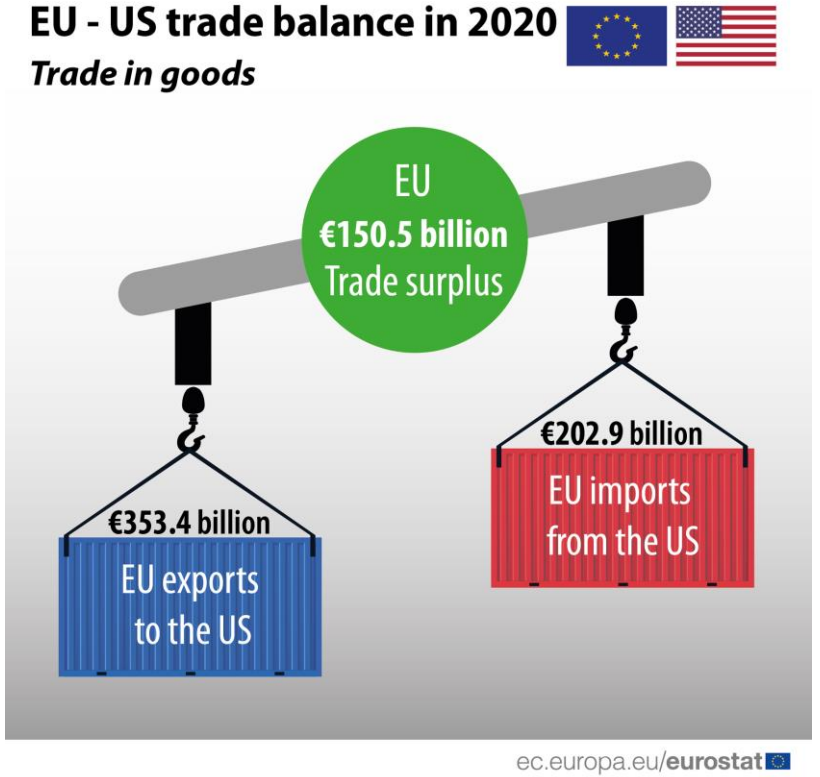




Fonte: https://ec.europa.eu/eurostat/en/web/products-eurostat-news/w/ddn-20230424-1

Sector farmacêutico: atividade impar na Europa





Pharma = 35,8%

Fonte: https://ec.europa.eu/eurostat/en/web/products-eurostat-news/w/ddn-20230424-1

Industria farmacêutica: balanço comercial 2021 (USD)

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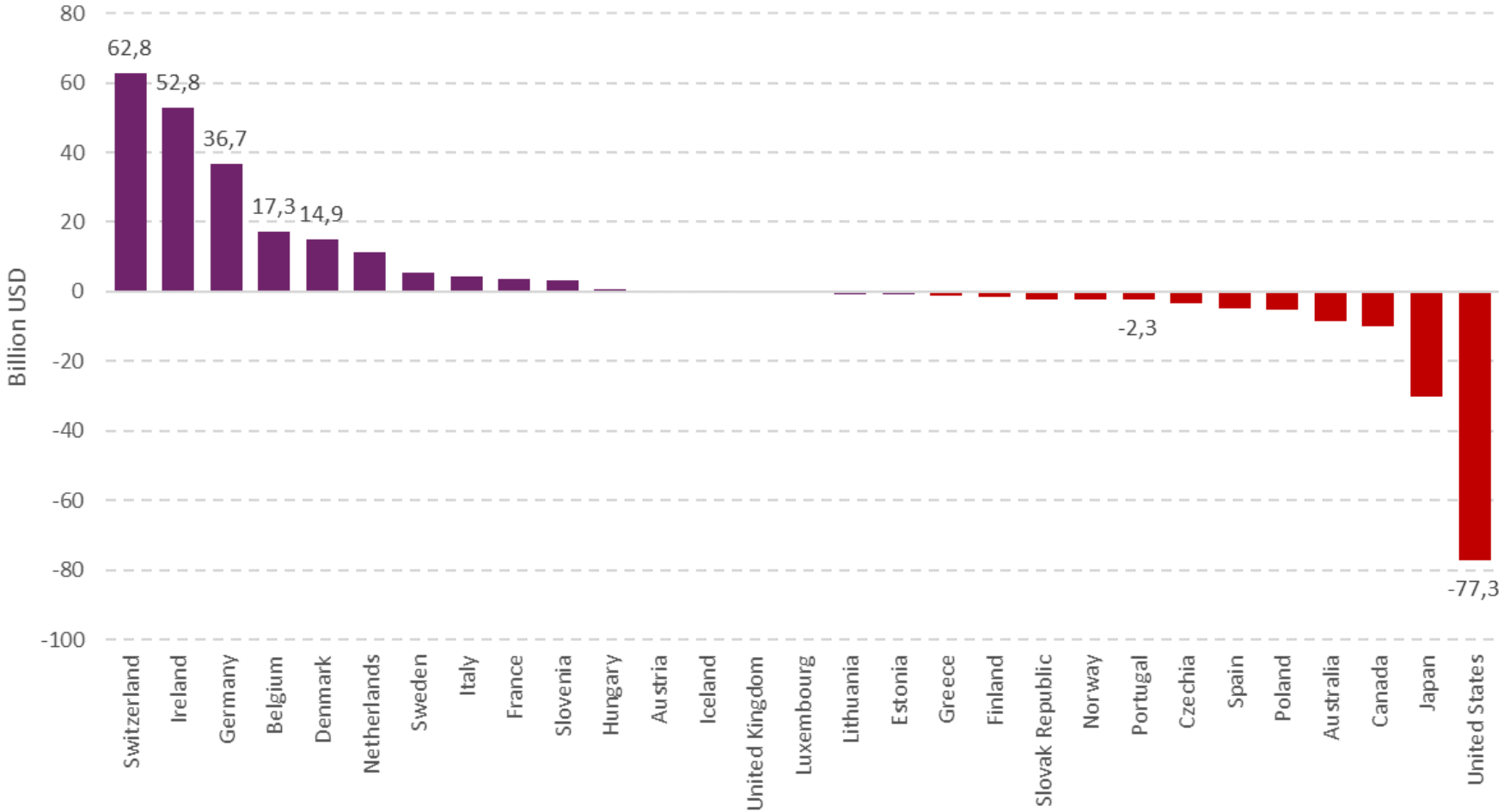

Fonte: https://www.oecd-ilibrary.org/science-and-technology/main-science-and-technology-indicators/volume-2022/issue-2_1cdcb031-en

Competitividade: perspetiva da politica de saúde

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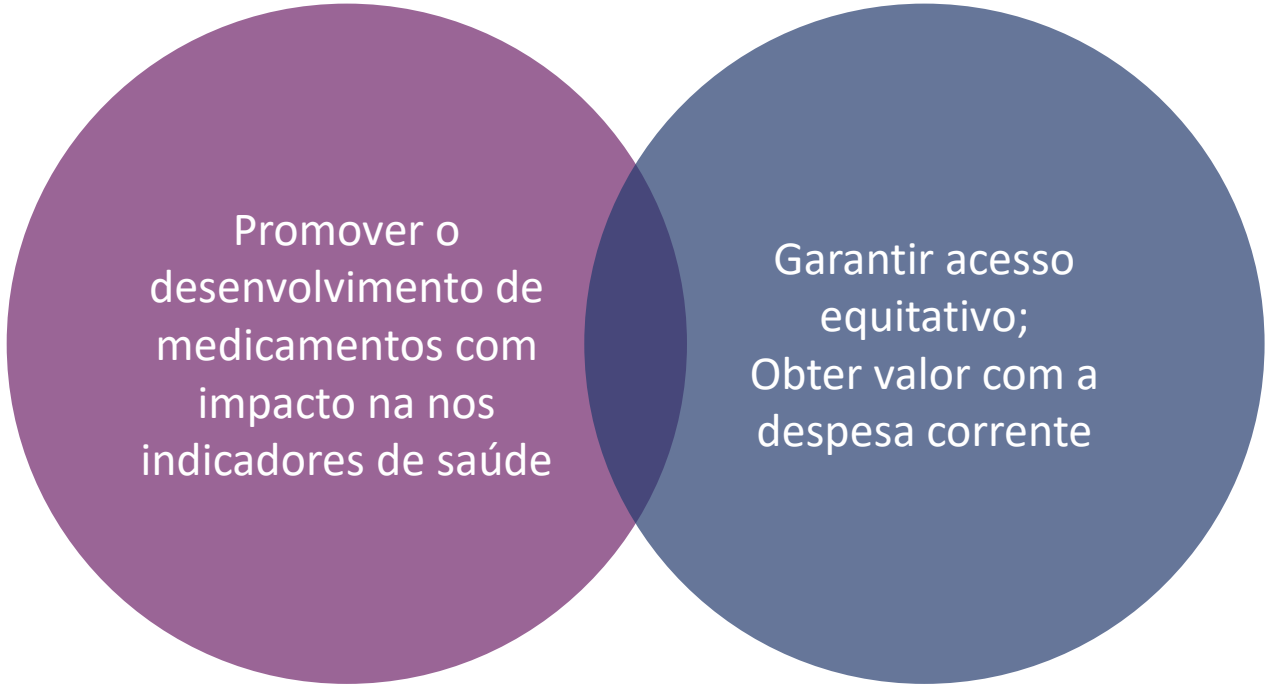
… **pagar o suficiente** de modo a incentivar mais investimento em I&D

… **não pagar mais do que necessário** para tal incentivo

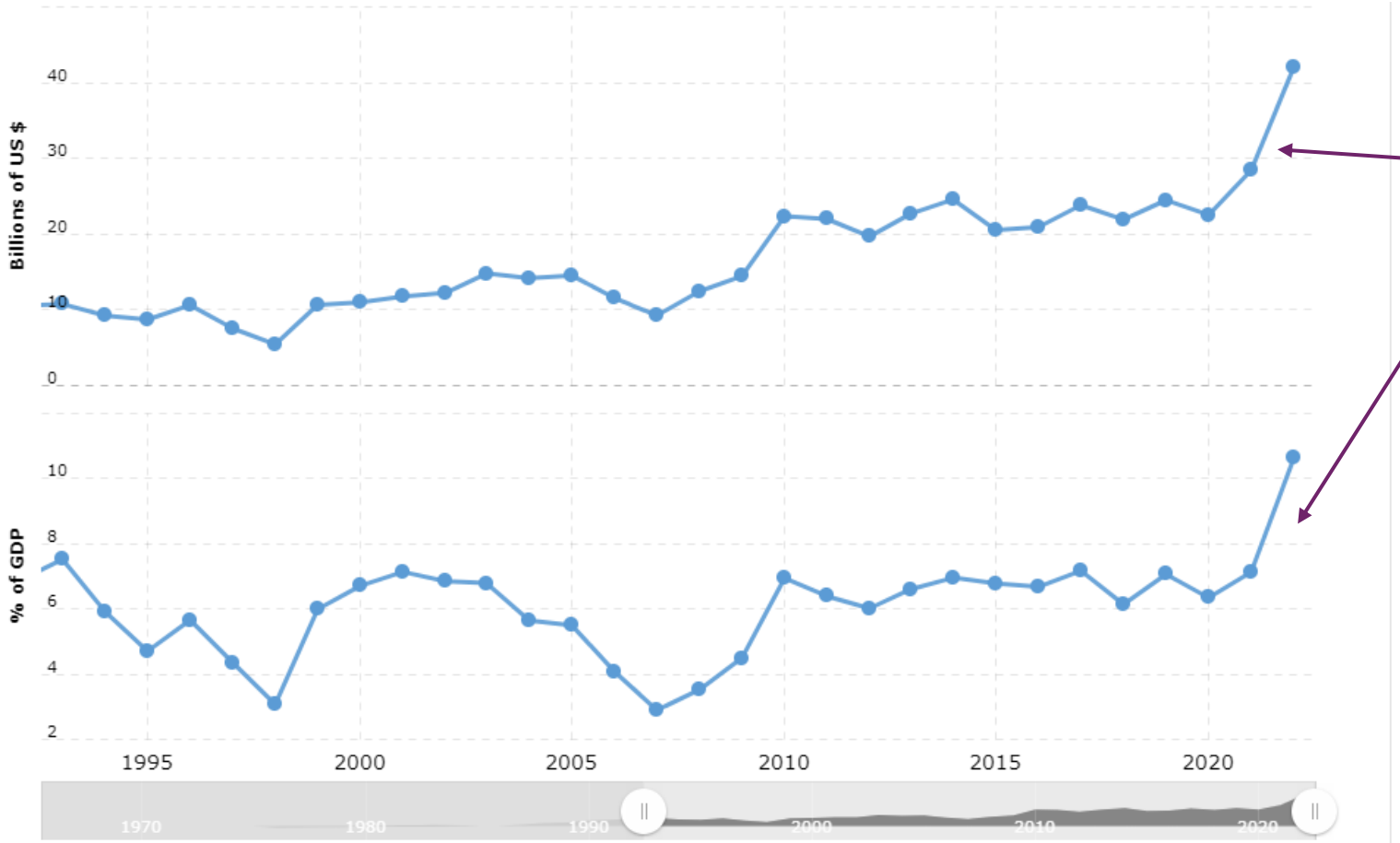


Fonte: adaptado a partir de https://www.oecd.org/daf/competition/excessive-pricing-in-pharmaceuticals.htm

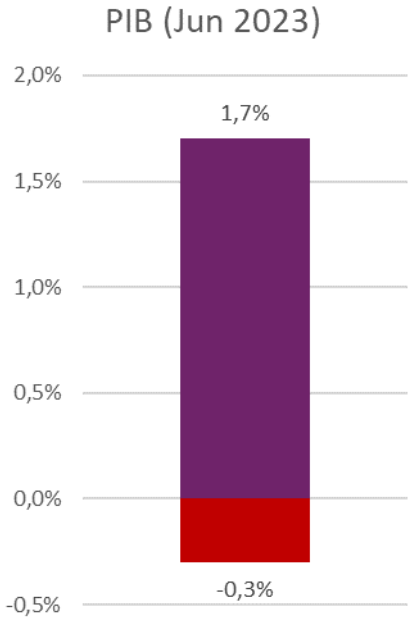
Competitividade do sector farmacêutico europeu

- Commissioner Stella Kyriakides at the Competitiveness Council- EU pharmaceutical reform (25 September 2023)
- “We had two main objectives in the pharmaceutical reform”:
- “Firstly, ensuring that safe and effective medicines reach patients”
- “Secondly, at the same time to ensure that our rules foster innovation and competitiveness in Europe's pharma industry”
- Usually, the revision of the pharmaceutical legislation is discussed by health ministers in the Employment, Social Policy, Health and Consumer Affairs Council (EPSCO)
- However following fuss from industry, **Austria** and **Germany** urged the Spanish and upcoming Belgian presidency to include the matter in the Competitiveness Council (COMPET)

Macroeconomia: Dinamarca balanço comercial e Novo Nordisk

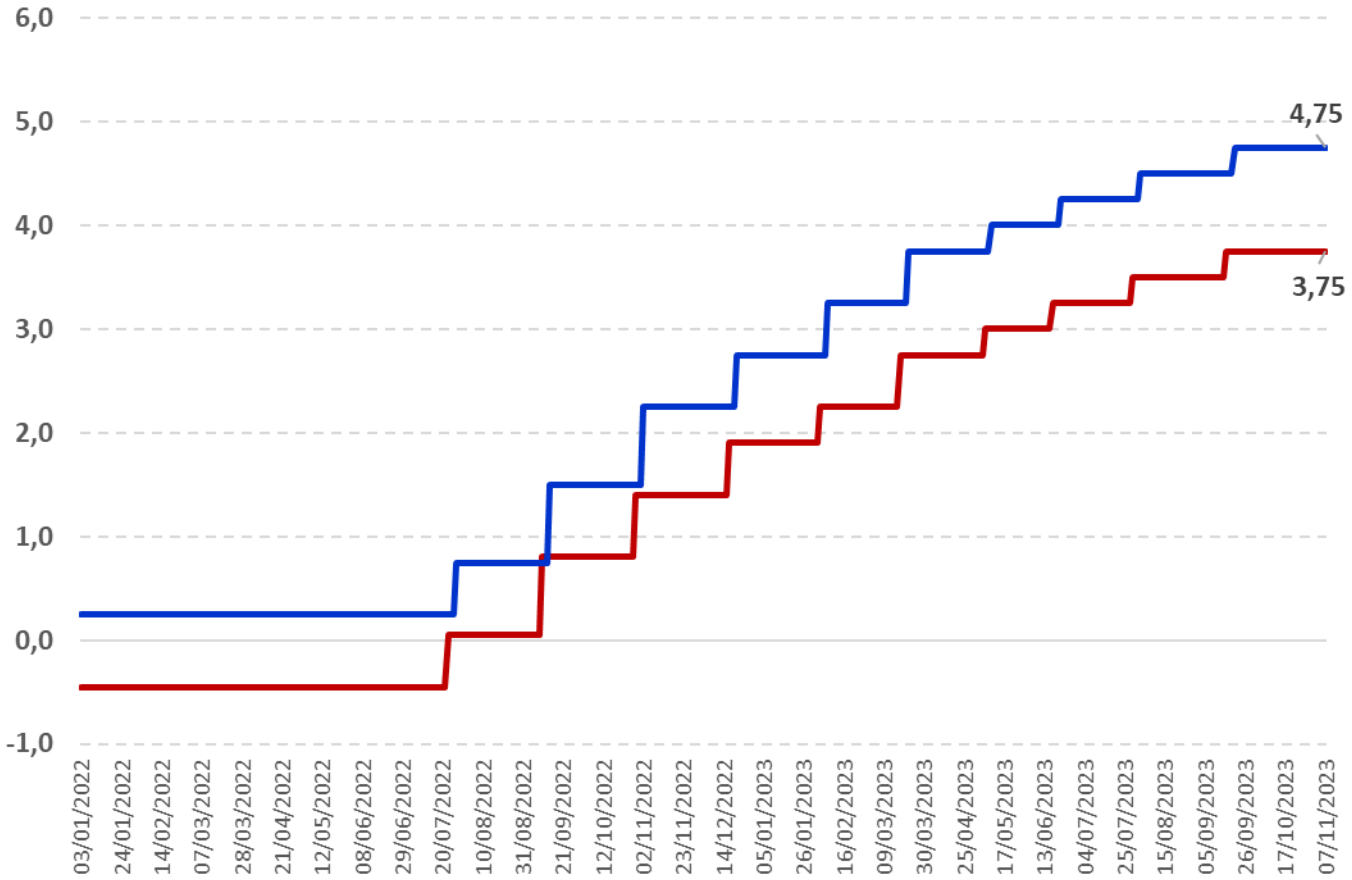


Novo Nordisk
Semaglutido: diabetes mellitus tipo 2 ; perda de peso



Fonte: https://www.macrotrends.net/countries/DNK/denmark/trade-balance-deficit

Taxa que os bancos podem obter liquidez



Fonte: https://www.nationalbanken.dk/en; https://www.ecb.europa.eu/home/html/index.en.html

Denmark's Economic Transformation

- By August 2023, Novo Nordisk's market capitalisation exceeded $427 billion > GDP ($398 billion)
- Europe's second-largest company (1st LVMH Louis Vuitton Moët Hennessy)
- Denmark's central bank has further had to maintain **lower interest rates** to stabilise the Danish krone, strengthening from Novo Nordisk's booming sales. As a result, Danish homeowners enjoy **lower mortgage rates** in comparison to their European counterparts

The New York Times · DANMARKS NATIONALBANK · Bloomberg · REUTERS · Le Monde · FT FINANCIAL TIMES · CNN · The Guardian

Evolução da legislação farmacêutica europeia

- Balanço difícil entre a politica de saúde e a politica económica
- Quem retém o valor da inovação
- Ótica da despesa em saúde
  - Custos, diretos e indiretos
  - Custos de oportunidade
- Não há tempo para “ingenuidades”
  - State Secretary at the Federal Ministry for Economic Affairs and Climate Action of Germany, Sven Giegold, agreed, warning that there is “no time for naivety” and that “we have seen in other sectors what happens when we are late”.
- Necessidade de debater e refletir o pacote farmacêutico da UE também numa perspetiva industrial e da competitividade