

Guidebook for patients: Drug approval & HTA



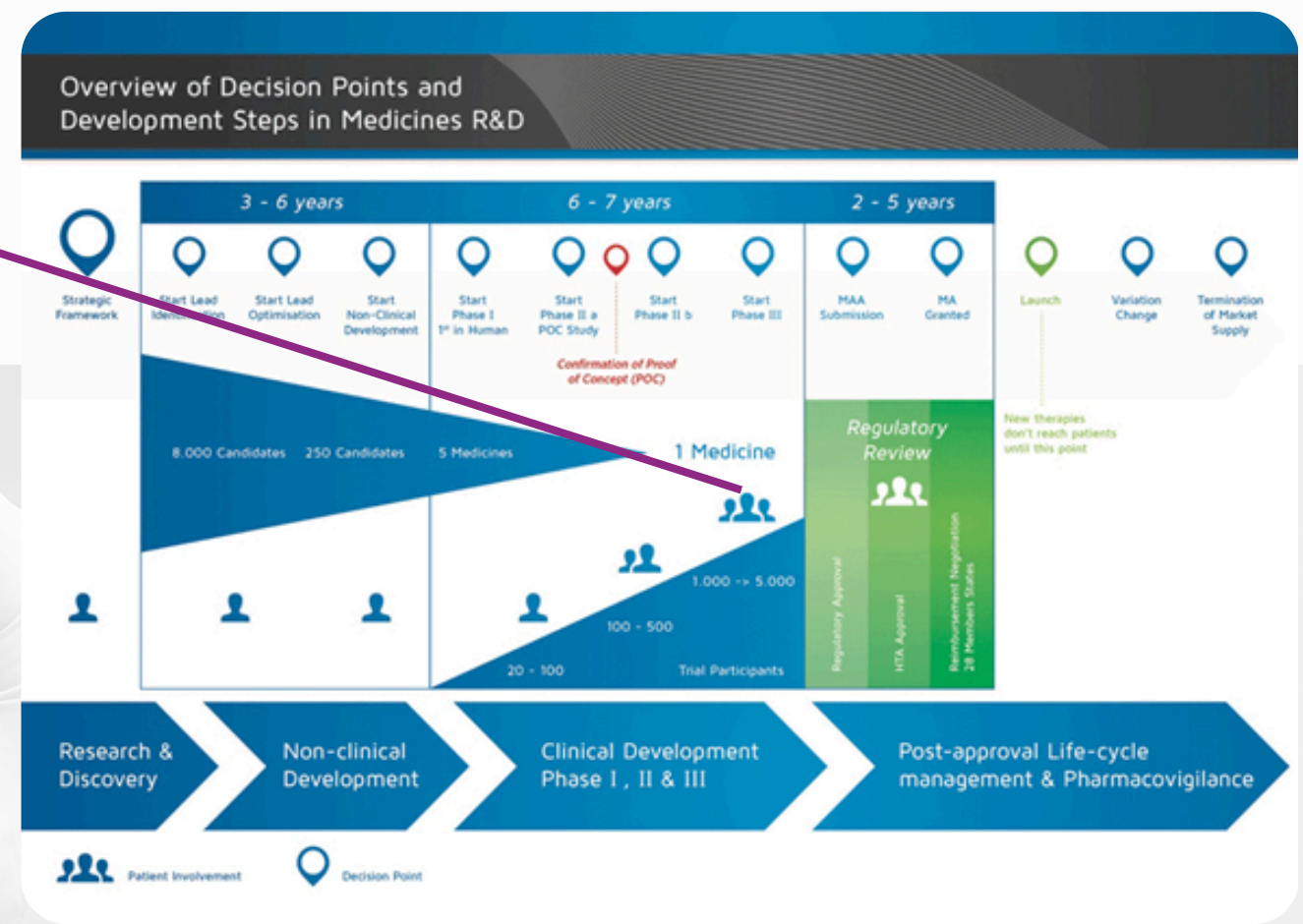
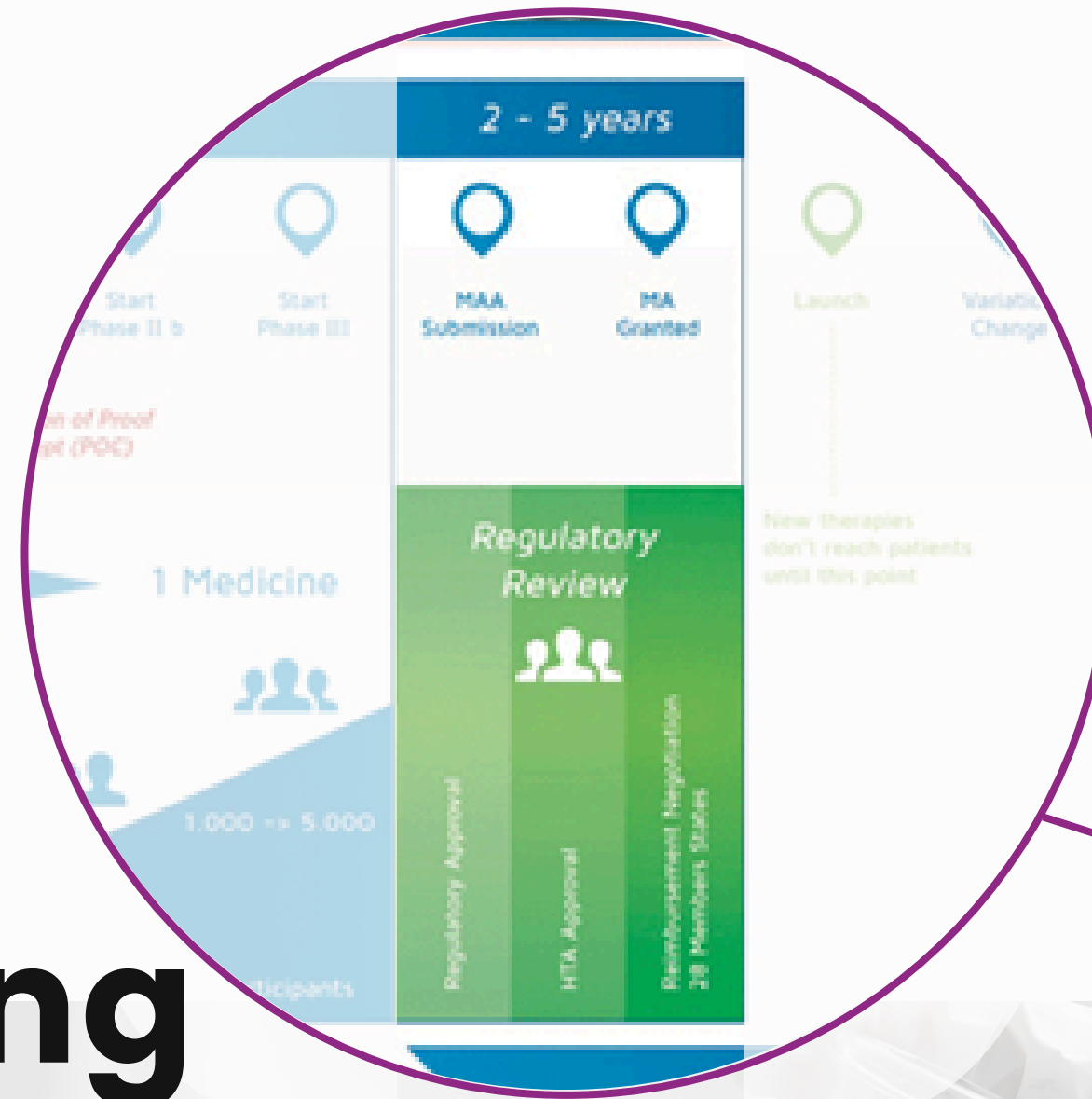
EFCCA Conference

Vienna, 12 October 2024

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Engagement Coordinator

Setting the scene

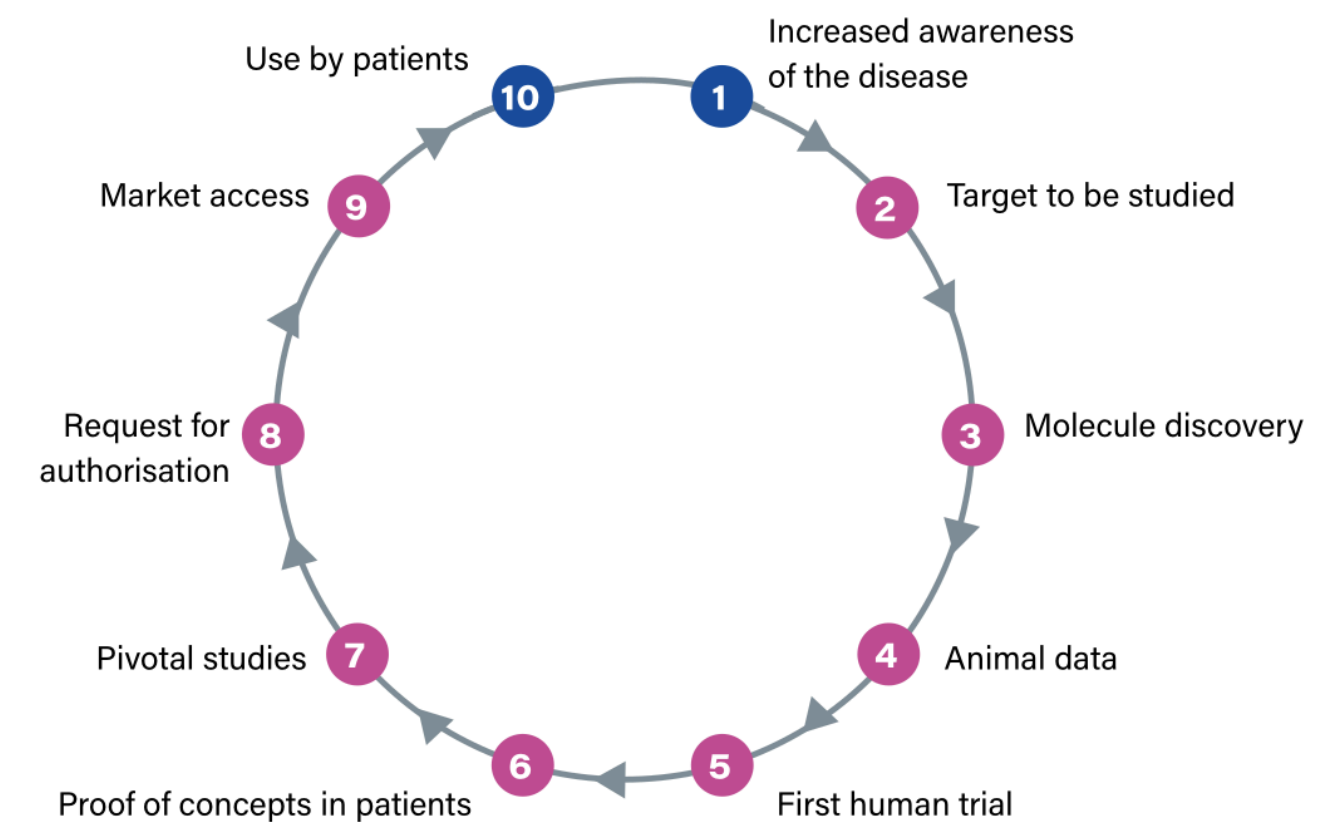


Commitment to:

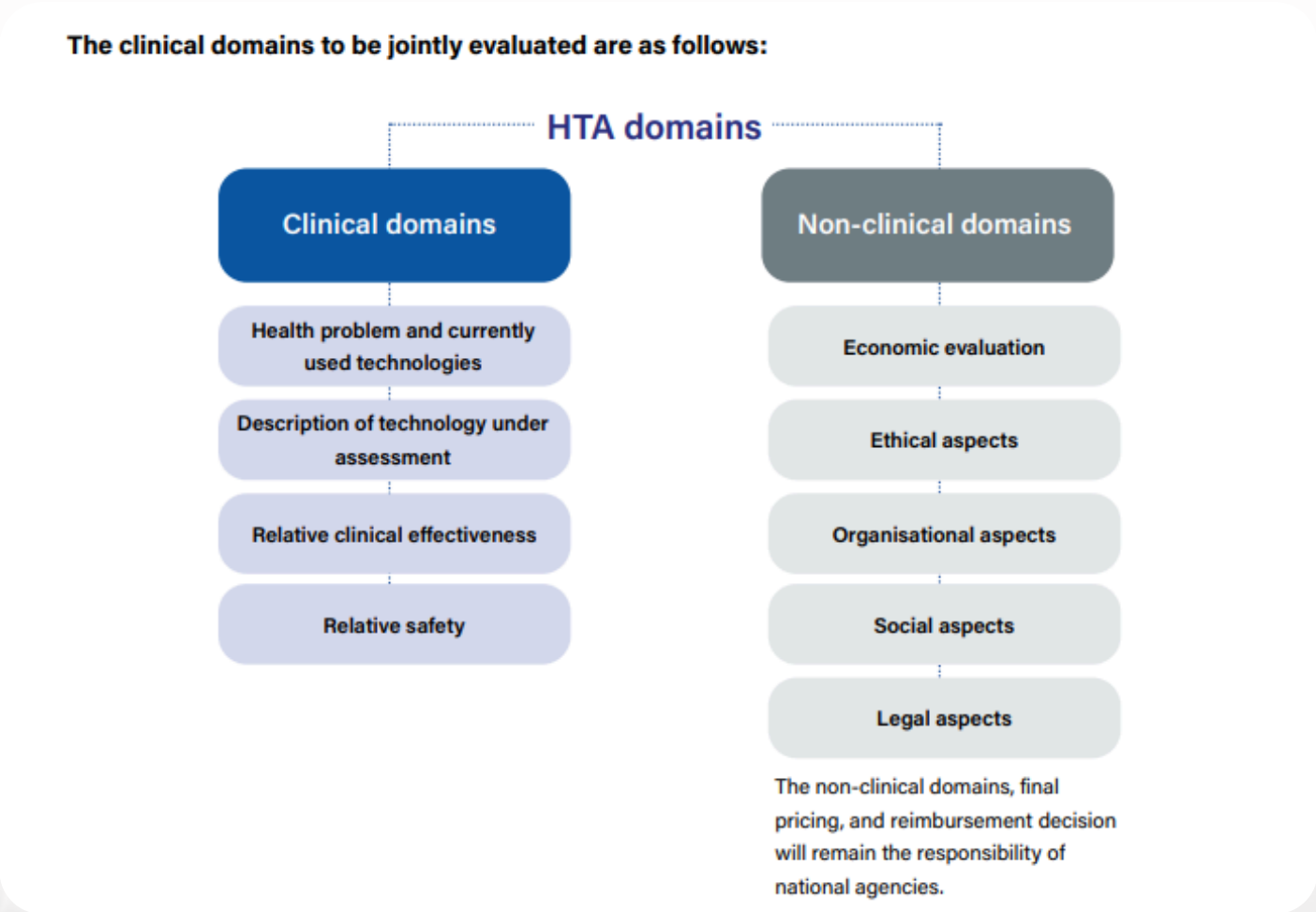
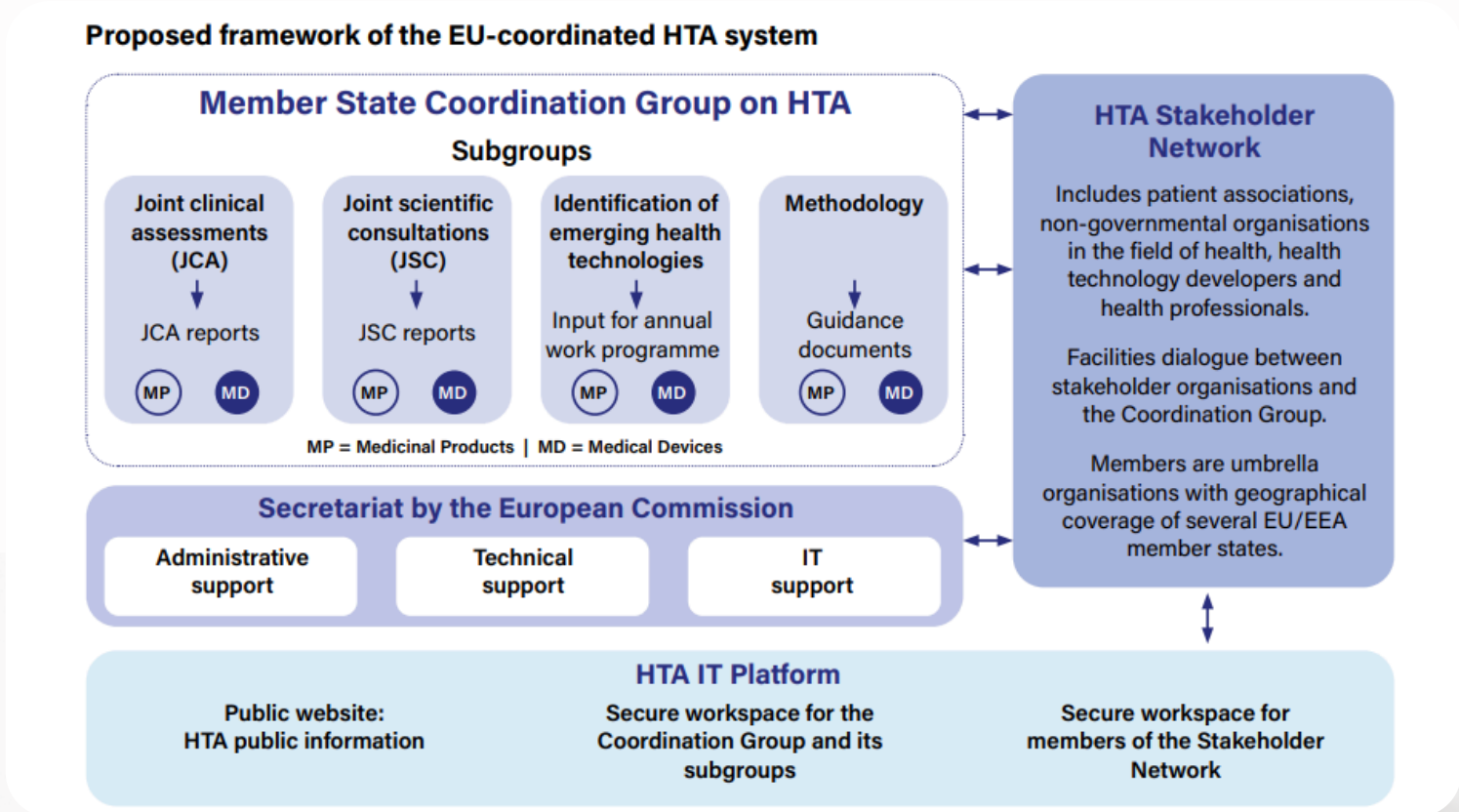
- Design studies for patient-relevant outcomes and preferences.
- Increase access to CTs by reducing barriers.
- Simplify informed consent documents.
- Engage patients in two-way communication
- Connect patients to support programs.
- Provide uninterrupted access to CT therapies.



Setting the scene



Clinical trials are followed by drug authorization and evaluation



**HTA is complex, but we
were able to explain it.**



EFCCA HTA Guidebook for patients



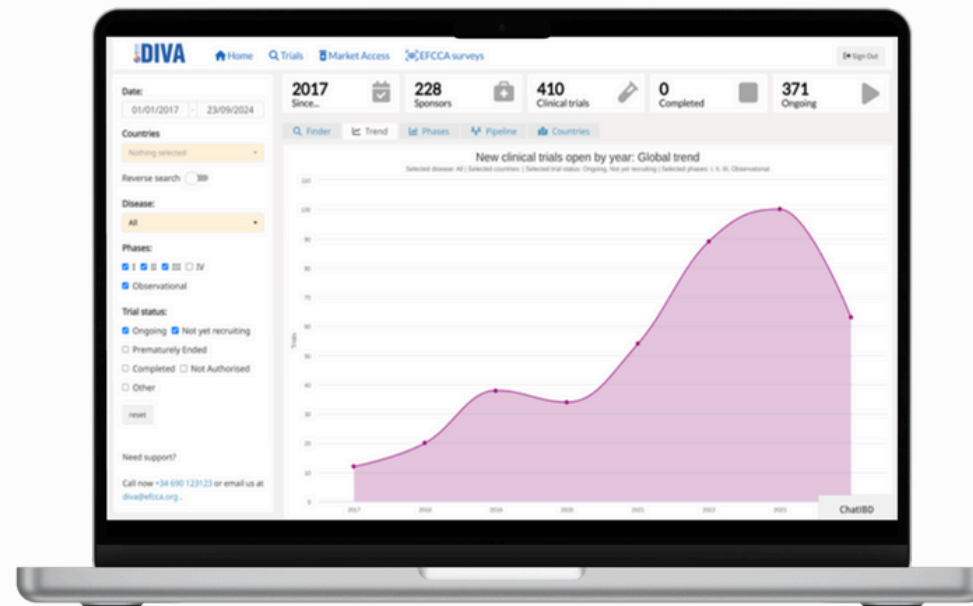
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1. Introduction
2. Inflammatory bowel disease and innovation
3. The drug approval process
4. The health technology assessment process.
5. Patient-reported outcomes to support health technology assessment

More drugs are coming: what can we do to help?


Patients can **participate** in the following HTA-led processes:

- Identifying and prioritizing the technologies to evaluate.
- Identifying goals and outcomes that are important to patients.
- Assigning values and preferences to available scientific knowledge.
- Adapting or simplifying reports to be more easily understood by patients.
- Collaborating in activities to disseminate findings from HTA reports.



Sponsor	Trial ID	Offert
Abbvie	11 abv464 (1, 2, 3)	11 abv464 (1, 2, 3)
Abivax	11 abv464 (1, 2, 3)	11 abv464 (1, 2, 3)
Amgen	11 am7798 (1)	11 am7798 (1)
AstraZeneca	11 az7798 (1)	11 az7798 (1)
Celgene	11 cel7798 (1, 2)	11 cel7798 (1, 2)
Janssen	11 jan7798 (1)	11 jan7798 (1)
Lilly	11 lily7798 (1, 2)	11 lily7798 (1, 2)
MSD	11 msd7798 (1)	11 msd7798 (1)
Pfizer	11 pfz7798 (1, 2)	11 pfz7798 (1, 2)
Sandoz	11 san7798 (1)	11 san7798 (1)
centro de educación médica e investigaciones clínicas norberto pirotta	11 cen7798 (1)	11 cen7798 (1)

...but **innovation** does not come.



EFPIA Patients W.A.I.T. Indicator 2023 Survey

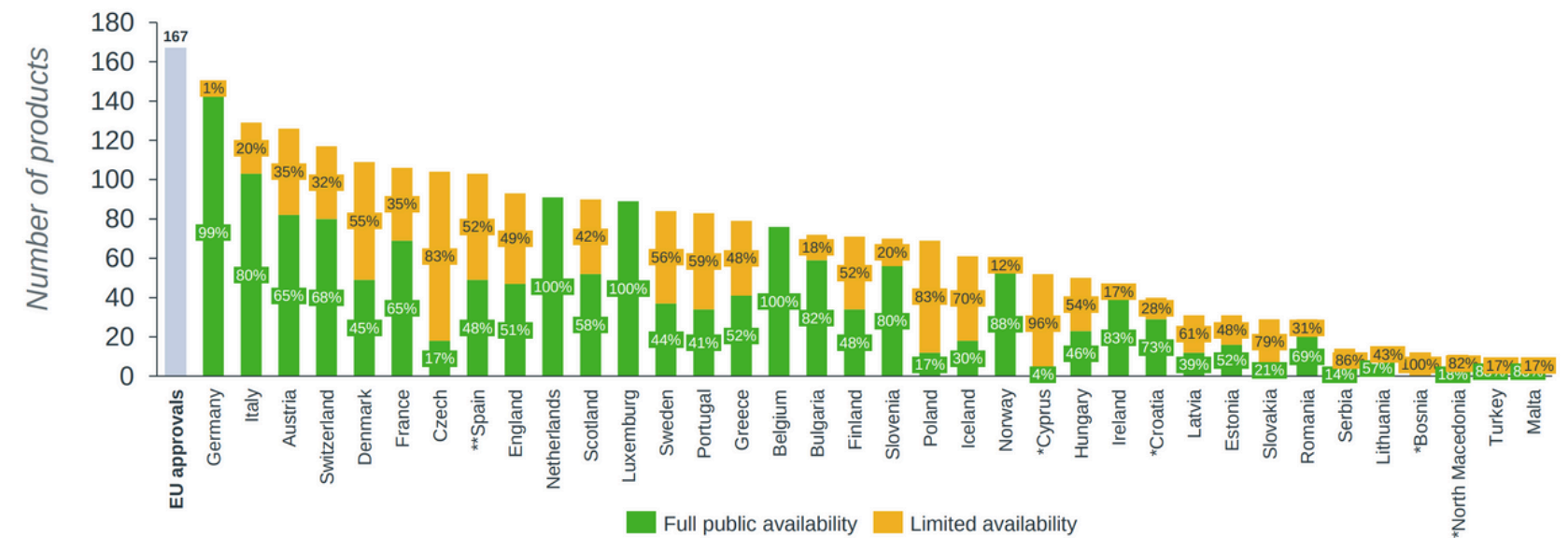
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Rate of full availability (% , 2019-2022)

The **rate of full availability** shows the proportion of medicines available to patients in European countries as of 5th January 2024 (for most countries this is the point at which the product gains access to the reimbursement list[†]) without any restrictions to the patient population, or through named patient basis schemes which have increased significantly in recent years and were not always captured in survey submissions.



European Union average: 72 products available (43%), Limited Availability (40% of available products) Netherlands did not submit complete information on restrictions to available medicines meaning LA* is not captured in these countries. †In most countries' availability equates to granting of access to the reimbursement list, except in DK, FI, LU, NO, SE where some hospital products are not covered by the general reimbursement scheme. *Countries with asterisks did not complete a full dataset and therefore availability may be unrepresentative. **In Spain, the WAIT analysis does not identify those medicinal products being accessible earlier in conformity with Spain's Royal Decree 1015/2009 relating to Medicines in Special Situations

EFCCA HTA Guidebook for patients

Why now?



Three main levels of involvement have been described in relation to HTA-levels in Europe



Involvement

- Patients collaborate with advisors from HTA agencies and other stakeholders by exchanging information and participating in decision making at different stages and activities at an individual level, or by joining a committee or working group.

Communication

- Patients and patient organisations receive information about products evaluated by HTA
- Information is disseminated among other patients, caregivers, or users



Some of the **questions** that EFCCA representatives have had to answer in recent years



What symptoms or side effects are patients experiencing?

How long do patients' symptoms or side effects last?

How intense and frequent are patients' symptoms?

Which symptoms are patients most concerned about and why?

How often are patients hospitalised?

Are there differences between men or women, younger or older age groups, or geographical regions?

How are patients' lives changed by the disease?

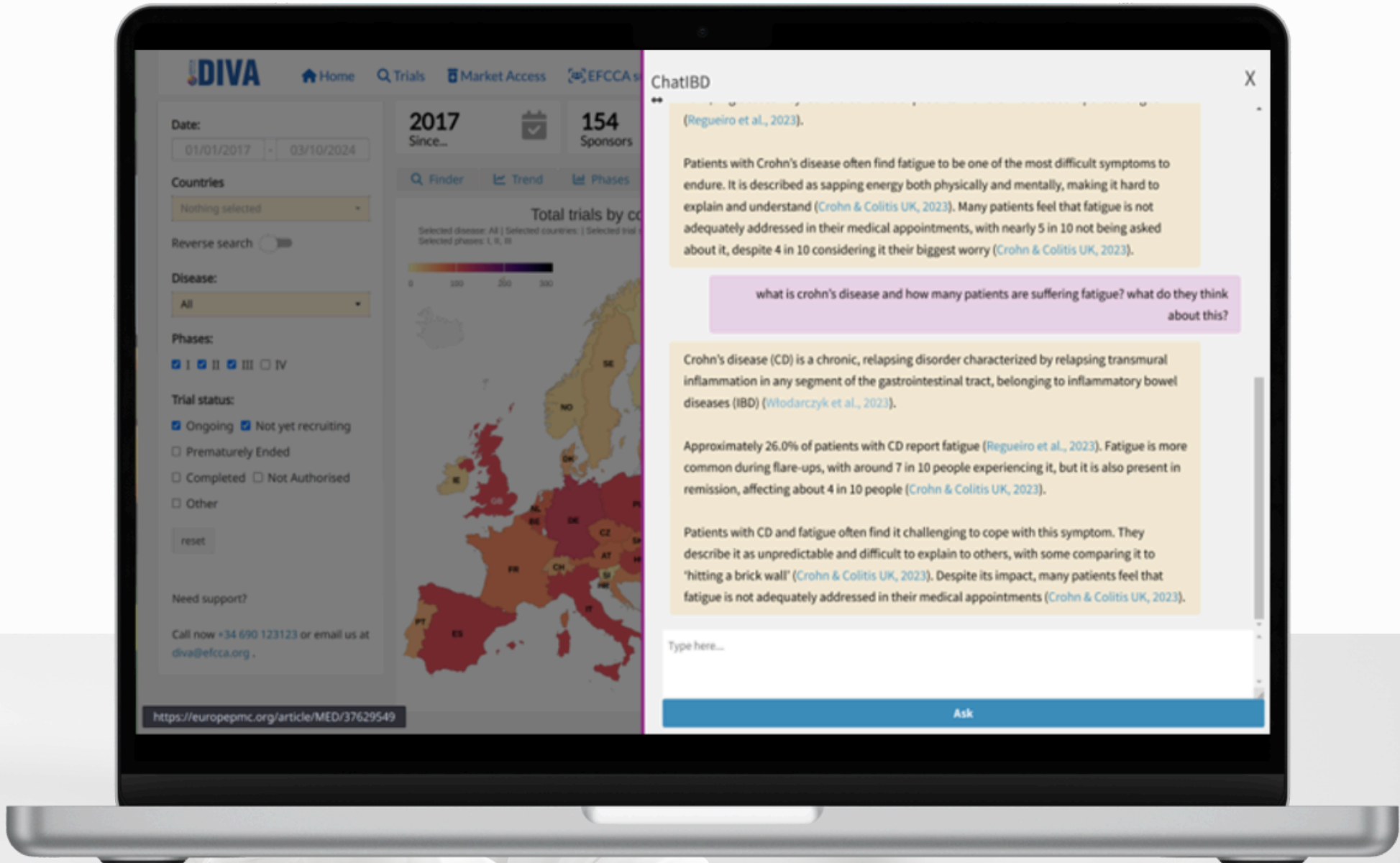
What benefit-risk are patients willing to accept to resolve symptoms?

To what extent do patients experience a sense of vulnerability during or following hospitalisation?

To what extent are patients' symptoms resolved by currently available treatments or surgery?

How much money do patients spend on managing their disease?

What characteristics do patients desire from a new treatment?



+ info on DIVA (Data Insights for Added therapeutic VALUE): diva.efcca.org

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Monitoring

Choose an option from the drop-down menu

DIMENSION	COMMENTS	STATUS	WEIGHT	COMMENT
Efficacy				
	Prevent surgery	not included ▼		
	Onset of action	not included ▼		
	Clinical remission	not included ▼		
	Steroid free remission	not included ▼		
	Abdominal pain and cramping	not included ▼		
	Other disease-related pain	not included ▼		
	Bowel frequency	not included ▼		
	Bowel urgency	not included ▼		
	Fatigue	not included ▼		
Safety				
	Aesthetic complications	not included ▼		
	Occurrence of infections	not included ▼		
	Risk of cancer	not included ▼		
	Others mild to moderate complications	not included ▼		
QoL				
	Emotional status	not included ▼		
	Social life	not included ▼		
	Sexual life	not included ▼		

Treatment 1

Select to Compare

Improvement in Itch



Improvement in Quality of Life



Improvement in Rash



treatment 2

Select to Compare

Improvement in Itch



Improvement in Quality of Life



Improvement in Rash



Thank you!



EFCCA

European Federation of Crohn's
& Ulcerative Colitis Associations

Follow us!

