EFPIA is redefining our messages for the new chapter

What and how should we communicate now and moving forwards

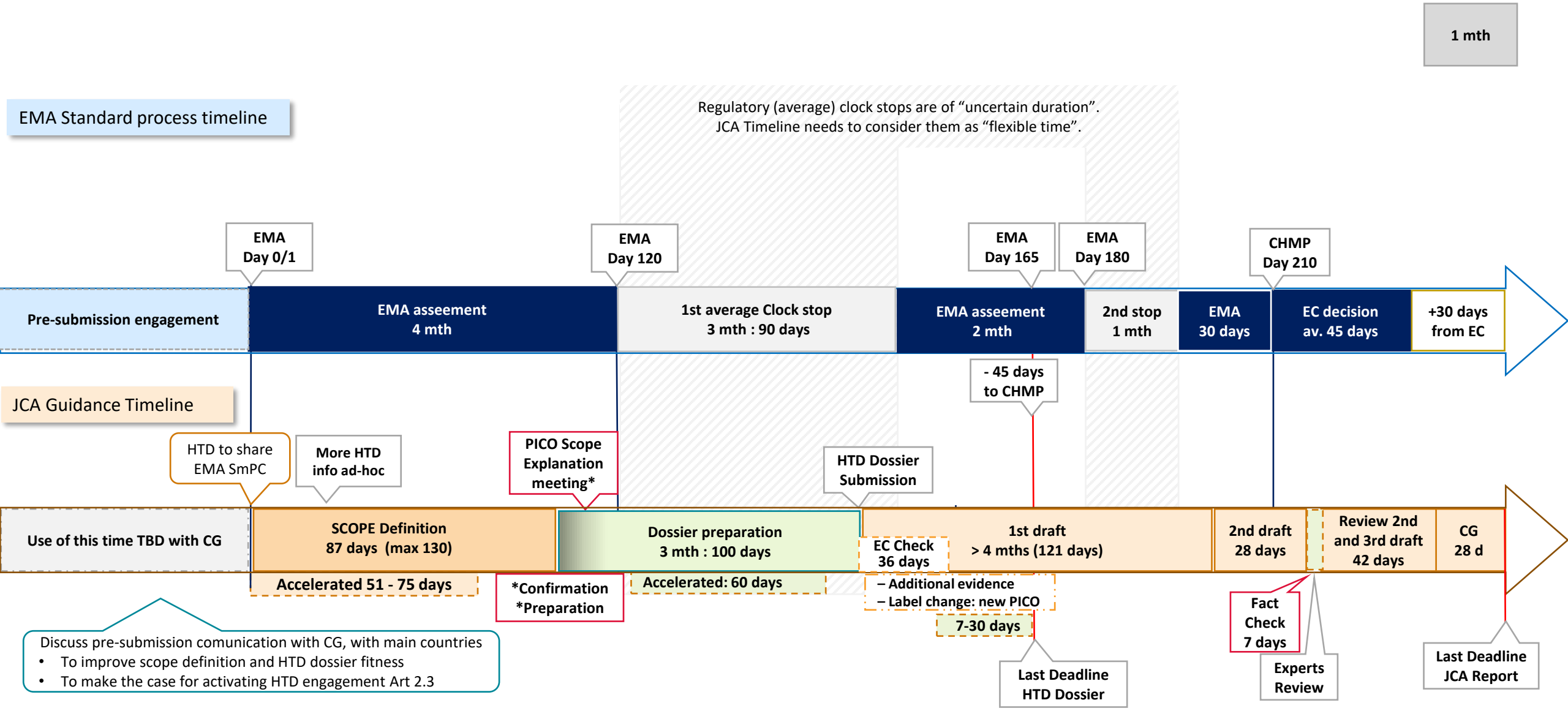
- What will be our key messages in the next months?
- Are we going to keep them for 2024 or adjust in 2025?

HTA ADVOCACY WORKSHOP – Brussels February 25th, 2025

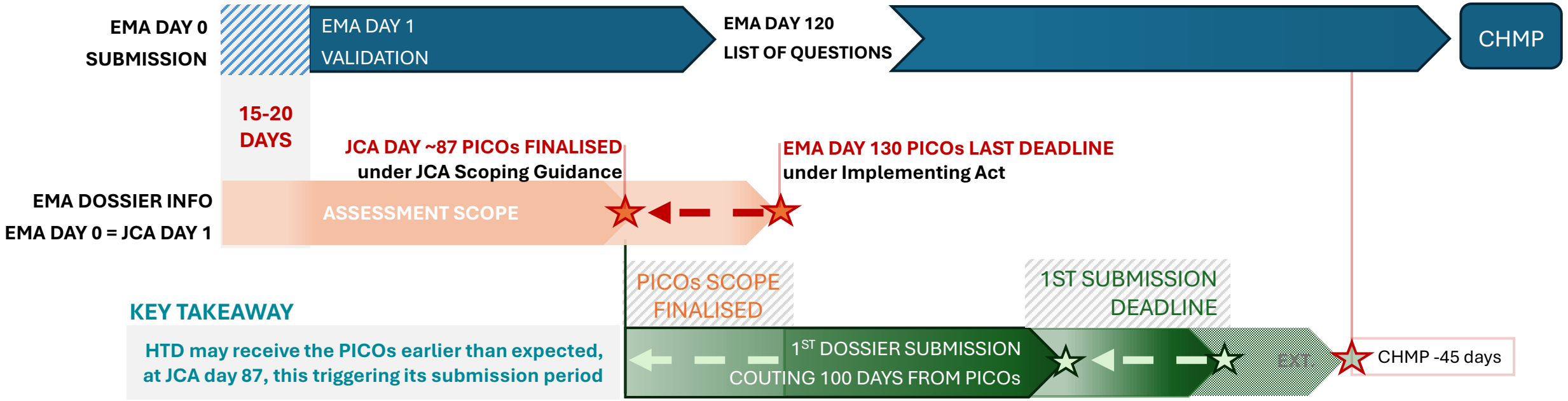
Strategy	
Insisting on EU HTA benefit (and objectives)	**Insisting on implementation shortcomings**
Highlight shortcomings	Impossible to met the objectives
Ask for improvement	Anticipate the failures

Communication tone	
This cannot work	**This must work**
As it is today, it will fail	Not perfect but workable
We told you	Industry will make every effort to make it work

Which message to whom	
EU Commission	Regulators
Coordination group	EU HTA leaders vs National Agencies
Other stakeholders	Policy / Access


1 mth
EMA Standard process timeline
Regulatory (average) clock stops are of "uncertain duration".
JCA Timeline needs to consider them as "flexible time".
EMA Day 0/1
EMA Day 120
EMA Day 165
EMA Day 180
CHMP Day 210
Pre-submission engagement
EMA asseement 4 mth
1st average Clock stop 3 mth : 90 days
EMA asseement 2 mth
2nd stop 1 mth
EMA 30 days
EC decision av. 45 days
+30 days from EC
- 45 days to CHMP
JCA Guidance Timeline
HTD to share EMA SmPC
More HTD info ad-hoc
PICO Scope Explanation meeting*
HTD Dossier Submission
Use of this time TBD with CG
SCOPE Definition 87 days (max 130)
Dossier preparation 3 mth : 100 days
EC Check 36 days
1st draft > 4 mths (121 days)
2nd draft 28 days
Review 2nd and 3rd draft 42 days
CG 28 d
Accelerated 51 - 75 days
*Confirmation
*Preparation
Accelerated: 60 days
– Additional evidence
– Label change: new PICO
7-30 days
Fact Check 7 days
Experts Review
Last Deadline HTD Dossier
Last Deadline JCA Report
Discuss pre-submission comunication with CG, with main countries
• To improve scope definition and HTD dossier fitness
• To make the case for activating HTD engagement Art 2.3

JCA timeline. New interpretation based on first JCAs. Interplay with EMA process



KEY TAKEAWAY

HTD may receive the PICOs earlier than expected, at JCA day 87, this triggering its submission period

EFPIA RECOMMENDATION TO MEMBERS

Companies to work assuming the most time-challenging scenario: reception of the PICO scope at calendar day 87 from EMA filing

CHALLENGES

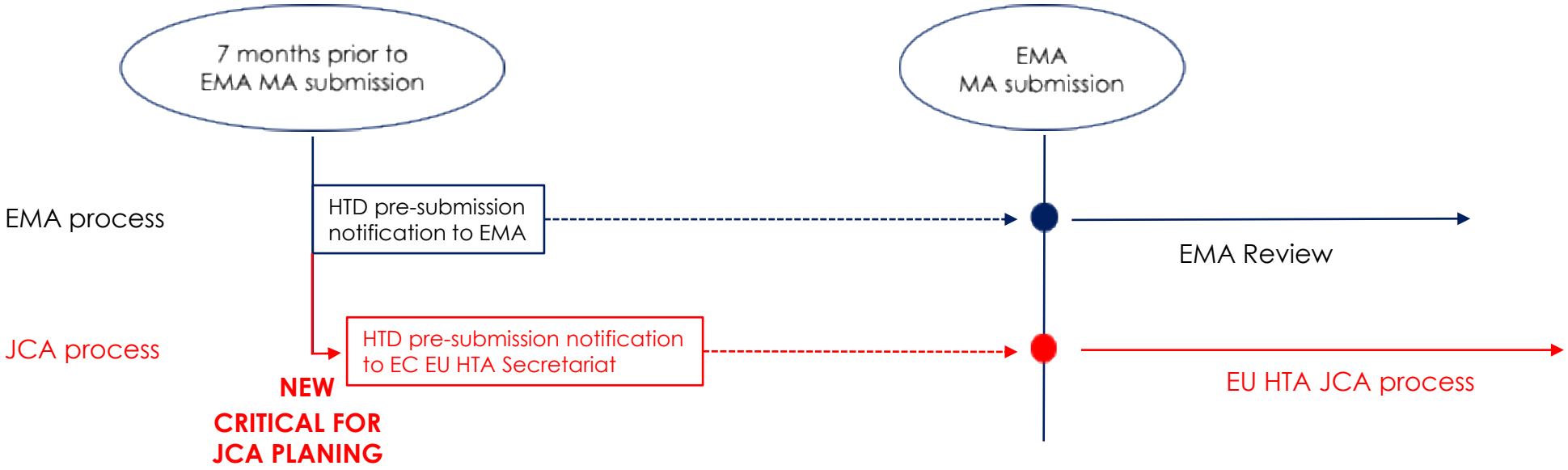
- **No full predictability on the HTD's side**
- **Need for a justified request for extension**

OPPORTUNITIES

- **Earlier visibility on the scope**
- **More rationale for extension requests**

Pre-submission notification : a key piece of the system

Early notification enables assessors readiness



For products in scope of JCA (2025: all ATMPs + oncology NAS in first indication):

-> **When company pre-notifies EMA** about an upcoming submission
-> then **also pre-notify the EU HTA secretariat** (voluntary and proactively)

First, **this was an industry ask**:

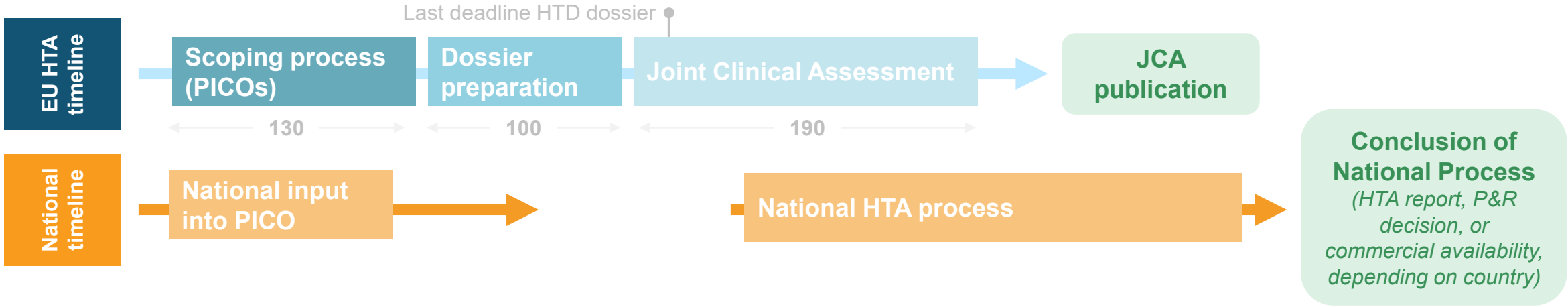
1. Time pre-JCA can be used to select assessors
2. Enables JCA to start as early as EMA day 0/1

Learning by doing. Monitoring to improve

New system not perfect : need to learn and improve from very first JCAs and JSCs

- MONITORING THE FIRST PHASE SYSTEM PERFORMANCE
- PREPARING TO BRING POSSIBLE IMPROVEMENTS

We wish to align on the most relevant time-points to monitor, regarding the JCA impact and its use in the HAS process



	Collection time point	Source
EU level data points	From day of EMA submission	Data collected by EFPIA from publicly available documents
	Day of JCA publication	Data collected by EFPIA
National level data points	Day of HTD dossier submission at HAS + days of acceptance	HTDs/NTAs

Are there any product-specific HAS process time-points which are already monitored by Leem?

The information provided by HTDs aims to capture whether the existence of JCA report leads to a more streamlined process for HTDs

NATIONAL LEVEL

Data requested	KPI	Policy objective
Is the JCA report referenced in the national HTA report? (Y/N/Unknown)	**KPI14** The extent to which national HTA reports reference and integrate findings from the JCA report. If no national HTA report exists, this KPI is not applicable	**Ensuring effective integration of JCA findings in national HTA reports**
For country-relevant PICOs, does the national HTA report use the same endpoints as the JCA for: • Primary trial endpoints (Y / N / N/A) • Patient-Reported Outcome Measures (PROMs) (Y / N / N/A) • Health-related quality of life (HRQoL) endpoints (Y / N / N/A)		
Did the national HTA body request additional PICOs beyond those included in the JCA report? (Y/N)	**KPI15** Percentage of national HTA bodies requesting additional clinical data beyond what is available in the JCA report. If no additional data requests are made, this KPI is not applicable.	**Minimizing unnecessary additional clinical data requests beyond JCA**
For PICOs included in the JCA report, did the national HTA body request additional clinical data beyond that included in the JCA report? (Y/N)		

- Data collected for KPI14 is a **proxy for the extent of JCA report integration** at a national level
- This focuses on the integration of clinical efficacy outcomes and disregarded other elements of the PICO to **reduce HTD collection burden**

- Collecting data on the frequency of additional data requests can help understand whether **JCA report is relevant on a national basis,** and ultimately **streamlines the process for HTDs**

Next slide contains aligned definition of 'additional data'

We want to discuss whether details on additional evidence requested should be captured, or whether this should remain a binary response

NATIONAL LEVEL

Data requested	KPI	Policy objective
Did the national HTA body request additional PICOs beyond those included in the JCA report? (Y/N) For PICOs in JCA report, did the national HTA body request additional clinical data beyond that included in the JCA report? (Y/N)	**KPI15** Percentage of national HTA bodies requesting additional clinical data beyond what is available in the JCA report. If no additional data requests are made, this KPI is not applicable.	**Minimizing unnecessary additional clinical data requests beyond JCA**

What counts as "Additional Clinical Evidence"?:
Mandatory clinical data for a particular PICO requested by national HTA body which is not part of JCA or standard national dossier requirements

This includes, but is not limited to:

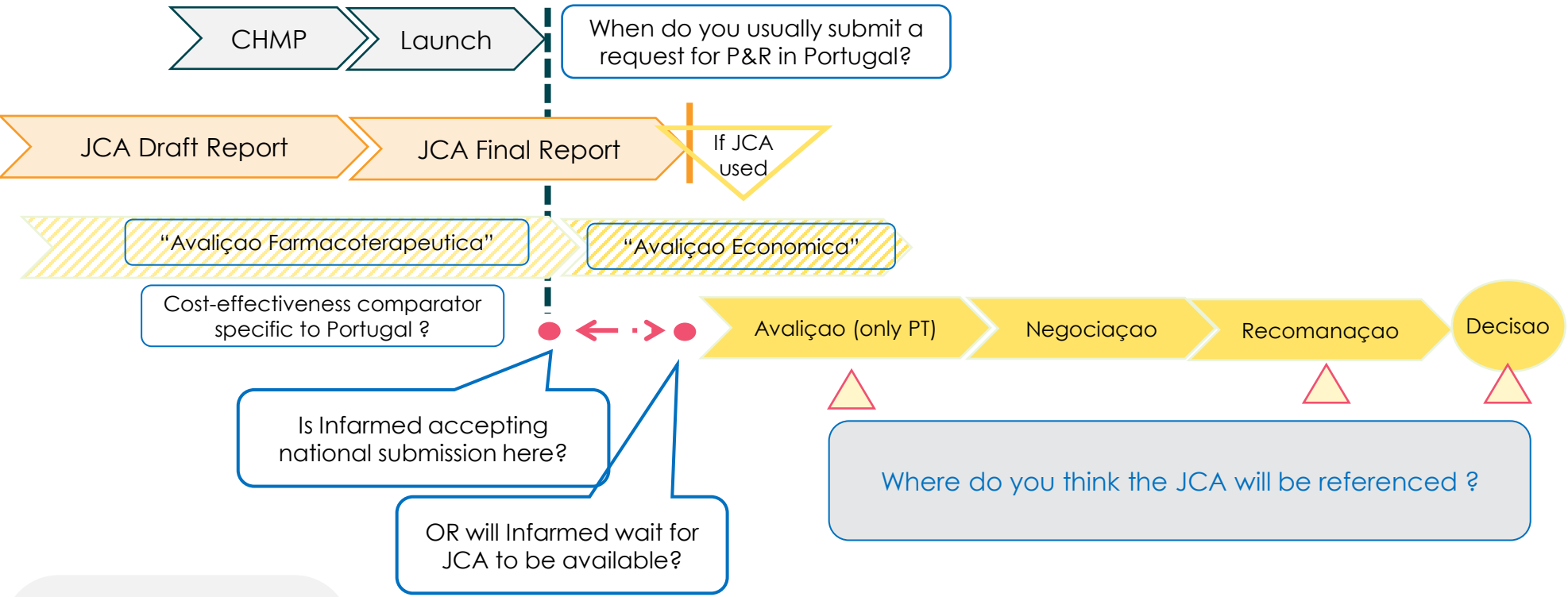
- New analyses
  - Additional subgroup or subpopulation analyses that were not in the JCA
  - Requests for a fresh cut of data (e.g., updated survival data)
- Different or extra comparators
  - Comparators not included in the JCA scope
  - Requests for head-to-head data where only indirect comparisons were submitted (or vice versa)
- Alternative endpoints or outcomes
  - Endpoints not assessed in the JCA but demanded at national level
- Supplementary real-world evidence
  - Demands for new registries, observational data, etc., when these were not part of the JCA
- Methodological re-analyses
  - National HTA requiring the manufacturer to redo analyses in a different statistical framework

This excludes non-clinical data required for standard national dossier submission, such as national epidemiological or economic data

One remaining question is whether we should ask HTDs to provide details on what additional evidence was requested, instead of Y/N response

Understanding the interplay of EU JCA and Infarmed submission

Question: how to best measure the impact of JCA in Portugal ?



QUESTION FOR YOU TODAY

How would you capture the impact of JCA into your national HTA process ?