EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Regulator's perspective & experience on prior knowledge and accelerated access

Session 5: Experiences of accelerated access approaches (i.e. PRIME)

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An agency of the European Union

Problem statement

PRIME eligibility criteria:

'a major therapeutic advantage over existing treatments, or benefit patients without treatment options'

medicine to show its potential to benefit patients with unmet medical needs based on early clinical data

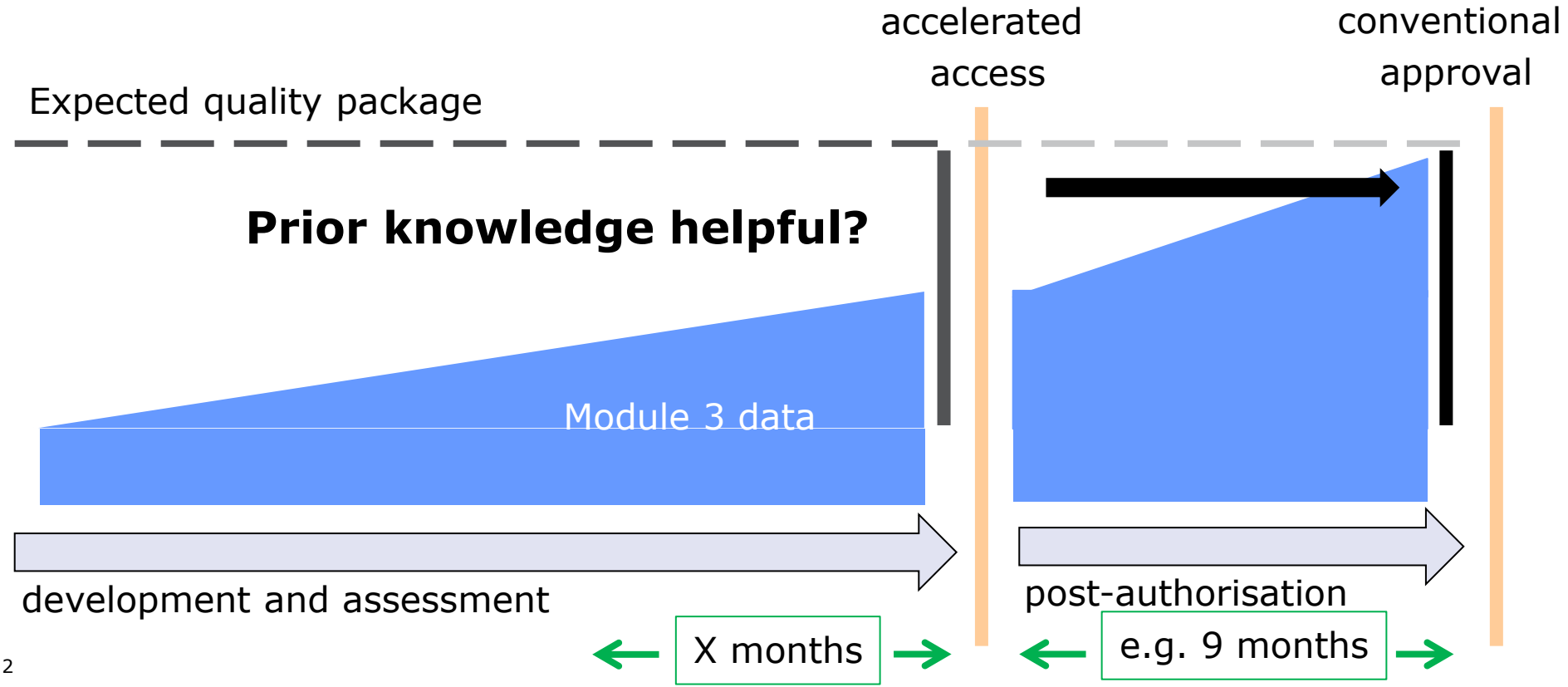
'reduced clinical testing' period

Assessment time

Conventional approval: 210 d + clock stops (3+3 m, 1-3 m) **11 – 16 m**

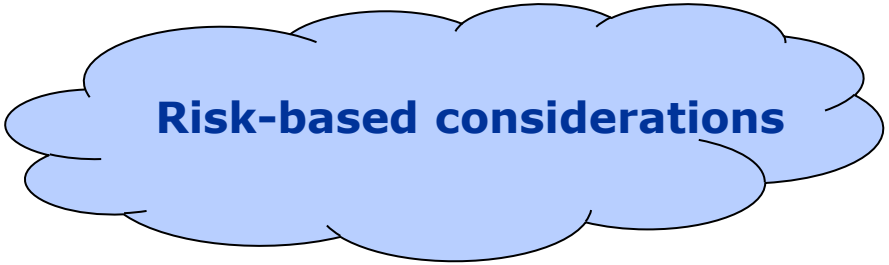
Accelerated assessment: 150 days + clock stops (1-3 m, 1 m) **7 – 9 m**

Problem statement



Considerations for prior knowledge

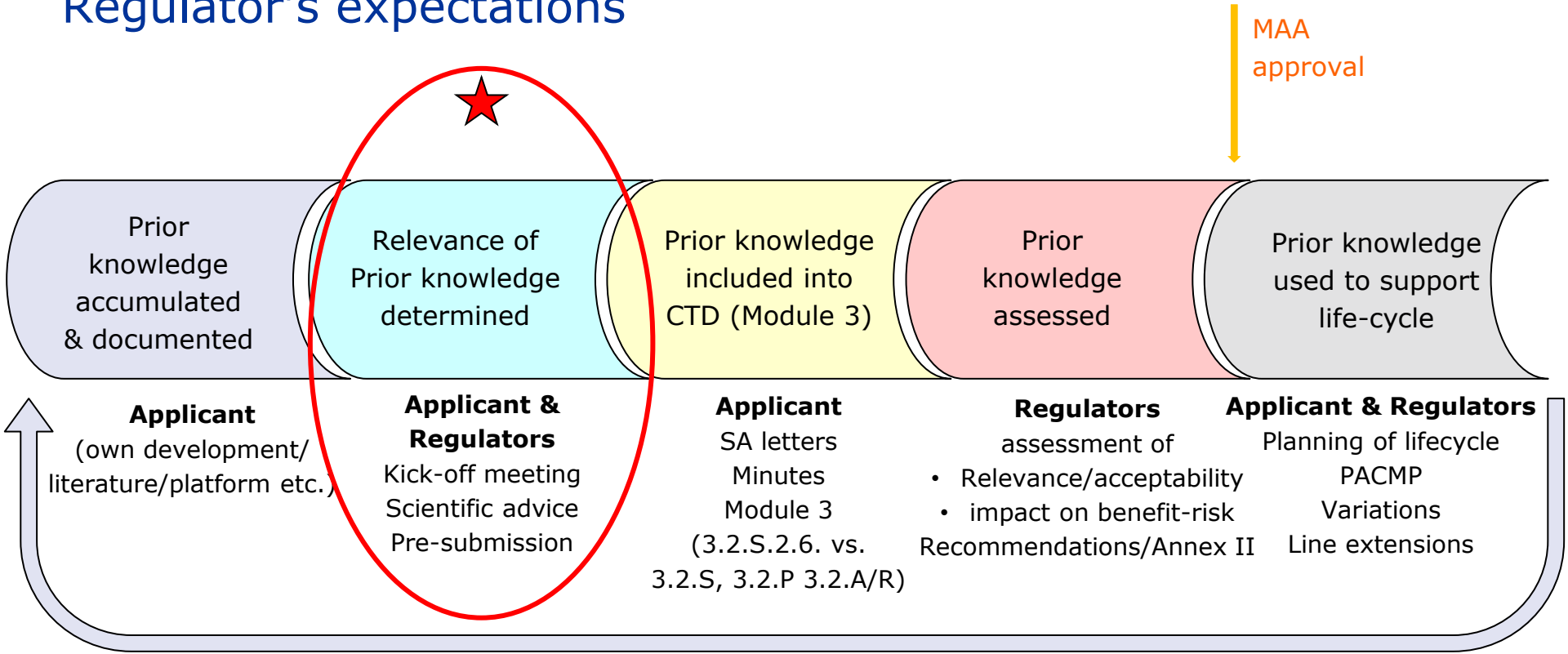
- **How** can flexibility/deferral of quality information be **justified** with prior knowledge?
- **How** & **where** can prior knowledge be described in the dossier?

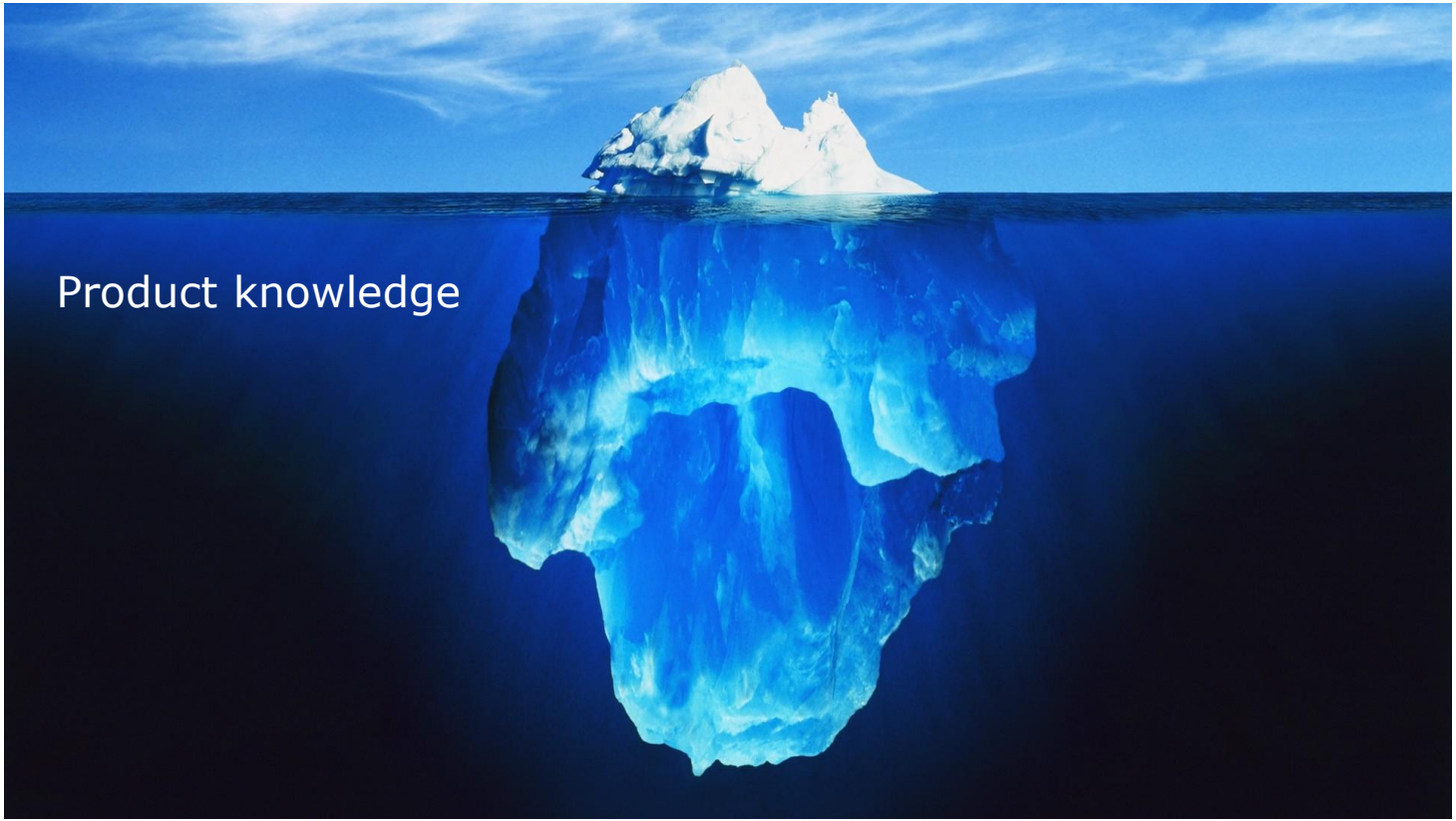


Regulators position:

- Case-by-case decision depending on the product type, unmet need, B/R ratio, totality of data approach
- Plan early for use of prior knowledge (with Regulators) and adjust development and dossier compilation accordingly

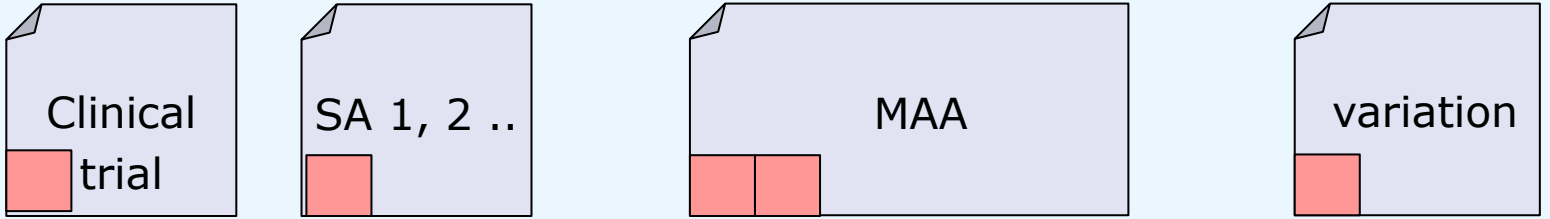
Regulator's expectations




Product knowledge

Accumulation & visibility of prior knowledge

Product development - *visible to Regulators*



static

Company's knowledge - *not visible to Regulators*

Growing prior knowledge & applicability

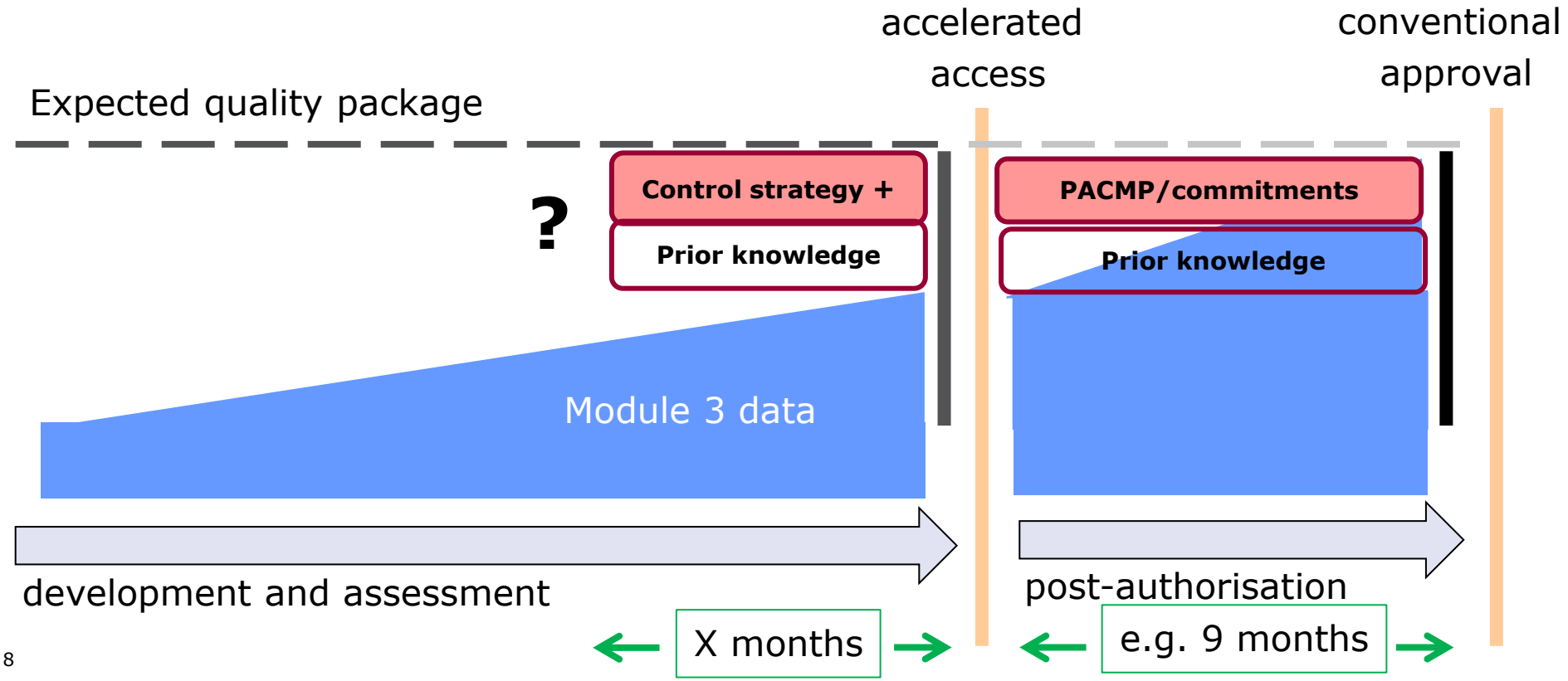
dynamic

Product lifecycle timeline

Prior knowledge during accelerated assessment

- **made available for assessment** in order to facilitate a consistent review across regulators
- Its **relevance and justification** for use should be agreed with Regulators (SA) for better plannability
- Its **way of documentation** should be agreed
- dynamic and evolving amount of data should be made available over regulatory submission (incl. regulatory tools → recommendations, PACMPs)

Point for further discussion



Experience on PRIME quality issues

PRIME designated candidate products (-October 2017)

Product type

11 ATMPs

9 Biological substances

9 Chemical substances

Applicants

12 SME

17 Other

17 candidates have undertaken SA/PA

15 scientific advice reports examined

Topic areas of scientific advice requests PRIME

Additional areas of prior knowledge

Critical areas

Number of question > 5

Product

Areas	Raw materials	Orphan similarity	Cell banks	**Starting materials**	Vector
# Q	**2**	**2**	**2**	**5**	**1**

Process

Areas	Process development	**Comparability**	Change management	**Validation**
# Q	**4**	**13**	**2**	**5**

Control

Areas	Potency assay	**Analytical control strategy**	**Specifications**	Adventitious agents	**Stability**	Product-rel. impurities
# Q	**4**	**6**	**7**	**2**	**6**	**3**

Conclusions:

Share prior knowledge data with regulators and agree on its relevance

- Kick-off meeting
- Scientific advice
- Pre-submission

Agree with regulators on format to capture prior knowledge data, e.g.

- SA letters
- Minutes
- Module 3 (3.2.S.2.6. vs. 3.2.S, 3.2.P 3.2.A/R)

Acceptability of prior knowledge to support quality package & conclusions → matter of assessment (case-by-case approach)

16:10 – 16:20	Considerations for Accelerated CMC programs *Ronald Imhoff, EBE, Janssen / Richard Keane, EBE, Biogen*
16:20 – 16:30	Case Study 1: Atezolizumab: A Case Study of Accelerated Development *Andrea Challand, EBE, Roche*
16:30 – 16:40	Case Study 2: Avelumab integrated Mab example *Isabelle Colmagne-Poulard, EFPIA, Merck*
16:40 – 17:00	Panel discussion *Panel Chair: Peter Richardson, EMA, Head of Quality* *Sol Ruiz, BWP chair, CHMP, CAT, AEMPS, Spain* *Nanna Kruse, BWP vice-chair, CAT, DKMA, Denmark* *Marie-Hélène Pinheiro, EMA, Industry Stakeholder Liaison* *Jordi Llinares Garcia EMA, Head of Scientific and Regulatory Management Department* *Ronald Imhoff, EBE, Janssen* *Richard Keane, EBE, Biogen* *Mairéad Duke, EBE, BioMarin*