# Setting the scene

## Current status of RP on platform trials

**Benjamin Hofner**
Head of Data Science & Methods

Bundesinstitut für Impfstoffe und biomedizinische Arzneimittel
Federal Institute for Vaccines and Biomedicines

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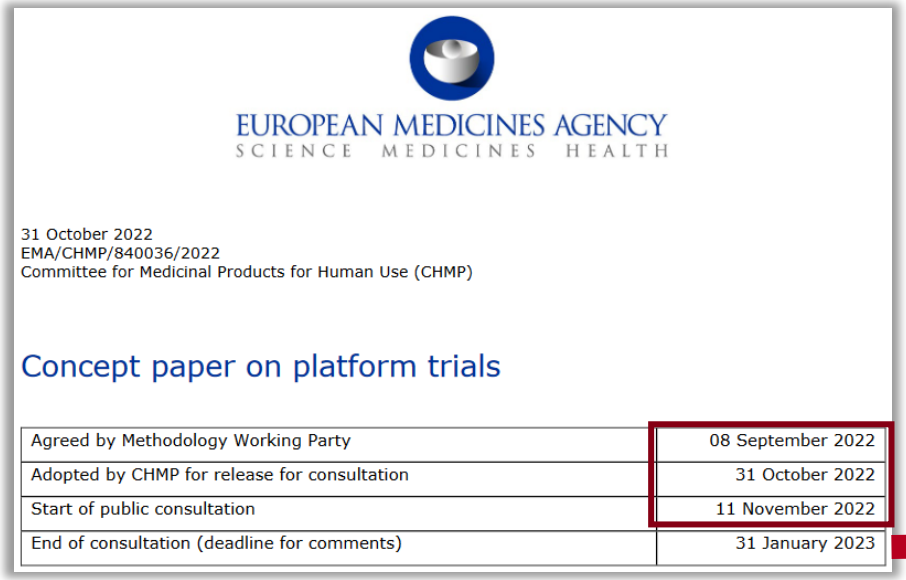
# Concept paper and draft reflection paper



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

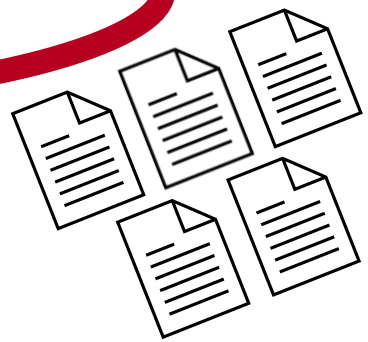
31 October 2022
EMA/CHMP/840036/2022
Committee for Medicinal Products for Human Use (CHMP)

Concept paper on platform trials

| | |
|---|---|
| Agreed by Methodology Working Party | 08 September 2022 |
| Adopted by CHMP for release for consultation | 31 October 2022 |
| Start of public consultation | 11 November 2022 |
| End of consultation (deadline for comments) | 31 January 2023 |

Comments from 12 stakeholders were received, in total around **25 pages** (on a ~2 page document)

✓ **Thank you to all who commented!**



Reflection paper on platform trials
Draft[2]

**Initial timelines**

| | |
|---|---|
| Discussion at CHMP: | 12/2023 |
| Draft Reflection Paper: | 03/2024 |

➢ It might take longer than expected.
**However, we remain ambitious!**

# Plans for Reflection Paper

## Reaction to Comments Received

### Aspects considered in scope of the RP

Primary focus: **methodological issues** in **confirmatory** trials

- Clarify which **elements render a study exploratory** and under which circumstances a study is **suitable for confirmatory** regulatory **decision-making**
- **Multiplicity** will have a key role in the discussion
- (Non-) **Concurrent controls**
- **Blinding** / Unblinding due to (interim) analyses
- **Adaptive design** aspects specific to platform trials (e.g. RAR, change of control arm)

**Broad definition** of platform trials will be used (incl. multi-arm trials without AD)

- Trials with different (sub)populations (“basket”/“umbrella”) not in primary scope

### Aspects considered NOT in scope of the RP

➢ **Note: All these aspects were forwarded to other stakeholders (e.g. ACT EU and CTCG) or are covered already in existing (see brackets) or upcoming guidelines.**

- (Purely) **operational aspects** (EMA/298712/2022)
- **Safety** considerations
- Intellectual property (**IP**) / data protection (**DP**)
- Specific guidance on
  - **rare diseases** (CHMP/EWP/83561/2005)
  - **paediatric extrapolation** (ICH E11A)
- **Historic controls** (ICH E10) **&**
  **Single-arm trials** (EMA/CHMP/564424/2021)
- **Bayesian methods** (EMA/298712/2022)

# Other EU guidelines on platform trials

- CTFG Recommendation Paper on the Initiation and Conduct of Complex Clinical Trials
  - potential opportunities and challenges of complex clinical trials (CCTs)
  - key recommendations for initiating and CCTs
  - regulatory considerations and communication
- Complex clinical trials - Questions and answers (EMA/298712/2022)
  - Q1: planning and conduct of CCTs
  - Q2: additional considerations that are needed for the design and conduct
  - Q3: Bayesian statistics in (complex) clinical trials
  - Q4:  shared controls / non-concurrent controls
  - Q5: biomarkers
  - Q6: safety
  - Q7: transparency (balance with integrity) and communication