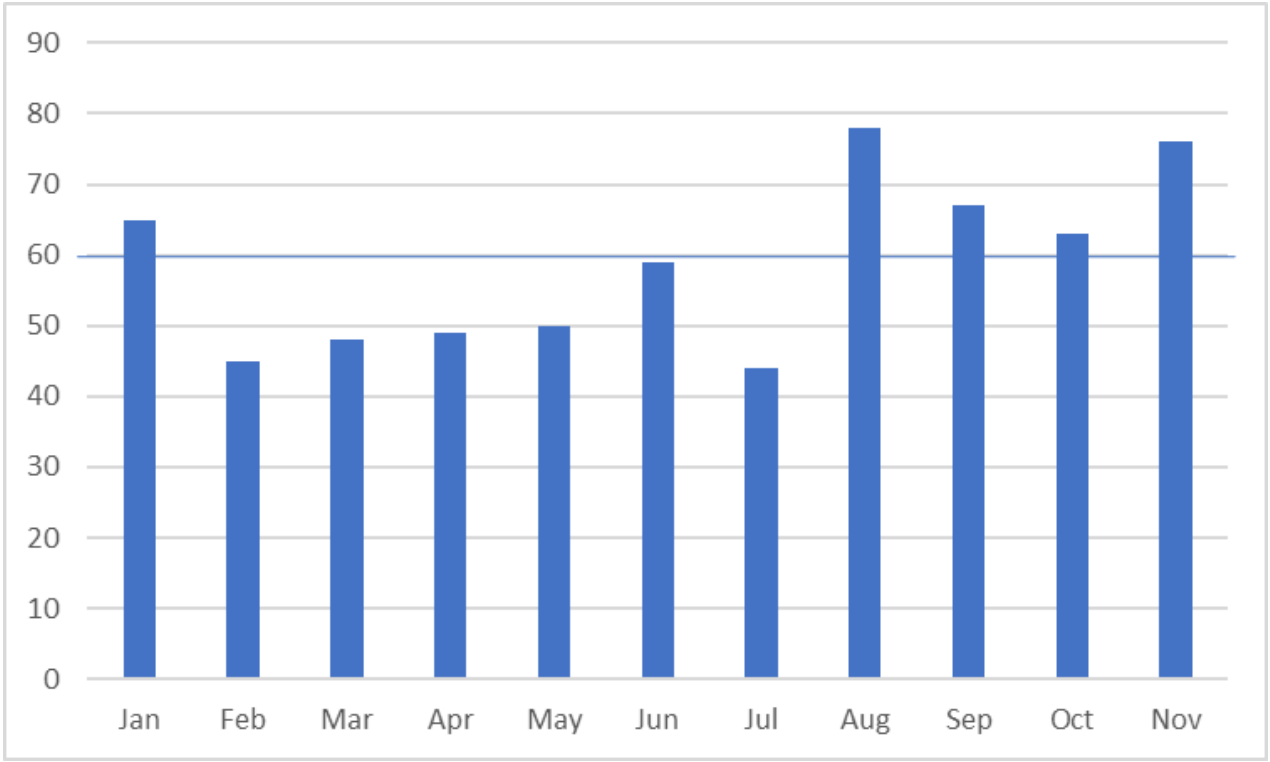
Insight from EMA on recent scientific advice developments

11th Industry stakeholder platform on research and development support

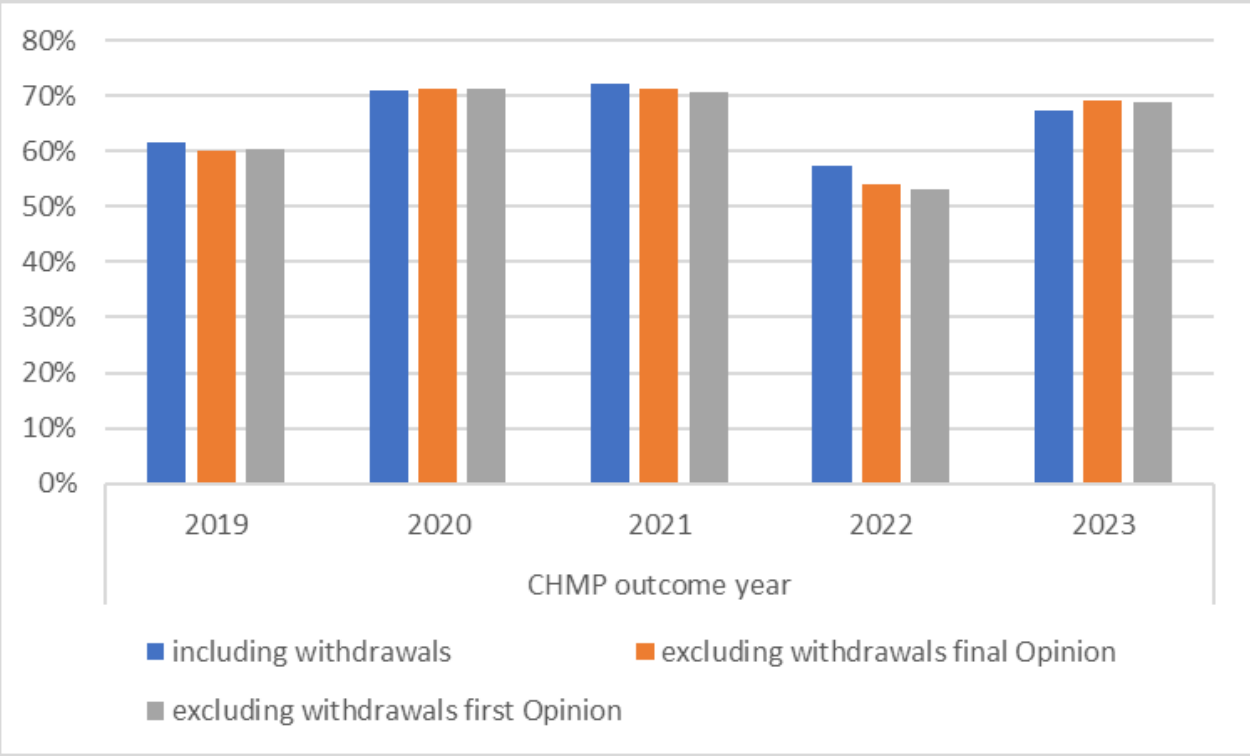
Presented by Iordanis Gravanis and Kevin Cunningham on 4 December 2023
Scientific Advice Office, Evidence Generation Department, Human Medicines Division, EMA

Scientific advice volumes and updates

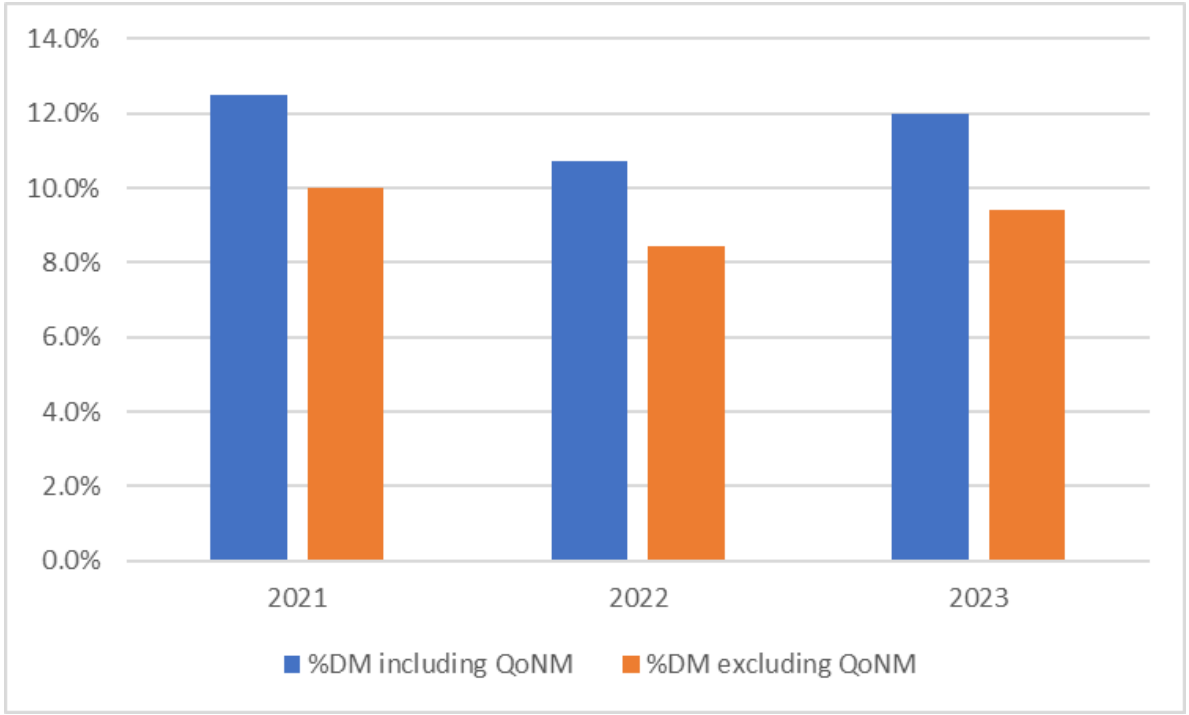
Scientific advice submission volumes in 2023



Percentage of MAA outcomes with prior scientific advice



Percentage of scientific advice cases with Discussion Meeting



- Nov2023 procedure starts excluded
- ETF cases excluded
- data before 2021 currently unavailable

Scientific advice on paediatric developments

REGULATION (EC) No 1901/2006 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

of 12 December 2006

on medicinal products for paediatric use and amending Regulation (EEC) No 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004

Whereas:

(15) Free scientific advice should be provided by the Agency as an incentive to sponsors developing medicinal products for the paediatric population. To ensure scientific consistency, the Agency should manage the interface between the Paediatric Committee and the Scientific Advice Working Group of the Committee for Medicinal Products for Human Use, as well as the interaction between the Paediatric Committee and the other Community committees and working groups concerning medicinal products.

Article 26

Any legal or natural person developing a medicinal product intended for paediatric use may, prior to the submission of a paediatric investigation plan and during its implementation, request advice from the Agency on the design and conduct of the various tests and studies necessary to demonstrate the quality, safety and efficacy of the medicinal product in the paediatric population in accordance with Article 57(1)(n) of Regulation (EC) No 726/2004.

In addition, this legal or natural person may request advice on the design and conduct of pharmacovigilance and risk management systems as referred to in Article 34.

The Agency shall provide advice under this Article free of charge.

Acceptable and non-acceptable paediatric scientific advice questions

Does the CHMP agree that a paediatric formulation is (not) needed? 

Does the CHMP agree on the development plan of the intended paediatric formulation? 

Does the CHMP agree that juvenile animal studies are (not) needed? 

Does the CHMP agree on the design of the proposed animal studies? 

Does the CHMP agree that studies in children <2 years are not needed/can be waived? 

Does the CHMP agree on the design of paediatric study XYZ? 

Does the CHMP agree that the planned paediatric development plan can support an indication in children (above X months/Y years of age)? 

Implementation of pilot initiatives based on first 5 years' experience of PRIME: experience to date

PRIME pilot initiatives: Experience to date

- Experience of 4 **Expedited Scientific Advice requests**:

Request	Scope	Question(s)	Expedited criteria met	Outcome	Reason	Conclusion day	Submission to outcome
1	Clinical	Statistical analysis/statistical methods	yes	Expedited SA		Day 34	6 weeks
2	Clinical	Statistical analysis/statistical methods	Yes	Expedited SA		Day 35	6 weeks
3	Quality/Non-clinical	Comparability programme	Yes	Standard SA	Rapporteur team capacity to initiate review	Day 40	15 weeks (summer break)
4	Quality	Potency Assay	Yes	Expedited SA		Day 40	8 weeks

- Preliminary experience of **development tracker submissions**:
  - High level of quality, detail, comprehensiveness
  - Applicants sought PRIME team support on tracker, incorporated changes
  - Increasingly used to support KOM/SRM
- First **submission readiness meeting** held October 2023
  - High quality of submission and level of engagement from Applicant/Rapporteur
  - Discussion on data package/maturity, CMA/MAA under EC, accelerated assessment request
  - Pre-submission questions answered through standard EMA channels

PRIME pilot initiatives – Planned analyses

- Pilots Launched March 2023 and will conclude at **18 month (Sept 2024)** or **24 month (March 2025)** depending on experience gained
- Planned analyses based on IRIS submissions**:**
  - number of **expedited SA requests**, scope, duration, outcome (clarification/expedited/standard),
  - number of **development trackers** submitted, number of updates submitted, metrics compared to previous annual update.
  - number of **SRM**, number of subsequent pre-sub meetings held, analysis of AA outcome and maintenance, MAA duration and outcome
- Questionnaire to PRIME product developers/Rapporteurs/regulators at conclusion of pilot:
  - Experience of the **expedited SA** procedure, effectiveness
  - Company experience populating/maintaining the **development tracker**, user-friendliness, effectiveness to support internal processes, and EMA PRIME meetings and interactions
  - effectiveness of **SRM** (strengthened engagement, identification of outstanding issues, AA/MAA preparedness), utility of the meeting versus pre-submission activities
- EMA will consult industry on survey content and approach through PRIME contact points in 2024