Updates from EMA on scientific advice and PRIME

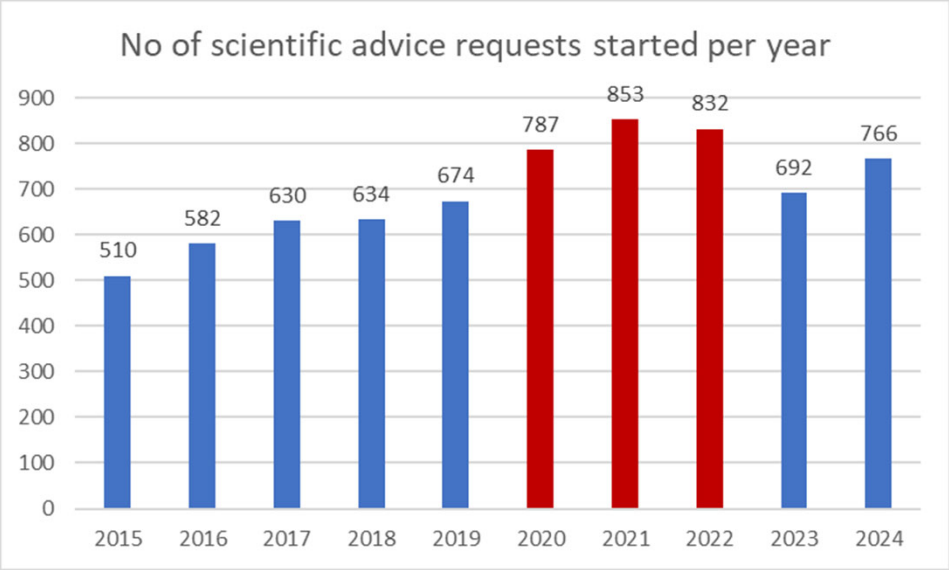
13th Industry stakeholder platform on research and development support

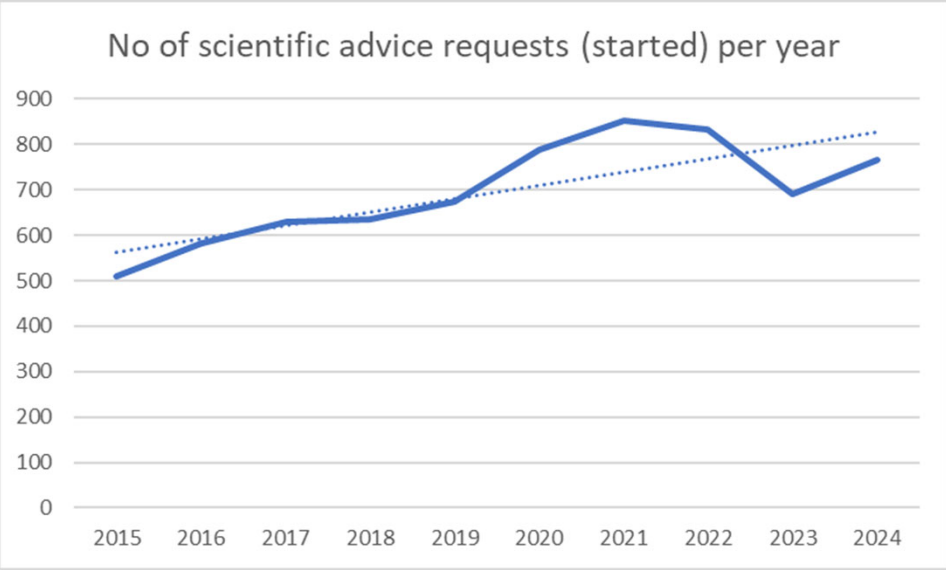
Presented by Iordanis Gravanis on 2 December 2024
Scientific Advice Office, Evidence Generation Department, Human Medicines Division, EMA

Outline

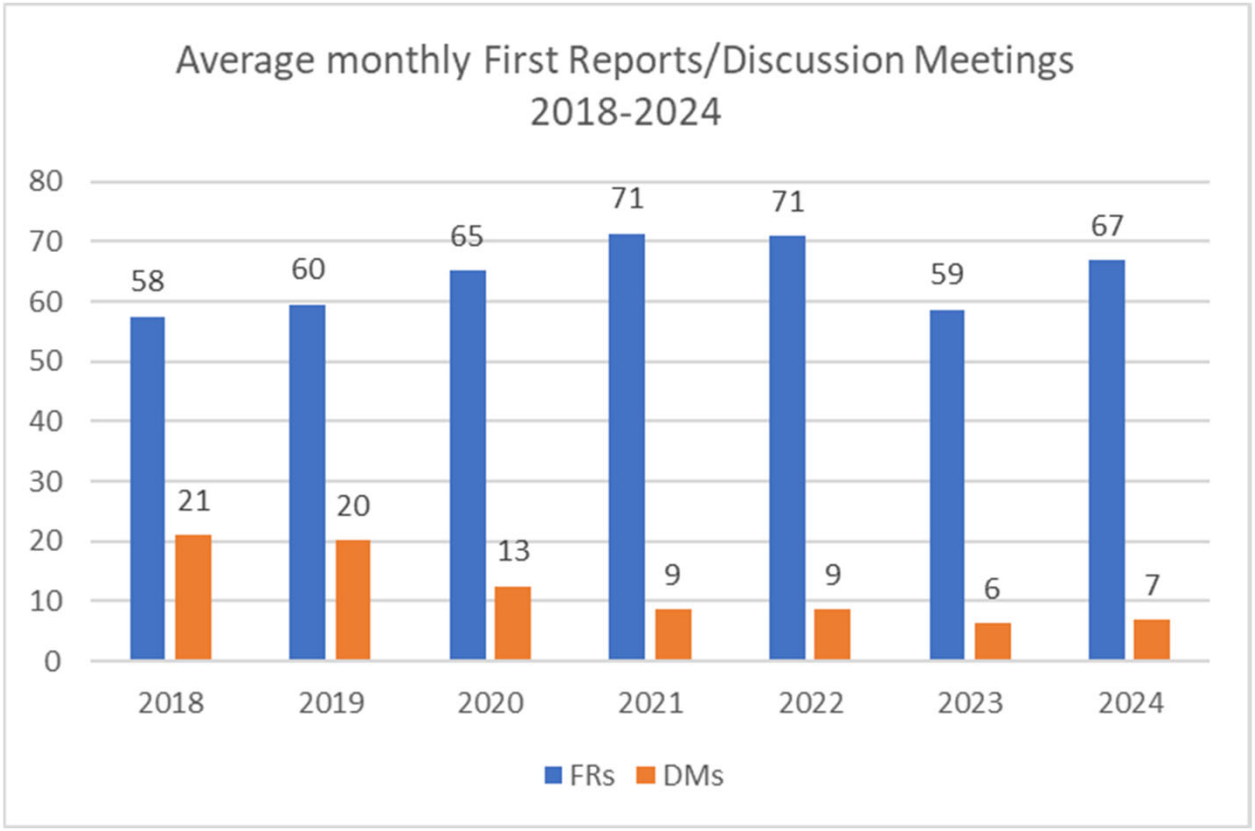
- 2024 scientific advice volumes
- Discussion meetings
- Experience with the SAWP/CTCG scientific advice pilot
- 2024 PRIME volumes and progress with pilots
- Progress with the Action Plan on future-proofing the Qualification of Novel Methodologies
- Update on activities related to combination products

2024 scientific advice volumes





Discussion meeting volumes in recent years

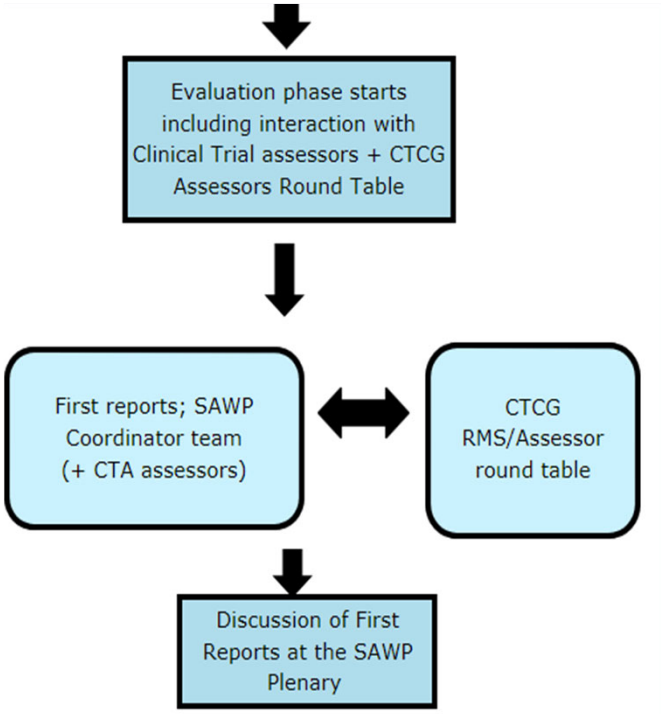


Update on proposals for improving discussion meetings (DMs)

- DMs provide opportunity for more interactive exchange and clarifications of discussion points **but they should be used efficiently**
- DMs focused on a small number of important issues may allow a shorter duration and allow more DMs to be held; however, the focus should be on meeting efficiency with shorter duration, **where possible,** being a by-product
- A default 1mo extension of the procedure with a draft D40 FAL **is no longer pursued**, but the option is being explored for a quick written exchange (short LoI to be responded to in writing initially) towards a tentative D70 DM which may finally not materialise in many cases **(no impact on current procedural timelines)**: this may help increase the number of DMs

SAWP-CTCG pilot: experience to date

- The SAWP/CTCG pilot gives the advantage of having the scientific views of the SAWP/CHMP and NCAs relevant to the planned CT(s) on the CT design in a single procedure
- 2 Procedures: 1 Concluded / 1 Ongoing
- First procedure was successfully concluded in accordance with the SAWP timeline
- No additional fees are requested for the involvement of the CTCG in the assessment



Updated guidance with clarification to be published shortly

Early feedback from applicants

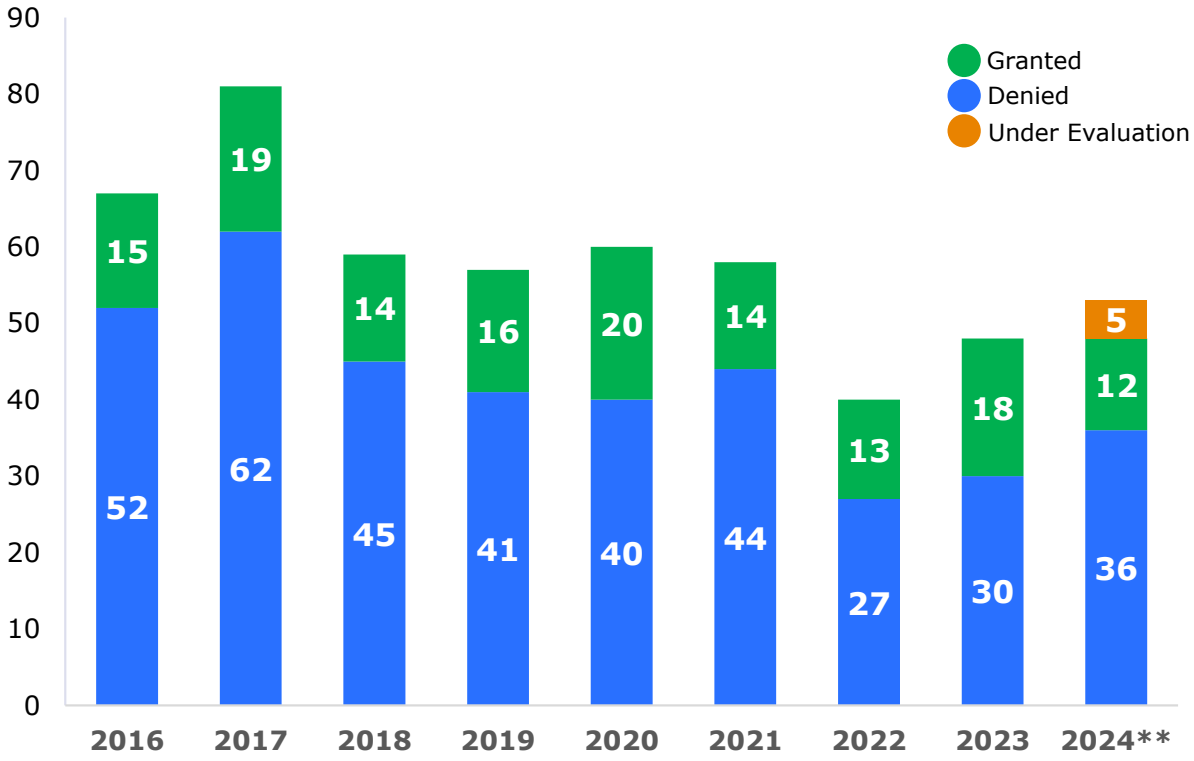
'It is helpful to be able to get feedback on issues that could be raised at the time of CTA review and address them prior to submission of the CTA. This should hopefully cut down the number of issues raised during the CTA procedure and make the 10-day RFI response period easier to manage and less likely to withdraw application'

'It is much easier to manage one procedure rather than EMA and separate national advice in parallel; it is less resource intensive'

'..this pilot process provides more reassurance that we can address potential issues up front to cut down on work during the start up period of the study when it is very busy for the team. The timelines from national agencies can depend on workload so it is good to know the timelines will be adhered to going via SAWP route'

'This was valuable for Phase 3 study as there is a longer start up period for Phase 3 so advice is timely'

PRIME applications: 2024 overview



53 Application outcomes 2024

- Applications increase 10% vs 2023
- Decrease in granted products vs 2023
- ATMPs:
  - 30% of applications
  - 50% of granted products
- Orphan medicines:
  - 26% of applications
  - 25% of granted products
- SME:
  - 52% of applications
  - 50% of granted

**5 Outcomes pending December 2024

PRIME pilot initiatives: Experience to date

12 **Expedited Scientific Advice requests**:

No.	Scope	Question(s)	Conclusion day	Submission to outcome	Comment
1	Clinical	Statistical aspects	Day 34	6 weeks	
2	Clinical	Statistical aspects	Day 35	6 weeks	
3	Quality/Non-clinical	Comparability programme	Day 40	15 weeks (summer break)	Standard timeline: Rapporteur team capacity to initiate review
4	Quality	Potency Assay	Day 40	7 weeks	
5	Clinical	CMA, Statistical aspects	Day 40	7 weeks	
6	Quality	Manuf. process validation	Day 40	7 weeks	
7	Quality/Clinical	Comparability programme	Day 36	7 weeks	Start postponed at Sponsor request
8	Clinical	Interim analysis	Day 67	18 weeks	Standard tlmeline: Reverted to allow Discussion Meeting
9	Non-clinical/ Clinical	Choice of endpoint, CMA	Day 40	12 weeks	
10	Clinical	Choice of endpoint	Day 37	11 weeks	
11	Quality/Non-clinical	Analytical methods, comparability/Non-clin package	TBC	TBC	
12	Clinical	Choice of endpoint; population	TBC	TBC	

57 **development tracker submissions** until Nov 2024:

- 31 periodic updates; 20 KOM submissions, 6 SRM submissions
- Increasing prominence in KOM and SRM preparatory discussions and meeting

6 **submission readiness meetings** held until Nov 2024 (3 more in planning until January 2025)

- High quality of submission
- Key Discussion topics: Data maturity to support (c)MA, GMP/inspections, quality data package to support (c)MA
- EMA PRIME team coordinating with MAA Forecasting

PRIME pilot initiatives – Planned analyses

- Pilots Launched March 2023 and will conclude at **24 months (March 2025)**
- Planned analyses based on IRIS submissions**:**
  - number of **expedited SA requests**, scope, duration, outcome (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
- Focused 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), impact of the meeting on subsequent pre-submission activities and MAA preparation
- EMA have initiated preparations with industry sounding board on survey content and approach in November – 2 scoping meetings to date

EMA Development Support Coordinator for PRIME products

- The Development Support Coordinator (DSC) will replace the PRIME scientific coordinator as primary point of contact for PRIME products
- Having relevant therapeutic area expertise, the DSC will handle scientific advice requests and ensure adequate coordination with other development support regulatory interactions
- Establishment of the role follows the principle of stewardship (also called 'lighthouse' or 'one-stop-shop') agreed in conclusion to the 2019-20 Focus Group on integrated development support
- The role will initially be piloted with a handful of EMA staff and a subset of PRIME products

Progress with the Action Plan on future-proofing the Qualification of Novel Methodologies

- The EMA published its Action Plan on future-proofing the Qualification of Novel Methodologies (QoNM) in early September 2024
- The QoNM core group has been meeting monthly since then
- Core group discussions have focused on lifecycle management, checklist/briefing document template for qualification of registries and possible procedural adaptations
- Templates and updates of the public guidance are being drafted
- Work is progressing according to the action plan

Update on activities related to combination products

- Scientific publication on the use cases identified by the 2023 Focus Group on combination products is nearing its submission to a journal
- Scientific advice questions under the remit of EMA/CHMP continue to be submitted
- Overlap with medical device expert panels is minimal/rare
- EMA continues to support the COMBINE programme as part of the programme group, and with involvement in 3-4 cross-sector projects

Let’s discuss!