EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Stepwise PIP

Review of the stepwise PIP pilot

13th R&D industry stakeholder platform

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An agency of the European Union

Stepwise PIP pilot

• In general, it is expected that all **PIP measures** can be agreed upon at the time of the **initial PIP application**

• For the rare cases when **crucial data are not yet available** to sufficiently define the key elements (KEs) of the planned measure at the time of the initial PIP application → **stepwise PIP approach** → **Opinion on a plan not yet fully defined**

• **KEs that cannot be defined → data required for defining them and by when**

• **Minimum set of data** (condition, age subsets, preliminary outline of planned studies and completion date) required

• The sPIP will be modified when relevant data available as agreed in milestones → **fully developed PIP at the end** (same details as for conventional PIP)

• **Paediatric development must not be delayed**

Requests

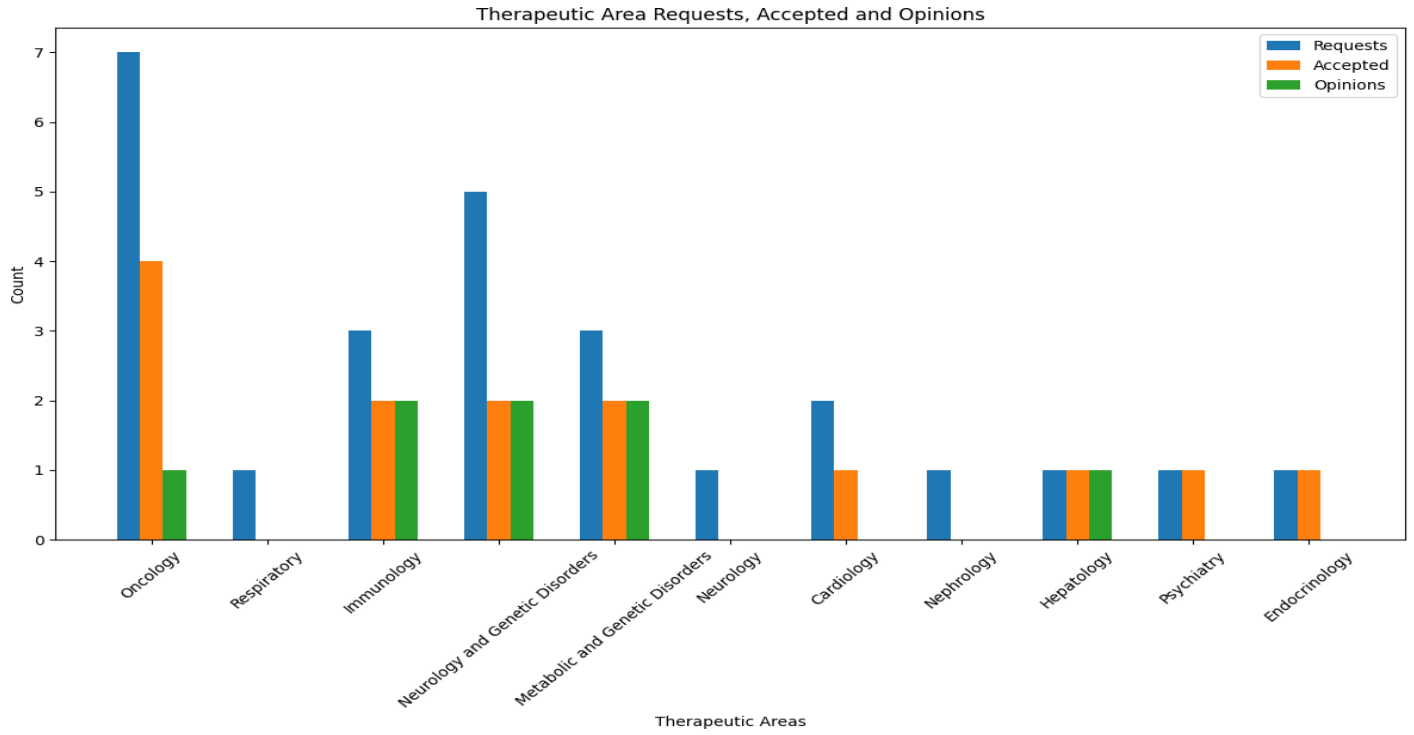
Number of requests: 28 (no information on therapeutic area for 2 requests)

Submitted sPIPs: 15

Refused during PDCO discussion: 1

Opinions adopted: 7 (and 1 in January 2025)

Therapeutic area



Reasons for rejection

- Ongoing PIP
- Previous PIP for the same active
- After the pre-submission meeting or discussion at the PDCO plenary it became clear how the development programme could be defined
- Too many requests in the same therapeutic area (for the pilot)

sPIP process timelines

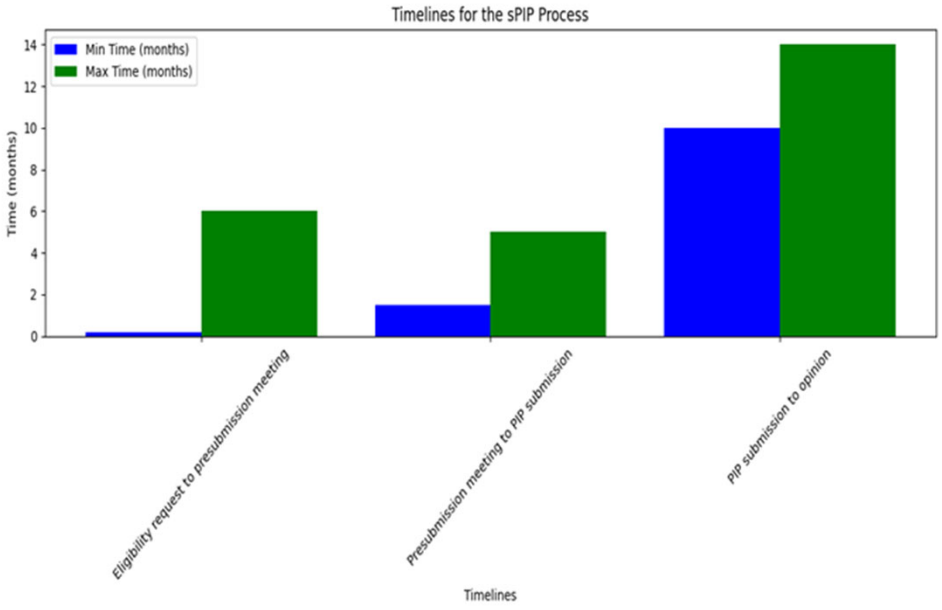
Number of pre-submission meetings: 10

Eligibility request to presubmission meeting: 5 days to 6 months (median 1.5 months)

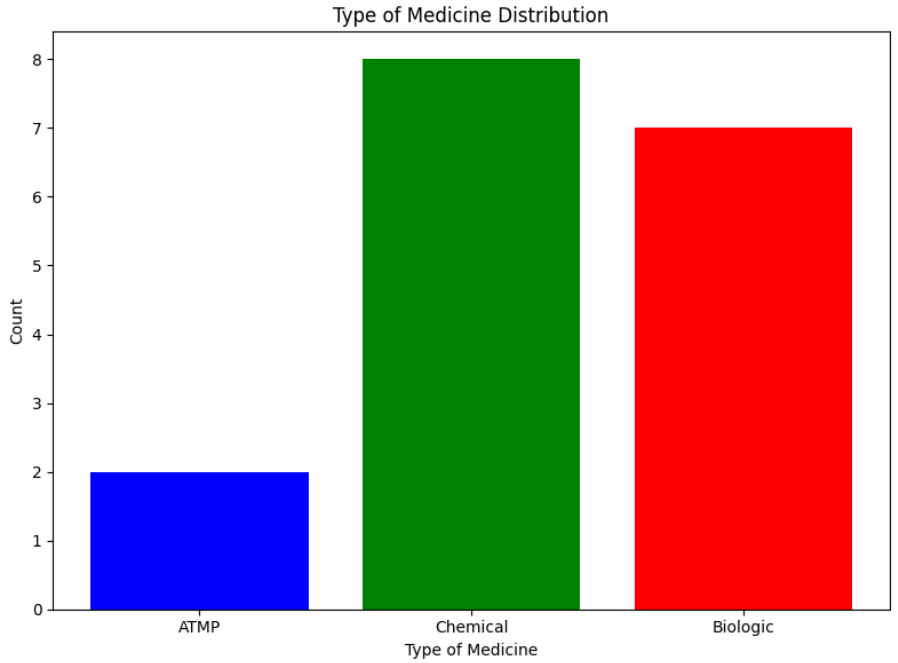
Presubmission meeting to PIP submission: 1.5 months to 5 months

PIP submission to opinion: 10 months to 14 months

PIP completion: 2029 to 2035

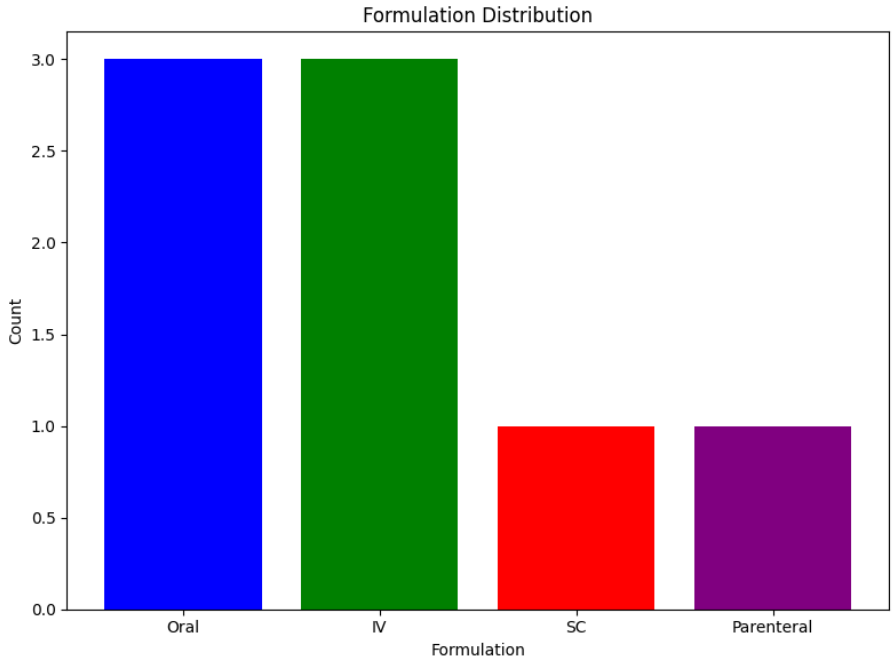


Type of medicine



Quality

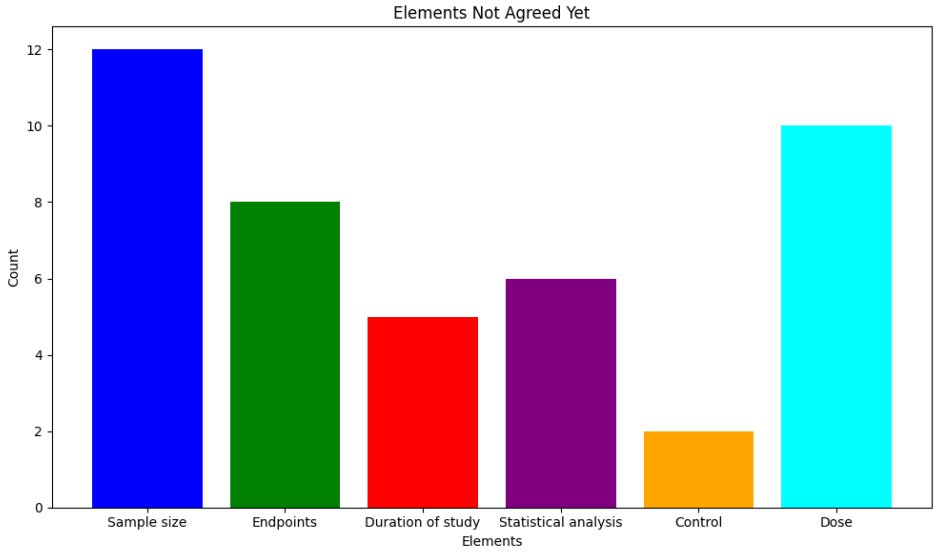
A quality study was included in 6 of the 8 agreed sPIPs



Clinical studies

Number of Clinical Studies

- **Per sPIP:** 2 to 4
- **Total studies:** 21
- **Elements not defined yet:** most frequently sample size and dose



Additional elements

Non-clinical studies:

- Included in 5 of 8 agreed PIPs
- Critical for go/no-go decision: 1

Condition: predominantly paediatric in 6 of the agreed sPIPs

Waivers: no waivers in 5 of the agreed sPIPs

Conclusion

- pilot well received by PME and PDCO
- pre-submission meetings useful to guide applicants through the process
- most requests in rare genetic diseases (metabolic and neurological) and oncology
- sPIP intended for situations when many elements cannot be defined (e.g a study in a paediatric subset)
- principles of sPIP applied to 'conventional' PIPs (e.g. sample size, dose)

Next steps

- Conclusion of pilot and moving to established procedure
- Discussion and agreement by PDCO
- Update of guidance
- Facilitate process

Any questions?

Further information

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