European Commission expert group on Safe and Timely Access to Medicines for Patients (STAMP)
- update -

Health Technology Assessment Network
20 May 2016

Presented by Helen Lee
Unit B5 – Medicines: policy, authorisation and monitoring
Directorate General for Health and Food Safety

Health and Food Safety

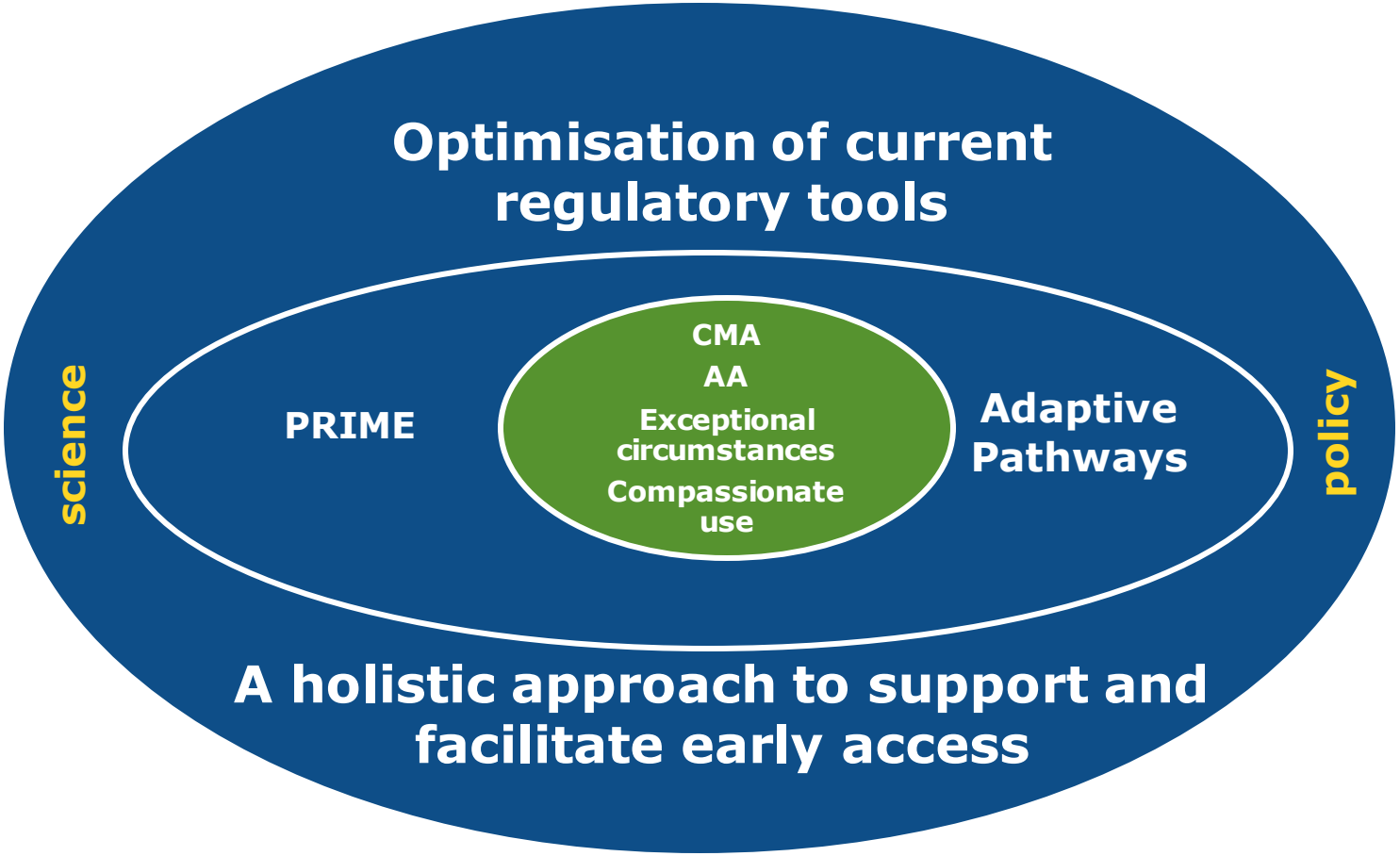
European Commission

STAMP discussions

- Experiences from national schemes for early patient access to innovative medicines
- Optimisation of existing regulatory tools for early access
  - → **Conditional marketing authorisations (CMA)**
  - → **Accelerated assessment procedure and PRIME**
  - → **EMA's adaptive pathways pilot project**
- Repurposing of established medicines
- Real world evidence
- Compassionate use
- Personalised medicine

European Commission

Bigger picture



Conditional Marketing Authorisation

Health and
Food Safety

European Commission

Conditional marketing authorisation Discussion at STAMP

- **Change negative perception:**

✓ Prospectively planned CMA vs reactive CMA ('rescue solution')

✓ early dialogue including with HTA bodies (an opportunity ?)

✓ Reassurance for post-authorisation phase:

- **Feasibility of Specific Obligations (SOs) at the time of imposition**
- **Regulatory actions** to be taken in case of delays/negative outcome of SOs
- **Streamlining annual renewals** with Periodic safety update reports (PSUR) assessments, rather than requiring (re) submission of PSUR data

Key changes to CHMP Guideline on CMA

- Encouragement of early dialogue and **prospective planning**
- '**Positive benefit-risk balance**' vs. comprehensive dossier
- Scope of CMA to cover serious debilitation and life-threatening effects also in the **long-term** *
- Exceptionally, **improvements in patient care** as a possible major therapeutic advantage *
- Guidance on situations when a **second product** can still address the same unmet medical need
- Confirmation of **significant benefit for orphan medicinal products**
- Clarifications on some further aspects (e.g. compatibility with **accelerated assessment**)

Slide courtesy of the European Medicines Agency

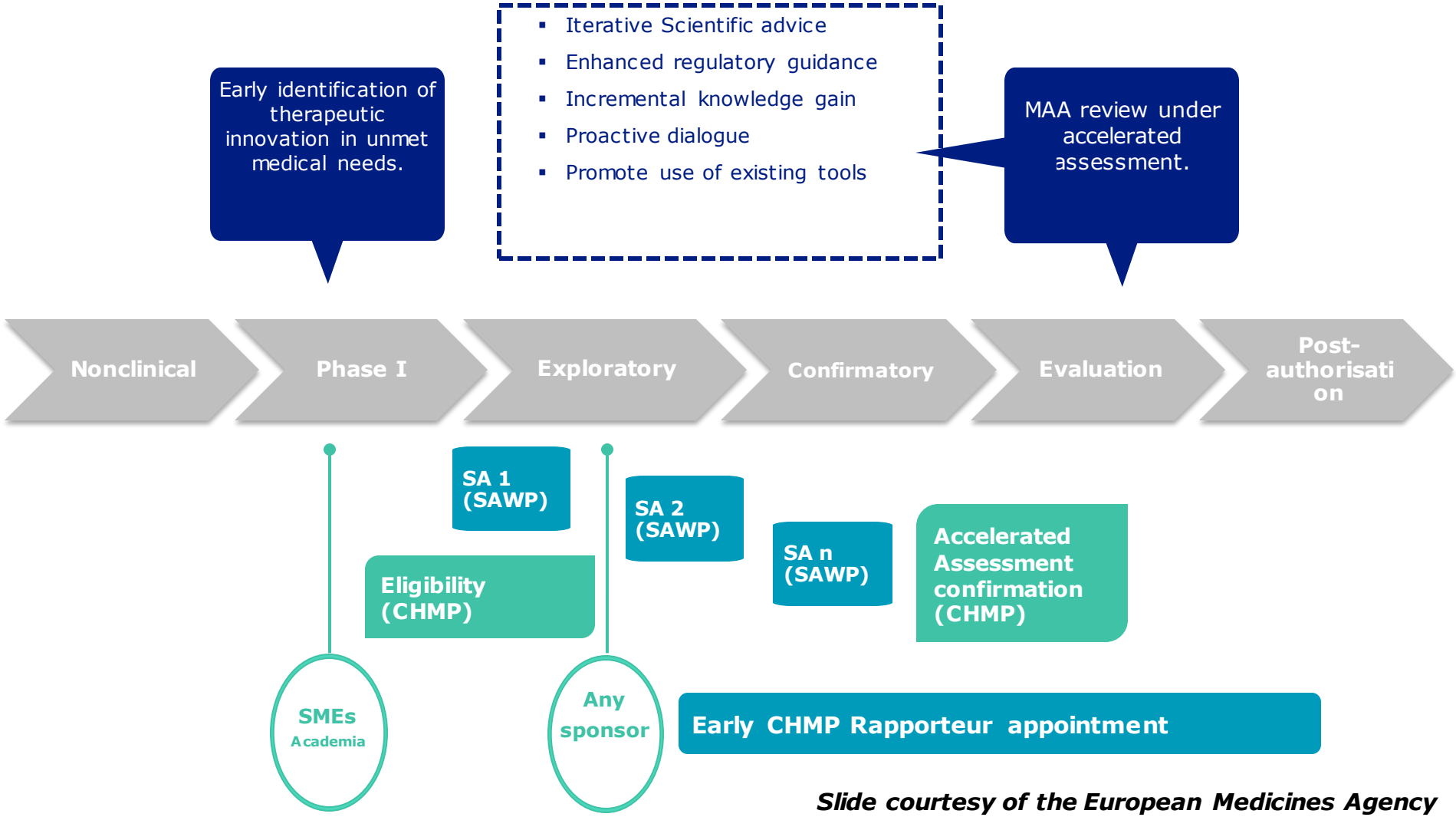
European Commission

PRIME – Priority Medicines

- Intended for innovative medicines with compelling evidence to fulfil unmet medical need

Health and Food Safety

Overview of PRIME scheme



Slide courtesy of the European Medicines Agency

European Commission

EMA's pilot project on Adaptive Pathways

Health and Food Safety

European Commission

EMA questionnaire on adaptive pathways
replies from Member State representatives

- other stakeholders need to be involved, for planning and implementation
- product prioritisation - Who should select the products?
- meaning of “need” (clinical, public health, cost reduction?)
- entry and exit schemes
- joint guideline development
- prescription controls
- feasibility of post-authorisation data acquisition
- making the most use of available data, access to other stakeholders

European Commission

Presidency expert meeting 1 -2 March:

- positive interaction between different experts
- demand for better involvement of whole “chain” from beginning
- upstream: already quite a lot of exchange of information between marketing authorisation and HTA bodies
- downstream: complex as different health systems
- opportunities: common problems, alignment of patient

European Commission

Discussion on Real World Evidence

- Examples of collection of real world data presented
- Access to and use of data
- Link to adaptive pathways pilot

STAMP meeting 10 March 2016

- Repurposing of established medicines/active substances
- Compassionate use
- Personalised medicine

European Commission

Thank you for your attention

More information:

http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp/index_en.htm

Health and Food Safety