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SCIENCE MEDICINES HEALTH

Adaptive Pathways – Learnings from pilot

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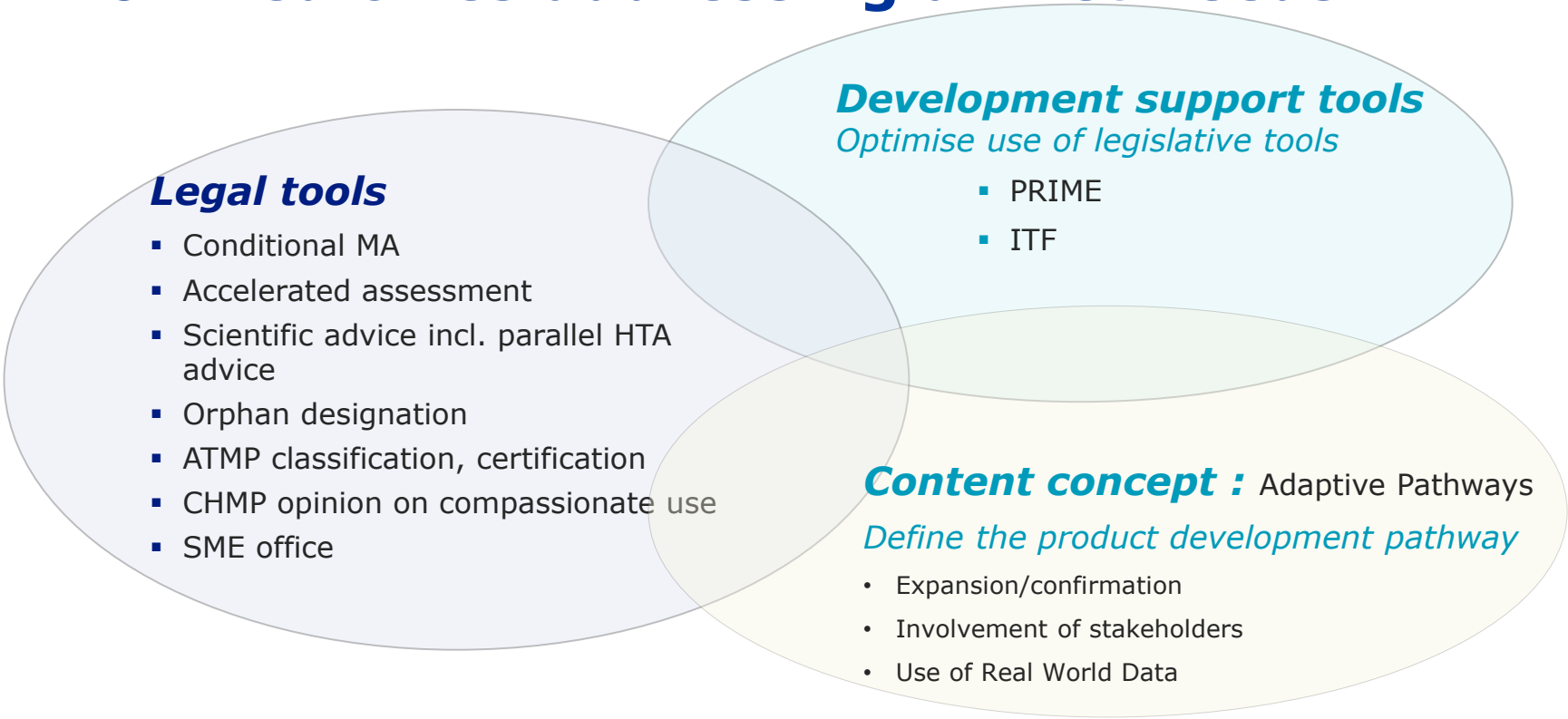
EMA development support and early access for medicines addressing unmet needs



Real World Data–making the best use of all information

RWD are viewed in contrasting ways, often in the same publication:

Case reports: a reliable source of information on safety

Well planned registry: unreliable to investigate effectiveness.

Methodological challenge to RWD acceptability for decision making.

RWD should:

- **Address justified uncertainties** emerging during the evaluation.
- **Confirm long term effects** if initial approval is based on early or surrogate endpoints

Already done-could be improved! (prospectively+optimised)

RWD can offer a **monitoring** advantage (capture real clinical practice, rare, long-term events, useful for geriatrics and paediatrics, to validate biomarkers, personalised medicine)

Learnings (1)

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To realise the benefit and smooth the road to access:

- A **prospective, life-span** discussion of product development with different stakeholders is possible and desirable in cases where decision making could be delayed by suboptimal planning.
- **Product selection** vs limited resources. Selection criteria and meaning of "need": clinical, public health, cost reduction(?).
- Increase **patient participation** (product selection, risk management, feasibility, ethical aspects, support enrolment in trials and registries).
- Making the most use of available RWD data, feedback/access to other stakeholders for their decision making.
- Prescription controls are important (STAMP discussion March'16)

Learnings (2)

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- Input in peri-approval advice,
- Choose clear-cut, **actionable** endpoints for decision making (for B/R, value, pricing)
- Joint guideline development with HTAs to streamline requirements
- Design of entry and exit schemes and data gathering for pricing commensurate to performance can only be answered with payer's input on feasibility/desirability (NB no price discussion!!).
- **Trust** is important (in safe harbour and in capability to conduct the studies).
- Confidentiality of discussions is part of the normal SA process.
- **Resource** intensive procedure: felt particularly by HTAs. Challenge to bring right stakeholders with right expertise into the discussion.

Proposed next steps (report soon finalised)

To make the process sustainable and utilise a well-tested and established framework , future submissions will be treated as parallel HTA/SA advice requests, granting an additional presubmission meeting to discuss the early draft:

- Established framework for patient participation
- More sustainable HTA input
- Publication of statistics and report annually as for other SA
- Two additional presubmissions for SMEs
- Other stakeholders (payers, FDA, WHO) may be invited where relevant

Pilot will be closing with publication of the report

- Pilot planned to close when 6 parallel SA/HTA advices were given (May 16)
- Report incorporates discussions and feedback from different sources (experience from pilot , STAMP, Dutch presidency meetings, Council Conclusions in June 2016, ADAPT-SMART, publications in scientific journals…)
- Applications continue to be accepted in the framework of parallel HTA/SA – statistics to be published as per Scientific Advice.