EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Adaptive Pathways:
Can we build better links between decision makers?

Francesca Cerreta
EMA – Scientific Advice

Why adaptive pathways?

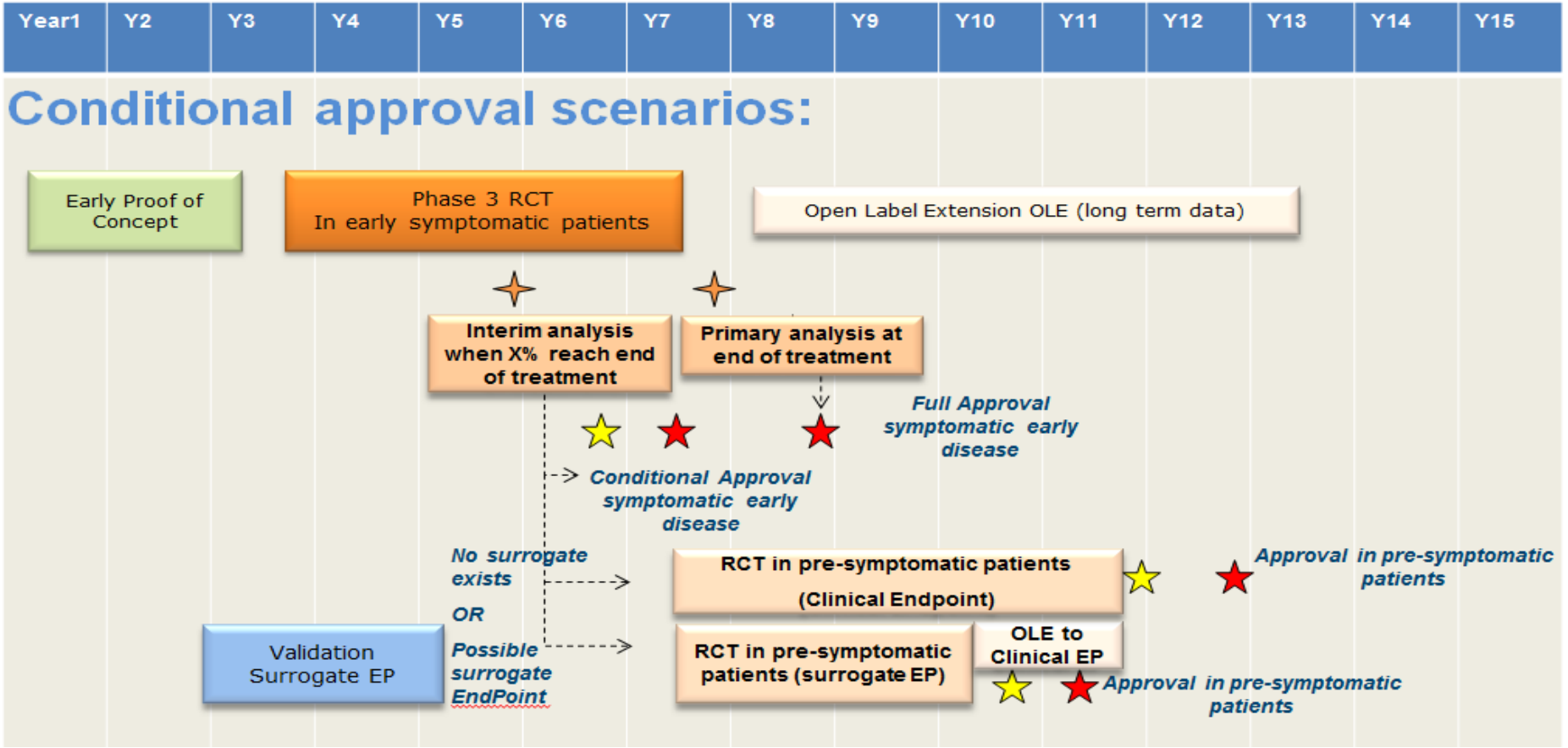
Adaptive pathways offers the opportunity to avoid a situation where a conditional MA is granted but a decision on value and reimbursement cannot be reached without collection of additional data. It is not for all medicines:

Iteration – RWD – Downstream decision makers

Real-world data used to **complement** RCTs in cases where conduct of trials is difficult

Standards for regulatory decision making remain the **same**: the amount of uncertainty acceptable in a marketing authorisation decision correlates to the degree of unmet medical need in the target population

An example of adaptive pathways development



Pilot Learnings (the glass half full)

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- AP was a learning exercise with wide acceptance criteria
- The adaptive approach can take place within the existing regulatory tools and processes.
- 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.
- Choose clear-cut, methodologically reliable, **actionable** endpoints for decision making (for B/R, value, pricing)
- There is **added benefit in well-planned post authorisation** activities.
- Input in peri-approval advice should be explored
- **Trust** is important (in safe harbour and in capability to conduct the studies).

Pilot Learnings (the glass half empty)

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- **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, 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!!).
- **Resource** intensive procedure: felt particularly by HTAs. Challenge to bring right stakeholders with right expertise into the discussion.

Next steps (1): Integration in Scientific Advice

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

Next steps (2): Consult stakeholders at workshop

Workshop 8 December to discuss with stakeholders the areas for further discussion identified in report:

1.Patients and health care professionals involvement

2. RWD methodological challenges

3 Payers and HTA conditions of participation

Topics raised by civil society will also be discussed.

Briefing book available on EMA website and workshop will be broadcast