EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Adaptive Pathways Pilot Project (formerly Adaptive Licensing)

STAMP meeting 27 January 2015- presented by Francesca Cerreta

1995 2015 EMA

An agency of the European Union

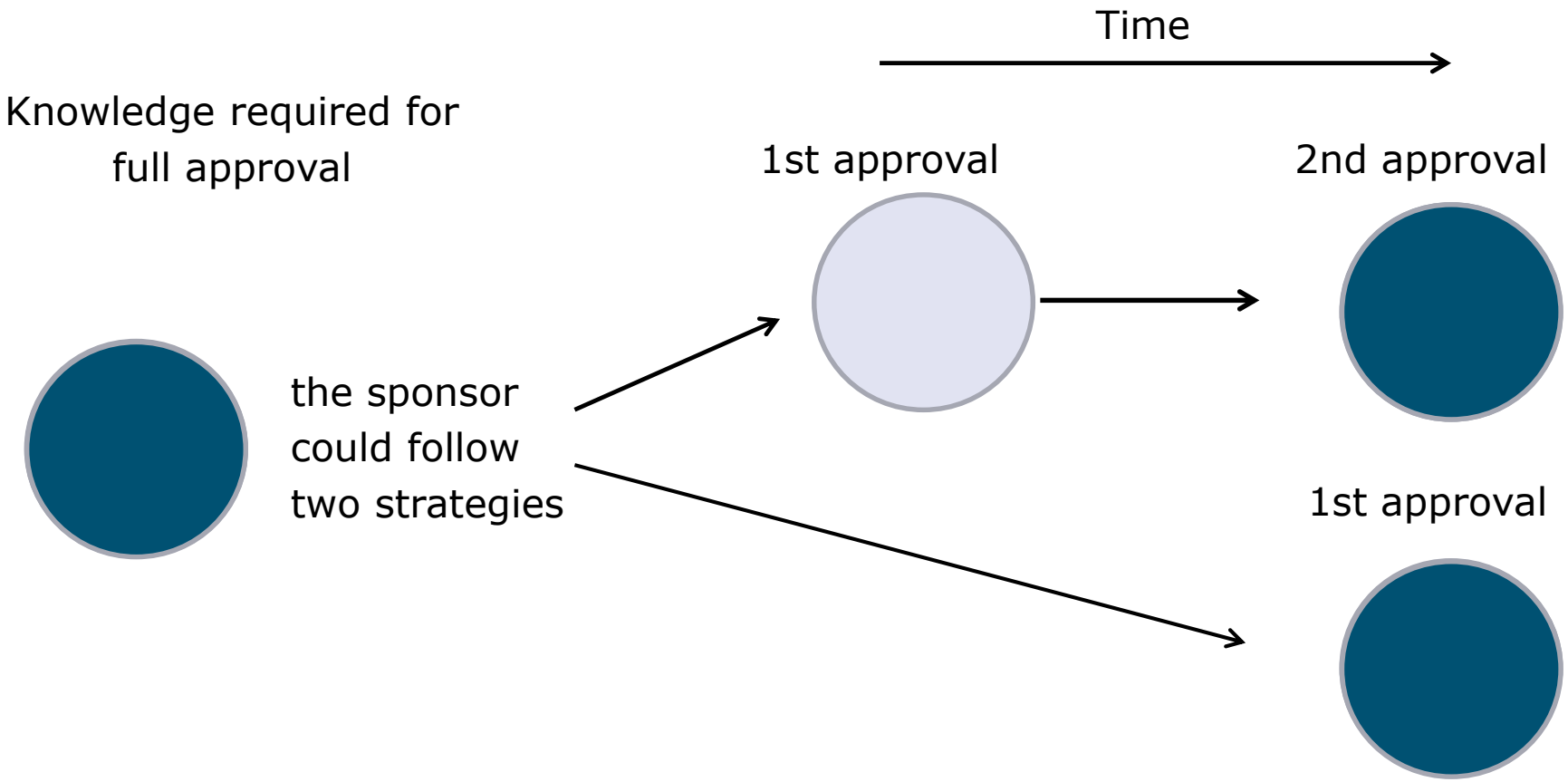
Aim

Support the selection of pathway of product development
and (potential) earlier access to medicines through early dialogue involving all stakeholders (regulators, HTAs, payers, patients, learned societies…)

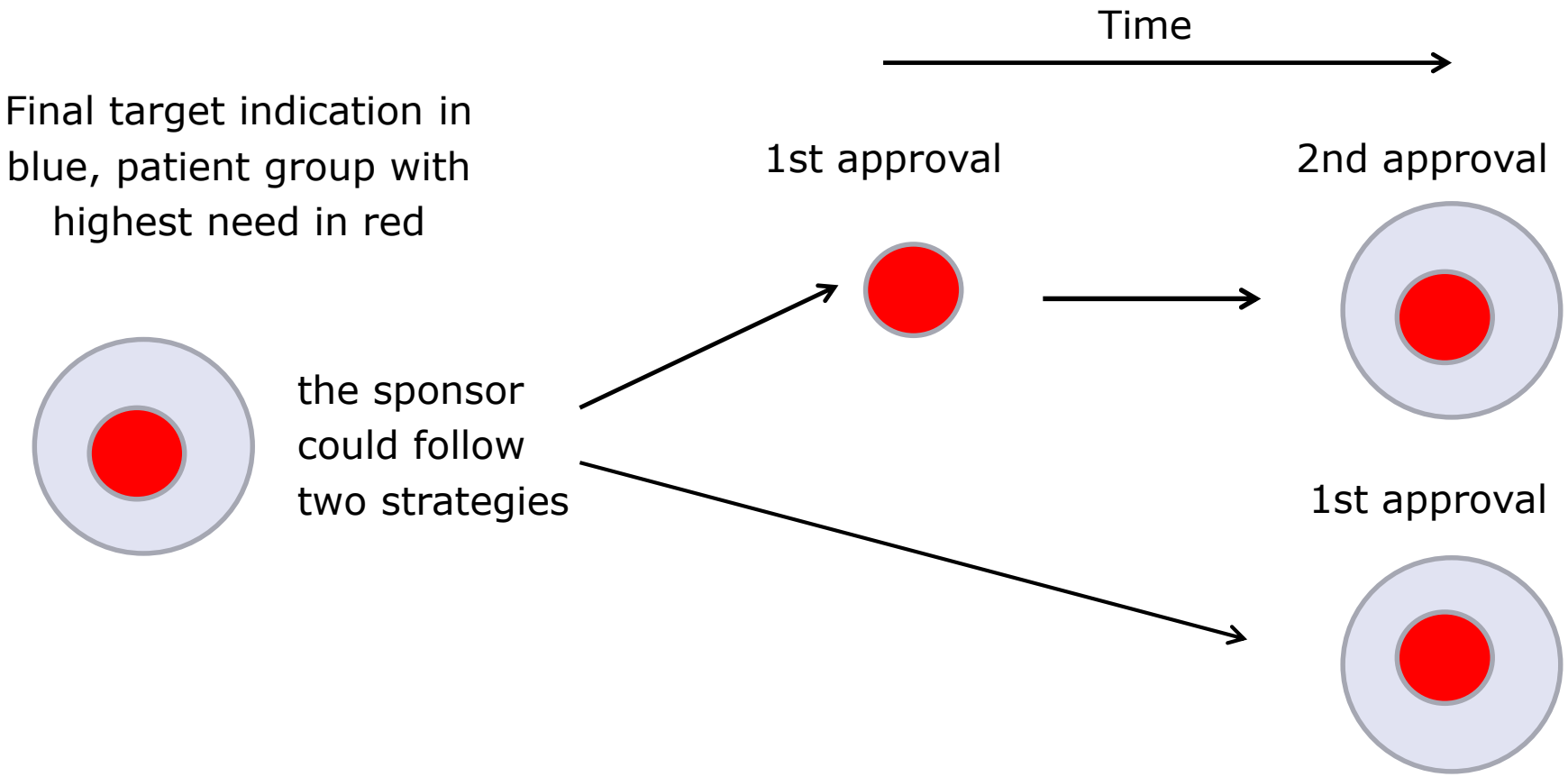
Criteria for candidate selection

1. An iterative development plan (start in a well-defined subpopulation and expand, or have a Conditional Marketing Authorisation, maybe surrogate endpoints and confirm)
2. Real World Data (safety and efficacy) can be acquired to supplement Clinical Trials
3. Input of all stakeholders, particularly HTAs, is fundamental
4. Unmet medical need

Adaptive pathways concept ("conditional approval")



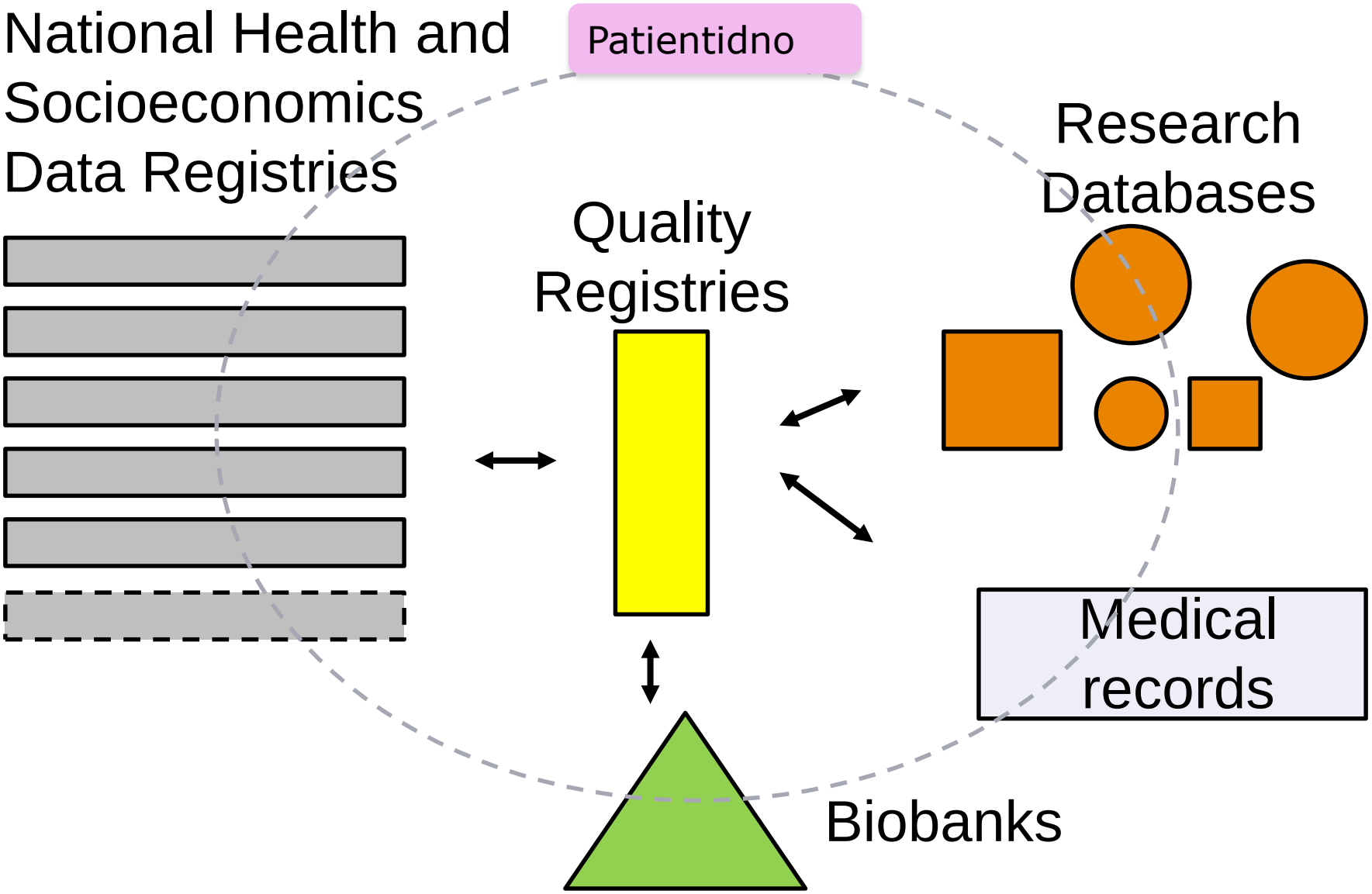
Adaptive pathways concept ("widening of the indication")



Other “rules of the game”

- Demonstration of positive Benefit/Risk will be required for approval. Involve all stakeholders to discuss how to demonstrate, and how to optimise requirements.
- Only existing regulatory tools to be used. Unmet need allows their full use.
- The discussion is a non binding, safe-harbour brainstorming. Not a new procedure, not a new approval route.
- A request for parallel EMA/HTA advice is expected to follow, to discuss science and requirements in depth, and for a formal advice letter.
- Acceptance/rejection in the AL pilot has no inference about approval potential

Potential data sources



Initial experience

- 39 products submitted as candidates
- 11 selected for in-depth discussion with company
  Of these:
  - 4 SMEs
  - 5 are Orphan drugs
  - 2 are ATMP (Advanced Therapy Medicinal Products)
- 7 stage I discussions have taken place
- 2 stage II discussions have taken place (1 product)

- Main reasons for rejection were:
- Development too advanced (too late to change anything)
- Limited learning potential for a pilot (only one iteration in terms of CMA – we may revisit some of these)

Lessons learned

- Incorporation in Scientific Advice provides optimisation of resource use and facilitates high quality input.
- AL is a lifecycle approach, involve PRAC, PDCO, COMP, CAT
- Companies should be well prepared to involve other stakeholders, particularly HTA, for a meaningful discussion
- HTA involvement at earlier stage (case product selection) would be useful
- Expectations need to be managed; perplexities to be addressed.

Suggested points for discussion at STAMP

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Feedback on merits/weaknesses of AP from the regulatory/policy point of view.

Food for thought

Compatibility of proposals with current framework

How to facilitate harmonisation/interchange between data sources

Tools to control prescription/input; partnership PRAC/HTA?

Quality of RWD/build on national experience on registries