EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

# Adaptive Licensing (AL) or …….. Adaptive Pathways Pilot project

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It's cheaper than a gastric band
and stops you using knife and fork

# AIM

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

# Criteria for candidate selection

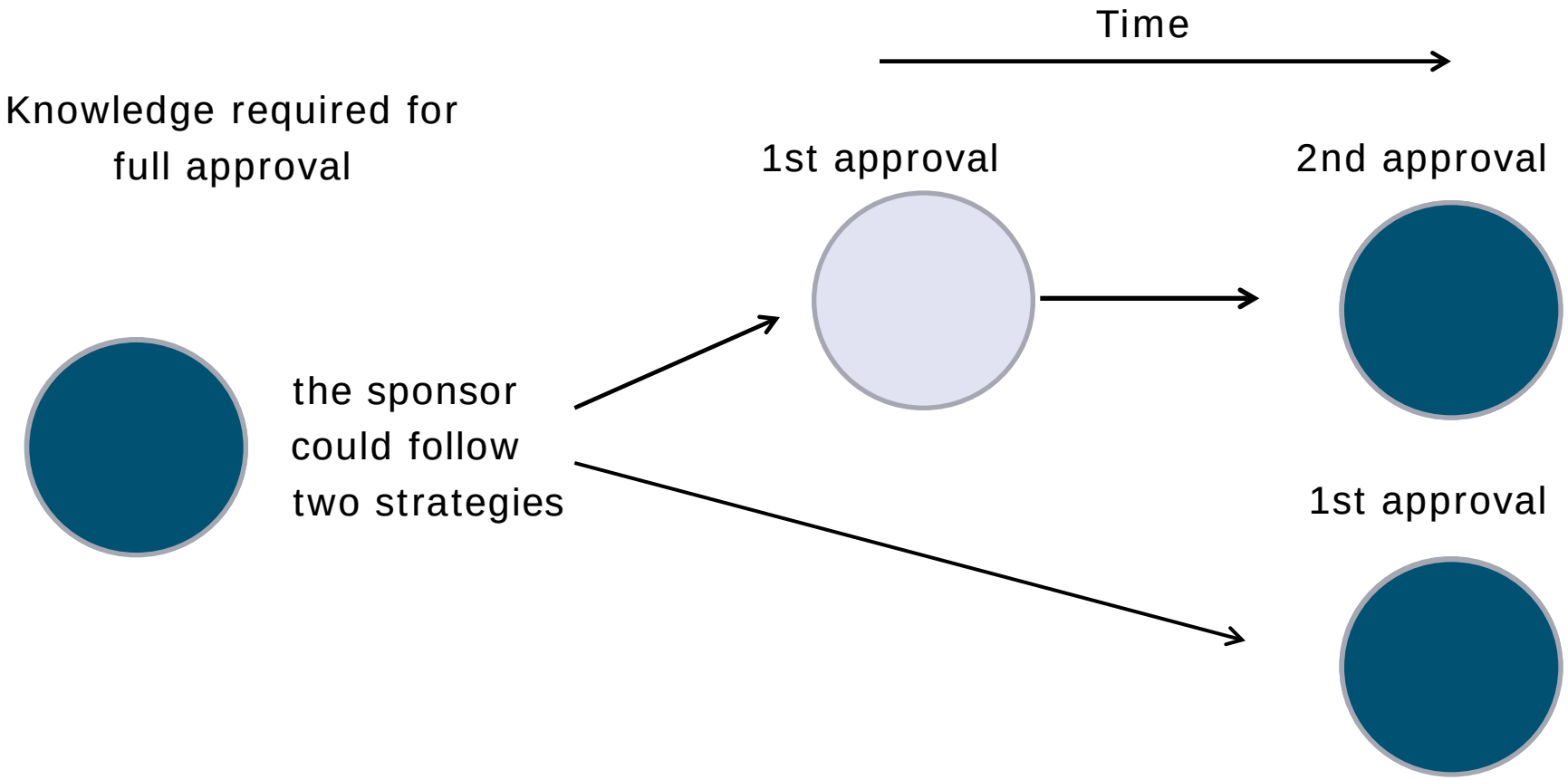
1. An iterative development plan (start in a well-defined subpopulation and expand, or have a Conditional Marketing Authorisation, maybe surrogate endpoints and confirm)

2. Real World Data (safety and efficacy) can be acquired to supplement Clinical Trials

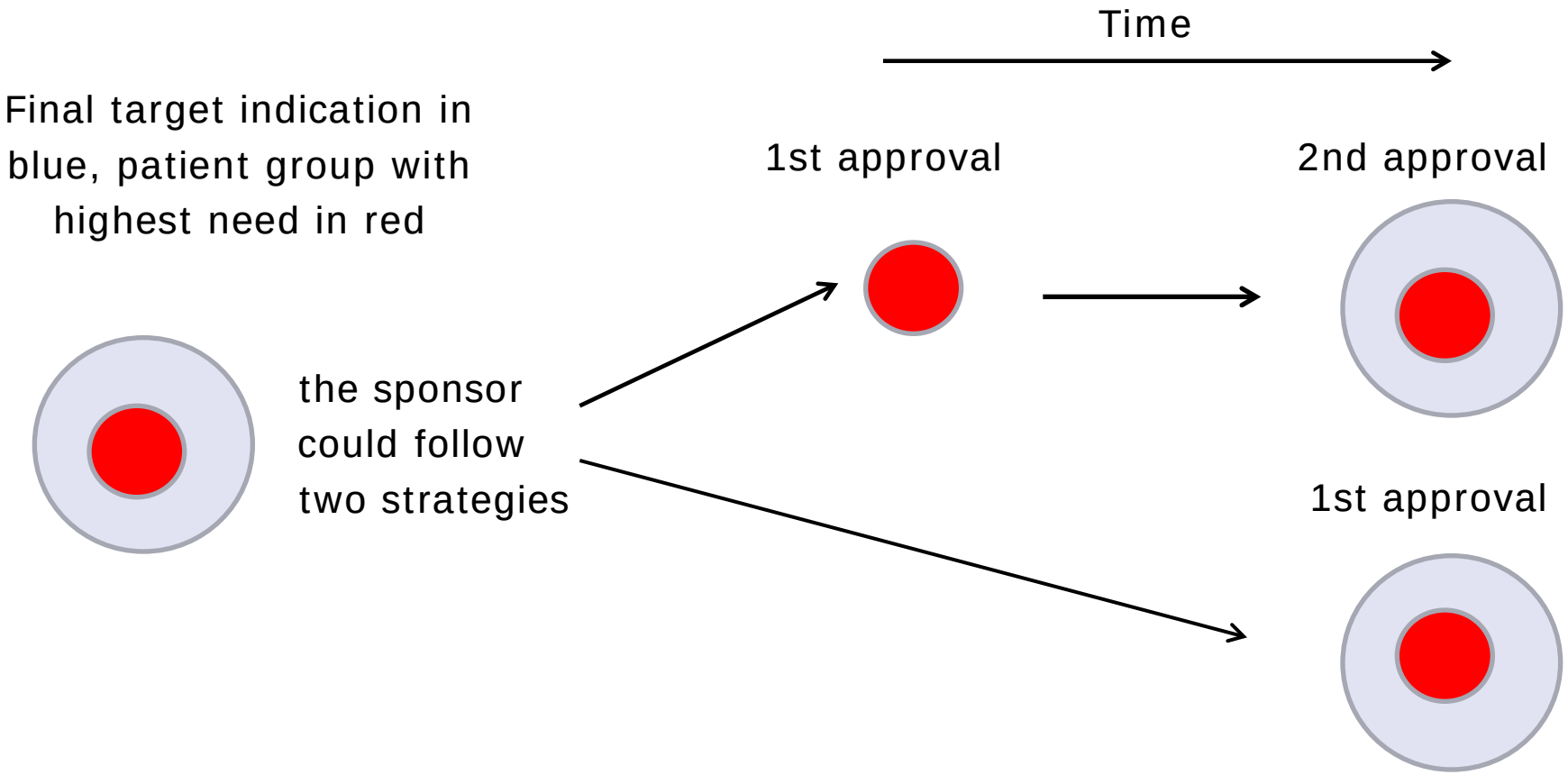
3. Input of all stakeholders, particularly HTAs, is fundamental

Unmet medical need is an important feature that allows full use of regulatory tools

# Adaptive pathways concept ("conditional approval")



# Adaptive pathways concept ("widening of the indication")



# Other “rules of the game”

- The discussion is a non binding, safe-harbour brainstorming. Not a new procedure, not a new approval route.
- Involve all stakeholders to discuss how to optimise development path and satisfy stakeholder requirements .
- Demonstration of positive Benefit/Risk is –as usual- required for approval.
- Only existing regulatory tools to be used.
- A request for parallel EMA/HTA advice is expected to follow, to discuss science and HTA requirements in depth, and for a formal advice letter.
- AL is flexible
- Acceptance/rejection in the AL pilot bears no inference about approval potential

# Initial experience

- 29 products submitted as candidates
- 9 selected for in-depth discussion with company (Step I)
- 7 discussions have taken place

Of these:

  - 9 SMEs
  - 9 are Orphan drugs
  - 5 are ATMP (Advanced Therapy Medicinal Products)

- 6 proposals selected for Step II (in-depth meeting)
- 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 have revisited 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.
- Companies should be well prepared to involve other stakeholders, particularly HTA, for a meaningful discussion
- Earlier HTA involvement would be useful (choice of candidates, prioritisations, involvement of appropriate partners)
- Expectations need to be managed; perplexities to be addressed.

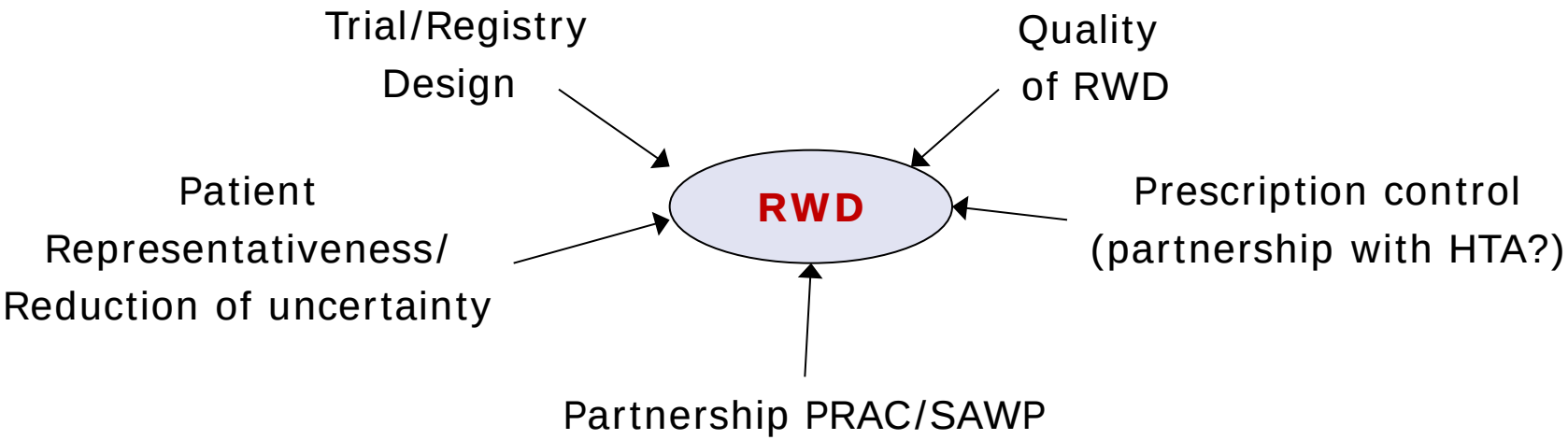
# Ongoing initiatives include….

- Interactions with EUnetHTA – including structure of EPARs, use of Effects Tables etc
- Joint advice with national HTA/payer bodies
- SEED advice with EMA
- Increased focus on post approval development
  - PhV including PASS
  - PAES
  - Specific areas eg Geriatrics
  - "Real World Data" for regulatory use (including IMI initiatives)
  - Registries
  - ENCePP survey on capacity to perform HTA studies

# Post-authorisation aspects

The adaptive licensing concept includes involvement of a wide range of stakeholders HTAs and patient representatives early in the planning of drug development and throughout the life-cycle of a product. This is key for facilitating timely access to patients of the right drugs.

## PRAC expertise



# Next steps

- December: publication of report on initial experience
- Closure of Phase I of the pilot in February (no more new candidates) to allow concentrating resources in the in-depth Phase II meetings
- Be mindful of several initiatives in the field (explore synergies, integration)
- AL is a learning exercise: run survey among participants (feed back to STAMP/ PharmaCommittee)
- Evaluation of impact after 5/6 procedures have gone through parallel SA/HTA advice