NICE National Institute for Health and Care Excellence

Adaptive pathways: a decision-makers perspective

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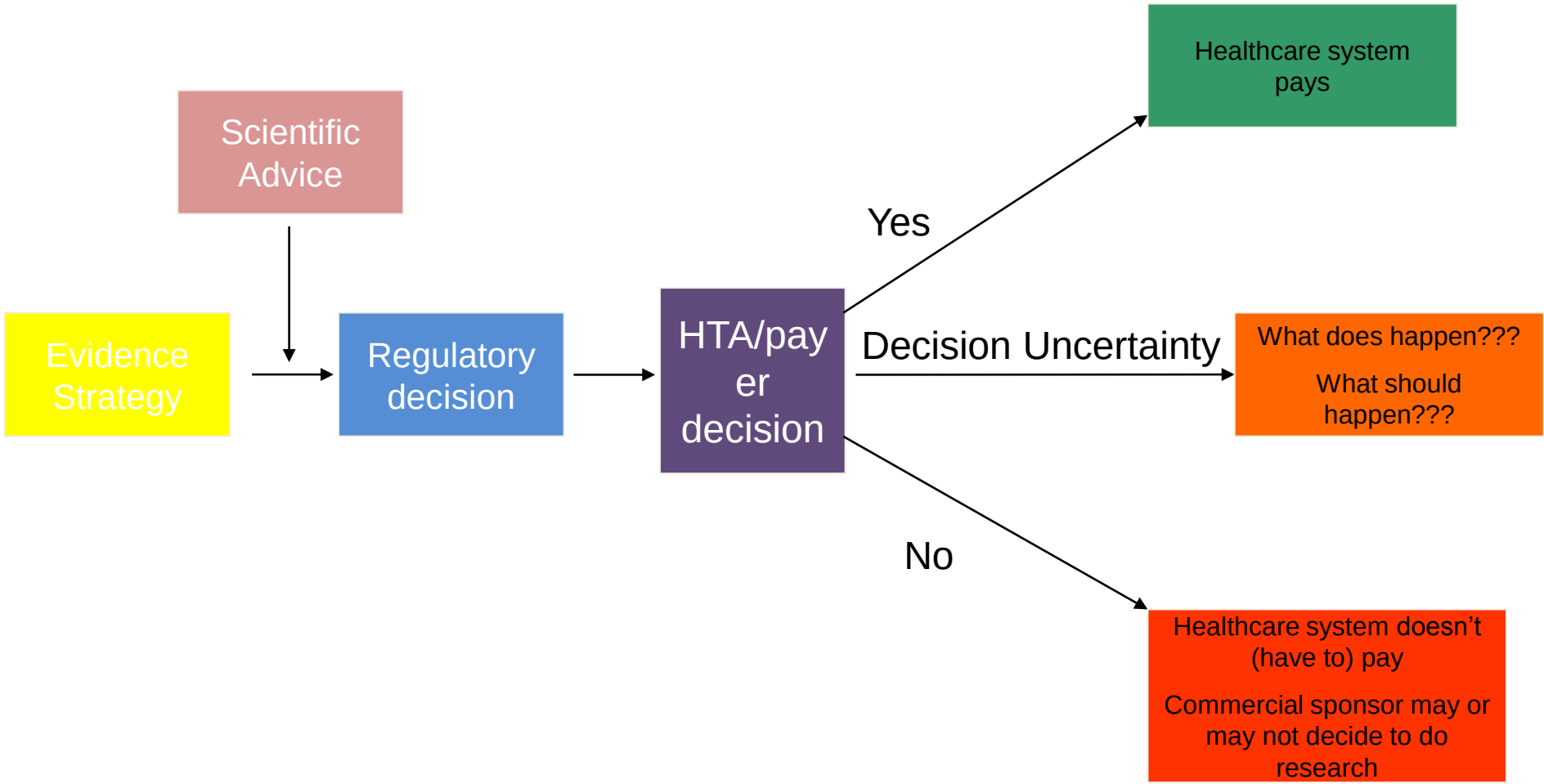
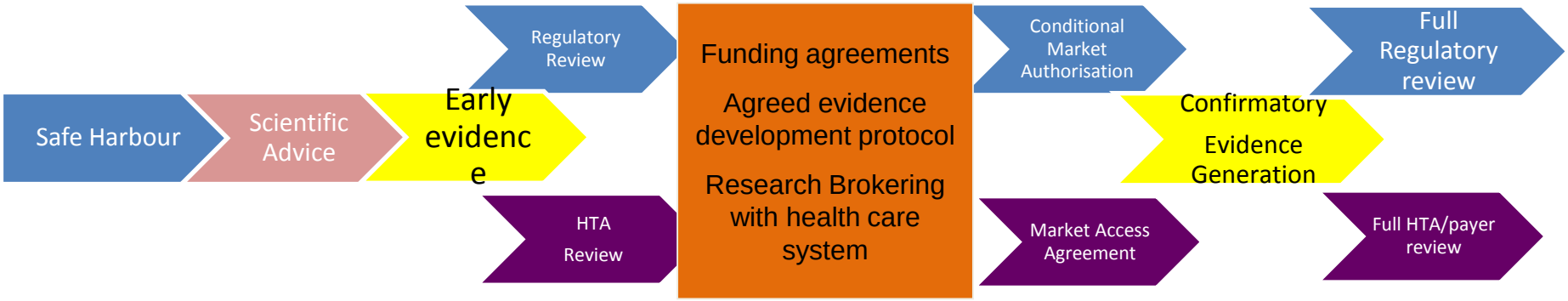
Current UK framework



Adaptive framework



What could an AP look like at UK level?

1. PIM designation
2. Office for Market Access: Safe harbour:
3. Scientific Advice (joint with UK regulators)
4. NICE submission
    - Demonstrated potential to be clinically and cost-effective
    - Uncertainties acknowledged
    - Evidence generation plan utilising existing UK capability
    - Managed Access proposal
        - Initial price with trust that can go up
        - Payment model
        - Exit strategy
5. Discussion to ensure strategy acceptable and viable in UK
6. NICE Recommendation ‘with evidence generation’
7. Evidence collection
8. Review

Pilot progress

- 62 products submitted
- 20 Stage 1 safe harbour
- 18 progressing to Stage 2 safe harbour
- 2 straight to Scientific Advice
- 2 UK safe harbour pilots in progress
  - Hosted by NICE Office for Market Access
  - NICE, MHRA, NHS England, patient organizations, clinicians, research groups

Personal insights: process

- 62 applications: the interest/need is there
  - Variable quality
  - Conditional licensing still seen as a ‘last ditch’ regulatory route
  - Vital to have robust selection mechanisms
- Collaboration of the willing: mixed HTA/payer views
  - Usually input from UK, Netherlands and Sweden
  - France interested bystander
  - Italy has advanced capabilities
- Safe harbour – valuable opportunity to discuss options
- Challenging for stakeholders to change culture
- Engagement is resource-intensive: fee for service essential

Personal insights: development

- Companies are very cautious
  - Time-lines still out to 2020 in some cases
- Some ‘curious’ scientific perspectives
  - Longer trials needed
  - More placebo despite these being drugs for unmet need
  - Lack of understanding about the potential role of real-world evidence
- Companies unaware of the need to demonstrate ‘value proposition’
- Lack of awareness of UK opportunities
  - Research infrastructure
  - Existing registers
- Development strategy still focused on US/Japan
- Manufacturing issues
- Patent issues

Personal insights: incentives

- Concerns about impact on pricing
  - less up front investment, earlier market access, more uncertainty for stakeholders, healthcare system sharing risk, potential for rapid label extension

  Versus

  - smaller patient numbers and lack of trust over ability of price to increase

- Novel reimbursement models being proposed BUT
  - companies still want to price at highest the market may bear
  - lack of trust that initial price may increase
  - unclear whether UK will discuss new models particularly outcomes-based
  - price is just one of potential incentives UK offers

Challenges (and opportunities) ahead…

- Data Science/RWE: UK's biggest asset
  - No UK funding for decision-makers to develop methodology or establish skills
  - Project funding has to go through research panels
  - Fragmented national agencies with overlapping responsibilities
  - Overlapping potential data sources
- Limited ability for UK to engage with IMI projects and capitalize on that EU investment
  - No UK-based co-ordination centre : currently competing
  - No funding for UK-focused projects (Alzheimer's, Hematological malignancies)

Adaptive Pathways summary

- NICE is fully supportive of expedited routes intended to get products to patients that are:
    - Innovative/transformative
    - Address unmet medical needs
- Some of the initiatives we are involved with:
    - NEWDIGS
    - EMA pilot
    - IMI projects: GetReal, ADAPT SMART, BD4BO
    - Office of Market Access and UK ‘safe harbour’ pilots
    - Scientific Advice
    - Early Access Review

BUT…

- Safety of patients must be ensured
  - Full pharmacovigilance to meet regulatory standards
  - Prescribing needs to be managed
- Regulatory and HTA evidence standards must be met during lifecycle
  - Greater collaboration required
  - Safe harbours offer this opportunity
  - Uncertainty must be managed
- Must be financially sustainable
  - Involvement in payers required to develop managed entry agreements with fair risk sharing
  - Exit strategies agreed
- Public-sector resource limitations need to be mitigated