NICE National Institute for Health and Care Excellence

Adaptive pathways: why involve other decision-makers

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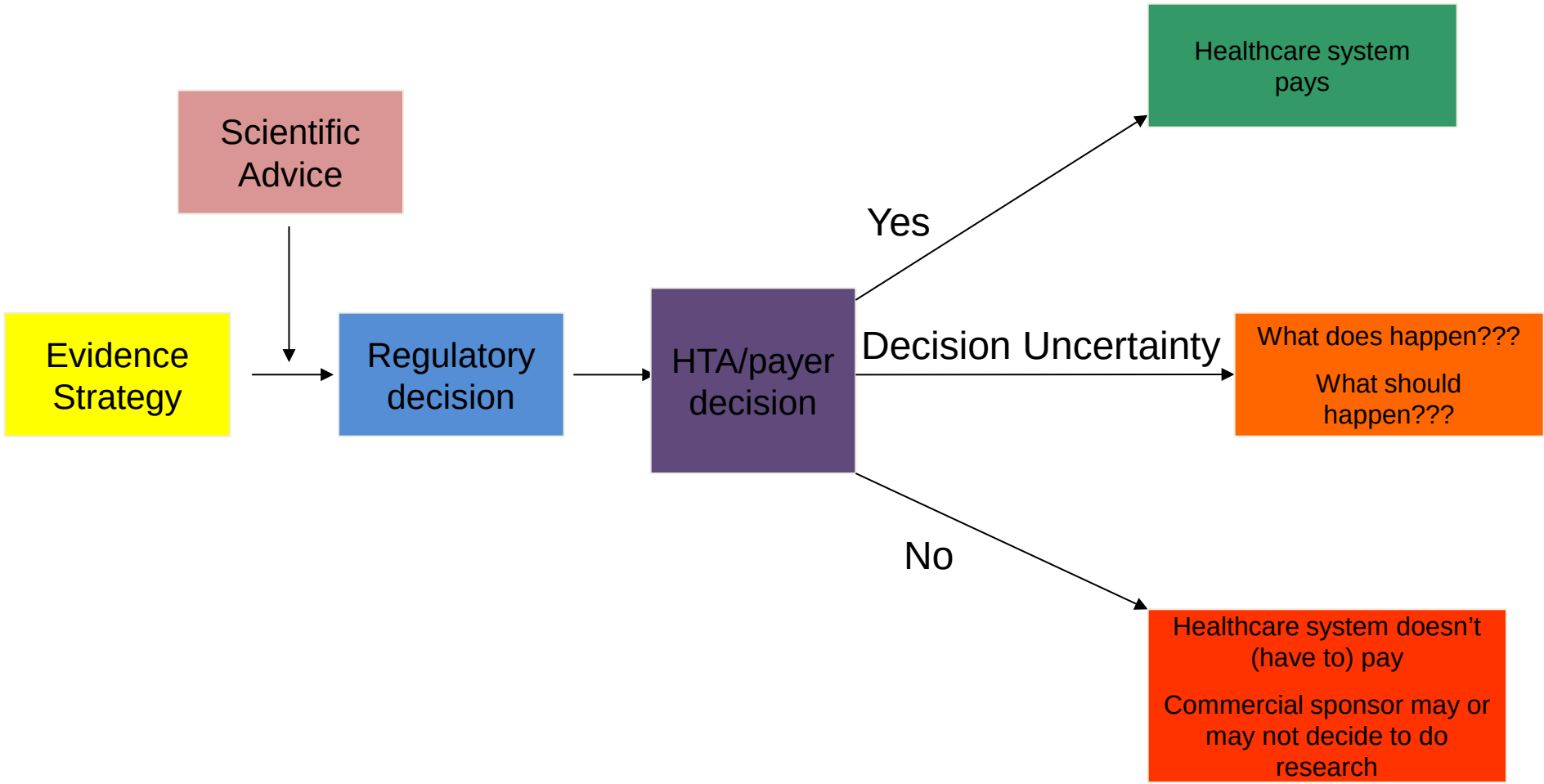
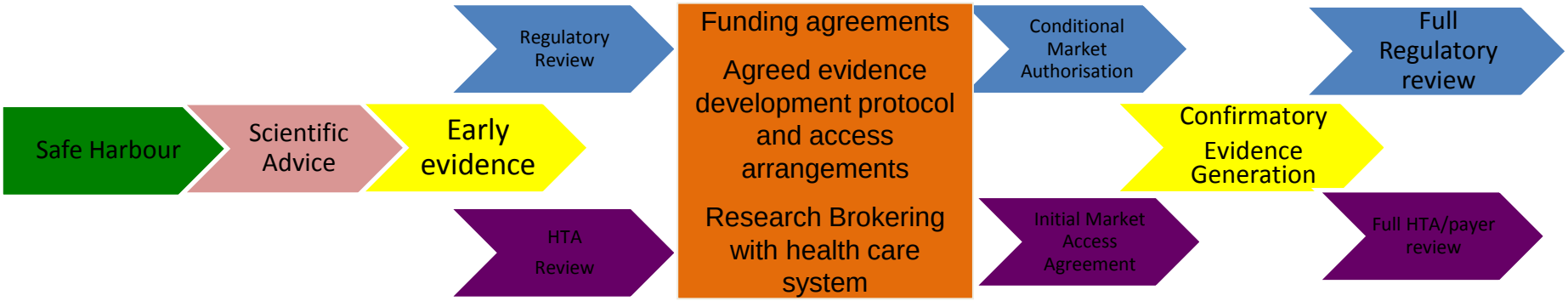
Current framework



Adaptive framework



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 views
- HTA insights needed into pilot design and product selection
- Safe harbour – valuable opportunity to discuss options
- New collaborative process challenging for all stakeholders
- Pilot is resource- intensive

Personal insights: development

- Companies are very cautious *
- FDA requirements driving development decisions
- Some ‘curious’ perspectives
  - Longer trials
  - More placebo despite these being drugs for unmet need
  - RCT despite no appropriate comparator
  - Lack of understanding about the potential role of real-world evidence
- Some companies unaware of the need to demonstrate ‘value proposition’
- Lack of awareness of research assets
  - Research infrastructure
  - Existing registers/register networks

*As are the public systems!

Personal insights: observational data

1. This is a technical/methods/practical issue NOT a policy problem.
2. The role of such data is still being explored
   - IMI projects: GetReal; EMIF; BD4BO, ADAPT SMART…
3. The biases are topic specific and must be understood and mitigated
   - Further methodological investment essential.
   - Opportunity for collaboration.
4. Evidence standards
   - Must still be met for regulation/HTA/payer
   - Will **not** remove need for confirmatory trials when appropriate

5. Will eventually be able to utilise health-system capability but infrastructure still in development and variable across Europe
6. Fragmentation compounding issues
7. Substantial ‘up-skilling’ and resources required.
8. Roles and responsibilities generally and for specific projects must be agreed up front including costs.
9. Data privacy and ethics must be assured.
   - Informed consent essential given risks associated with products

Personal insights: incentives

- Concerns about impact on pricing
  - less investment and more uncertainty
  - BUT smaller patient numbers and lack of trust over ability of price to increase
- Novel reimbursement models being proposed
  - BUT concern that companies may still want to price at highest the market may bear
- The aim is to make development and access more efficient
  - Lower research costs prior to market access
  - Earlier market access
  - Alignment of stakeholders with respect to evidence plan
- Health care systems are now sharing risk
  - Enhanced pharmacovigilance
  - Data collection infrastructure
  - Supporting development
  - Early advice ensures system needs described

ADAPT SMART research on managed entry agreements

- Need to get ‘selection criteria’ right:
  - Highest support from payer/HTA perspective for truly transformative products
  - Decision-making for products with questionable benefits remains problematic
- Outcome-based agreements are rare due to administrative burden and complexity
- Progress will be made only by discussions around specific products in specific healthcare systems
- Further opportunities identified for exploration
  - Discounts based on evidence targets being met
  - Down-stream evidence requirements voluntarily specified in risk management plans

What have I learnt?

- Opinions on Adaptive Pathways are based on perceptions of current system
- Solutions will only be found by changing culture of interactions and building trust
- Need for sectors to work more closely together: resources?
- Care or research: which ethical paradigm?
- Issues and solutions are product/disease specific
- Role of 'Real World Data'
  - IMI GetReal project (http://www.imi-getreal.eu/)
- ADAPT- SMART (IMI Co-ordination and Support Action- http://adaptsmart.eu/)
  - Managed entry agreements
  - Acceptability of single-arm studies
  - Payer evidence requirements

Regulation and HTA