EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Managing uncertainty over the life-span of drug development and use: Recent EMA developments

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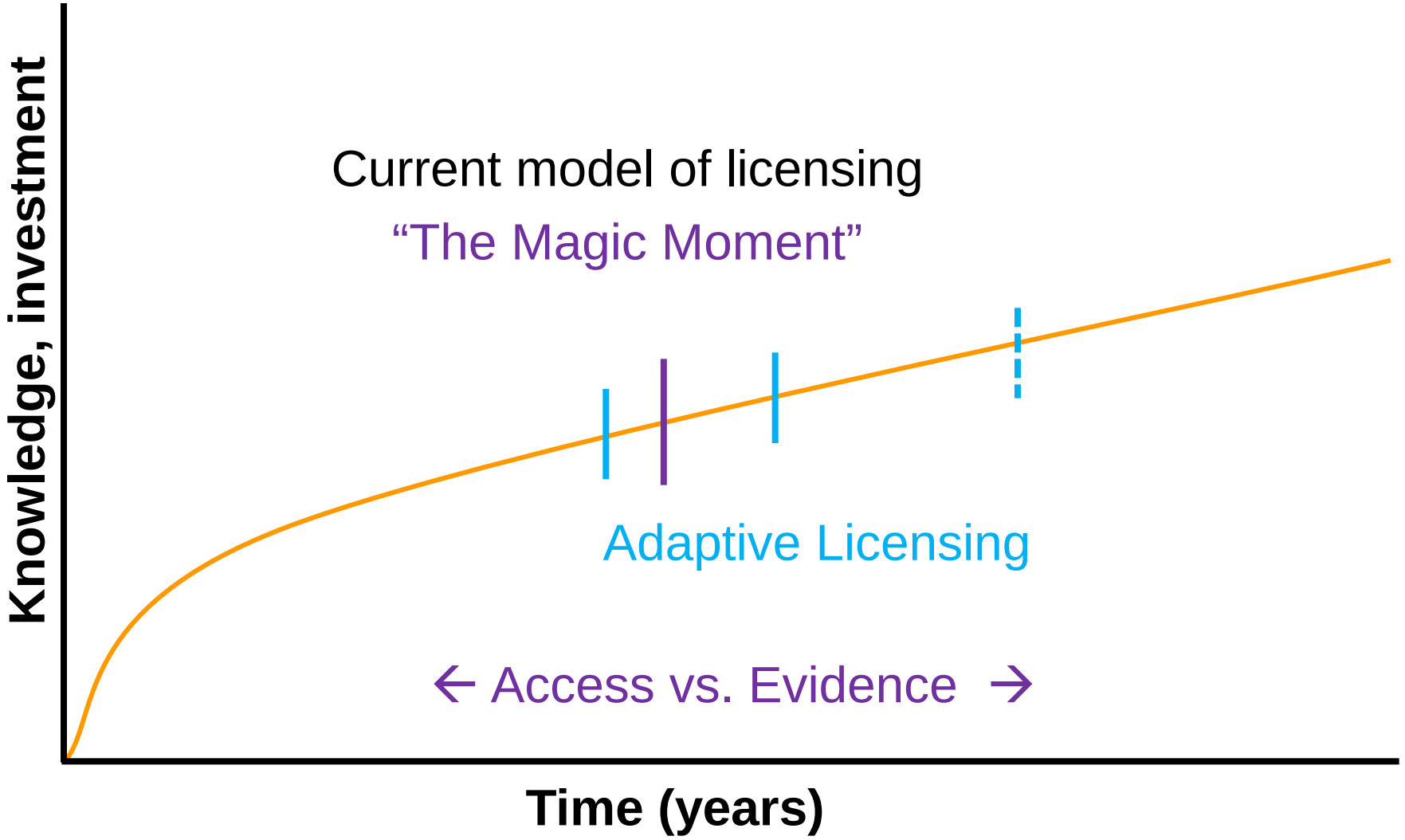
How can we address the access vs. evidence trade-off?

Competing objectives

- Allow timely access for patients to address unmet medical need: “the safest drug that arrives too late is of no benefit to a patient”
- Provide an environment supportive of innovation

- Provide ‘complete’ information on benefits, risks, relative effectiveness

From magic moment to life-span management



Drivers of adaptive pathways

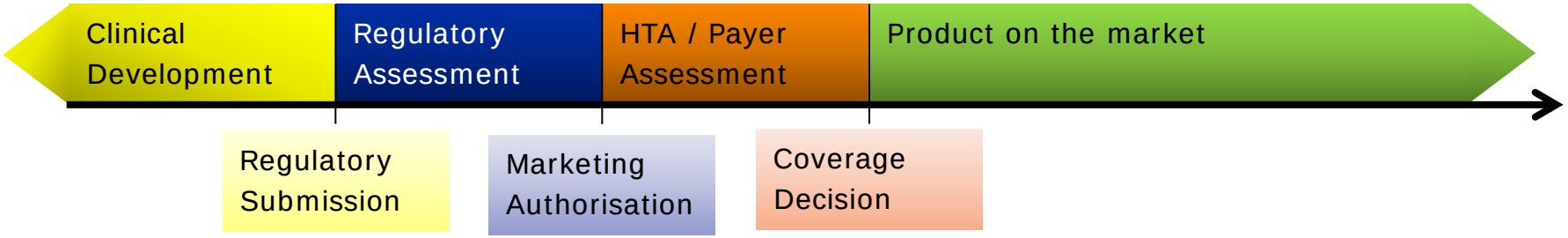
Why change a ‘tried and tested’ concept?

- Patient expectations: demand for timely access and emphasis on unmet medical need
- Emerging science: fragmentation of treatment populations and early disease interception
- Healthcare systems under pressure: rise of payer influence
- Pharma/investors under pressure: sustainability of drug development

A systems approach

Comprises the entire life-span:

Development → licensing → coverage → utilization → monitoring



Adaptive Licensing → Adaptive Pathways

What will change with adaptive pathways?

Transition from …

Magic moment	→	life-span management
Prediction	→	monitoring
RCT only	→	toolkit for evidence generation
Big populations	→	small populations
Focus on licensing	→	focus on patient access
Open utilisation	→	managed utilisation

From prediction to monitoring

Realised versus inherent risk

- 1950/60s: thalidomide (phocomelia; 10.000 cases) high-visibility, low background event!
- 2005: natalizumab (PML; 3 cases)
- 2009: Pandemrix (narcolepsy; 15 cases), *but…*
- high-background or low visibility events (e.g. MI in diabetics) ?

What needs to be in place to enable adaptive pathways? 1/2

- Culture of collaboration with patients and physicians to agree on level of unmet need and acceptable uncertainty

  EMA initiatives: pilot programs to elicit patient preferences

- Collaboration of sponsor, regulators, payers/HTA bodies throughout the life-span of a product

  EMA initiatives: ample experience with parallel scientific advice with HTA bodies

What needs to be in place to enable adaptive pathways? 2/2

- Rapid learning systems for data generation across whole life-span → to minimise *realised* risk (as opposed to *inherent* risk)

  EMA initiatives: Risk management plans, data infrastructure / analysis projects

- Tools to provide reasonable assurance of appropriate Rx

  EMA initiatives: ?? (Risk minimisation activities)

Conclusion

- We are on a trajectory to more adaptive pathways
- The speed of change will depend on how fast preconditions can be met
- Adaptive pathways are likely the best (only?) way to address the access versus evidence trade-off
- EMA initiatives: ‘Adaptive Licensing Pilots Project’; to date: 28 products submitted, 9 selected for pilot – watch this space!

Thank you

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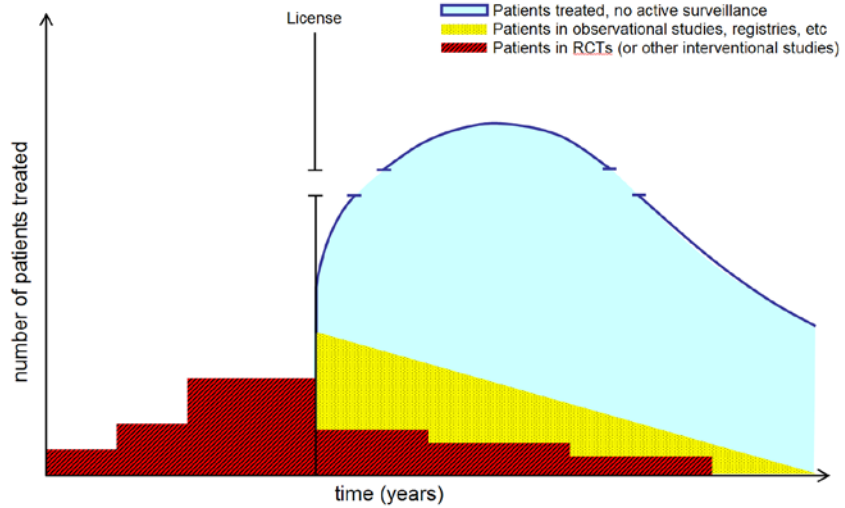
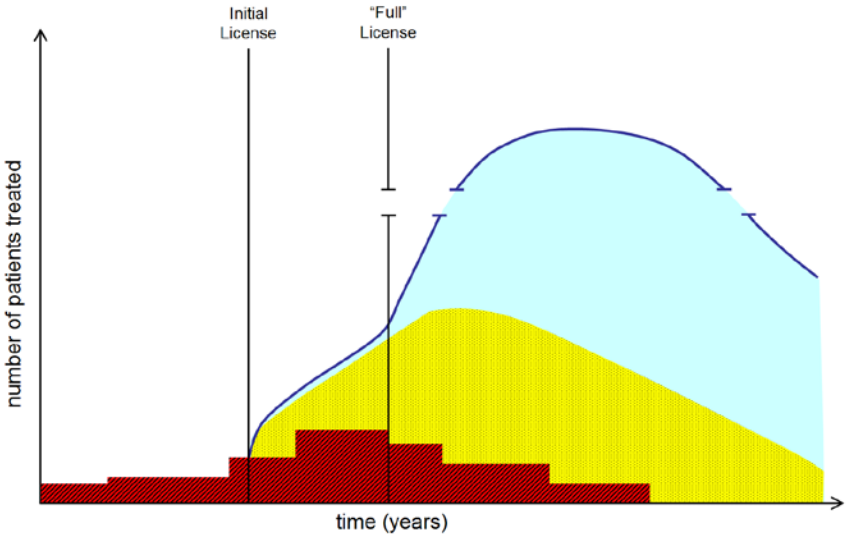
Discussion slides – will not be presented during main talk

From RCT to toolkit for evidence generation



Current scenario:

Post-licensing treatment experience of many patients does not contribute to evidence generation



Adaptive Licensing:

After initial license, patient experience is captured to contribute to real-world information

From licensing focus to patient access