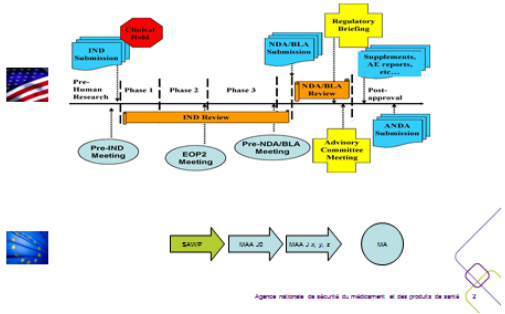
Conclusions and perspectives (EU)

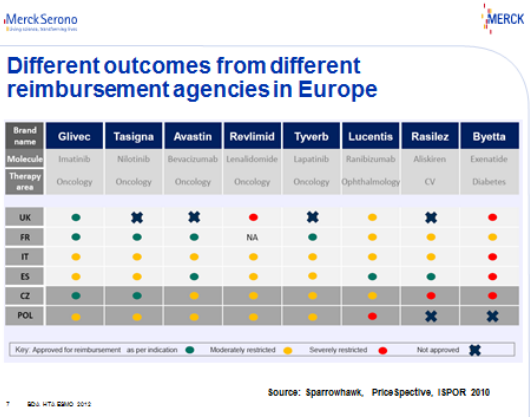
Francesco Pignatti
European Medicines Agency, London, UK

Vienna, BDA, Sept 2012

Bridging the gaps

US/EU
Rolling Assessement / Stepwise Process





Patient Access to Health Technologies

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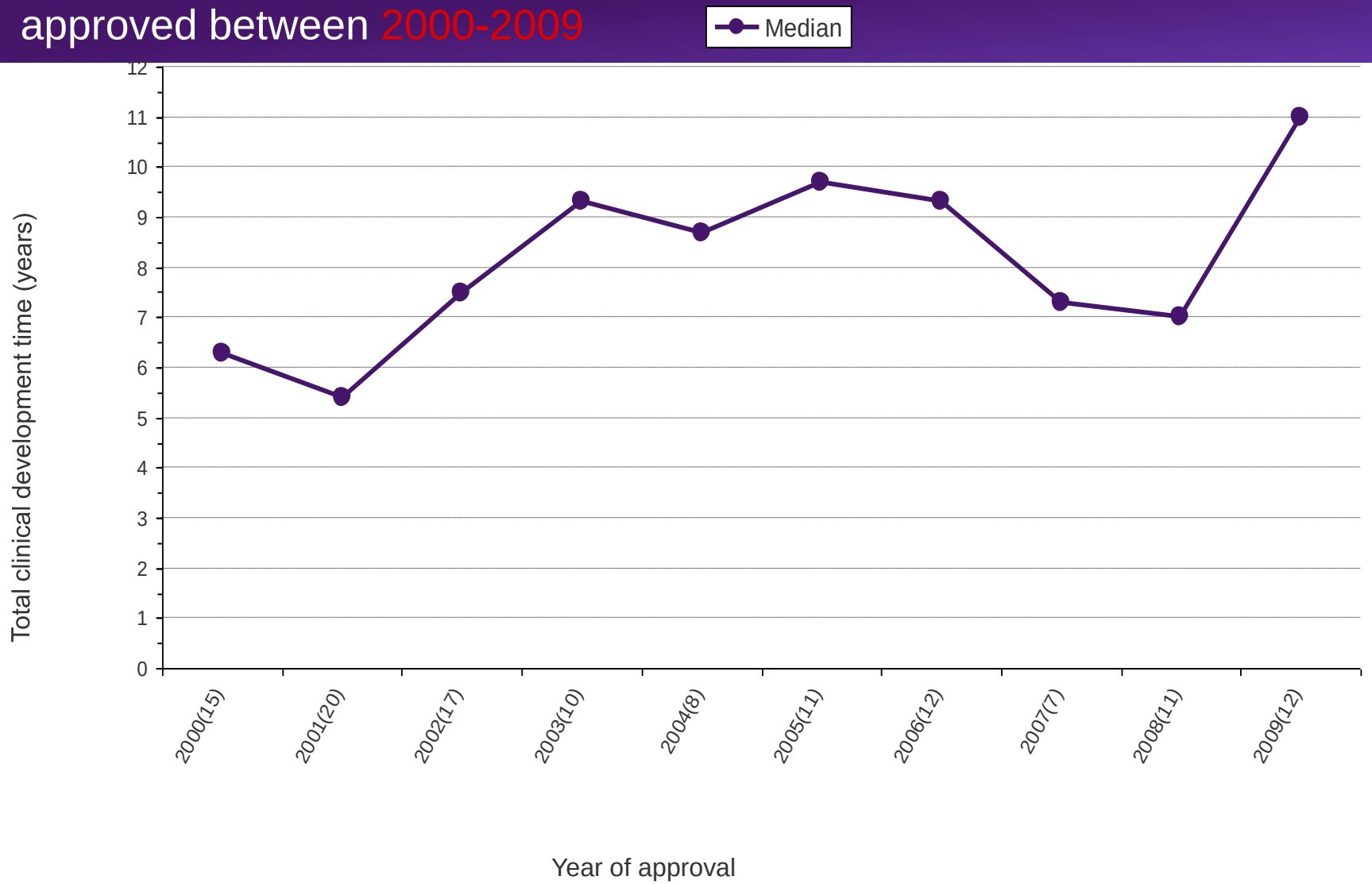
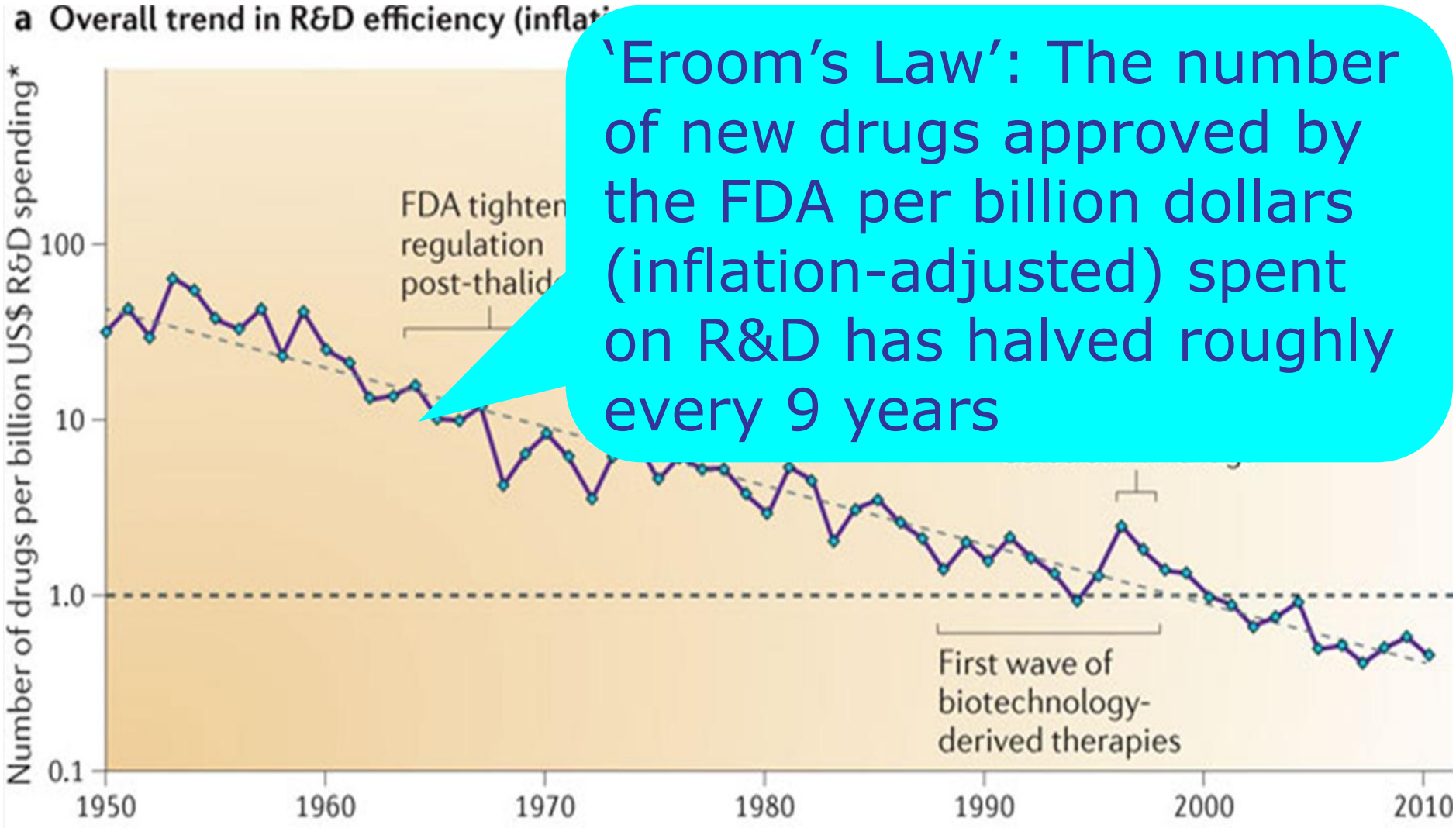
EU	Drugs *(therapeutic, preventive)*	Devices *(implantable, etc)*	Diagnostics *(lab tests, imaging, etc)*	Procedures *(surgery, physio ther.)*	Other *(educational, campaigns, etc)*
Benefit Risk *(Marketing Authorisation)*	EMA				
Relative Efficacy Assessment *(CAV)* Rel. Effectiveness					
Health Technology Assessment *(incl. cost consequences)*					
Coverage decision *(incl. appraisal, soc. preferences)*					
Utilisation *(on-/off-label, med. errors)*					

Horizontal and vertical inconsistency between member states

Trend in actual clinical development time for new development projects approved between 2000-2009



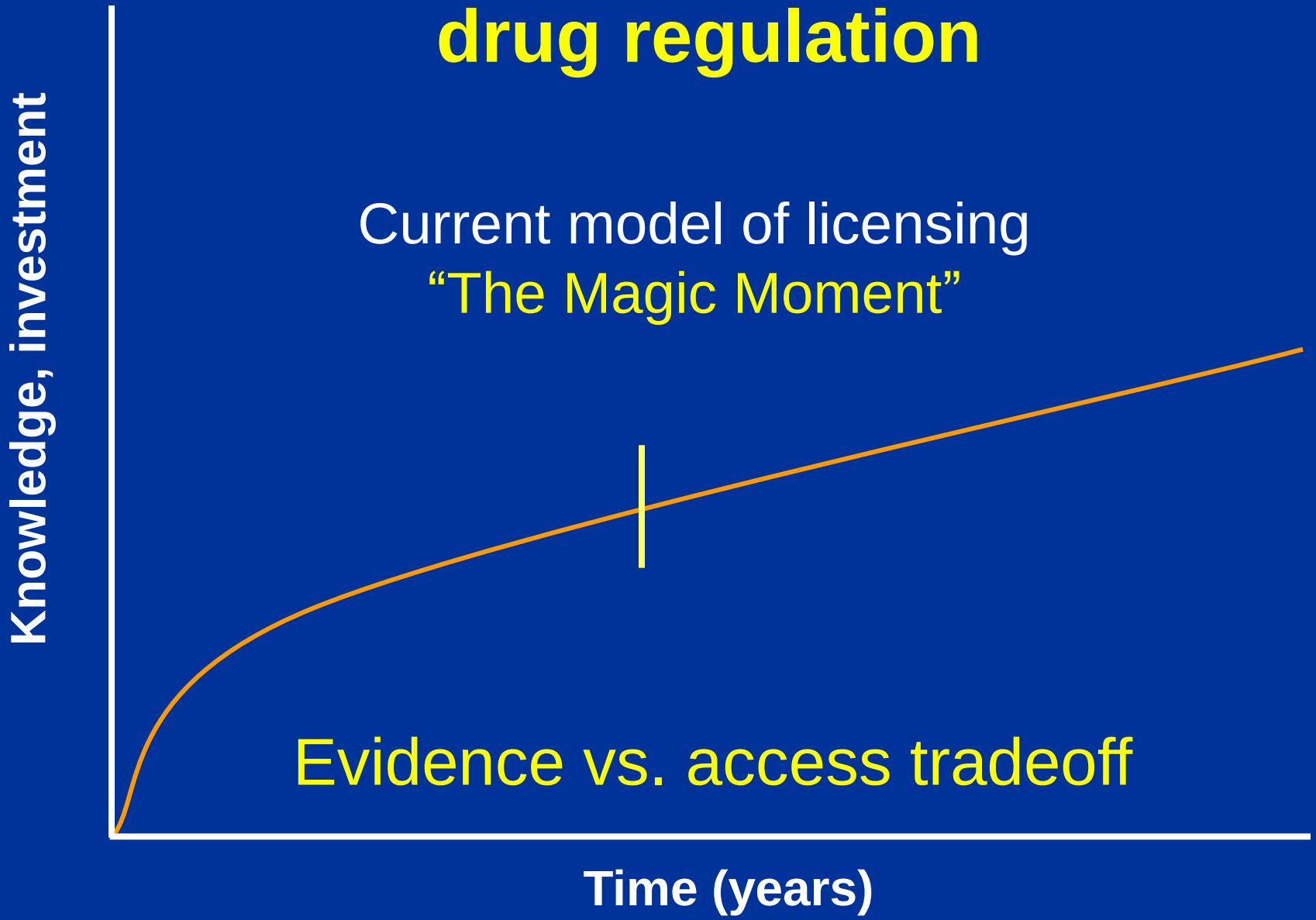
Actual clinical development time is calculated for new development projects as the time between 'First human dose' (T-1-1) and 'First approval' (T-4-2). Data represent all new development projects that reached 'First approval' (T-4-2) between 2000-2009, where the start and end milestone dates for the interval are available. (n) = number of projects analysed in each year. This analysis is based on data from a consistent cohort of 17 companies participating each year between 2001 and 2010.



'Eroom's Law': The number of new drugs approved by the FDA per billion dollars (inflation-adjusted) spent on R&D has halved roughly every 9 years

Scannell JW et al. Nature Rev Drug Disc, March 2012

The binary nature of drug regulation



The regulator's dilemma

"…it has been said that the FDA has just two speeds of [drug] approval – too fast and too slow."

Hamburg MA & Sharfstein JM. NEJM 360;24: 2493-5; 2009

Adaptive licensing *in a nut shell…*

Taking a less ambitious regulatory review route

that would limit the drug to a far smaller and

higher-risk group of patients, at least initially

San Diego Union-Tribune (10 Feb 2011)

"Precursors" to Adaptive Licensing

- Conditional Marketing Authorization
- New Pharmacovigilance legislation
- Risk Management Plans
- Periodic Safety Update Reports
- Five-year renewal of marketing authorization
- (Compassionate use programs)

A better model for evolution?

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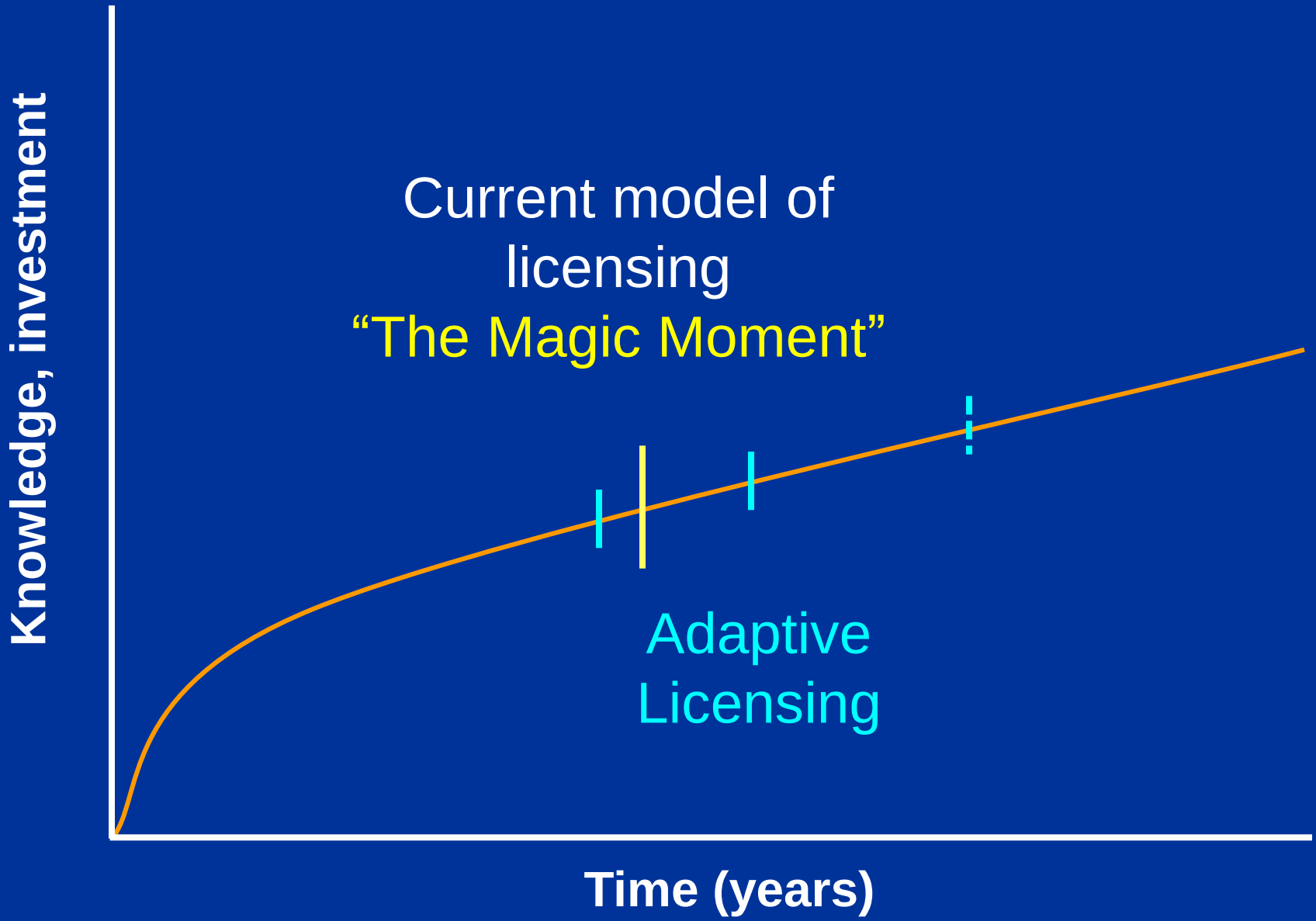
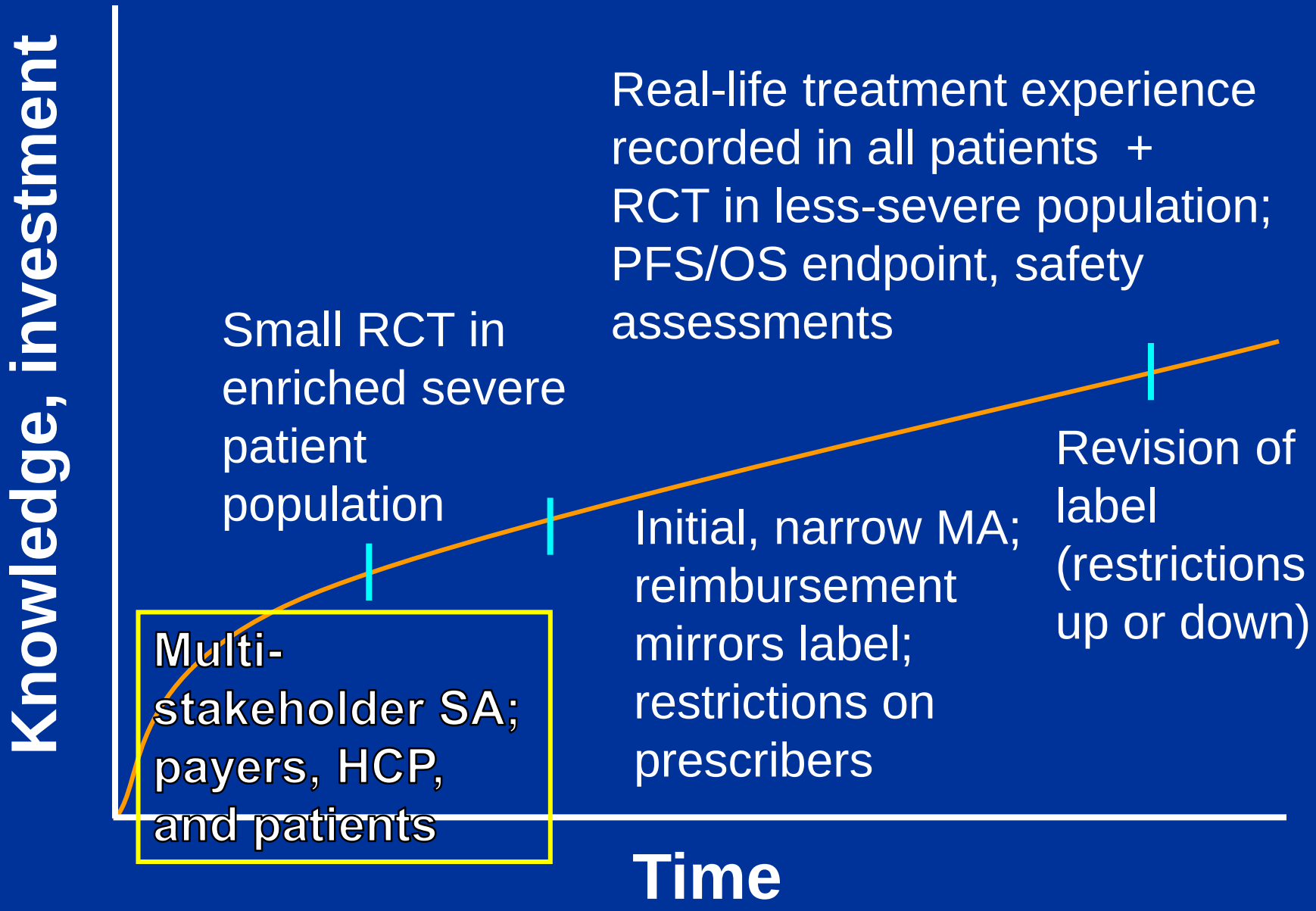


Possible AL model rare cancer



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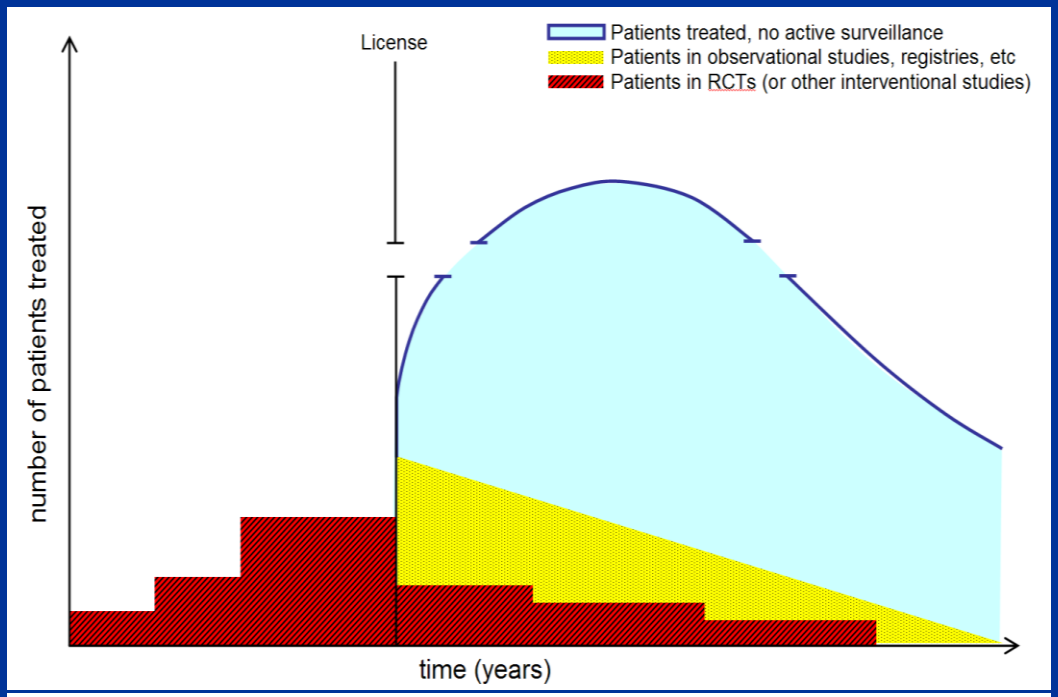
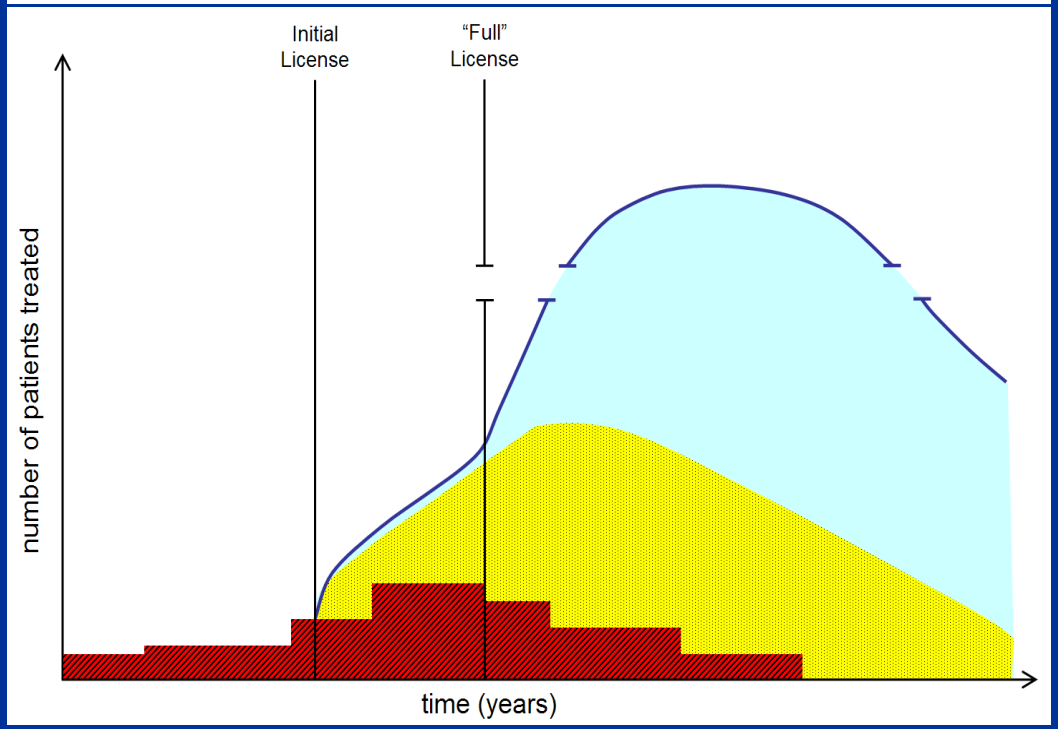
Different names, same ideas

- EMA: staggered approval
- FDA: progressive reduction of uncertainty
- Health Canada: progressive authorization
- HSA Singapore: test bed for adaptive regulation
- Payers: managed entry (HTAi), CED
- MIT/NEWDIGS: adaptive licensing project





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Current scenario:

Post-licensing, treatment population grows rapidly; treatment experience does not contribute to evidence generation

Adaptive Licensing:

after initial license, number of treated patients grows more slowly, due to restrictions; patient experience is captured to contribute to real-world information

Obstacles to Adaptive Licensing

- concerns over lowered standards
- how to communicate uncertainty?
- doable under current statute?
- getting commitment from industry to conduct “stage n+1 studies”?
- are follow-on studies doable after “loss of equipoise”?
- alignment between regulators and payers
- different reward structure required to incentivise drug development enterprise?
- ensuring appropriate prescriptions

Addressing the obstacles; next steps?

- Address economic consequences for drug development
- Design pilots cases using current sponsor assets
- Address legal underpinnings of AL
- Explore opportunities for collaboration with payers
- Obtain buy-in from all ranks of regulatory community
- Conduct pilots (EMA work program 2012)

EMA Road map to 2015

[…] a key issue for regulators will be whether a more ‘staggered‘ approval (or progressive licensing) concept should be envisaged for situations not covered by conditional marketing authorisations […]

The Agency would like to launch a debate with all stakeholders on the appropriateness of introducing such a concept, including a consideration of appropriate incentives to support new medicines development.

Thank you!

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Acknowledgments: Hans-Georg Eichler (EMA)