EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

- Launch of the Adaptive Licencing Project

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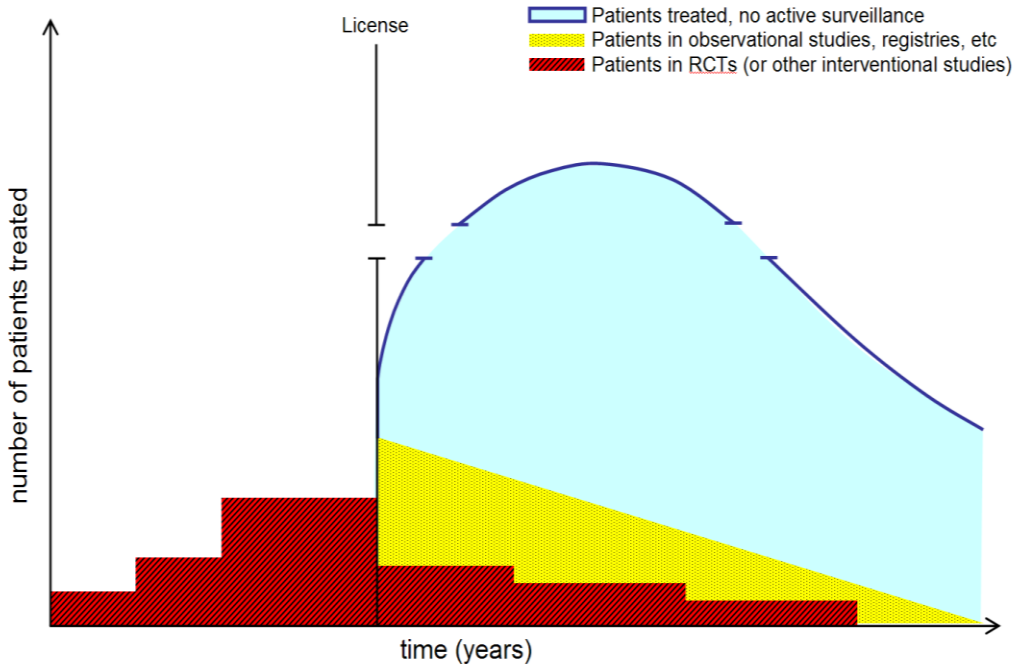
Launch of the Adaptive Licencing Project

On March 19 the Agency launched the project:

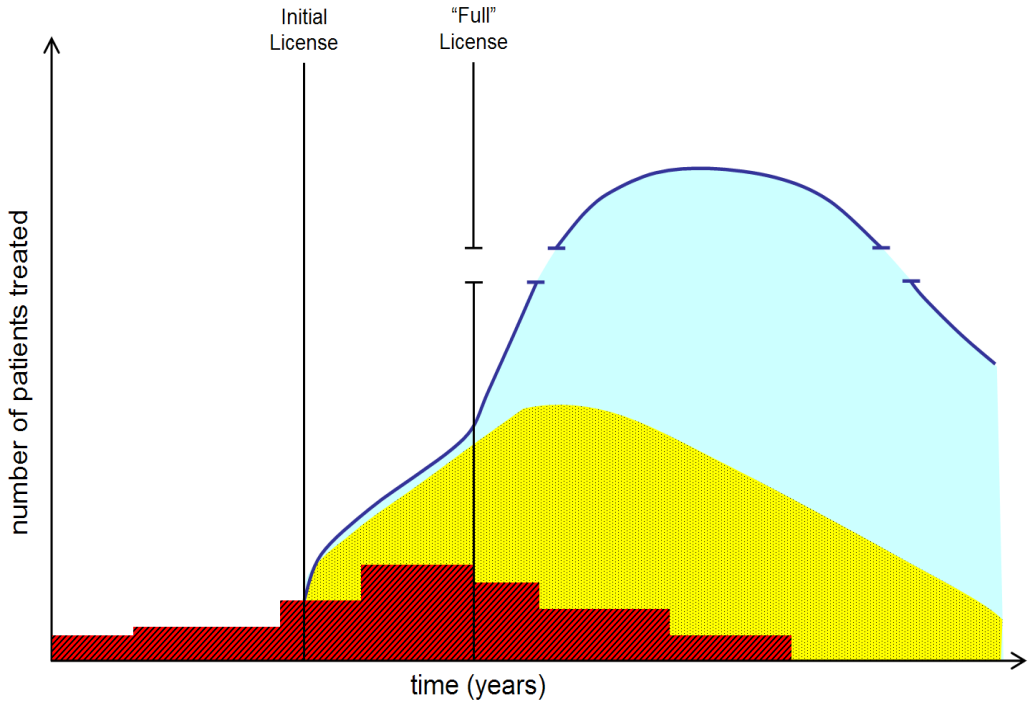
What is this concept about?

- early access for patients starting from a niche indication with a high unmet medical need,
- continued collection of data (both on efficacy & safety) in this niche indication and extension to broader patient groups.
- Involve HTAs, patient representatives and health care professionals throughout.



Current scenario:

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



Adaptive Licensing:

after initial earlier license, number of treated patients grows more slowly, due to restrictions; patient experience is captured to contribute to real-world information [2]

Launch of the Adaptive Licencing Project

- Agency invites sponsors to submit real examples* to be discussed informally by the Adaptive Licencing Discussion Group
- Project is managed by the Scientific Advice Office
- 26 proposals submitted so far, 3 discussed with companies
- If established, AL implementation in current legislative framework: e.g. SA for planning, CHMP opinion for initial MA in niche indication and variations for extending to broader populations.

* Dedicated e-mail: adaptivelicensing@ema.europa.eu

Adaptive Licencing

It is an approach that builds on advances in medical science, in particular genomics and personalised medicine to facilitate an early approval of innovative medicines in a targeted population with specific characteristics linked to the drug. it is envisaged that by targeting initially such a specific group of patients with a very serious condition the benefit will be large enough also on a surrogate endpoint to outweigh the uncertainties so that the benefit/risk balance is positive at the early initial licensing.

The proposal has to include tools how to control prescription in the targeted population.

Similar to personalised medicines the AL concept includes going beyond the current broad definition of diseases.

Adaptive Licencing

There needs to be a plausible plan for expansion of the initial patient population based on an iterative development pathway between developers, regulators and HTAs

For this, evidence on efficacy needs to be continuously gathered post-licensing with special focus on the use of real world data! not only on randomised clinical trials. the adaptive licensing concept has the goal to move away from the traditional magic moment of the initial licensing in a broad population or in a small population and switch to market with not much control or evidence generation afterwards. Efficacy data from observational studies, registries and so on will be used to adapt the access to patients

Systemic Amyloidosis

Affects all organs; different patterns of organ involvement, varying stages of disease at presentation

But involvement of heart and kidney contributes most to mortality;

Product: Antibody to remove amyloid.

Initial licencing: Most severe form based on amyloid depletion, cardiac and renal function

Increase evidence in initial indication/ Expand: Survival in the most severe form, focus in renal function and quality of life in less severe forms.

Adaptive Licencing

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.

Adaptive Licencing Project: An experiment

- Advantages: Early approval and access to patients with real need, with involvement of all stakeholders and prospective planning to collect data

- Risks: Increased number of withdrawals; uncertainties may be higher in the initial licencing