EARLY ACCESS TO MEDICINAL PRODUCTS

1st STAMP meeting

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The French scheme of Temporary Authorisation for Use (TAU)

DGS
Direction générale de la santé

ansm
Agence nationale de sécurité du médicament et des produits de santé

ATU scheme: Principles

- Legal provision laid down since 1994:
  - Exceptional derogation to the MA by the national authority
  - Early access to new and promising drugs
  - Drug fitting an unmet medical need
  - No option for patient' enrollment into clinical trials

- Criteria for authorisation:
  - Drug for treatment, prevention or diagnosis of a ***rare or serious disease***
  - With no ***satisfactory*** alternative available
  - And for an Urgent medical need (*new law basis, 2011*)
  - When the risk/benefit balance is ***presumed*** to be positive

Developments and current status

- Two types of TAU status:
  - Named-patient basis (*art 5-Directive 2001/83/CE*) :
    - « compassionate use » on a case-by-case analysis
    - temporary granting only if the company is pursuing a MAA or a cohort TAU in France
  - Cohort TAU (*art 83- Regulation CE n°726/2004*):
    - commitment of the company to apply for a MA within a fixed timeframe
    - one predefined indication and population
    - 1-year granting with possible renewal
- For both, specific binding conditions:
  - Improved patients follow-up :
    - robust pharmacovigilance for both TAU schemes
    - efficacy data for cohort TAU and some named TAU
  - New provision for drug reimbursment

Drug supply & Reimbursment

- Drug supply:
  - Prescribing restricted to hospital physicians
  - Delivery only by hospital pharmacists
- Reimbursment & transition phase to standard market access :
  - Initial Drug pricing fixed by the company and directly negociated with the hospitals
  - For the patients and hospitals : 100% reimbursment by the national Health care payor
  - When MA decision by the EC :
    - End-date of the TAU transmitted to the Health Ministry → new phase of HTA
    - Transition from free price to negotiated, controled pricing system
    - If the final agreed price is inferior, the company may be asked to reimburse the difference (adjusted to the volume of sales during the TAU period)

Experience (1): Impact on early access to innovative drugs

- Status :
  - >130 cohort TAUs in total, 24 currently ongoing
  - In 2013:
    - 9 new cohort TAUs, including 6 000 patients followed-up
    - 240 drugs granted for individual TAUs
    - Overall 20 000 patients under both TAUs status
- Early access *(analysis ANSM, 2012):*
  - 10-12 months (average) before EU MA for centralized drugs under cohort TAU status
  - Time-laged of 11 months between licensing and final pricing → 4 months longer than for non-TAU drugs
  - For orphan diseases: 36 months on average (*historical data*)

Experience (2) : Limitations

- High work load with no improvement since exceptional /conditional approvals pathways
- Access depending on the company's willingness
- No binding option for a *mandatory* MA application and positive outcome …
- Issue with drug availabilty :
  - TAU granted but no drug supplied by the company
  - Due mainly to drug manufacturing (and company's good willing for the individual TAU)
- Divergent views on the therapeutic indication & population(s) to be granted between health professionals/ authorities /company

Experience (3): Challenges

Pre-approval assessment of the risk / benefit balance:

- Scientific expertise at national level or EMA (often linked to clinical trials involvement)
- Based on interim data from the ongoing phIII trials, sometimes from phII trials (e.g. targeted therapies in oncology)
- Limited capacity of data analysis after TAU granting
- Reliability of efficacy data in new indications/ sub-populations
- Risk management of the off-label usage (implementation of a guideline « protocole temporaire d'utilisation »)