EARLY ACCESS TO MEDICINAL PRODUCTS

1st STAMP meeting

27 January 2015

The French scheme of Temporary Authorisation for Use (TAU)
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 negociated, 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-lagged 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 availability:
  – 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 »)