The Reform of the French Compassionate Use Program : The State and Perspective Before the Change

Zimmermann P[1], Tano M[1,2], Siorat V[1], Ribault M[1], Parent de Curzon O[1], Degrassat-Théas A[1,2], Paubel P[1,2]

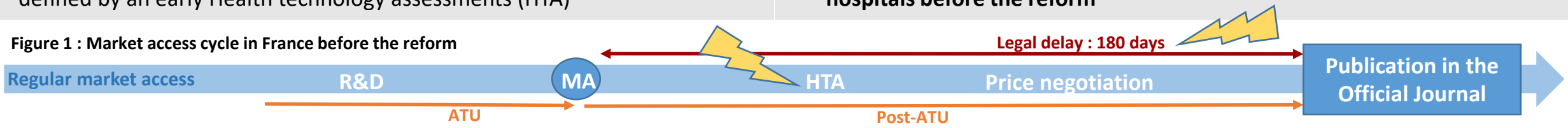
1 : General Agency of Equipment and Health Products (AGEPS), Assistance Publique-Hôpitaux de Paris (AP-HP), Paris, France ; 2 : Health Law Institute, INSERM UMR S 1145, University of Paris ; Faculty of pharmacy, University of Paris, Paris, France

Code : POSA217

Introduction

- A major reform of the French Compassionate Use Program (CUP), previously called Temporary Authorization for Use (ATU) (fig. 1), takes place in 2021
- First goal of the reform : reserving CUP to only presumed innovative drugs defined by an early Health technology assessments (HTA)
- Second goal of the reform : sanctioning too long price negotiation after the marketing authorization (MA) to shorten the free pricing window
- **Objective of this work : take stock of drugs under CUP in Paris public hospitals before the reform**

Figure 1 : Market access cycle in France before the reform



Method

- Scope : all drugs under ATU and so called "post-ATU" from 2018 to 2020 in Paris public hospitals
- Indicators : annual cost, time spending under post-ATU
- Innovative character evaluated by the French HTA agency after MA based on the added clinical benefit (ASMR) rated from I to III

Results

Figure 2 : Number of ATU and post-ATU drugs and spending

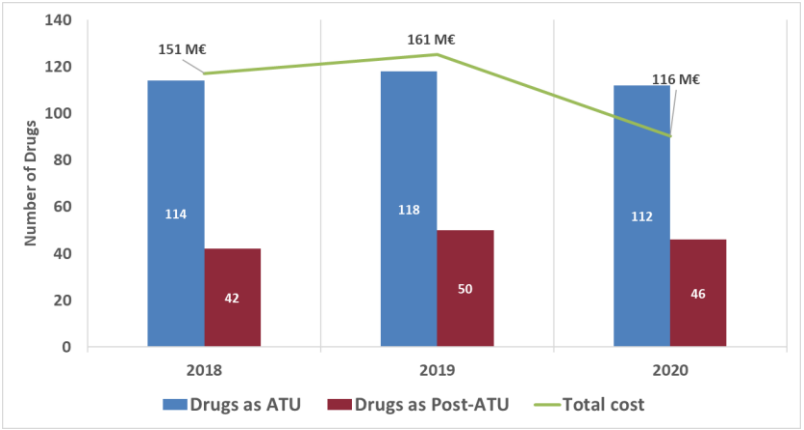
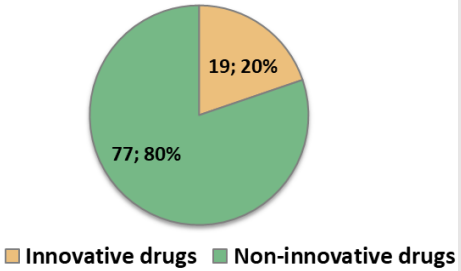


Figure 3 : HTA assessment



- With an amount of **116 to 151M€**, ATU & post-ATU drugs represent 16 % of annual AP-HP budget (Fig. 2)
- **Only 20% of drugs were deemed innovative** after the regular HTA process post-MA (Fig. 3)
- Average time for HTA and price negotiation : **593 days** [191;2069], way above the 180 days legal delay (22 drugs still under HTA review)

Conclusion

Beginning of July 2021, discounts could apply to manufacturers for drugs that do not respect the new CUP criteria. The reform put HTA at the core of the CUP in order to control the drug derogatory access but at the risk of being less attractive for companies.

✉ : philippe.zimmermann@aphp.fr