Faster, Simpler and Easier: Analyzing the Reform of the French Early Access Pathway

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Background

- The publication of the French Social Security Financing Act for 2021 enacted the reform of the authorizing procedure for early and derogation access to medicinal products by regrouping former derogation access processes into 2 distinct pathways:
  - Pre/post-marketing early access (EA)
  - Compassionate access
- The reform is effective in France since July 2021 and the HAS is now responsible for evaluating EA requests based on a set of four major criteria
- **We aimed at analyzing all EA opinions issued by the HAS since July 2021**

HAS evaluation criteria for EA requests

- Severe, rare or debilitating disease
- Lack of appropriate treatment
- Impossibility to defer treatment initiation
- Presumptively innovative nature, particularly compared to any clinically relevant comparator

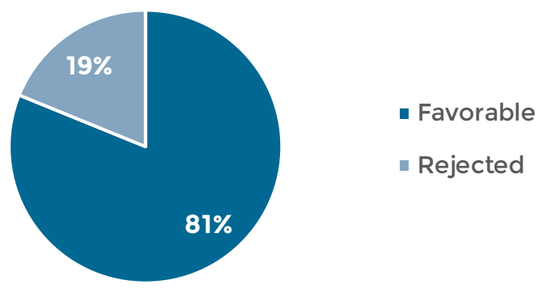
Methods

- A review of HAS EA opinions was conducted. All published opinions since the reform was implemented (i.e. July 2021) up to July 7th 2022 were included.
- Outcomes of interest were:
  - Approval/rejection rate
  - Therapeutic areas
  - Percentage of eligibility criteria met
  - Market authorization status
  - Mean/median review time

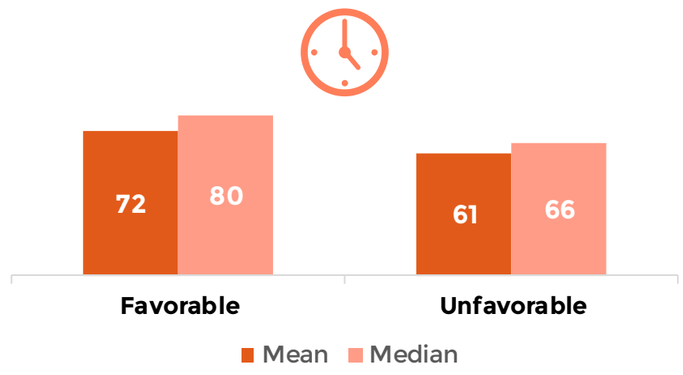
Results

- A total of 69 opinions was extracted, with 56 EA requests accepted (81%) and the main therapeutic areas being in oncology (n=22; 32%), infectiology (n=14; 20%, with 12 being related to COVID-19) and onco-hematology (n=9; 13%)
- Additionally, 30 requests (43%) were submitted pre-MA and 39 (57%) post-MA

Approval/rejection rates for EA requests (n=69)



Review time of early access requests (days)



Main therapeutic areas represented in EA (number of opinions)

Top 3 reasons for EA rejection

1. Absence of presumptively innovative nature (n=7 ; 53%)
2. Possibility to defer treatment initiation (n=5 ; 38%)
3. Alternative treatment available (n=3 ; 23%)

- Favorable opinions met the following HAS criteria (n=56):
  - ✓ 'Debilitating disease' (100%),
  - ✓ 'Severe disease', 'presumptively innovative nature' & 'impossibility to defer treatment initiation' (98%)
  - ✓ 'Lack of appropriate treatment' (95%)
  - ✓ 'Rare disease' (70%)
- For seven opinions, the HAS recommended the creation of a patient registry or patient cohort follow-up, regardless of whether the EA was approved or rejected

Discussion

- A comparable analysis was conducted by the HAS on May 2022 and reported similar results (based on 50 opinions), with a 80% approval rate and a mean review time of 60 days
- The HAS research also provides additional insights about 25 positive post-MA EA requests and their subsequent evaluation for reimbursement:
  - 12 of them received an ASMR III grade,
  - 8 received an ASMR IV grade,
  - 5 received an ASMR V grade

Conclusion

- The reform replaces the previous ATU system (formerly managed by the ANSM) and provides a simpler and clearer system for drug manufacturers
- EA requests and HTA are now both managed by the same national body (HAS), thus improving review time for reimbursement dossiers if an EA request is submitted beforehand
- This new system also gives a voice to patients, as patient associations can provide feedback when EA requests are being assessed by the HAS
- Further research could be conducted to look at the impact of this new pathway on pricing negotiation processes by the CEPS

Abbreviation: ANSM= Agence Nationale des Sécurité des Médicaments ; ASMR= Amélioration du Service Médical Rendu ; ATU= Autorisation Temporaire d'Utilisation ; CEPS= Comité Economique des Produits de Santé ; EA= Early Access ; HAS= Haute Autorité de Santé ; HTA= Health Technology Assessment ; MA= Market Authorization

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