Analysis: First Year of the New Early Access Authorization in France

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BACKGROUND

- The **temporary authorization of use (ATU) framework** in France, which was previously divided into 6 categories, was reformed in 2021 to provide patients with fast-track access to new medicinal products.[1]
- The reformed early access program is now divided into 2 main systems:
  - Early access authorization
  - Compassionate access

OBJECTIVE

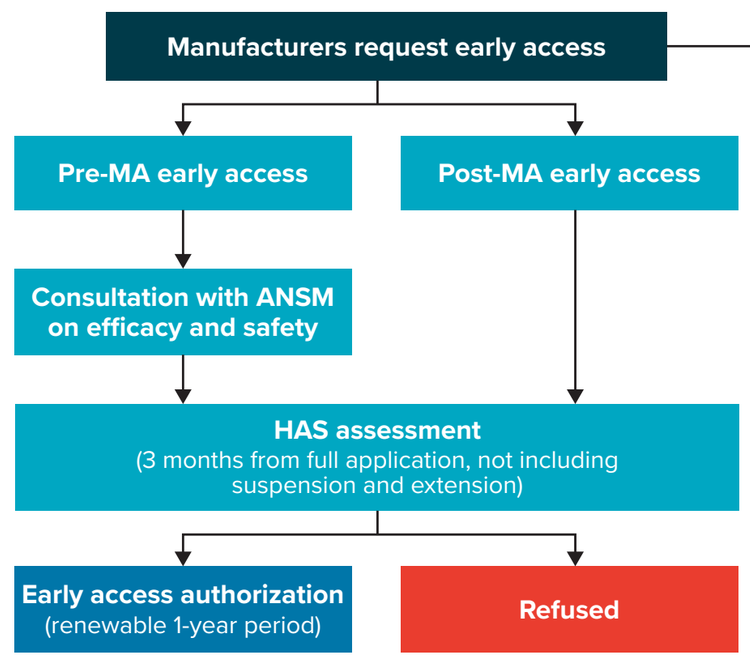
- Our objective was to review and analyze the new early access authorization program launched in France in July 2021.

METHODS

- 66 assessments on treatments that were processed under the early access authorization program of Haute Autorité de Santé (French National Authority for Health; HAS) and published online between July 2021 and June 2022 were analyzed.[2]
  - The assessments were examined for regulatory status, added clinical benefit rating, therapy area, processing timeline, and data used in the evaluation.

RESULTS

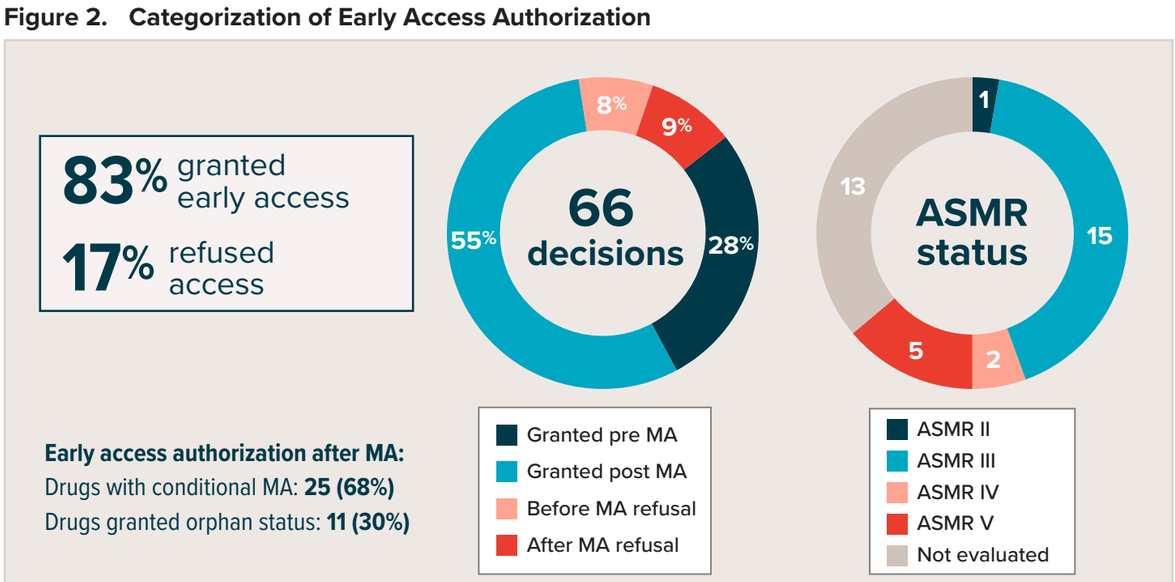
Figure 1. Overview of Early Access Authorization Scheme



ANSM = Agence Nationale de Sécurité des Médicaments et des produits de santé [French National Agency for Medicines and Health Products Safety); MA = marketing authorization.

Overview of Early Access Authorization Decision

Figure 2. Categorization of Early Access Authorization



ASMR = Amélioration du service médical rendu.

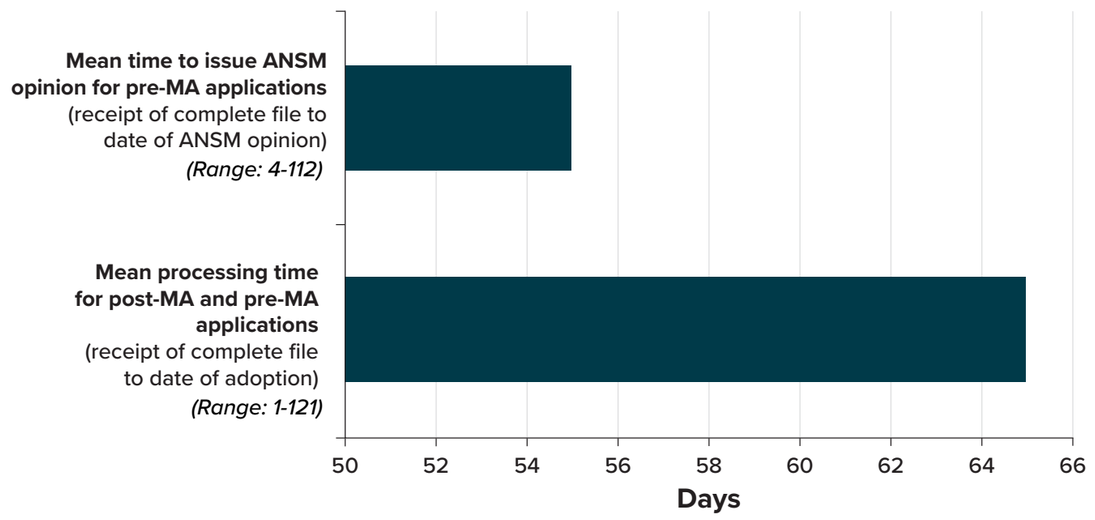
Note: ASMR accounts for the added clinical benefit of the new drug relative to existing treatment. The HAS Transparency Committee assesses the degree of ASMR on a scale from I (major benefit) to IV (minor benefit). A level V means absence of or no additional benefit.

- The main overarching reason for the refusal of early access was the "not presumed to be innovative." The specific reasons ranged as follows:
  - Availability of appropriate other treatment in the indication
    - Difficulty quantifying therapeutic contribution with preliminary data regarding medicinal alternatives
  - Lack of availability of pivotal superiority study results
  - Study methodological limitations
    - Lack of studies—performed or planned—in the very specific subpopulation of patients targeted
  - Development plan not adapted in the indication concerned

Processing Timeline

- The average time taken for the processing of all applications (receipt of complete file to date of adoption) was 65 days (range, 1-121).
- Mean time for ANSM opinion on the product's presumed efficacy and safety in the absence of MA was 55 days (range, 4-112) (Figure 3).

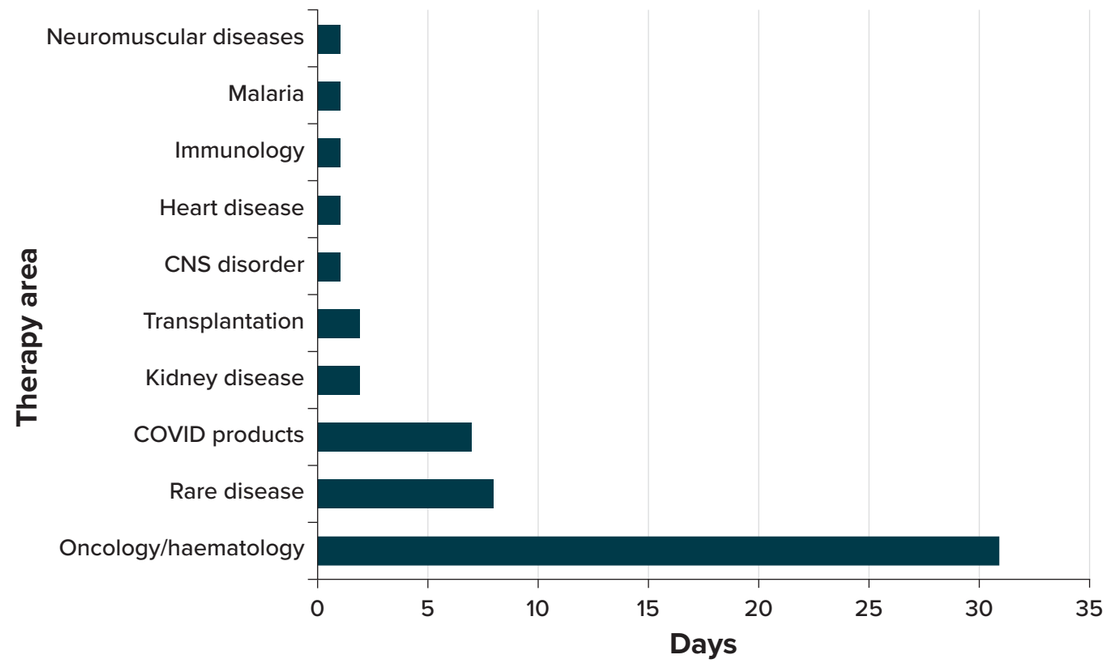
Figure 3. Application Processing Time (Days)



Distribution by Therapy Area

- Oncology/haematology treatments accounted for most of the applications that were granted early access, followed by treatments for rare diseases and COVID-19 (Figure 4).

Figure 4. Early Access Authorization by Therapy Area



Clinical Data Used and Stakeholder Contribution

- Most decisions were based on phase 2-3 clinical data for technologies with or without MA.
- Only 2 early access authorization requests that were granted before market authorization used phase 1 clinical data:
  - Treatments indicated for pre-exposure prophylaxis of COVID-19
  - Unresectable or metastatic triple-negative breast cancer
- Contributions from stakeholders, including patient and user groups, were included in 39 (59%) applications (both before and after MA).

CONCLUSION

Overall, the time taken to evaluate early access applications is significantly lower than the 3-month examination period stipulated by the program; this facilitates quick access for patients with high unmet need.

REFERENCES

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