

New framework for early access to drugs in France: feedback after a one-year use in university public hospitals of Paris



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OBJECTIVES

France has been a pioneer in setting up regulations allowing patients to be offered early access to drugs with a potential benefit. A new framework for early access to drugs was implemented in France in July 2021.



Fig 1. Pre-MA and post-MA early access assessment process

The aim of this study is to review the **use of early access drugs** (EAD) at Public Hospitals of Paris (AP-HP), in terms of consumption (expenditures and quantities), types of hospitals (general, follow-up care and rehabilitation and paediatric), and therapeutic areas involved (Anatomical Therapeutic Chemical (ATC) classes).

METHODS

PMSI hospital database (French medical information system program) was used to collect data, over the July 2021 - June 2022 period, on both the EAD use (quantities, expenditures and ATC Classification) and consumers (hospitals and hospital wards).

RESULTS

During the study period, **53 medicines** of EAD were used in AP-HP, representing **€34.115 million** of expenditures, **52 %** of which were due to a gene therapy drug and a CAR-T cell.

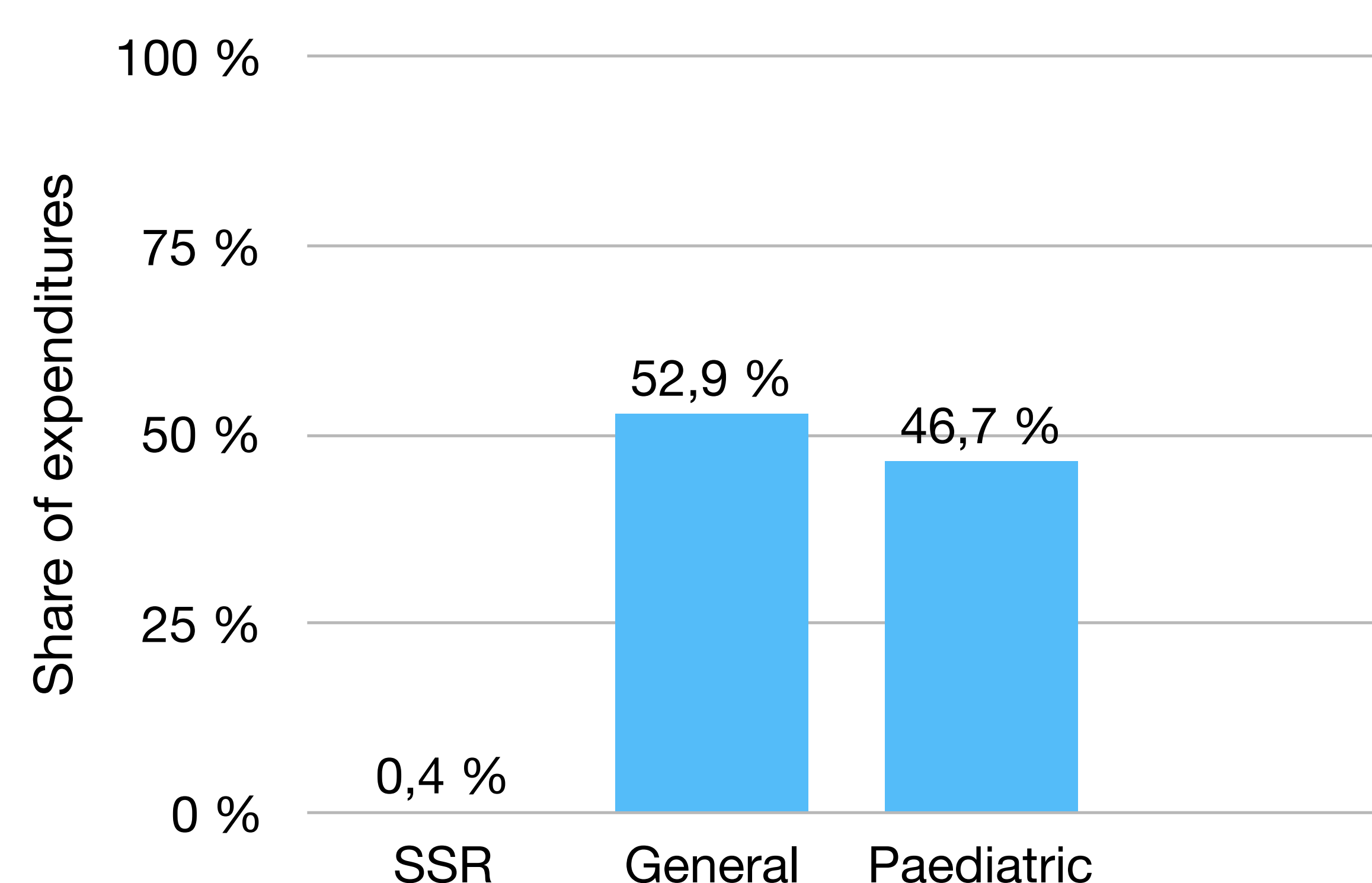


Fig 2. Share of EAD expenditures by hospital type

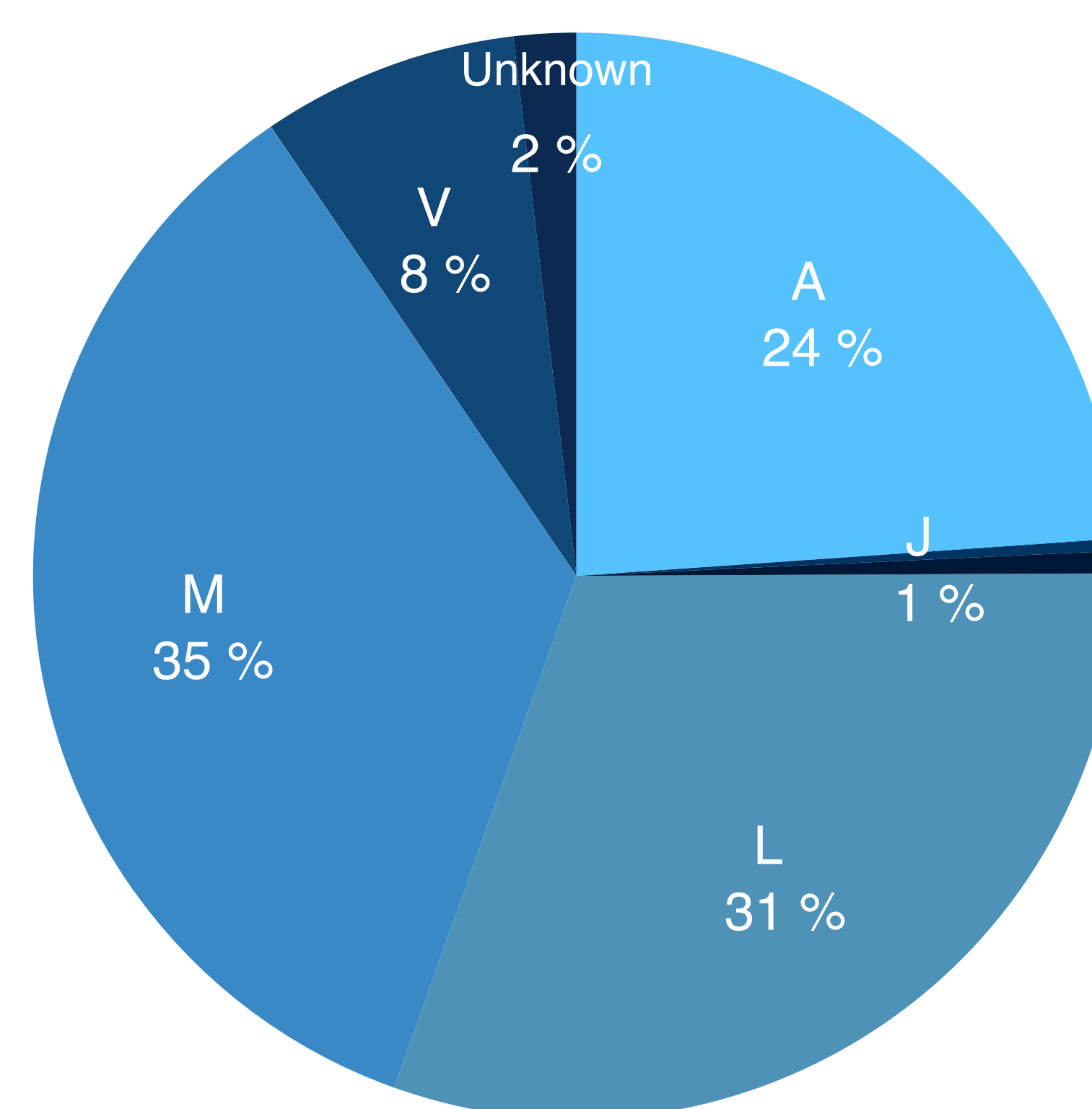


Fig 3. Share of EAD expenditures by ATC class

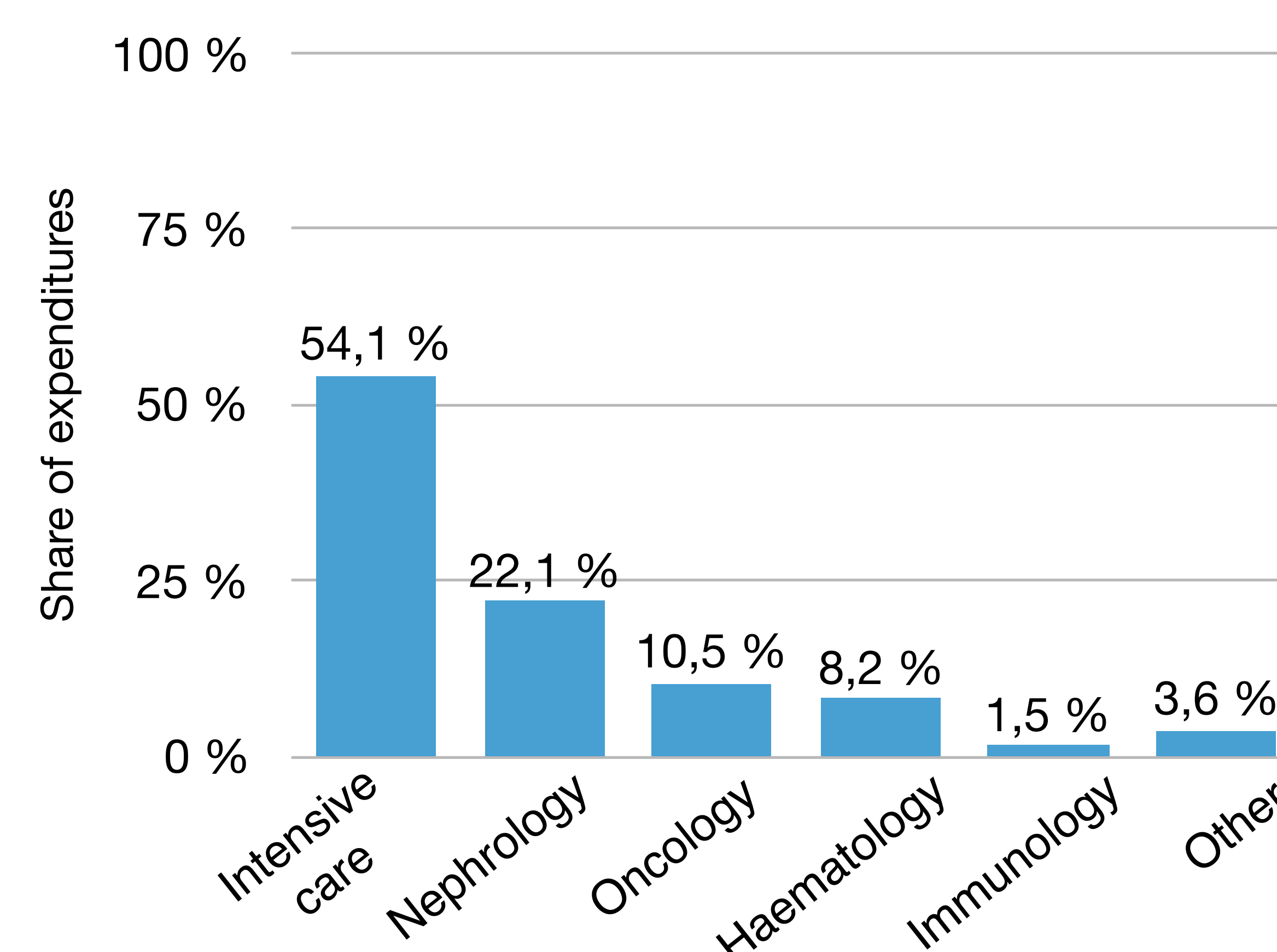


Fig 4. Share of EAD expenditures by hospital department

21 out of 37 hospitals in AP-HP used EAD. All types of hospitals are represented, **19 general** hospitals (medicine-surgery-obstetrics wards), **3 paediatric** hospitals, and **2 follow-up care and rehabilitation** (SSR) hospitals.

The three most represented ATC classes are **M - Musculo-skeletal system** (35 % of expenditures and <1 % quantities), **L - Antineoplastic and immunomodulating agents** (31 %, 20%) and **A - Alimentary tract and metabolism** (24 %, 60 %).

Intensive care, nephrology, and oncology-haematology departments were the main users of EAD, accounting for 95 % of expenditures.

CONCLUSION

EAD are widely used, in nearly **two-thirds of AP-HP hospitals**. The three main therapeutics areas involved (90 % of expenditures for M, L and A classes) are representative of EAD used in the three main departments mentioned (intensive care, nephrology, and oncology-haematology). EAD represent **significant expenditures, but low quantities**, due to the high individual cost of **innovative drugs**, in particular a gene therapy drug and a CAR-T cells. Maintenance of price and innovative nature will have to be confirmed after the French Health-Technology-Assessment process.