

EIGHTEEN-MONTH DESCRIPTIVE ANALYSIS OF EARLY ACCESS AUTHORIZATION AND COMPASSIONATE USE AUTHORIZATION DRUG PREPARATIONS IN A PHARMACY AT A CANCER CENTER

E.COSME¹; P.ROY-DJORDJEVIC¹; L.DENIS¹; M.ANNEREAU¹; B.DO¹

¹Gustave Roussy, Department of clinical pharmacy, 114, rue Édouard Vaillant - 94805 Villejuif Cedex - France
Contact : edouard.COSME@gustaveroussy.fr

Poster 4CPS-064

INTRODUCTION

DEROGATORY ACCESS FOR INNOVATIVE MEDICINES IN FRANCE:

Early Access Authorization (EAA) and Compassionate use authorization (CUA) are key regulatory pathways to provide innovative medicines to patients in France. This makes it possible to provide medicines that are not yet authorized or covered by common law. Hospital pharmacies are central to the order, preparation, traceability, and reporting of these drugs, which generates valuable real-world data. According to the Haute Autorité de Santé (HAS), 140,000 patients in France benefited from an EAA between 2021 and 2025 [1]. Oncology is the main therapeutic area with 75 EAA approvals [1]. Management of EAA and CUA is an important activity of our pharmacy at a cancer center.

AIM AND OBJECTIVES

The aim was to describe the characteristics of patients and medicines managed under EAA and CUA in our pharmacy at a cancer center over 18 months

METHODS

We retrospectively collected all drug preparations declared by our pharmacy under EAA or CUA between January 2024 and June 2025. Data included drug name, pharmaceutical class, route of administration, regulatory status (EAA or CUA), indication, and medical committee. Preparations were grouped by therapeutic area and analysed descriptively.

RESULTS

A total of **28 drugs** were used, **mainly injectables** (26/28). The main therapeutic classes were **immunotherapies** (16 drugs, 57%) and antibody–drug conjugates (5 drugs, 18%) (table 1).

Most patients were treated in oncology-related committees: **breast diseases** (387 patients, 2635 preparations), **haematology** (54, 532), **urology** (55, 426) (figure 1)

Route of administration	Total		Therapeutic classes	Total					
	Injectables drugs	Oral therapies		Immunotherapy	Antibody-drug conjugates	Conventional chemotherapy	Tyrosine kinase inhibitor	Others	
Number of drugs	26	2		16	5	2	1	4	28
Distribution (%)	93%	7%	100%	57%	18%	7%	4%	14%	100%

Table 1 : distribution of drugs per route of administrations and therapeutic classes

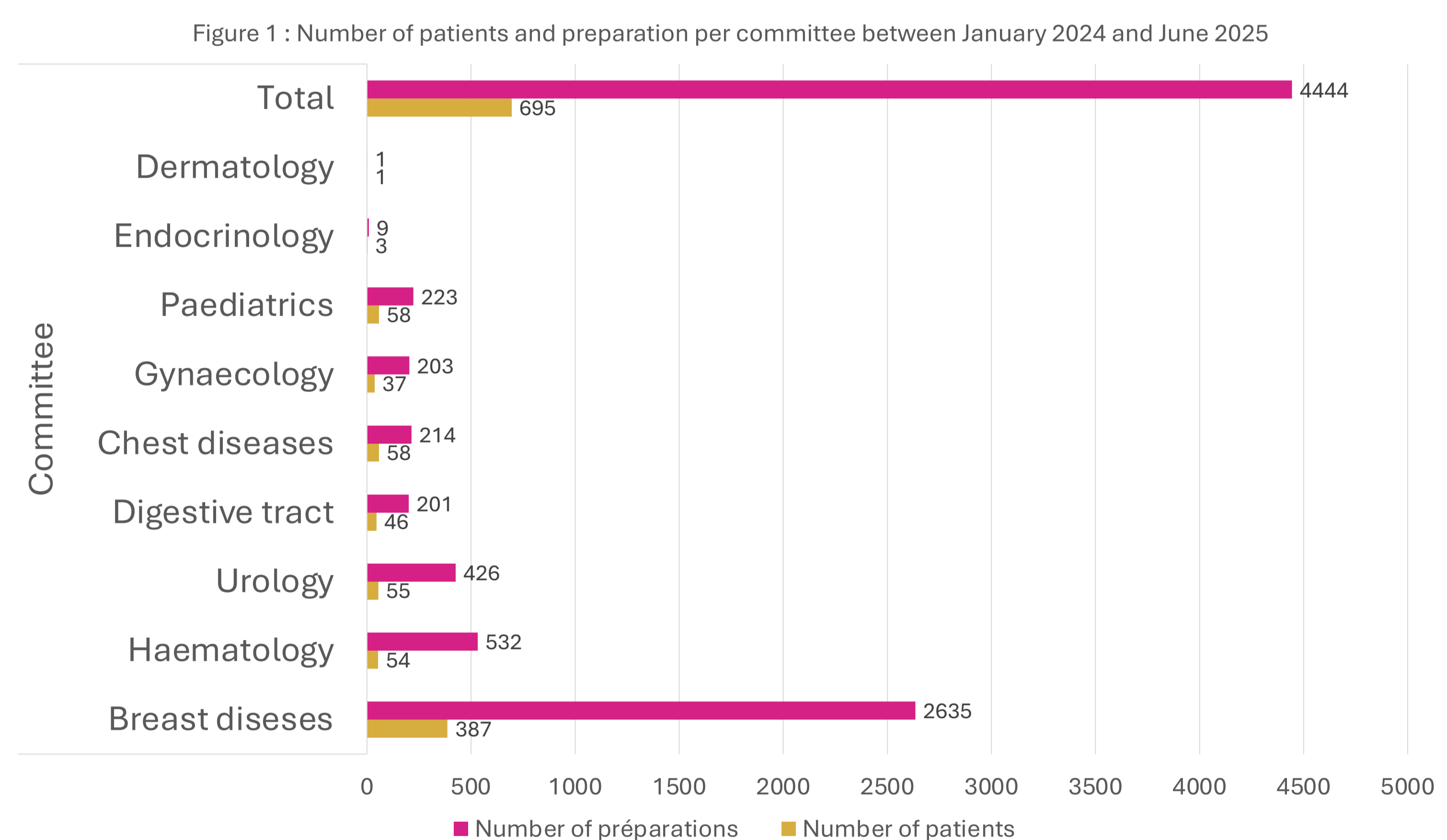
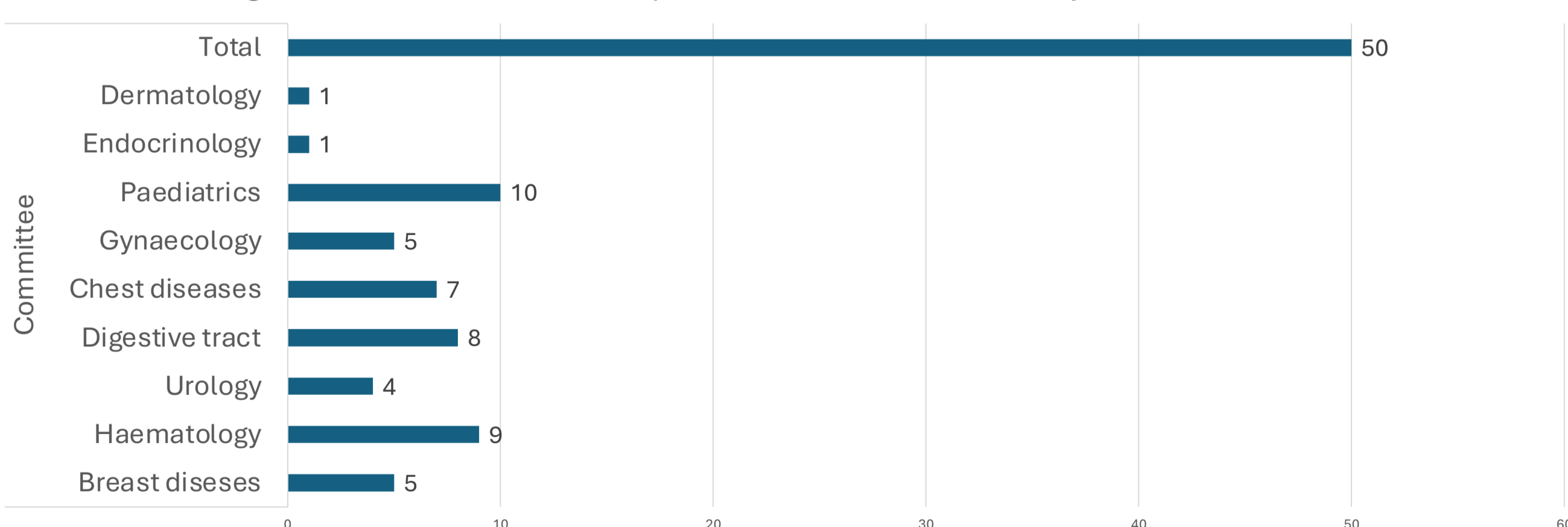


Figure 2 : Number of indication per committee between January 2024 and June 2025



In total, **50 different indications** were managed, mainly in **paediatrics** (10), **haematology** (9), **digestive tract** (8) (figure 2)

Main results
4444 preparations
695 patients
Main therapeutic class : immunotherapy
Main committee per patients : breast diseases
Main committee per indication : paediatrics

CONCLUSION

Our analysis highlights the major involvement of hospital pharmacy in EAA and CUA. This analyze if a first step to our future analyze of 5 years. Preparations allow to give access to 6% of our new patients to the most advance treatment. Paediatrics has the most indications, we will investigate this further. Beyond operational impact, these data could help anticipate transitions to routine care and support the collection of real-world evidence to complement clinical trial findings, thus contributing to drug evaluation and market access strategies.

REFERENCES

1. Haute Autorité de Santé [Internet]. [cité 7 févr 2026]. Accès précoce des médicaments : un bilan positif après deux ans de mise en place du dispositif. Disponible sur: https://www.has-sante.fr/jcms/p_3470178/fr/acces-precoce-des-medicaments-un-bilan-positif-apres-deux-ans-de-mise-en-place-du-dispositif

