

EIGHTEEN-MONTH DESCRIPTIVE ANALYSIS OF THE PAEDIATRIC POPULATION INCLUDED IN EARLY ACCESS AUTHORIZATION, COMPASSIONATE USE AUTHORIZATION, COMPASSIONATE PROGRAM IN A PHARMACY AT A CANCER CENTER

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INTRODUCTION

DEROGATORY ACCESS FOR INNOVATIVE MEDICINES IN FRANCE:

Early Access Authorization (EAA), Compassionate use authorization (CUA) and Compassionate Program (CP) are key regulatory pathways to provide innovative medicines to patients to paediatrics patients in France. Thanks to these programs many young patients at our cancer center have benefited from innovative treatments. 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, CUA and CP is an important activity of our pharmacy at a cancer center.

AIM AND OBJECTIVES

The aim was to describe the characteristics of paediatrics patients and medicines managed under EAA, CUA and CP in our hospital pharmacy over 18 months.

METHODS

We retrospectively collected all drugs provided by our pharmacy under EAA, CUA, CP between January 2024 and June 2025. Data included drug name, route of administration, regulatory status (EAA, CUA, CP) at dispensation and on 30th June 2025, indication. Dispensations were grouped by therapeutic area and analysed descriptively.

RESULTS

453 dispensations were done for 76 patients in the paediatrics committee (PC). This represents 8.5% of our population treated in these programs.

PC had the most indications of all our committee with 19 different indications. The second was chest disease (CD) (18 indications for 103 patients) and haematology the third (16 indications for 80 patients) (table 1)

	Committee				Total
	Paediatrics	Chest disease	Haematology	Other	
Number of indication code	19	18	16	46	98
Number of patient	76	103	80	623	882
Number of dispensation/preparation	453	373	693	3818	5337
Proportion of dispensation (%)	8,5%	7%	13%	71,5%	100%

Table 1 : distribution of drugs dispensed between 1st January 2024 and 30th June 2025 according committee

Compassionate (table 2) :

- At the time of dispensing : **12 indications** was in compassionate access (CUA + CP) for PC (9 for CD , 7 for haematology). This represented 12,2% of all our indications.

- On 30th June 2025 : **12 indications** for PC (7 for CD, 6 for haematology)

EAA (table 2) :

- At the time of dispensing : **2 indications** for PC (2% of all our indication) (5 for CD , 7 for haematology)

- On 30th June 2025 : **1 indication** for PC (3 for CD , 6 for haematology)

Reimbursement (table 2) :

For PC, **only one indication** obtained reimbursement (2 for CD , 3 for haematology)

	Committee				Total
	Paediatrics	Chest disease	Haematology	Other	
Number of indication code on CUA at the time of dispensing	11	9	6	21	47
Number of indication code on CP at the time of dispensing	1	0	1	1	3
Number of indication code on EAA at the time of dispensing	2	5	7	22	36
Number of indication code on "other" at the time of dispensing	5	5	2	6	18
Number of indication code on CUA on 30/06/25	11	7	5	7	30
Number of indication code on CP on 30/06/25	1	0	1	2	4
Number of indication code on EAA on 30/06/25	1	3	6	15	25
Number of indication code on "other" on 30/06/25	5	6	1	8	20
Number of indication code reimbursed on 30/06/25	1	2	3	14	20

Table 2 : distribution of indications code and their status of drugs dispensed between 1st January 2024 and 30th June 2025 according committee

A total of 10 drugs were used for the PC, mainly oral (9/10).

The main therapeutic classes were tyrosine kinase inhibitor (6 drugs) (table 3).

Route of administration	Injectables drugs	Oral therapies	Total	Therapeutic classes	Immunotherapy	Conventionnal chemotherapy	Tyrosine kinase inhibitor	Others	Total
Number of drugs	1	9	10		1	1	6	2	10
Distribution (%)	10%	90%	100%		10%	10%	60%	20%	100%

Table 3 : distribution of drugs dispensed between 1st January 2024 and 30th June 2025 according their route of administration and therapeutic classes

Main results for paediatrics committee

453 dispensations

76 patients

19 different indications

1 indication obtained a reimbursement on 30th June 2025

CONCLUSION

Our analysis highlights the major difficulties for access market in paediatric population. PC is the only committee in our hospital that has not seen a decrease in the number of indications. Plus, only 5,2% and the indication in PC obtained a reimbursement on 30th June 2025. This highlights the need to collect more real-world data in pediatrics to facilitate assessments and market access. The development of new pharmaceutical forms adapted to pediatrics by our pharmacy may also be a lever to facilitate market access.

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

