

Background and importance

Juvenile Myelomonocytic Leukaemia (JMML) is a paediatric haematological malignancy with a poor prognosis¹. In August 2019 in our paediatric hospital, we had a case of JMML. A protocol pharmacy related to medicinal product indication preparation, and flow chart instructions was made based on the information given by the patient's doctor, marketing authorization, and internet research. In Europe, azacitidine is indicated for the treatment of adult patients who are not eligible for haematopoietic stem cell transplantation (HSCT) with specific diagnostic criteria. The reconstituted solution should be injected subcutaneously². For this reason, diagnosis and route of administration, this request was an off-label use. Azacitidine is cytotoxic and is prepared in a centralized production unit, under the pharmacist's responsibility. In 2022 we intended to research the current state of the art.

Aim and objectives

A systematic review of azacitidine in the treatment of JMML, in a recent period of time. Based on the results the objective is to update the pharmacy protocol of our hospital and refresh the knowledge of the specific use of a cytotoxic drug.

Material and methods

To perform this work, we used the following databases: PubMed and Embase, limited to publication years from 01 January 2020 to 09 September 2022. Keywords included: azacitidine AND Juvenile Myelomonocytic Leukaemia.

- Step 1 – Doing the Database Search
- Step 2 – Articles excluded by the title
- Step 3 – Lecture and analysis of the summary of the articles
- Step 4 – Reading total articles previously selected and final selection.

An Excel table was made with the results (table 1).

Results

	Step 1	Step 2		Step 3			Step 4	
Database	Broad search	Reading the title		Lecture of the summary			Full reading of the articles	
PubMed	n= 10	Excluded (n=2)	Included (n=8)	Excluded (n=0)	Included (n=8)	duplicate articles (n= 6)	Excluded (n=8)	Included (n=6)
Embase	n= 47	Excluded (n=25)	Included (n=22)	Excluded (n=10)	Included (n=12)			

Table 1. Data synthesis

- We found 57 articles. Among them, 27 were excluded by reading the title and 10 by the summary. Among the 20 analysed manuscripts, 6 were repeated and 8 were excluded after reading the full text. Thus, 6 articles were selected for this review.

Conclusion and relevance

A significant change occurred in May 2022. Food and Drug Administration (FDA) approved azacitidine monotherapy as a suitable option for children with newly diagnosed JMML based on the results of the AZA-JMML-001 trial. Although long-term safety and efficacy remain to be fully elucidated in this population, the data demonstrate that azacitidine provides valuable clinical benefit to JMML patients prior to HSCT. In Europe, it has not yet been approved for this clinical situation. It is important to share treatments for rare diseases. Pharmacists are medication experts and play a critical role in this. Accordingly, we review a pharmacy protocol and updated azacitidine new findings.

References and acknowledgements

1. Gupta, A. K., Meena, J. P., Chopra, A., Tanwar, P., & Seth, R. (2021). Juvenile myelomonocytic leukemia-a comprehensive review and recent advances in management. American Journal of Blood Research, 11(1), 1–21. Embase.
2. EMA. SmPC Azacitidine. Available online https://www.ema.europa.eu/en/documents/product-information/vidaza-epar-product-information_en.pdf (accessed on 10 Sept 2022).

No conflict of interest.

