

Clinical activity of venetoclax and azacitidine in children with *de novo* or secondary multiple relapsed/refractory acute myeloid leukemia: a real-world experience

Agathe Arcourt,^{1*} Uri Ilan,^{2*} Laura Murillo,³ Nira Arad-Cohen,⁴ Alba Rubio,⁵ Sarah K. Tasian,^{2,6,7} André Baruchel,⁸ C. Michel Zwaan,^{2,9} Stephane Ducassou^{1#} and Bianca F. Goemans^{2#}

¹Department of Pediatric Hematology and Oncology, Bordeaux University Hospital, Bordeaux, France; ²Princess Máxima Center for Pediatric Oncology, Utrecht, the Netherlands; ³Pediatric Oncology and Hematology Department, Hospital Vall d'Hebrón, Barcelona, Spain; ⁴Rambam Medical Center, Haifa, Israel; ⁵Pediatric Oncology and Hematology Department, Hospital Infantil Universitario Niño Jesús, Madrid, Spain; ⁶Children's Hospital of Philadelphia, Division of Oncology and Center for Childhood Cancer Research, Philadelphia, PA, USA; ⁷University of Pennsylvania School of Medicine, Department of Pediatrics, Philadelphia, PA, USA; ⁸University Hospital Robert Debré (APHP) and Université de Paris, Paris, France and ⁹Department of Pediatric Oncology, Erasmus MC-Sophia Children's Hospital, Rotterdam, the Netherlands

*AA and UI contributed equally as first authors.

#SD and BFG contributed equally as senior authors.

Correspondence: B.F. Goemans
b.f.goemans@prinsesmaximacentrum.nl

Received: June 10, 2025.
Accepted: October 7, 2025.
Early view: October 16, 2025.

<https://doi.org/10.3324/haematol.2025.288246>

©2026 Ferrata Storti Foundation

Published under a CC BY-NC license



Clinical activity of venetoclax and azacitidine in children with de novo or secondary multiple relapsed/refractory acute myeloid leukemia: a real-world experience

Agathe Arcourt*¹ Uri Ilan*^{2,9}, Laura Murillo³, Nira A. Cohen⁴, Alba Rubio⁵, Sarah K Tasian^{6,7} André Baruchel⁸, C. Michel Zwaan^{2,9}, Stephane Ducassou**¹, Bianca F. Goemans**²

SUPPLEMENTARY DATA

Supplementary Table 1. Active and recently completed clinical trials of venetoclax and azacitidine-based therapies, including pediatric patients with acute leukemias.

Num	NCT Number	Study status	Phase	Disease type	Age	Venetoclax regimen and dose	Azacitidine regimen and dose	Funded By
1	NCT03194932	Completed	I	Acute Myeloid Leukemia	2 Years to 20 Years	for 21 days	for 7 days	academic
2	NCT03941964	Completed	III	Acute Myeloid Leukemia	12 Years and older	OD buiding up in 3 days for 28 days - 400mg	75mg/m2 OD for 7 days	Industry
3	NCT04161885	Recruiting	III	Acute Myeloid Leukemia	12 Years and older	OD for 28 days	OD for 5 days	Industry
4	NCT04588922	Recruiting	I/II	all Hematologic Malignancies	12 Years and older	NA	NA	Industry
5	NCT04904237	Recruiting	II	Acute Myeloid Leukemia	16 Years to 65 Years	NA	NA	academic
6	NCT05659992	Recruiting	I	Acute Myeloid Leukemia	14 Years to 75 Years	OD buiding up in 3 days for 14 days - 400mg	75mg/m2 OD for 7 days	academic
7	NCT05823714	Recruiting	II	Acute Myeloid Leukemia/MDS	Age 8 to 65 years	200mg/day for 7days	75mg/ m ² /day for 7days.	academic
8	NCT05955261	Recruiting	II	Acute Myeloid Leukemia	29 Days to 21 Years	NA	NA	Academia Industry
9	NCT06068621	Recruiting	II	Acute Myeloid Leukemia	14 Years to 75 Years	400 mg/day, days 1 to 14	75 mg/m2/day, days 1 to 7	academic
10	NCT06177067	Recruiting	I	Acute Myeloid Leukemia	1 Year to 30 Years	NA	NA	Academia Industry
11	NCT04161885	Recruiting	III	Acute Myeloid Leukemia	12 Years and older	OD for 28 days	OD for 5 days	Industry
12	NCT05292664	Not yet recruiting	I	Relapsed/Refractory Acute Leukemia	2 Years to 21 Years	NA	NA	Academia Industry
13	NCT05317403	Not yet recruiting	I	Acute Myeloid Leukemia	1 Year to 25 Years	OD buiding up in 3 days for 14 days - 400mg	75mg/m2 OD for 5 days	academic
14	NCT06397027	Not yet recruiting	I	Relapsed/Refractory Acute Leukemia	2 Years to 21 Years	NA	NA	Academia Industry

Supplementary Table 2: Other studies reporting venetoclax-based therapies in children with relapsed/refractory AML or MDS.

Publication	Total Patients Treated with Ven/Aza	Response to Treatment (% out of total)	Number of Patients Continuing to HSCT (% out of CR/CRi)
Trabal et al. 30 (Cancers 2023)	17	CR: 7 (41%) CRi: 1 (6%)	4 patients (44%)
Winters et al. 21 (Pediatric Blood & Cancer, 2020)	8	CR/CRi: 6 patients (66%)	4 patients (66%)
Niswander et al. 22 (Hematologica, 2023)	19	CR with MRD negativity: 9 patients (47%)	7 patients (78%) 1 continue to HSCT with MRD +
Masetti et al. 31 (Blood Advances, 2023)	22 (VEN/DEC)	CR: 7 patients (36.8%)	4 patients (57%) 1 continue to HSCT with PR
Total	65	CR/CRi: 31 patients (46%)	19 patients (61%)

CR - complete remission, CRi - complete remission with incomplete blood count recovery, HSCT - hematopoietic stem cell transplant, NA - not available.

Supplementary Figure 1: Overall survival for the entire cohort (n=38) since Day 1 of Ven/Aza

