

# KNOW AML

## Menin inhibitors:

What are they, and how are they used in AML?

**Menin inhibitors are a targeted treatment for AML with particular genetic changes.**

Menin inhibitors may help some patients with AML that has come back (relapsed) or that has not responded to treatment (refractory).

They can be used for patients with specific genetic changes

***KMT2A* gene mutations**



or

***NPM1* gene mutations**



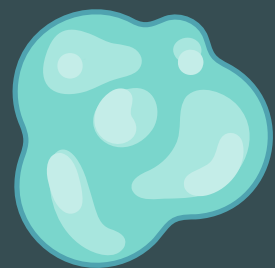
Genetic testing can be used to check whether patients have these gene changes and whether menin inhibitors could be used in their treatment.



# How do menin inhibitors work?

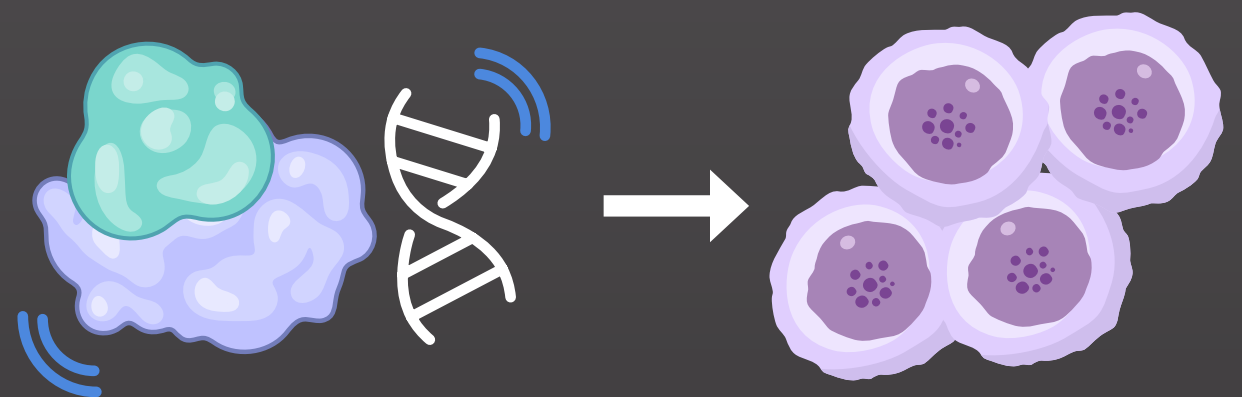
In AML, blood cells do not grow and develop in the way that they should, and become leukemia cells.

In types of AML with changes in the *KMT2A* or *NPM1* genes, these leukemia cells grow quickly and do not function normally.

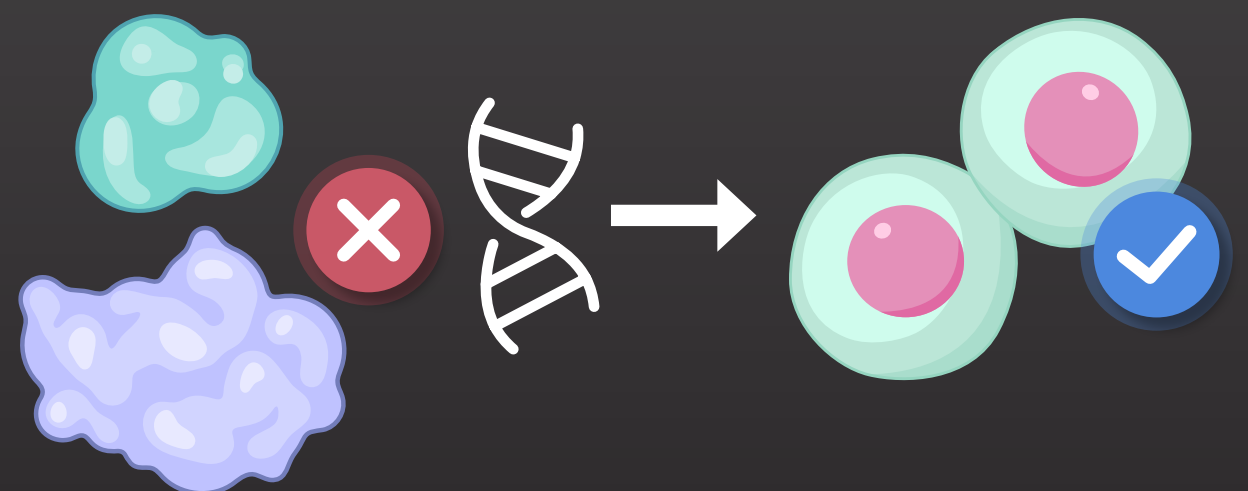


**Menin** is a protein that helps leukemia cells in these types of AML stay immature and keep growing, instead of developing into normal blood cells.

In AML with changes in the *KMT2A* or *NPM1* genes, **menin** forms a group with **other proteins** that together keep leukemia cells immature and growing quickly.



Menin inhibitors stop **menin** from working in this protein group, slowing leukemia cell growth and helping blood cells to grow normally.



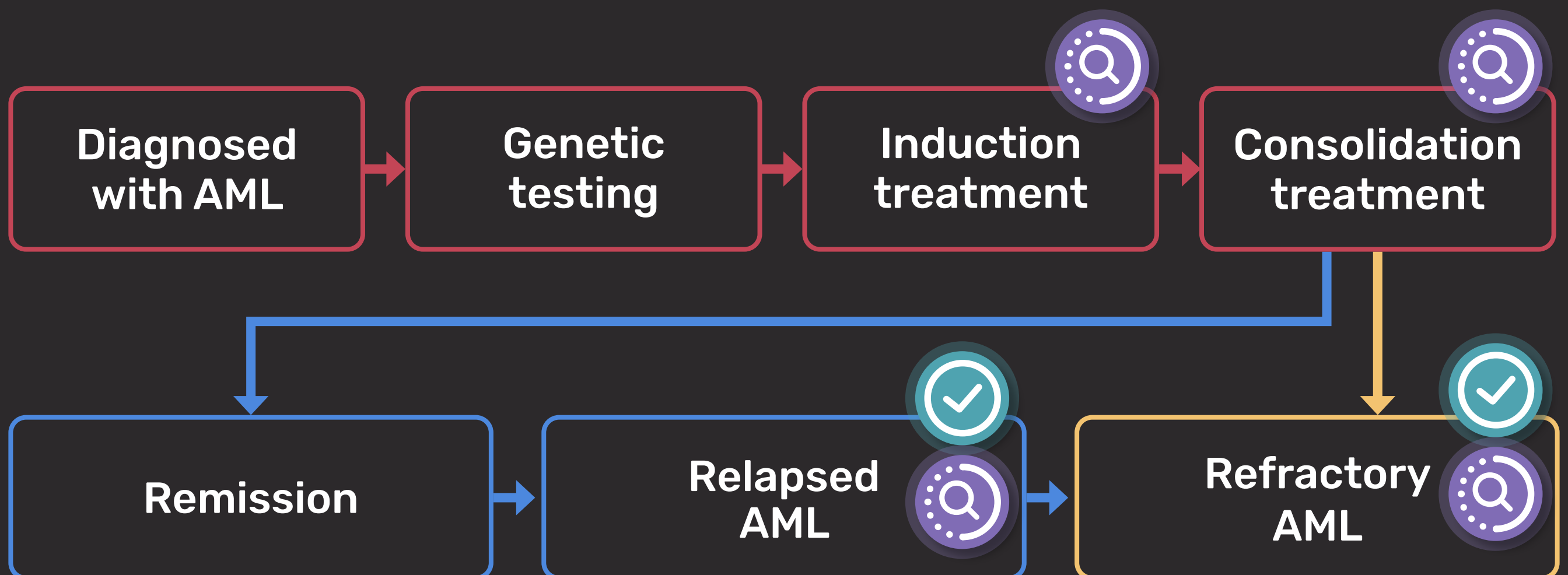
# When are menin inhibitors used?



Two menin inhibitors are currently **available for patients** whose AML has come back after treatment or hasn't responded to treatment, and has changes in the *KMT2A* or *NPM1* genes.



Other menin inhibitors are **being studied in clinical trials** to see how well they work and what side effects they cause. Trials are also testing menin inhibitors in combination with other treatments, such as chemotherapy, and in people who are newly diagnosed with AML.



# What are the possible side effects of menin inhibitors?

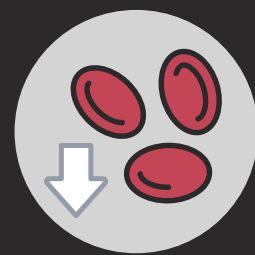
Like all cancer treatments, menin inhibitors have side effects. Not all patients will experience them, and they can be managed with supportive care and adjustments to treatment. Some possible side effects are:



Nausea



Diarrhea



Low blood cell counts



Fatigue



Heart rate changes



**Differentiation syndrome** is a known side effect of menin inhibitors. It does not occur in most patients, but it can be serious. Close monitoring allows early identification and treatment. It happens when white blood cells start maturing (differentiating) very quickly in response to treatment. Signs and symptoms include:

**fever • rapid weight gain • feeling unwell • breathlessness • swelling**

It most often appears between 1 and 4 weeks after starting treatment.



Menin inhibitors can also **interact with other medicines**, such as antifungals, so healthcare teams monitor patients closely and adjust doses if needed.

# Key points to remember



In types of AML with changes in the *KMT2A* or *NPM1* genes, menin helps leukemia cells to grow quickly.



Menin inhibitors stop menin from working with other proteins, preventing leukemia cells from growing and helping blood cells to mature normally.



Possible side effects of menin inhibitors include diarrhea, low blood cell counts, nausea, fatigue, and differentiation syndrome, which can usually be managed with supportive care and treatment adjustments.



Two menin inhibitors are approved for use in patients, and others are being studied in clinical trials to find out more about how well they work and their side effects.

This educational resource is independently supported by Sumitomo Pharma. All content was developed by SES in collaboration with an expert ambassador group. Funders were allowed no influence.

### References:

Nadiminti K, *et al. J Hematol Oncol.* 2024;17(1):113.

Mansour R, *et al. Int J Mol Sci.* 2026;27(4):1775.

Wang ES, *et al. J Clin Oncol.* 2025;43(31):3381-3390.

Arellano ML, *et al. Blood.* 2025;146(9):1065-1077.

Issa GC, *et al. J Clin Oncol.* 2025;43(1):75-84.

Syndax Pharmaceuticals. Medication guide: *Revumenib*.

<https://cms.syndax.com/wp-content/uploads/Revuforj-med-guide-IFU.pdf>.

Revised Oct 2025. Accessed May 13, 2026.

Kura Oncology, Inc. Medication guide: *Ziftomenib*.

<https://kuraoncology.com/wp-content/uploads/medicationguide.pdf>.

Published Nov 2025. Accessed May 13, 2026.

**KNOW  
AML**

**Know-AML.com**

Providing patients and caregivers  
with the information they need  
to deal with acute myeloid leukemia.

Brought to you by  
 **SES**