NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Decitabine–cedazuridine with venetoclax for treating acute myeloid leukaemia when intensive induction chemotherapy is unsuitable ID6601

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of decitabine–cedazuridine with venetoclax within its marketing authorisation for treating acute myeloid leukaemia when induction chemotherapy is unsuitable.

Background

Acute myeloid leukaemia (AML) is a cancer of the blood and bone marrow. It is characterised by the overproduction of early immature myeloid cells (blasts), which build up in the blood and bone marrow, interfering with normal blood cell production. AML progresses quickly over weeks or months and is fatal if not treated. Symptoms of AML include anaemia, bleeding problems, and serious infections. People with AML also feel fatigued, which can affect daily life.

In 2023, there were around 2,500 new cases of AML in England, with the highest incidence in people aged 75 to 79.[1] The 5-year survival rate of AML is around 15%.[2]

The aim of treatment for AML is to cure it. People who are fit enough can have intensive treatment. It is done in 2 phases: induction chemotherapy to reduce the number of blast cells, then consolidation chemotherapy to reduce the risk of recurrence. For people with good general health, the treatment options are intensive chemotherapy and allogeneic haematopoietic stem cell transplant (HSCT).

Over half of patients with AML are ineligible for intensive chemotherapy and HSCT because of factors such as age or comorbidities. Other treatment options for this population include azacitidine, ivosidenib, low dose cytarabine and venetoclax.

NICE technology appraisal guidance 218 recommends azacitidine for adults who are not eligible for HSCT and have AML with 20% to 30% blasts and multilineage dysplasia, according to the World Health Organization classification.

NICE technology appraisal guidance 765 recommends venetoclax with azacitidine for untreated AML in adults when intensive chemotherapy is unsuitable.

NICE technology appraisal guidance 787 recommends venetoclax with low dose cytarabine for untreated AML in adults when intensive chemotherapy is unsuitable, if they have over 30% bone marrow blasts.

NICE technology appraisal guidance 979 recommends ivosidenib plus azacitidine as an option for untreated acute myeloid leukaemia (AML) with an IDH1 R132 mutation in adults who cannot have standard intensive induction chemotherapy.

The technology

Decitabine-cedazuridine (Inaqovi, Otsuka Pharmaceutical) with venetoclax (Venclyxto, AbbVie) does not currently have a marketing authorisation in the UK for AML. It has been studied in a single arm clinical trial in adults with AML who are ineligible for intensive induction chemotherapy.

Decitabine-cedazuridine has a marketing authorisation as monotherapy for the treatment of adult patients with newly diagnosed AML who are ineligible for standard induction chemotherapy.

Intervention(s)	Decitabine–cedazuridine with venetoclax
Population(s)	Adults with acute myeloid leukaemia in whom intensive induction chemotherapy is not suitable
Comparators	• Venetoclax with azacitidine • Venetoclax with low dose cytarabine (for adults with over 30% bone marrow blasts) • Azatidicine (for adults not eligible for HSCT and have AML with 20% to 30% blasts and multilineage dysplasia) • Ivosidenib with azacitidine (for adults with an IDH1 R132 mutation) • Low dose cytarabine
Outcomes	The outcome measures to be considered include: • overall survival • event-free survival • disease-free survival • response rates, including remission • blood transfusion dependence • rate of complete remission and complete remission with partial haematologic recovery • adverse effects of treatment • health-related quality of life.

Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. If the technology is likely to provide similar or greater health benefits at similar or lower cost than technologies recommended in published NICE technology appraisal guidance for the same indication, a cost comparison may be carried out. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective. The availability of any commercial arrangements for the intervention, comparator and subsequent treatment technologies will be taken into account.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations	**Related technology appraisals:** Ivosidenib with azacitidine for untreated acute myeloid leukaemia with an IDH1 R132 mutation (2024). NICE technology appraisal guidance 979. Venetoclax with low dose cytarabine for untreated acute myeloid leukaemia when intensive chemotherapy is unsuitable (2022) NICE technology appraisal guidance 787. Venetoclax with azacitidine for untreated acute myeloid leukaemia when intensive chemotherapy is unsuitable (2022) NICE technology appraisal guidance 765. Azacitidine for treating acute myeloid leukaemia with more than 30% bone marrow blasts (2016) NICE technology appraisal guidance 399. Azacitidine for the treatment of myelodysplastic syndromes, chronic myelomonocytic leukaemia and acute myeloid leukaemia (2011) NICE technology appraisal guidance 218 . **Related NICE guidelines:** Haematological cancers: improving outcomes (2016) NICE guideline NG47.

	Related quality standards: Haematological cancers (2017) NICE quality standard 150

Questions for consultation

Where do you consider decitabine–cedazuridine with venetoclax will fit into the existing care pathway for AML?

Have all the relevant comparators been included?

Can people have intensive induction therapy and then "standard" consolidation therapy rather than intensive chemotherapy at both steps?

Please select from the following, will decitabine–cedazuridine with venetoclax be:
A. Prescribed in primary care with routine follow-up in primary care
B. Prescribed in secondary care with routine follow-up in primary care
C. Prescribed in secondary care with routine follow-up in secondary care
D. Other (please give details):
For comparators and subsequent treatments, please detail if the setting for prescribing and routine follow-up differs from the intervention.

Would decitabine–cedazuridine with venetoclax be a candidate for managed access?

Do you consider that the use of decitabine–cedazuridine with venetoclax can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

Please indicate if any of the treatments in the scope are used in NHS practice differently than advised in their Summary of Product Characteristics. For example, if the dose or dosing schedule for a treatment is different in clinical practice. If so, please indicate the reasons for different usage of the treatment(s) in NHS practice. If stakeholders consider this a relevant issue, please provide references for data on the efficacy of any treatments in the pathway used differently than advised in the Summary of Product Characteristics.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which decitabine–cedazuridine with venetoclax will be licensed;

- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;

- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE is considering evaluating this technology through its cost comparison evaluation process.
Please provide comments on the appropriateness of appraising this topic through this process.
(Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

Technologies can be evaluated through the cost-comparison process if they are expected to provide similar or greater health benefits, at a similar or lower cost, compared with technologies that have been previously recommended (as an option) in published NICE guidance for the same indication. Companies can propose cost-comparison topics to NICE at any stage during topic selection and scoping. NICE will route technologies for evaluation through the cost-comparison process if it is agreed during scoping that the process is an appropriate route to establish the clinical and cost effectiveness of the technology.

NICE's health technology evaluations: the manual states the methods to be used where a cost comparison case is made.

- Is the technology likely to be similar in its clinical effectiveness and resource use to any of the comparators? Or in what way is it different to the comparators?
- Will the intervention be used in the same place in the treatment pathway as the comparator(s)? Have there been any major changes to the treatment pathway recently? If so, please describe.
- Will the intervention be used to treat the same population as the comparator(s)?
- Overall is the technology likely to offer similar or improved health benefits compared with the comparators?
- Would it be appropriate to use the cost-comparison methodology for this topic?

References

1. NHS Digital (2025). Cancer registration statistics in England, 2023.Accessed December 2025.
2. Cancer Research UK (2023). Survival for acute myeloid leukaemia (AML). Accessed December 2025.