



Deposited via The University of York.

White Rose Research Online URL for this paper:

<https://eprints.whiterose.ac.uk/id/eprint/244888/>

Version: Published Version

Article:

Williamson, Joab, Searle, Emma J, Louramo, Elina et al. (2026) Health-system burden of higher-risk myelodysplastic syndromes in England: a literature-based micro-cost analysis. BMJ Open. e119858. ISSN: 2044-6055

<https://doi.org/10.1136/bmjopen-2026-119858>

Reuse

This article is distributed under the terms of the Creative Commons Attribution-NonCommercial (CC BY-NC) licence. This licence allows you to remix, tweak, and build upon this work non-commercially, and any new works must also acknowledge the authors and be non-commercial. You don't have to license any derivative works on the same terms. More information and the full terms of the licence here:

<https://creativecommons.org/licenses/>

Takedown

If you consider content in White Rose Research Online to be in breach of UK law, please notify us by emailing eprints@whiterose.ac.uk including the URL of the record and the reason for the withdrawal request.

BMJ Open Health-system burden of higher-risk myelodysplastic syndromes in England: a literature-based micro-cost analysis

Joab Williamson ¹, Emma J Searle,² Elina Louramo,¹ Ralph Hughes,¹ Petri Bono ¹, Vijay S Gc ³

To cite: Williamson J, Searle EJ, Louramo E, *et al.* Health-system burden of higher-risk myelodysplastic syndromes in England: a literature-based micro-cost analysis. *BMJ Open* 2026;**16**:e119858. doi:10.1136/bmjopen-2026-119858

► Prepublication history and additional supplemental material for this paper are available online. To view these files, please visit the journal online (<https://doi.org/10.1136/bmjopen-2026-119858>).

Received 19 March 2026

Accepted 12 August 2026



© Author(s) (or their employer(s)) 2026. Re-use permitted under CC BY-NC. No commercial re-use. See rights and permissions. Published by BMJ Group.

¹Faron Pharmaceuticals Oy, Turku, Southwest Finland, Finland

²The Christie NHS Foundation Trust, Manchester, UK

³Centre for Health Economics, University of York, York, UK

Correspondence to

Dr Joab Williamson;
joab.williamson@faron.com

ABSTRACT

Objectives To quantify the per-patient-per-month (PPPM) cost for each phase of care in higher-risk myelodysplastic syndromes (HR-MDS) and the overall cost of a base-case (illustrative) non-curative management pathway followed by a patient with HR-MDS in England.

Design We conducted a retrospective, literature-based, micro-costing analysis from the National Health Service (NHS) England provider perspective using published sources and publicly available price lists. No individual-patient data were used. Based on literature, a base-case management pathway followed by a patient with HR-MDS was defined as a diagnostic work-up at month 0, 12 cycles of azacitidine (given for 7 days in each 28-day cycle), 5 months of post-hypomethylating agent (HMA) failure supportive care and 1 month of terminal care.

Setting Healthcare resource utilisation was analysed for adults with HR-MDS who received first-line azacitidine in routine practice if cycle-level or phase-level transfusion and admission rates were reported in the literature. Patients who required allogeneic haematopoietic stem cell transplantation or whose HR-MDS transformed to acute myeloid leukaemia were excluded because the diagnostic and/or therapeutic pathways differ. Unit costs for 2024/2025 were taken from published English national sources and an English trust tariff.

Results PPPM costs were £8721.58 during active azacitidine therapy, £5399.58 after HMA failure and £12 554.58 for hospital-dominant terminal care; a one-off diagnostic work-up with genomics cost £2710 to £2810. The total cost for the base-case management pathway was £146 921 per patient. An alternative pathway excluding genomic testing and assuming hospice-dominant terminal care reduced the total cost to approximately £136 840 to £140 990, depending on whether the lower or upper bound of hospice bed-day costs is applied.

Conclusions The direct NHS-provider cost burden of this non-curative HR-MDS management pathway is concentrated in azacitidine acquisition and administration during active treatment, transfusions and admissions after HMA failure and setting-dependent costs at the end of life. The total cost for the illustrative management pathway followed by a patient with HR-MDS (£146 921) is of a similar order to the estimate in the National Institute for Health and Care Excellence technology appraisal for azacitidine updated to £124 848 for 2024/2025. This comparison is provided for context only and should not

STRENGTHS AND LIMITATIONS OF THIS STUDY

- ⇒ Uses only publicly available English sources (British National Formulary, National Health Service (NHS) Blood and Transplant price lists, Royal Devon University Healthcare NHS Foundation Trust tariffs and Personal Social Services Research Unit costs), making the costing framework reproducible and auditable.
- ⇒ Disaggregates higher-risk myelodysplastic syndromes care into four clinically meaningful phases and reports per-patient-per-month costs for each phase, which can inform budget-impact models.
- ⇒ Anchors resource utilisation in contemporary real-world studies and UK clinical experience rather than expert opinion alone, improving the plausibility of healthcare-resource assumptions.
- ⇒ Excludes costs after transformation to acute myeloid leukaemia and after allogeneic stem cell transplantation, so the estimates do not capture lifetime haematological costs for those subgroups.
- ⇒ Does not include primary-care activity or community-based care, and some unit prices (eg, for hospital haematology inpatient stay) are drawn from a single trust's tariff; local adaptations should therefore substitute local prices where available.

be interpreted as validation of the present model, given differences in population, model structure, treatment duration and price year. The phase-specific PPPM estimates can inform, subject to local validation and scenario testing, UK budget-impact analyses, service planning and future economic models.

INTRODUCTION

Higher-risk myelodysplastic syndromes (HR-MDS) are generally incurable in routine practice; allogeneic haematopoietic stem cell transplantation (allo-HSCT) remains the only treatment with curative potential, but is unsuitable for many patients because of age, comorbidity and donor constraints; consequently, approximately 5% of patients receive transplants.^{1 2} The natural history of HR-MDS is dominated by complications of bone marrow failure, including infections

and bleeding and by a substantial risk of progression to acute myeloid leukaemia (AML).^{2,3}

In the UK, hypomethylating agents (HMAs) constitute first-line disease-modifying therapy for most higher-risk cases and azacitidine is the established standard.⁴ Although the survival benefit of azacitidine over conventional care was demonstrated in trial AZA-001 and related analyses, real-world cohorts typically show lower treatment persistence and substantial ongoing supportive-care needs.^{5–8} This is because baseline patient and disease characteristics often differ between clinical trial and real-world populations.^{6–8} In contrast to the controlled setting of clinical trials, real-world studies tend to include frailer patients with HR-MDS and several comorbidities. In addition, in AZA-001, the patient population was not confined to patients with HR-MDS but also included patients with AML and chronic myelomonocytic leukaemia, and used the original International Prognostic Scoring System (IPSS) to assess risk,⁵ which has since received multiple enhancements.

In the AZA-001 trial, azacitidine was given for a median of nine cycles (IQR 4–15) with a median cycle-length of 28 days (IQR 28–35). In a recent Danish real-world study, patients with HR-MDS received azacitidine for a median of 6 (IQR 3–11) cycles with a median cycle length of 28 days.⁸ In contrast, the overall population with HR-MDS of a real-world study conducted across three European countries received azacitidine for a median of 12 cycles, and the UK subgroup of this study received azacitidine for a median of 13.5 cycles.⁷ The patients included in the latter study appeared to have fewer comorbidities than patients in previously reported studies. In previous real world, international studies, the duration of treatment for azacitidine in populations with HR-MDS has been vastly different based on the demographics, with one real-world study finding a median HMA duration of 7.4 months⁹ and another finding a median of 15.6 months.¹⁰

UK tariffs for outpatient chemotherapy attendances, day-case transfusion administration and haematology inpatient bed-days, together with nationally set blood-component prices and palliative-care day rates, provide a transparent basis for micro-costing the burden of HR-MDS care within National Health Service (NHS) England.^{11–13} Globally, the burden of myelodysplastic syndromes is increasing in parallel with population ageing. A recent Global Burden of Disease analysis of combined myelodysplastic syndromes (MDS) and myeloproliferative neoplasms estimated that incident cases almost doubled between 1990 and 2021 (from 171 132 to 341 017 cases), with disability-adjusted life-years (DALYs) and deaths rising in absolute terms despite some attenuation in age-standardised rates; the burden was disproportionately concentrated in high-Socio-demographic Index countries such as the UK.¹⁴ At the patient level, a recent scoping review collated 56 epidemiological and 46 health-related quality of life (HRQoL) studies and reported crude prevalence estimates of 6.2–12.0 per 100 000 in registries, 5-year survival of 27–46% and substantial decrements in

HRQoL, with European Organisation for Research and Treatment of Cancer QLQ-C30 global health scores in the range of 50–67 points and EQ-5D-based utilities around 0.73–0.83.¹⁵

Economic evaluations and cost-of-illness studies have similarly highlighted the resource intensity of MDS, particularly among patients with higher-risk disease. A recent systematic review of economic burden identified wide variation in methods and costs across 56 studies, but consistently found that inpatient care, systemic therapies (notably HMAs) and transfusions were dominant components of direct medical costs.¹⁶ In a US claims analysis of treated HR-MDS, mean per-patient-per-month (PPPM) costs were approximately US\$17 361 (2015 US dollars), of which around two-thirds were attributable to medical services, with MDS-related chemotherapy and supportive care representing the major drivers.¹⁷ Earlier UK work used a burden-of-disease framework and estimated the annual national cost of managing intermediate-2 and high-risk MDS at £12 to £16 million, driven chiefly by hospitalisations and transfusions, with smaller contributions from community nurse visits and caregiver time.¹⁸

Despite this, few UK-specific studies disaggregate costs by clinically meaningful phases of care for HR-MDS (ie, diagnosis, active HMA therapy, post-HMA failure, terminal care) and they do not report PPPM costs that commissioners and service planners can reuse. In the National Institute for Health and Care Excellence (NICE) guidelines for azacitidine, a manufacturer analysis from trial AZA-001 found that the expected total healthcare cost for a patient treated with azacitidine was estimated to be £91 800 in 2011 (£124 848 in 2024/2025).⁴

Recent real-world evidence also highlights the intensity of healthcare resource utilisation (HCRU) during azacitidine therapy of HR-MDS in Denmark.⁸ Hospitalisation occurred in 75% of these patients. Red blood-cell (RBC) transfusions were required by 89% of patients. This emphasises the need to reflect transfusion and hospital admission patterns in financial estimates.

There are no approved standard of care disease-modifying therapy options after HMA failure in the UK.¹⁹ Median overall survival after azacitidine failure remains short at approximately 5–6 months, implying a distinct, high-burden post-HMA failure phase.²⁰

Therefore, this analysis couples contemporary English price benchmarks with HCRU reported in key real-world studies to quantify the PPPM cost for each phase of care in HR-MDS and the overall cost of a base-case non-curative management pathway followed by a patient with HR-MDS in England. To focus purely on the impact of non-curative management of HR-MDS on health-system burden, this analysis excludes costs associated with HR-MDS transformation to AML and potentially curative stem-cell transplantation because the diagnostic and/or therapeutic pathways are vastly different.²¹

METHODS

Study design and perspective

We conducted a retrospective, literature-based micro-costing analysis from the NHS England provider perspective. Costs were derived using contemporaneous, publicly available sources in England. No individual-patient data were used; all data were taken from public sources and published literature as cited. Reporting follows the Consolidated Health Economic Evaluation Reporting Standards (CHEERS) 2022 guidance for economic evaluations of health interventions.²²

Patient and public involvement

Patients and members of the public were not involved in the design, conduct, reporting or dissemination plans of this research, which was based entirely on published sources and publicly available price lists.

Study population

As MDS is primarily a disease of adults, this analysis of HCRU included adults ≥ 18 years of age diagnosed with HR-MDS (IPSS revised (IPSS-R) intermediate/high/very high risk) and treated with azacitidine.

The analysis excluded patients who required allo-HSCT or whose HR-MDS transformed to AML as the diagnostic and/or therapeutic pathways differ. Because azacitidine was the standard first-line hypomethylating agent in this setting, the terms 'HMA failure' and 'azacitidine failure' are used synonymously throughout; the post-HMA failure supportive care phase denotes the period following azacitidine discontinuation for treatment failure.

Identification of evidence on healthcare resource use

We undertook a targeted literature search to identify observational studies reporting HCRU among adults with HR-MDS treated with azacitidine in routine practice. Searches were conducted in MEDLINE (via PubMed) and Embase from 2004, when azacitidine registration studies began to appear, to January 2025; the search was updated in January 2026. Although the evidence base in this clinical area is evolving, the update did not identify any additional study that could inform the model parameters beyond the primary and supporting sources already included. Searches used combinations of the terms 'myelodysplastic syndromes', 'higher-risk', 'azacitidine', 'resource use', 'transfusion', 'hospitalisation' and 'real-world'. We also screened reference lists of key clinical trials and health technology assessments, and hand-searched recent haematology conference abstracts (American Society of Hematology, British Society of Haematology and European Hematology Association). Studies were eligible if they included adults with HR-MDS who received first-line azacitidine in routine practice and reported cycle-level or phase-level transfusion and admission rates. Records were screened against these criteria by one author, with an independent screen by a second author and discrepancies resolved through discussion. Because the objective was to parameterise a deterministic micro-costing model

rather than to synthesise comparative effectiveness, this was a targeted (pragmatic) evidence review rather than a formal systematic review; formal dual independent screening and quantitative risk-of-bias scoring, which are designed for effectiveness syntheses, were therefore not applied. No individual patient-level data were used and there were consequently no missing patient-level data to impute; where a specific resource quantity was not directly reported in a source, it was informed by triangulation across cohorts and UK clinical input, as described below. The full search strategy for each database, a flow summary of screening and tables of the included and of the cross-checked or excluded studies (with reasons and a structured assessment of their applicability) are provided in the online supplemental material. Two contemporary multicountry chart reviews, Drummond *et al*⁷ and Brommann *et al*,⁸ were prioritised as primary data sources because they provided detailed cycle-level utilisation data and included European and UK centres.^{7,8} Other publications were used to cross-check transfusion intensity, admission rates and survival, but did not contain sufficient detail to parameterise the model. For end-of-life assumptions, we supplemented these sources with studies that described the intensity of terminal care, hospice use and hospitalisation among patients with MDS and other haematological malignancies.^{23–25}

Costing approach

All costs are expressed in British pound sterling (£). Unit costs in 2024/2025 were taken from published English national sources and an English trust tariff. If costs were provided for the previous year, they were inflated to 2024/2025 prices using the NHS Cost Inflation Index (NHSCII). The price of each healthcare resource unit used in the analysis, along with its source, is provided in table 1.

The British National Formulary (BNF) price was used for an azacitidine 150 mg vial; this price was similar to the total price of two 100 mg vials listed at the lowest price.²⁶ Transfusion components were costed using NHS Blood and Transplant price lists which are set centrally.¹¹ For any costs where the 2024/2025 costing was unavailable, the costs from the previous year (2022/2023) were uprated to 2024/2025 using the NHSCII. A Royal Devon University Healthcare NHS Foundation Trust non-contract tariff was used for costing outpatient chemotherapy attendances, outpatient reviews, hospital day admissions, bone-marrow procedures and hospital haematology inpatient stays.¹² This trust's tariff was used because it is published, comprehensive and reflective of NHS England practice. The unit costs from the Personal Social Services Research Unit (PSSRU) were used for specialist palliative-care bed-days.¹³

We applied micro-costing (bottom-up) by multiplying the monthly amount of a resource item used by a patient for a phase by its unit cost and summing all these values to obtain the PPPM for each phase. Details are provided in table 2.

Table 1 Price in 2024/2025 of each healthcare resource unit used in the analysis and the source of its price

Type of healthcare resource unit	Cost per unit (£)	Source of price
HMA therapy		
Azacitidine (150 mg vial)	£481	BNF 2025 ²⁶
Outpatient attendances		
Chemotherapy	£280	Royal Devon University Healthcare NHS Foundation Trust 2024 ¹²
Non-chemotherapy	£165	Royal Devon University Healthcare NHS Foundation Trust 2024 ¹²
Hospital day admission for transfusions		
≤4 hours	£340	Royal Devon University Healthcare NHS Foundation Trust 2024 ¹²
4–8 hours (typical for 2 × RBC units)	£545	Royal Devon University Healthcare NHS Foundation Trust 2024 ¹²
Transfusion components		
One RBC unit	£153.30	NHS Blood and Transplant 2024 ¹¹
One therapeutic dose of platelets for adults	£275.21	NHS Blood and Transplant 2024 ¹¹
Hospital haematology inpatient stay per night	£915	Royal Devon University Healthcare NHS Foundation Trust 2024 ¹²
Procedures/diagnostics		
Bone-marrow aspirate and trephine	£870	Royal Devon University Healthcare NHS Foundation Trust 2024 ¹²
Myeloid NGS sequencing	Separate scenario: £1200	NHS England 2024, NHS England 2025 ^{29 30}
Karyotype test/FISH	Separate scenario: £300 to £400	National Genomic Test Directory 2024, National Genomic Test Directory 2025 ^{29 30}
Specialist palliative-care bed per day	£397 to £812 (uplifted to 2024/2025 pricing per NHSCII)	Jones <i>et al</i> 2022, Jones <i>et al</i> ^{13 31}
BNF, British National Formulary; FISH, fluorescence in situ hybridisation; HMA, hypomethylating agent; NGS, next-generation sequencing; NHSCII, NHS Cost Inflation Index; RBC, red blood cell.		

Phases of care

We modelled four phases of care that are consistent with clinical decision-making: (1) diagnostic work-up; (2) active HMA therapy; (3) post-HMA failure supportive care; and (4) terminal care. The total cost of each phase was calculated using a base-case non-curative management pathway followed by a patient with HR-MDS that was consistent with real-world persistence on HMA therapy (12 cycles/months of azacitidine as reported in a

European study including UK patients) and with approximately 5–6 months of survival after HMA failure.^{7 20} Literature indicates that around 50% of patients received end of life hospice care with the duration usually <1 month.^{23 24} Therefore, the base-case management pathway followed by a patient with HR-MDS in our analysis consisted of a diagnostic bundle at month 0, 12 cycles of subcutaneous azacitidine (given for 7 days in each 28-day cycle), 5 months post-HMA failure supportive care and 1 month of terminal care.²⁷

Healthcare resource-utilisation and assumptions

Monthly HCRU for one-off diagnostics and each phase of care was parameterised as PPPM counts for each resource (eg, outpatient chemotherapy attendances, day-case transfusions, inpatient nights and palliative-care bed-days) used by a patient with HR-MDS following the base-case non-curative management pathway. Where possible, PPPM values were anchored in recent real-world datasets describing HCRU, transfusion burden and costs for HR-MDS treated with HMAs or best supportive care, and informed by UK clinical practice and clinical plausibility checks.^{7 8 16 17 28} For the terminal care phase, the assumed frequency of hospital nights, hospice days and transfusions was calibrated to reflect the high intensity of end-of-life care reported for patients with MDS and other haematological malignancies.^{23 24}

Table 3 shows the amount and cost of each healthcare resource used for the one-off diagnostic work-up and the source/s used to determine the extent of utilisation. Table 2 provides a breakdown of the PPPM amount and cost of each healthcare resource used in each of the other phases of care in HR-MDS, and the source/s used to determine the extent of utilisation of each healthcare resource.

Diagnostic work-up

This used a representative one-off bundle for bone-marrow aspirate and trephine plus a ≤4 hours hospital day admission. A separate scenario in the diagnostic work-up included the cost of a one-off myeloid next-generation sequencing (NGS) panel commissioned centrally via the National Genomic Test Directory and delivered by Genomic Laboratory Hubs, and karyotype test/fluorescence in situ hybridisation (FISH); this was conducted as a separate analysis to avoid embedding non-standard local prices because national prices are not published, prices vary between NHS trusts (£1000 to £1500 for myeloid NGS sequencing) and testing is episodic.^{29 30}

Active HMA therapy

Each month (28-day cycle) included azacitidine 75 mg/m² given subcutaneously once daily for 7 days to a patient of average body surface area of 1.7 m².^{7 8 27} Each administration of azacitidine incurred an outpatient chemotherapy attendance cost. A base month included 3.5 RBC units given during two hospital day admissions of 4–8 hours. Two and a half platelet doses were included and given

Table 2 Healthcare resource utilisation and costs (per month per patient with HR-MDS) by phase of care and the sources used to determine the extent of utilisation

Phase of care	Amount of HCRU PPPM	Unit cost of HCRU PPPM* (£)	Source
Active HMA therapy†	Azacitidine 75 mg/m ² given subcutaneously once daily for 7 days to a patient of average body surface area of 1.7 m ² . Azacitidine, 7 × 150 mg vials required.	£3367	7 8 27
	Outpatient attendance for chemotherapy × 7	£1960	
	RBC units × 3.5	£536.55	7 8 16 17 28
	Hospital day admission of 4–8 hours for co-administration of RBC and platelets × 2	£1090	
	Platelet dose × 2.5	£688.03	
	Outpatient review × 1	£165	7 8 16 17
	Hospital haematology inpatient overnight stay × 1	£915	
			7 8 16 17 20 28
Post-HMA failure supportive care	Hospital day admission of 4–8 hours for co-administration of RBC and platelets × 4	£2180	
	RBC units × 3.5	£536.55	
	Platelet dose × 2.5	£688.03	
	Outpatient review × 1	£165	
	Hospital haematology inpatient overnight stay × 2	£1830	
Terminal care			
Hospital-dominant	Hospital haematology inpatient overnight stay × 10	£9150	23–25
	Hospital day admission of 4–8 hours for co-administration of RBC and platelets × 4	£2180	7 16 17 23 24 28
	RBC units × 3.5	£536.55	
	Platelet dose × 2.5	£688.03	
Hospice-dominant	Specialist palliative-care bed-days × 10	£3970 to £8120	23–25

*See table 1 for unit costs and the source of price.
†Each month is a 28-day cycle.
HCRU, healthcare resource utilisation; HMA, hypomethylating agent; HR-MDS, higher-risk myelodysplastic syndromes; PPPM, per-patient-per-month; RBC, red blood cell.

during the same session as the RBC transfusion(s). A base month also included one outpatient review and one night of hospital inpatient stay. These patterns reflect high transfusion dependence and hospitalisation rates reported in real-world cohorts who received azacitidine.⁸

Post-HMA failure supportive care

Worsening cytopenias and infections drive HCRU. Each month included four 4–8 hours hospital day admissions

for supportive care, co-administration of 3.5 RBC units and 2.5 platelet doses, 1 outpatient review and an average of 2 nights of hospital inpatient stay; much in-line with Drummond *et al.*⁷

Terminal care

Two scenarios were considered for 1 month of terminal care. The hospital-dominant care scenario included 10 nights of hospital inpatient stay and allowed for frequent

Table 3 Healthcare resource utilisation and costs for one-off diagnostic work-up and the sources used to determine the extent of utilisation

HCRU	Total cost of HCRU* (£)	Source
Bone-marrow aspirate and trephine	£870 ^b	Royal Devon University Healthcare NHS Foundation Trust 2024 ¹²
One ≤4 hours hospital day admission	£340	Royal Devon University Healthcare NHS Foundation Trust 2024 ¹²
Alternative scenario: NGS sequencing and karyotype test/FISH	Alternative scenario: £1500 to £1600	National Genomic Test Directory 2024, National Genomic Test Directory 2025 ^{29 30}

*See table 1 for unit costs and source of price.
FISH, fluorescence in situ hybridisation; HCRU, healthcare resource utilisation; NGS, next-generation sequencing.

Table 4 One-off cost for the diagnostic work-up and the overall PPPM cost for each of the other phases of care in HR-MDS (main analysis and alternative scenarios)

Phase of care	Cost by phase
Diagnostic work-up (one-off cost)	£1210
Alternative scenario including the price of NGS sequencing and karyotype test/FISH	£2710 to £2810
Active HMA therapy (PPPM)	£8721.58
Post-HMA failure supportive care (PPPM)	£5399.58
Terminal care - hospital-dominant (PPPM)	£12 554.58
Alternative scenario of specialist hospice dominant terminal care (PPPM)	£3970 to £8120

FISH, fluorescence in situ hybridisation; HMA, hypomethylating agent; HR-MDS, higher-risk myelodysplastic syndromes; NGS, next-generation sequencing; PPPM, per-patient-per-month.

transfusions (four 4–8 hours hospital day admissions for transfusion of 3.5 RBC units and 2.5 platelet doses). The hospice-dominant care scenario included 10 specialist palliative-care bed-days costed at both the upper and lower end of the price range in the PSSRU¹³; transfusions were not included in this scenario because transfusion practice at the end of life is highly variable and active transfusion support is commonly de-escalated as the goals of care shift towards comfort; a hospice-dominant pathway that retained occasional transfusions would fall between the two scenarios presented and would not change the finding that end-of-life costs are strongly setting-dependent. The literature varies regarding the duration of terminal inpatient stays, with one study reporting an average of 24 days²⁵ and another reporting 6

days,²⁴ therefore, an intermediate assumption of 10 inpatient nights was adopted as a balanced midpoint between these reported extremes (6–24 days), with the hospice-dominant scenario capturing the lower-cost end of the range.

RESULTS

The primary outcomes of the analysis (ie, a one-off cost for the diagnostic work-up and the overall PPPM cost for each of the other phases of care in HR-MDS) together with outcomes of the alternative scenario analysis, shown in [table 4](#).

The cost composite breakdown for the one-off diagnostic work-up and the PPPM cost for each of the other phases of care in HR-MDS is provided in [figure 1](#).

Diagnostic work-up

The cost for the Diagnostic Work-up was £1210 using a representative one-off bundle for bone-marrow aspirate and trephine plus a ≤4 hours day-case attendance. In a separate scenario, that included the cost of one-off NGS sequencing and karyotype test/FISH, the one-off cost increased to £2710 to £2810.

Active HMA therapy

Under tariff-aligned costs and co-administration of platelets within RBC transfusion sessions, the PPPM cost during Active HMA therapy was £8721.58.

Post-HMA failure supportive care

In the post-HMA failure supportive care phase, the PPPM cost was £5399.58.

Terminal care

Terminal care costs were setting-dependent: the PPPM cost for hospital-dominant care was £12 554.58. Meanwhile,

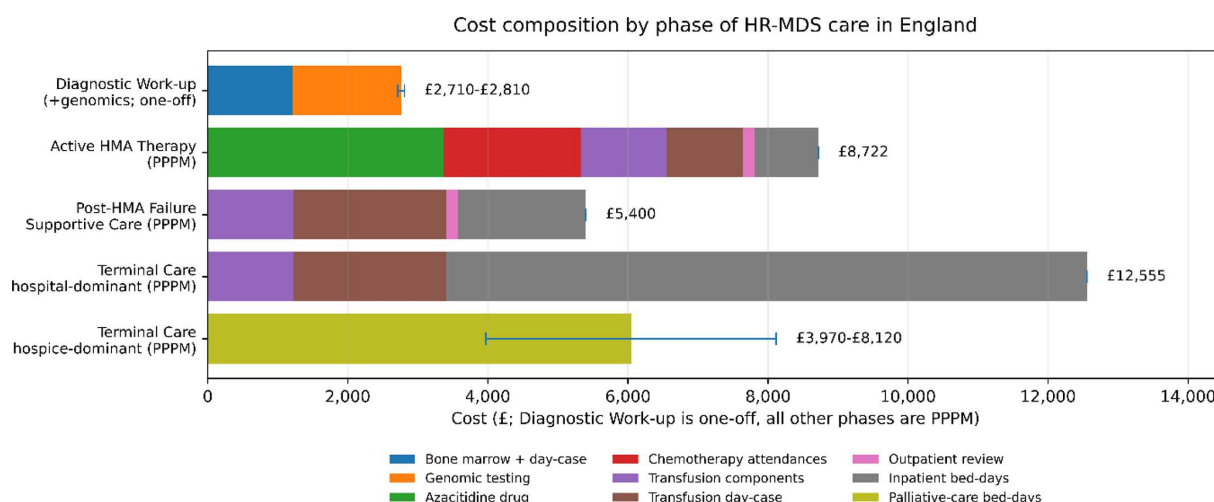


Figure 1 Cost composition breakdown for the one-off diagnostic work-up and phase-specific PPPM costs. Diagnostic work-up with genomic testing is shown using the midpoint of the scenario range (£2710 to £2810). Hospice-dominant terminal care is shown using the midpoint of the NHSCII-uprated PSSRU specialist palliative-care bed-day range (£3970 to £8120); the horizontal interval represents this deterministic unit-cost range and is not a CI, IQR or other measure of statistical uncertainty. All other bars are deterministic point estimates. HMA, hypomethylating agent; HR-MDS, higher-risk myelodysplastic syndromes; NHSCII, National Health Service Cost Inflation Index; PPPM, per-patient-per-month; PSSRU, Personal Social Services Research Unit.

the separate scenario of hospice-dominant care cost £3970 to £8120 (at the PSSRU bounds (see [table 1](#))).

Cost of a base-case management pathway followed by a patient with HR-MDS

Applying the PPPM cost for each phase to the base-case non-curative management pathway followed by a patient with HR-MDS (ie, diagnostic bundle at month 0 including NGS, 12 cycles of azacitidine, 5 months post-HMA failure supportive care and 1 month of hospital-led terminal care) produced a tariff-aligned cumulative total cost of £146 921 per patient. An alternative scenario analysis excluding genomic testing at diagnosis and including hospice-dominant terminal care reduced the total cost to £136 840 to £140 990.

Scenario analyses

Because azacitidine treatment duration is the principal determinant of total cost and varies across real-world cohorts, the total pathway cost was re-estimated for shorter and longer treatment durations while holding all other base-case assumptions constant (diagnostic work-up including genomic testing and hospital-dominant terminal care). Assuming 9 cycles of active azacitidine therapy reduced the total to approximately £120 760, whereas 15 cycles increased it to approximately £173 090, compared with approximately £146 921 in the 12-cycle base case.

Substituting a 5-day azacitidine administration schedule for the base-case 7-day schedule (reducing both drug acquisition and chemotherapy-attendance costs) lowered the monthly active-therapy cost from £8721.58 to £7199.58 and the total base-case pathway cost to approximately £128 660. The alternative pathway excluding genomic testing and assuming hospice-dominant terminal care produced a total of approximately £136 840 to £140 990, depending on whether the lower or upper bound of specialist palliative-care bed-day costs was applied.

A full per-phase breakdown of all scenarios is provided in online supplemental tables S3 and S4. Across every scenario the rank order of cost drivers was unchanged, with azacitidine acquisition and administration during active therapy and inpatient bed-days at the end of life remaining the dominant contributors.

DISCUSSION

This analysis provides estimates of the health-system burden of HR-MDS in England, using a transparent micro-costing framework with PPPM costs disaggregated by clinically meaningful phases of care. We estimated that HR-MDS imposes substantial direct costs on NHS England. Phase-specific PPPM costs were approximately £8700 during active azacitidine treatment, £5400 during post-azacitidine supportive care and £12 600 during hospital-dominant terminal care, with hospice-dominant terminal care costing around £4000 to £8100 per month. Applying these estimates to a representative management sequence comprising 12 months of active

azacitidine, 5 months of post-HMA failure supportive care and 1 month of terminal care yielded a total pathway cost of £146 921 including hospital-dominant terminal care or £136 840 to £140 990 including hospice-dominant terminal care.

During Active HMA therapy, monthly costs are dominated by azacitidine acquisition, with medium contributions from transfusions and outpatient attendances for administration of the chemotherapy, and a smaller inpatient component. The results are consistent with contemporary real-world utilisation patterns and evidence that supportive care remains substantial even while on HMA therapy.^{4 5 8 11–13}

After HMA failure, outcomes are poor, the pattern shifts towards transfusions and admissions, reflecting the clinical deterioration and short survival in this phase.^{8 20} Terminal care is strongly setting-dependent, with hospital-dominant trajectories driven by haematology inpatient bed-days and hospice-dominant trajectories aligning with specialist palliative-care bed-day rates.^{12 13} The range in costs for terminal care capture the observed variation in end-of-life pathways and are consistent with national benchmarks for cost.^{7 20}

Because the model uses publicly available national price benchmarks for medicines, blood components and palliative care and a published non-contract tariff for day-case and inpatient services, the estimates are auditable and can be replicated or adapted locally.^{4 11–13}

Importantly, the total cost of a base-case non-curative management pathway followed by a patient with HR-MDS in England was £146 921 in our analysis. Despite differences in model structure and price years, this is in a similar price range as the total healthcare cost of £91 800 per patient reported in the NICE technology appraisal for azacitidine in 2011 and updated to £124 848 in 2024/2025 to take account of NHSCIL.^{4 31} This similarity provides reassuring external context but should not be interpreted as validating the England-benchmarked estimates used in our analysis, given the differences in population, model structure, treatment duration and price year noted above. The residual ~18% difference between the two figures is explained by several factors: the present model uses a longer median treatment duration (12 cycles vs approximately 9 cycles in AZA-001), higher transfusion intensities drawn from more recent real-world cohorts, and a different model structure; whereas generic azacitidine and the availability of a 150mg vial have partly offset these upward pressures by reducing drug acquisition costs compared with the originator price used in 2011. Taken together, the proximity of the two estimates suggests that the core cost drivers of HR-MDS have remained broadly stable over time, but the difference underscores that the two analyses are not directly comparable and should not be used interchangeably.²⁶

These findings are broadly consistent with previous economic analyses of MDS while extending the evidence base in several ways. At the macro level, Gou and colleagues have shown that the global incidence and DALY burden of MDS/myeloproliferative neoplasms has risen steadily over the last three decades, particularly in high-income settings,¹⁴ underscoring the need for robust, country-specific cost estimates. Our PPPM values sit within the upper range of per-patient

costs reported in the recent systematic review of economic burden in MDS, which found that higher-risk disease and use of HMAs were consistently associated with greater expenditures.¹⁶ In the US claims analysis of treated higher-risk MDS, mean total costs were approximately US\$17 361 PPPM (2015 US dollars), with roughly two-thirds attributable to medical services and the remainder to outpatient pharmacy costs; MDS-related chemotherapy and supportive care, including transfusions, were the main drivers.¹⁷ Although direct comparison across health systems and price years is limited, the pattern of cost drivers in our English micro-costing: HMA acquisition and administration during active therapy, transfusions and admissions after HMA failure and setting-dependent end-of-life care aligns closely with these international observations.

Earlier UK work estimated the annual national burden associated with managing intermediate-2 and high-risk MDS at £12 to £16 million, again highlighting hospitalisations and transfusions as predominant contributors, with community nurse visits and caregiver time representing smaller, non-negligible elements of cost.¹⁸ Our analysis complements that top-down perspective by providing a bottom-up, phase-specific decomposition of NHS England secondary and tertiary care costs from diagnosis through active HMA therapy, post-failure supportive care and terminal care. The resulting PPPM and pathway values are, therefore, well suited for direct use as cost estimates in future economic models of emerging therapies and for local budget-impact analyses.

We did not include costs incurred after transformation to AML, or after allogeneic transplantation. Both pathways involve different diagnostics, treatments and admission patterns and published modelling demonstrates a distinct cost profile for AML.²¹ Moreover, only a small minority of patients with HR-MDS proceed to potentially curative transplantation in routine practice. Contemporary data indicate that fewer than 5% undergo allogeneic transplantation, which supports treating transplant as a separate pathway for economic purposes while acknowledging its clinical importance.³² Excluding post-AML and post-transplant costs, therefore, improves interpretability for NHS planning specific to myelodysplastic syndromes, but it also means the figures do not represent lifetime haematological costs. The net effect of these exclusions is likely to be an underestimate of total lifetime expenditure in the subset of patients who transform to AML or receive a transplant; users aiming to model the full disease trajectory should consider AML and transplant cost and usage sourced from dedicated literature.^{20 21 32}

The findings of our analysis have several implications for practice and policy. Reducing transfusion requirements lowers both component expenditure and the time-banded day-case administration cost, so interventions that achieve haemoglobin stability or transfusion independence may deliver meaningful system savings alongside clinical benefit.^{1 3 7 11–13} Minimising admissions, particularly after HMA failure and near the end of life, is important because small changes in length of hospital stay shift monthly costs appreciably.^{8 20} Finally, hospice-dominant end-of-life

trajectories have markedly lower costs than hospital-dominant courses; ensuring access to timely palliative-care assessment may improve patient experience and moderate late-phase expenditure.¹³

Strengths of this work include the exclusive use of publicly verifiable UK sources for unit costs, a phase-based structure for care of patients with HR-MDS that aligns with clinical decision-making, and transparent calculations that allow direct substitution of local tariffs. The base-case management pathway followed by a patient with HR-MDS is anchored in a contemporary French/German/UK real-world study that reported a median of 12 azacitidine cycles per patient overall, and 13.5 cycles per patient in the UK subset. However, other real-world studies report shorter median durations (6–9 cycles), so 12 cycles should be regarded as a plausible upper-bound estimate.⁷ The PPPM values for each resource enable the costs to be estimated for alternative durations of each phase. The analysis also assesses the impact of administration and transfusion management making the drivers explicit for audit and replication.

Limitations should be acknowledged. The model is a static monthly cross-section rather than a time-to-event simulation and, therefore, does not capture patient-level heterogeneity or cycle-by-cycle trajectories in early treatment.⁸ Many of the included parameters, such as treatment cycles, transfusion frequency and duration of inpatient stay or terminal care, vary substantially across published real-world studies and NHS regions. Selecting upper and lower limits for these parameters that vary widely by population and setting, would be inherently subjective. Rather than attaching formal probability distributions to parameters whose plausible ranges vary widely by population and setting, we used explicit and transparent England-specific assumptions and, in response to reviewer feedback, undertook deterministic scenario analyses around the principal cost drivers, azacitidine treatment duration and administration schedule and the terminal-care setting (see Results, Scenario analyses and online supplemental material); these assumptions can be re-run directly with local data. Healthcare resource use was drawn from observational cohorts recruited between 2010 and 2022, whereas unit prices reflect more recent English schedules. This temporal mismatch is typical of cost-of-illness work but means that the analysis combines current prices with utilisation patterns that may not fully reflect very recent changes in clinical practice, including earlier use of novel agents. Unit prices include national benchmarks, a single trust's tariff and transfusion component prices drawn from publicly available lists that are updated periodically; local arrangements vary and blended payments are permitted under the NHS Payment Scheme, which is why alternative scenario analyses are provided; users are encouraged to substitute local prices if replicating the model locally, noting that reasonable variation does not overturn the ordering of cost drivers.^{11–13 29 33} The use of BNF list prices for azacitidine may overestimate the true costs as NHS trusts typically pay lower contracted prices—these however, are not publicly available. Although BNF list prices may overestimate true azacitidine acquisition costs, this may be partially offset by variability in supportive-care

use and related costs, as highlighted in this Discussion. Other adjunctive therapies such as erythropoiesis-stimulating agents, granulocyte colony-stimulating factors, iron chelation and anti-infectives were not itemised; evidence from other MDS economic studies indicates that these contribute to costs, particularly in lower-risk disease, but that in intermediate-2 and high-risk MDS anticancer therapies, transfusions and hospitalisations remain the main direct medical cost drivers.^{16–18,28} However, the analysis may overestimate the cost of HMA treatment because the model has assumed a consistent 7-day administration, when in practice, dose reductions to a 5-day treatment per cycle are common,³⁴ and additionally, the model has not accounted for possible treatment delays. Risk stratification follows IPSS-R to maintain alignment with current service planning; however, implications of IPSS-molecular for economic modelling warrant future study. Community and primary-care activity and concomitant medications given outside admissions were not itemised and may be incorporated in local adaptations; their omission is unlikely to change the ranking of cost drivers.

Generalisability is also limited. Unit costs are specific to NHS England, and a single English trust was used as the basis of the model. While the overall pattern of cost drivers is likely to be similar elsewhere in the UK, absolute costs may differ in devolved nations and in non-UK health systems and local adaptations should substitute relevant tariffs. Finally, the analysis reports direct NHS costs only and excludes patient travel, caregiver time and productivity losses, which are relevant to societal burden but fall outside the provider perspective adopted here. Recent global burden, quality-of-life (QoL) and caregiver studies suggest that these non-medical and indirect consequences of HR-MDS are substantial, implying that the true economic and humanistic impact is materially greater than the costs quantified in this model.^{14 15 28 35}

The present micro-costing focuses deliberately on provider-borne NHS costs and, therefore, does not capture the full burden of HR-MDS on patients and families. Recent reviews and observational studies emphasise that MDS is associated with substantial decrements in HRQoL; particularly in physical functioning, role functioning and fatigue domains, as well as impaired health utility.^{15 28} Transfusion dependence appears to be a key driver of both costs and QoL deterioration, with transfusion-dependent patients incurring higher direct medical costs and reporting worse symptoms than those who are transfusion independent.²⁸ Moreover, surveys in Europe and the USA indicate that caregivers often experience marked emotional distress, time away from work and disruption to daily life because of frequent hospital visits and transfusion schedules.³⁵ These humanistic and societal impacts sit alongside, but are not captured by the secondary and tertiary care costs estimated in the present analysis, suggesting that the true economic burden of HR-MDS in England is materially higher than our NHS perspective figures.

The phase-specific PPPM estimates, and pathway totals reported here can also serve as baseline costs in economic evaluations of emerging therapies that may modify both survival and healthcare utilisation in HR-MDS. Several agents

are under investigation in the post-HMA failure setting and in combination with azacitidine in the first-line HR-MDS setting. These include novel BCL2 inhibitors and novel immunomodulatory agents.³⁶ Agents that achieve durable transfusion independence or disease remission would be expected to reduce per-month costs in the post-failure phase while potentially extending the duration of time spent in each phase of care. If such agents are confirmed to extend survival and reduce transfusion intensity, they will inevitably increase cumulative NHS expenditure by lengthening time spent in costly phases of care, but they offer the prospect of better outcomes and potentially improved value per life-year or quality-adjusted life-year gained. The PPPM and phase-specific estimates presented here provide a transparent foundation on which incremental effects of new therapies on survival, transfusion dependence and admission risk can be overlaid in future cost-effectiveness and budget-impact analyses.

In summary, HR-MDS imposes a sustained direct cost burden on NHS providers that is concentrated in azacitidine acquisition and administration, transfusions and admissions after treatment failure and setting-dependent costs at the end of life. The phase-specific PPPM values and the base-case management pathway followed by a patient with HR-MDS provide a transparent basis, subject to local validation, for budget-impact assessments, capacity planning and future cost-effectiveness models that distinguish between on-treatment, post-treatment failure and end-of-life states. In practical terms, interventions that reduce the frequency of transfusions and chemotherapy administration, and that establish ambulatory pathways to avert or shorten hospital admission are likely to yield the greatest system savings while improving patient experience.

Acknowledgements Medical writing assistance was provided by TransCrip Group with funding from Faron Pharmaceuticals Ltd.

Contributors JW was responsible for study conception, model development, data extraction, cost calculations, analysis, interpretation of results and drafting of the manuscript. VSG contributed to the health-economic design, methodological approach, interpretation of findings and critical revision of the manuscript. EJS contributed clinical expertise on HR-MDS management, healthcare resource utilisation and interpretation of clinical assumptions. EL, RH and PB contributed to data verification and critical review of the manuscript. All authors provided intellectual input, reviewed and approved the final version of the manuscript. JW is the guarantor.

Funding This research received no specific grant from any funding agency in the public, commercial or not-for-profit sectors. Medical writing assistance was funded by Faron Pharmaceuticals Ltd. The funder (Faron Pharmaceuticals Ltd) contributed to the study design, the construction of the costing model and the selection and interpretation of parameters, and to the drafting, review and approval of the manuscript and the decision to submit it for publication. Several authors are employees of the funder, as detailed under Competing interests. The analysis is based entirely on published sources and publicly available price lists; all authors had full access to the model inputs and take responsibility for the integrity and accuracy of the work.

Competing interests JW, EL, RH and PB are employees of Faron Pharmaceuticals Ltd (and may hold stock or stock options in the company). EJS is a principal investigator in clinical trials sponsored by Faron Pharmaceuticals Ltd and is employed by the Christie NHS Foundation Trust, falling under NHS England. VSG reports no competing interests.

Patient and public involvement Patients and/or the public were not involved in the design, or conduct, or reporting, or dissemination plans of this research.

Patient consent for publication Not applicable.

Ethics approval Not applicable.

Provenance and peer review Not commissioned; externally peer reviewed.

Data availability statement Data are available in a public, open access repository. All data used in this analysis are from published sources and publicly available listings, as cited in the manuscript.

Supplemental material This content has been supplied by the author(s). It has not been vetted by BMJ Publishing Group Limited (BMJ) and may not have been peer-reviewed. Any opinions or recommendations discussed are solely those of the author(s) and are not endorsed by BMJ. BMJ disclaims all liability and responsibility arising from any reliance placed on the content. Where the content includes any translated material, BMJ does not warrant the accuracy and reliability of the translations (including but not limited to local regulations, clinical guidelines, terminology, drug names and drug dosages), and is not responsible for any error and/or omissions arising from translation and adaptation or otherwise.

Open access This is an open access article distributed in accordance with the Creative Commons Attribution Non Commercial (CC BY-NC 4.0) license, which permits others to distribute, remix, adapt, build upon this work non-commercially, and license their derivative works on different terms, provided the original work is properly cited, appropriate credit is given, any changes made indicated, and the use is non-commercial. See: <https://creativecommons.org/licenses/by-nc/4.0/>.

ORCID iDs

Joab Williamson <https://orcid.org/0000-0002-8306-1559>

Petri Bono <https://orcid.org/0000-0003-3963-0416>

Vijay S Gc <https://orcid.org/0000-0003-0365-2605>

REFERENCES

- Gupta S, Kulasekararaj AG, Costantino H, *et al.* Association between transfusion status and clinical and economic outcomes in patients with myelodysplastic syndromes from the physicians' perspective. *Cancer Rep (Hoboken)* 2023;6:e1680.
- Greenberg PL, Tuechler H, Schanz J, *et al.* Revised international prognostic scoring system for myelodysplastic syndromes. *Blood* 2012;120:2454–65.
- Kota V, Ogbonnaya A, Farrelly E, *et al.* Clinical impact of transformation to acute myeloid leukemia in patients with higher-risk myelodysplastic syndromes. *Future Oncol* 2022;18:4017–29.
- NICE. Azacitidine for the treatment of myelodysplastic syndromes, chronic-myelomonocytic leukaemia and acute myeloid leukaemia. 2011. Available: <https://www.nice.org.uk/guidance/ta218>
- Fenaux P, Mufti GJ, Hellstrom-Lindberg E, *et al.* Efficacy of azacitidine compared with that of conventional care regimens in the treatment of higher-risk myelodysplastic syndromes: a randomised, open-label, phase III study. *Lancet Oncol* 2009;10:223–32.
- Rajakumaraswamy N, Gandhi M, Wei AH, *et al.* Real-world Effectiveness of Azacitidine in Treatment-Naive Patients With Higher-risk Myelodysplastic Syndromes. *Clin Lymphoma Myeloma Leuk* 2024;24:260–8.
- Drummond M, Finelli C, Kristo F, *et al.* Real-World Treatment Patterns, Clinical Outcomes, and Costs in Patients with Higher-Risk Myelodysplastic Syndromes Across France, Germany, and the United Kingdom. *J Blood Med* 2025;16:307–19.
- Brommann SJ, Pilgaard L, Jensen JF, *et al.* Real-World Healthcare Resource Utilisation in Patients With Higher-Risk Myelodysplastic Syndrome During Azacitidine Treatment: A Population-Based Cohort Study. *EJHaem* 2025;6:e70066.
- Zeidan AM, Zhao R, Pierre-Victor D, *et al.* Real-World Use Patterns and Clinical Outcomes for Myelodysplastic Syndrome Patients Initiating Oral Decitabine and Cedazuridine or Intravenous/Subcutaneous Hypomethylating Agents. *Blood* 2024;144:5189.
- Enjeti A, Ashraf A, Caillet V, *et al.* Real-world study of the use of azacitidine in myelodysplasia in Australia. *EJHaem* 2024;5:527–34.
- Blood NHS, List T. Blood and components - cost per item. Available: https://nhsbtdbeblobcorewindowsnet/umbraco-assets-corp/34945/price_list_bc_cost-per-item_2024-25pdf [Accessed 22 Aug 2025].
- Royal Devon University Healthcare NHS Foundation Trust. Non-contractual tariffs. Available: <https://www.royaldevonhshuk/media/e1wn2w4n/foi-rdf2433-24-non-contractual-tariffspdf> [Accessed 23 Aug 2025].
- Jones K, Weatherly H, Birch S, *et al.* Unit costs of health and social care 2022 manual. Available: <https://karkentacuk/100519/1/Unit%20>
- Costs%20of%20health%20and%20Social%20Care%202022%20%28amended%2013%20July%202023%29pdf [Accessed 23 Aug 2025].
- Gou X, Chen Z, Shangguan Y. Global, regional, and national burden of myelodysplastic syndromes and myeloproliferative neoplasms, 1990–2021: an analysis from the global burden of disease study 2021. *Front Oncol* 2025;15:1559382.
- Xie S, Yan J, Tse P, *et al.* Burden of Myelodysplastic Syndromes: A Literature Review of Epidemiological and Humanistic Aspects. *J Evid Based Med* 2025;18:e70065.
- Tse P, Xie S, Yan J, *et al.* Burden of myelodysplastic syndromes: a systematic literature review of economic burden. *Eur J Health Econ* 2025;26:1469–85.
- Bell JA, Galaznik A, Blazer M, *et al.* Economic Burden of Patients Treated for Higher-Risk Myelodysplastic Syndromes (HR-MDS) in Routine Clinical Care in the United States. *Pharmacoecon Open* 2019;3:237–45.
- Brereton N, Nicklasson L, Mufti GJ. UK Estimates of Burden of Disease Associated with Management of Intermediate-2 or High-Risk Myelodysplastic Syndromes. *Blood* 2011;118:4749.
- Cook MR, Karp JE, Lai C. The spectrum of genetic mutations in myelodysplastic syndrome: Should we update prognostication? *EJHaem* 2022;3:301–13.
- Prébet T, Gore SD, Esterni B, *et al.* Outcome of high-risk myelodysplastic syndrome after azacitidine treatment failure. *J Clin Oncol* 2011;29:3322–7.
- Wang H-I, Aas E, Howell D, *et al.* Long-term medical costs and life expectancy of acute myeloid leukemia: a probabilistic decision model. *Value Health* 2014;17:205–14.
- Husereau D, Drummond M, Augustovski F, *et al.* Consolidated Health Economic Evaluation Reporting Standards 2022 (CHEERS 2022) Statement: Updated Reporting Guidance for Health Economic Evaluations. *Pharmacoeconomics* 2022;40:601–9.
- Fletcher SA, Cronin AM, Zeidan AM, *et al.* Intensity of end-of-life care for patients with myelodysplastic syndromes: Findings from a large national database. *Cancer* 2016;122:1209–15.
- Sexauer A, Cheng MJ, Knight L, *et al.* Patterns of hospice use in patients dying from hematologic malignancies. *J Palliat Med* 2014;17:195–9.
- Konstantopoulos K, Lauren L, Hast R, *et al.* Survival, hospitalization and cause of death in 99 patients with the myelodysplastic syndrome. *Anticancer Res* 1989;9:893–6.
- Joint Formulary Committee. British national formulary (bnf). Available: <https://bnf.nice.org.uk/about/joint-formulary-committee/> [Accessed 22 Oct 2025].
- Bristol Myers Squibb. SmPC vidaza 25 mg/ml powder for suspension for injection. Available: <https://www.medicines.org.uk/emc/product/6468/smcp> [Accessed 22 Oct 2025].
- Lucioni C, Finelli C, Mazzi S, *et al.* Costs and quality of life in patients with myelodysplastic syndromes. *Am J Blood Res* 2013;3:246–59.
- NHS England. National genomic test directory (knowledge hub). Available: <https://www.genomicseducationheathshuk/genotes/knowledge-hub/the-national-genomic-test-directory/> [Accessed 23 Aug 2025].
- NHS England. National genomic test directory. Available: <https://www.england.nhs.uk/publication/national-genomic-test-directories/> [Accessed 23 Aug 2025].
- Jones K, Weatherly H, Birch S, *et al.* Unit costs of health and social care 2024 manual. Available: <https://karkentacuk/109563/> [Accessed 29 Dec 2025].
- Sekeres MA, Schoonen WM, Kantarjian H, *et al.* Characteristics of US patients with myelodysplastic syndromes: results of six cross-sectional physician surveys. *J Natl Cancer Inst* 2008;100:1542–51.
- NHS. NHS payment scheme – annex b: guidance on currencies. Available: <https://www.england.nhs.uk/long-read/25-26-nhsps-annex-b-guidance-on-currencies/> [Accessed 22 Aug 2025].
- Sapinho G, Alves-Ribeiro L, Infante J, *et al.* Full-Dose Azacitidine in 5 Days Versus 7 Days With a Weekend Break in Myelodysplastic Syndromes: A Retrospective Cohort Study. *Clin Lymphoma Myeloma Leuk* 2024;24:e13–20.
- Lewis K, Williamson M, Brown E, *et al.* Real-World Study of the Burden of Myelodysplastic Syndromes in Patients and Their Caregivers in Europe and the United States. *Oncol Ther* 2024;12:753–74.
- Kontro M, Stein AS, Rimpiläinen J, *et al.* Transfusion independence, hematological improvement and associated safety outcomes with bexmarilimab and azacitidine in HR-MDS: Results of the bexmab Phase 1/2 study. *Blood* 2025;146:5627.