

Decitabine monotherapy for pediatric myelodysplastic syndrome: A case report

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Abstract. The occurrence of myelodysplastic syndrome (MDS) in children is infrequently documented and most commonly manifests in the form of refractory cytopenia of childhood (RCC). The management of pediatric MDS remains controversial and involves primarily chemotherapy, immuno-suppressive therapy (IST) and allogeneic hematopoietic stem cell transplantation (HSCT). However, therapeutic data for patients with RCC who are ineligible for either HSCT or IST are scarce. In the present case, the 8-year-old patient achieved disease stabilization following decitabine monotherapy, thereby suggesting a potential novel therapeutic avenue for children with low-risk chronic MDS or RCC.

Introduction

Myelodysplastic syndrome (MDS) comprises a group of clonal hematopoietic malignancies characterized by dysplastic hematopoiesis, an increased risk of progression to acute myeloid leukemia (AML), and persistent single-stage or multilineage cytopenias. The incidence of MDS in children is significantly lower than that in adults and accounts for <5% of all hematological cancers in children, with an estimated annual incidence of 1-4 per 1,000,000 children (1). In contrast to adult MDS, the pediatric type of MDS is frequently associated with underlying inherited bone marrow failure syndromes (IBMFs) or a history of high-dose alkylating agent exposure. Consequently, pediatric MDS has distinct characteristics in terms of diagnosis, clinical presentation, laboratory findings, therapeutic management and prognosis (2,3). Refractory cytopenia of childhood (RCC) represents the most prevalent subtype of pediatric MDS and accounts for ~50% of cases. This condition is defined by persistent peripheral blood cytopenias (including thrombocytopenia, anemia, and/or neutropenia) and may manifest as pancytopenia (4).

The established indications for initiating treatment in RCC include the following: i) Transfusion dependency; ii) clinically significant neutropenia, which is defined as an absolute neutrophil count (ANC) <1.0x10⁹/l; and iii) the presence of high-risk chromosomal abnormalities, such as monosomy 7, deletion 7q or complex karyotypes (≥2 aberrations). For patients with RCC who do not meet these criteria (specifically those with a normal karyotype and no evidence of a predisposition syndrome), observational follow-up is considered an appropriate management strategy, as supported by relevant studies (5).

For eligible patients with RCC who meet the aforementioned indications, the primary treatment modalities include chemotherapy, immunosuppressive therapy (IST) and allogeneic hematopoietic stem cell transplantation (HSCT). Allogeneic HSCT remains the cornerstone of curative therapy and should be prioritized for all pediatric patients with MDS, particularly those exhibiting high-risk features, including monosomy 7, complex karyotypes, transfusion dependence or severe neutropenia with a significant risk of infection (2).

By contrast, IST may be considered for patients with hypocellular BM and associated cytopenias. Although IST yields favorable outcomes in a subset of adult patients with MDS (particularly those with low-risk, hypocellular subtypes), it has also demonstrated efficacy in pediatric RCC, with reported response rates ranging from 63 to 76% and a more favorable adverse effect profile than that found in adults (6).

In recent years, epigenetic therapies [particularly DNA methyltransferase (DNMT) inhibitors such as decitabine, have been increasingly utilized in adult patients with MDS, with a considerable proportion of patients achieving clinical benefits (7). However, clinical experience with these agents in treating pediatric patients with MDS remains limited, and robust clinical data from this population are scarce.

The present case report describes the use of decitabine in a pediatric patient with RCC who did not respond to prior immunosuppressive and supportive therapies. It also discusses the clinical implications of this approach for similar refractory cases where HSCT is not feasible.

Case report

In September 2015, an 8-year-old girl was admitted to Lishui Central Hospital (Lishui, China) due to recurrent skin

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petechiae and ecchymoses that had persisted for 3 weeks. The patient's medical history was otherwise unremarkable. A physical examination revealed scattered dark red petechiae and ecchymoses on the limbs and trunk, with no hepatosplenomegaly observed. A complete blood count revealed pancytopenia, with the following parameters being reported: Hemoglobin concentration, 92 g/l (normal range, 115-150 g/l); platelet count, $22 \times 10^9/l$ (normal range, $125-350 \times 10^9/l$); white blood cell count, $2.3 \times 10^9/l$ (normal range, $3.5-9.5 \times 10^9/l$); ANC, $1.2 \times 10^9/l$ (normal range, $1.8-6.3 \times 10^9/l$); lymphocyte count, $1.0 \times 10^9/l$ (normal range, $1.1-3.2 \times 10^9/l$); and reticulocyte count, $70 \times 10^9/l$ (normal range, $25-75 \times 10^9/l$). No blast cells were detected in the peripheral blood or BM aspirate.

BM aspiration revealed dysplastic hematopoiesis, which was characterized by small megakaryocytes and partial neutrophil maturation arrest (Fig. 1A-C). A BM biopsy confirmed the presence of hypocellular myeloid hematopoietic tissue (~50%), with a normal granulocyte-erythrocyte ratio. Granulocytic cells at all stages were present, accompanied by myelodysplasia of neutrophils. The erythroid lineage was dominated by intermediate and late erythroblasts. Megakaryocytes were not scarce and were composed mainly of lobulated forms; small megakaryocytes (with hyperchromatic nuclei, small cell bodies and few lobes) were easily detectable and scattered in distribution. Lymphocytes and plasma cells were scattered. Reticulin staining revealed a myelofibrosis-0 grade, defined as no significant fibrosis according to the European Consensus/WHO grading system (8) (Fig. 1D-F). Flow cytometry immunophenotyping (Data S1; Fig. S1) revealed no aberrant blast population, and conventional cytogenetic analysis revealed a normal karyotype. Next-generation sequencing (NGS) of a targeted gene panel (including ASXL1, BCOR, DNMT3A, EZH2, IDH1, IDH2, JAK2, KRAS, NRAS, RUNX1, SF3B1, TET2 and TP53, among other genes) revealed no significant somatic mutations.

Further investigations were performed to exclude underlying conditions. Genetic testing for genes indicating IBMFs (including FANCA, FANCB, FANCC, FANCD1, FANCD2, FANCE, FANCF, FANCG, FANCL, FANCM, SAMD9, SAMD9L, GATA2 and DKC1) yielded negative results. A chromosomal breakage test for Fanconi anemia was also negative. Additional workup ruled out parvovirus B19 infection, vitamin B12 deficiency and folate deficiency as causes of the hypoplastic BM. Serum antinuclear antibody and rheumatoid factor results were negative, and serum ferritin levels were within the normal range.

On the basis of these comprehensive findings, a definitive diagnosis of RCC was established. Human leukocyte antigen typing within the family failed to identify a matched donor. Given an initial ANC $>1.0 \times 10^9/l$ and the absence of high-risk cytogenetic features, a watch-and-wait strategy was initially adopted. However, at the 2-month follow-up, the ANC had decreased to $<1.0 \times 10^9/l$, and the patient had become platelet transfusion-dependent. The patient's parents declined HSCT due to concerns over procedural risks and financial burden.

Consequently, IST with cyclosporine A (CsA) at 150 mg daily (5 mg/kg/day) was initiated alongside transfusion support. However, after 2 weeks, the patient developed severe drug-induced liver injury, as evidenced by markedly elevated liver enzymes [alanine aminotransferase, 1,178 U/l (normal

range, 10-40 U/l); aspartate aminotransferase, 1,090 U/l [normal range, 10-40 U/l]]. CsA was promptly discontinued, and IST was deemed intolerable.

Following the recovery of liver function, decitabine treatment was initiated in January 2016 at a dosage of 10 mg/m²/day for 5 days per 28-day cycle. Hematological improvement was observed after two cycles, with a maximum hemoglobin concentration >60 g/l and a platelet count of $\sim 40 \times 10^9/l$. The transfusion frequency was decreased accordingly, and the patient achieved transfusion independence by the third cycle.

However, after the third cycle, the patient developed a severe secondary infection accompanied by high-grade fever (peak temperature, 39.5°C). Management included administration of granulocyte colony-stimulating factor at 5 µg/kg/day subcutaneously for 14 days and recombinant human thrombopoietin at 300 U/kg/day subcutaneously for 12 days to support hematopoietic recovery, along with meropenem at 20 mg/kg intravenously every 8 h for a 10-day course for anti-infective therapy. The infection was successfully controlled, and the body temperature normalized within ~2 weeks. A period of severe neutropenia ($<0.5 \times 10^9/l$) persisted for ~6 weeks, with blood counts recovering by the seventh week. Transfusion independence was maintained throughout this period.

The patient completed a total of eight cycles of decitabine. Complete peripheral blood recovery was achieved after the fourth cycle, with a platelet count of $100 \times 10^9/l$ and an ANC of $1.7 \times 10^9/l$ (Table I). The post-treatment evaluation included the collection of BM aspirates in June 2017 (Fig. 2), March 2018 and March 2019, all of which confirmed stable disease without evidence of progression. Repeated flow cytometry analyses during this period yielded negative results. Given the sustained normalization of the peripheral blood counts, a follow-up BM biopsy was deemed unnecessary. As of the most recent follow-up on August 21, 2024, the patient continues to maintain a stable clinical status under routine outpatient surveillance (every 3 to 6 months).

Discussion

Childhood MDS represents a diverse category of clonal hematopoietic diseases characterized by their rare occurrence. RCC represents the most common subtype of pediatric MDS and was first classified as a provisional entity in the 2008 World Health Organization classification revision (9).

Pancytopenia accompanied by reduced BM cellularity in children has diverse etiologies, with acquired severe aplastic anemia, IBMFs and RCC representing the three most common hematopoietic disorders (2). This overlap results in specific challenges regarding the diagnosis of RCC. The diagnosis of RCC is primarily based on morphological criteria for MDS, which specifically include $<5\%$ blasts in the BM, $<2\%$ blasts in the peripheral blood and the presence of dysplastic features (which are most frequently observed in the erythroid and megakaryocytic lineages) (1). A significant diagnostic hurdle involves the phenotypic mimicry between RCC and certain IBMFs, such as Fanconi anemia, which can present with similar morphological characteristics (10).

In this context, ancillary techniques are crucial for the differential diagnosis. Immunohistochemical staining of BM biopsies for megakaryocytic antigens (such as CD61, CD41, and

Table I. Peripheral blood indicator changes across different treatment cycles.

Decitabine cycles	WBCs ($\times 10^9/l$)	RBCs ($\times 10^{12}/l$)	ANC ($\times 10^9/l$)	HB, g/l	PLTs ($\times 10^9/l$)
Cycle 1	1.05 (0.73, 1.28)	2.16 (1.90, 2.89)	0.30 (0.13, 0.65)	70.0 (60.5, 89.0)	18.5 (9.0, 53.5)
Cycle 2	0.80 (0.73, 1.10)	1.98 (1.71, 2.23)	0.10 (0.10, 0.30)	61.5 (56.5, 70.8)	12.0 (8.3, 36.0)
Cycle 3	1.00 (0.80, 1.70)	2.23 (2.04, 2.55)	0.10 (0.10, 0.23)	69.5 (61.8, 76.3)	21.5 (7.8, 34.3)
Cycle 4	1.70 (1.18, 1.95)	2.79 (2.30, 3.10)	0.40 (0.10, 0.60)	86.5 (71.8, 103.3)	39.0 (14.0, 67.0)
Cycle 5	3.10 (2.48, 4.63)	3.72 (3.67, 3.78)	1.20 (0.48, 1.93)	125.5 (123.8, 131.3)	86.5 (72.5, 111.8)
Cycle 6	4.10 (3.70, 4.63)	3.71 (3.58, 3.87)	1.55 (1.28, 1.83)	126.0 (119.5, 130.3)	114.5 (77.5, 145.8)
Cycle 7	4.20 (3.93, 4.98)	4.12 (3.87, 4.19)	1.75 (1.45, 2.38)	135.0 (127.3, 135.8)	152.0 (146.5, 180.0)
Cycle 8	5.00 (4.25, 5.38)	4.12 (3.95, 4.17)	2.30 (1.80, 3.70)	139.5 (131.3, 143.3)	205.5 (170.5, 235.3)
2 years after treatment	5.1 (5.00, 5.20)	4.23 (4.10, 4.25)	2.80 (2.70, 2.82)	138.0 (134.0, 140.0)	264.0 (245.0, 268.0)

Data are presented as the median (lower quartile, upper quartile). WBC, white blood cell; RBC, red blood cell; ANC, absolute neutrophil count; HB, hemoglobin; PLT, platelet.

CD42b) can aid in identifying micromegakaryocytes, which is a feature that supports a diagnosis of RCC over aplastic anemia or other conditions (11). Cytogenetic abnormalities, which are detected in ~30% of RCC cases, provide another diagnostic clue, with monosomy 7 being the most frequent finding (12).

Flow cytometry is widely employed in MDS diagnosis due to its ability to detect abnormal maturation patterns or aberrant antigen expression that is indicative of dysplasia (13). However, its utility in RCC or low-grade MDS is limited, as the results are often observed to be within normal limits. The advent of NGS has revealed a distinct mutational landscape in childhood MDS (cMDS). Specifically, germline mutations in genes such as SAMD9/SAMD9L and GATA2 are detected in patients with MDS across the blast spectrum (14), whereas somatic mutations are more common in patients with MDS with increased blasts. Notably, somatic mutations in genes such as ASXL1, RUNX1, SETBP1 and KRAS are frequently identified in ~46% of patients with secondary MDS or IBMFs (15,16). By contrast, mutations that are typically detected in adult MDS (such as TET2, DNMT3A, SF3B1, and SRSF2) are typically absent in cMDS (17).

The prognosis of cMDS is influenced by the blast percentage, complex karyotypes and single-lineage dysplasia (18). To the best of our knowledge, there are no reports on decitabine monotherapy for cMDS, and data on the effect of genetic mutations on prognosis are scarce. A recent study showed that decitabine-based combination therapy or bridging to transplantation improves survival (19). Long-term remission with decitabine-based combination therapy has been reported in non-transplanted patients (20). Although the prognostic effect of specific genetic abnormalities remains an area of active investigation, emerging evidence suggests that SETBP1 mutations may be associated with shortened overall survival (OS) time (17).

The management of RCC remains controversial and is guided primarily by clinical and cytogenetic risk stratification. Allogeneic HSCT is considered a curative option for children with MDS, particularly those demonstrating a high risk of disease progression (20). For patients with low-risk RCC who are not transfusion dependent and who lack significant neutropenia, a watch-and-wait strategy is generally

recommended (5). Conversely, therapeutic interventions (such as immunosuppressive therapy) are indicated for those with persistent neutropenia and/or transfusion dependency for platelets or red blood cells (2). Despite these general principles, data on effective treatment strategies for patients with RCC who are ineligible for either HSCT or immunosuppressive therapy remain limited.

Epigenetic therapies (particularly DNMT inhibitors such as decitabine and azacitidine) are being increasingly utilized in the treatment of adult MDS. Although traditionally indicated for intermediate- to high-risk MDS, emerging evidence supports the use of hypomethylating agents in low-risk MDS with multilineage dysplasia, where they have demonstrated clinical benefit (21). Nevertheless, the optimal dosing and scheduling of decitabine remain to be fully established.

The efficacy of decitabine has been evaluated across different dosing schedules in clinical trials conducted at the MD Anderson Cancer Center. The standard 3-day regimen of decitabine was established through comparative studies of earlier dosing schedules, and subsequent trials have confirmed the efficacy of an alternative 5-day schedule (22,23). In one randomized study on adult patients with MDS, two regimens were compared, including 20 mg/m² decitabine administered daily for 3 days every 28 days vs. 20 mg/m² decitabine administered weekly for 3 weeks every 28 days. The two schedules demonstrated comparable efficacy, with an objective response rate of 23%, no drug-related mortality and transfusion independence being achieved in 67% of the patients. Moreover, the median OS time was not reached, and 70% of the patients were alive at 500 days, thereby supporting the use of low-dose decitabine in this patient population (7).

However, the use of hypomethylating agents in pediatric MDS remains limited. Emerging evidence suggests that high-risk pediatric patients with MDS may benefit from decitabine-containing conditioning regimens prior to treatment with allogeneic HSCT. In one study involving 27 children with high-risk MDS, decitabine-based conditioning was associated with an 84.8% 3-year OS rate, thus indicating excellent outcomes in this population (19). Similarly, another study including 20 children with MDS demonstrated that compared

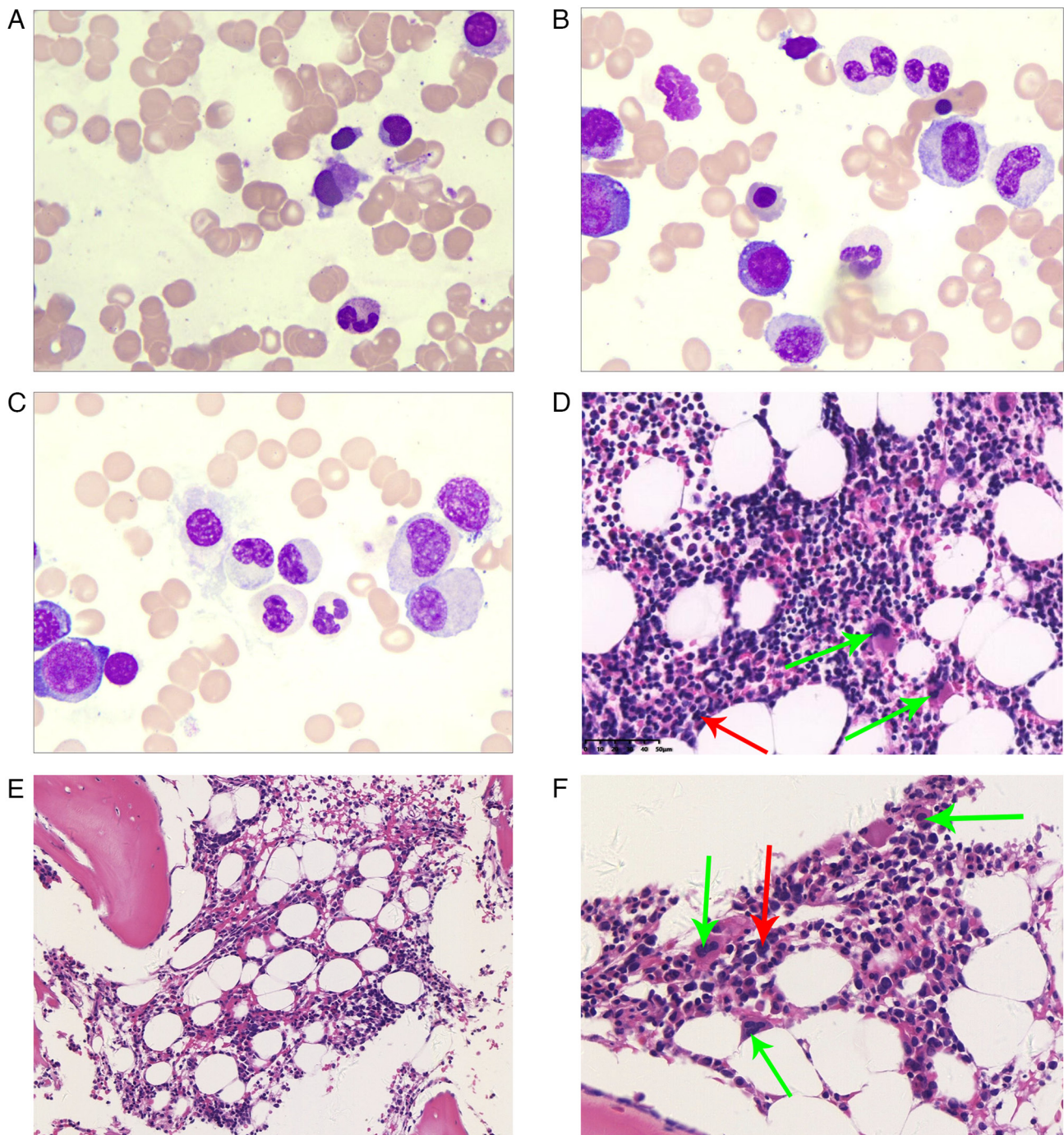


Figure 1. (A) BM aspirate demonstrating small megakaryocytes (Wright-Giemsa stain; x400 magnification). (B and C) Dysplastic neutrophils exhibiting nuclear hypoblobation and abnormal chromatin condensation (Wright-Giemsa stain; x400 magnification). (D) BM biopsy showing dysplastic features in the neutrophil lineage (red arrow) and megakaryocytes with nuclear hyperchromasia and irregular nuclear contours (green arrows) (H&E stain; x400 magnification). (E) BM biopsy revealing hypocellularity with ~50% cellularity (H&E stain; x200 magnification). For this pediatric patient, the normal range of the proportion of BM hematopoietic tissue was 80-90%. Therefore, hypocellularity was considered present. (F) High-power view demonstrating dysplastic granulocytes (red arrows) and hypolobated megakaryocytes (green arrows); no definitive morphological features diagnostic of refractory cytopenia of childhood were observed (H&E staining; x400 magnification). BM, bone marrow; H&E, hematoxylin and eosin.

with those receiving AML-type chemotherapy, the subgroup receiving decitabine combined with a minimally myelosuppressive regimen before transplantation achieved significantly better survival, with a 4-year OS rate of $84.6 \pm 10\%$, compared with $66.7 \pm 27.2\%$ in the decitabine alone group and $0.0 \pm 0.0\%$ in the chemotherapy group (20).

By contrast, data concerning the use of DNMT inhibitors in non-transplanted pediatric patients with MDS remain

scarce. A report by Glasser *et al* (24) described the cases of two children with secondary AML and MDS who achieved BM disease stabilization following treatment with decitabine and vorinostat, highlighting a potential role for epigenetic therapy outside the transplant setting.

In the present patient, the primary clinical manifestation was pancytopenia, without evidence of high-risk features for progression to advanced MDS or AML. Treatment was

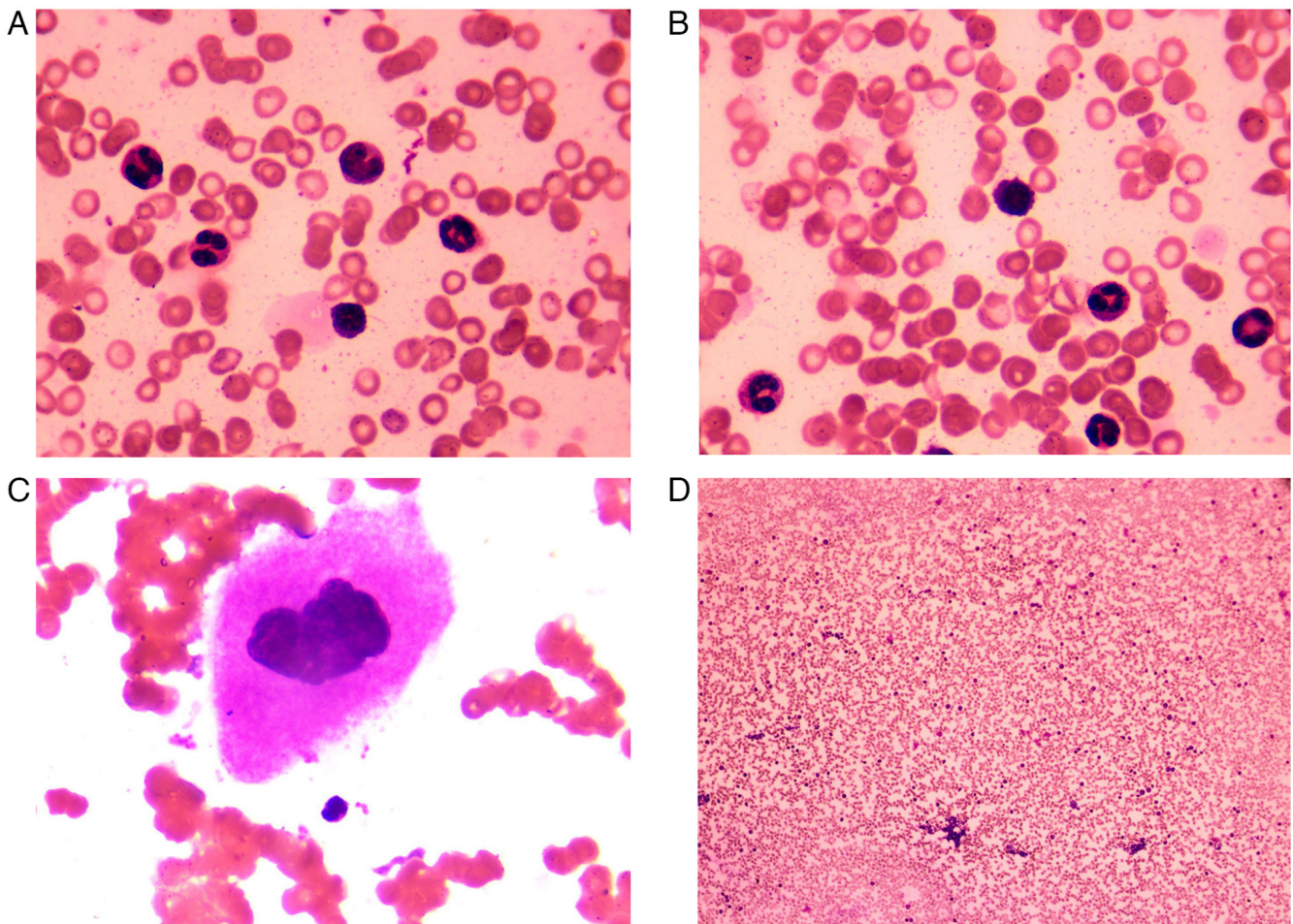


Figure 2. (A and B) Normal mature neutrophils and erythroblasts were observed, with complete resolution of dysplastic features in both granulocytic and erythroid lineages. (C) Morphologically normal granular megakaryocytes with adequate lobulation were also identified (Wright-Giemsa stain; x400 magnification). (D) Following decitabine treatment, bone marrow aspirate demonstrated active marrow hyperplasia (Wright-Giemsa stain; x100 magnification).

indicated due to transfusion dependency and significant neutropenia. However, therapeutic options for RCC or cMDS beyond IST and transplantation remain limited.

Currently, the use of decitabine in cMDS has rarely been reported and has been observed mainly in combination regimens or as a bridge to transplantation, with no report on its use as monotherapy. The present patient received 8 cycles of decitabine monotherapy, with peripheral blood recovery observed after 4 cycles; moreover, disease stability has been maintained to date.

To the best of our knowledge, there are no previous reports of decitabine monotherapy for cMDS, and this is the first case in which such monotherapy was used in this context. Moreover, the present case has the longest follow-up reported to date, with the patient continuing to demonstrate sustained remission with this treatment approach. This case suggests that decitabine may represent a viable treatment option for patients with low-risk cMDS or RCC, particularly those who are not suitable candidates for transplantation or who cannot tolerate immunosuppressive therapy. However, further data from larger prospective studies are necessary to confirm the safety and efficacy of epigenetic therapy (whether administered as monotherapy or in combination regimens) in pediatric patients with MDS.

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Availability of data and materials

The data generated in the present study may be found in the Genome Sequence Archive of the National Genomics Data Center, China National Center for Bioinformation/Beijing Institute of Genomics, Chinese Academy of Sciences under accession number HRA015221 or at the following URL: <https://ngdc.cncb.ac.cn/gsa-human/browse/HRA015221>. The remaining data generated in the present study may be requested from the corresponding author.

Authors' contributions

ZF and JZ contributed to the conception of the study. ZF collected the data and wrote the manuscript. AS, JZ and NX analyzed patient data and revised the manuscript. ZF and JZ

confirm the authenticity of all the raw data. All authors have read and approved the manuscript.

Ethics approval and consent to participate

Not applicable.

Patient consent for publication

Written informed consent was obtained from the parents of the patient for the publication of the present study.

Competing interests

The authors declare that they have no competing interests.

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