



Remarkable Recovery of Low-Risk Myelodysplastic Syndrome, A case of complete Hematologic Remission .

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A B S T R A C T

Background: Pediatric myelodysplastic syndrome is rare and typically presents with unexplained cytopenias. We report a case of a previously healthy 14-year-old male presenting with severe pancytopenia diagnosed as low-risk MDS ,who showed a marked hematologic response to immunosuppressive therapy with cyclosporine and eltrombopag.

Case Presentation: The patient presented with dizziness and severe pancytopenia (anemia , Leukopenia, thrombocytopenia). Initial workup excluded nutritional deficiencies, viral etiologies, and hemolysis. Bone marrow biopsy revealed hypocellular marrow with rare megakaryocytes and blasts at 2%, consistent with Myelodysplastic syndrome. Management included Intensive care unite admission, transfusions, corticosteroids, cyclosporine, and eltrombopag. Substantial hematologic improvement was seen within three months.

Conclusion: Immunosuppressive therapy resulted in full hematologic recovery and normalization of bone marrow function. This case underscores the importance of early recognition and prompt treatment of pediatric myelodysplastic syndrome .

1 Introduction

Myelodysplastic syndromes (MDS) represent a heterogeneous group of clonal bone marrow disorders characterized by ineffective hematopoiesis, pancytopenia, and risk of progression to acute myeloid leukemia (AML).(Daniel S et al ,:2021).

Pediatric MDS accounts for less than 5% of hematologic malignancies in children, making it significantly rarer than adult form(Martina R et al ,:2022).

Clinical presentation often includes fatigue, recurrent infections, or bleeding tendencies due to cytopenias.(Franco L et al ,: 2018).

Diagnosis relies on a combination of laboratory findings, bone marrow morphology, cytogenetic analysis, and the exclusion of secondary causes. (Zaina Iet al ,: 2025)

Treatment options vary depending on age, risk category, and severity of pancytopenia. Here, we present a case of low-risk MDS in an adolescent male successfully managed with immunosuppressive therapy.(Yoshida N et al ,:2020).

Supportive care, Red blood cell transfusions are indicated for symptomatic anemia, and platelet transfusions are reserved for severe thrombocytopenia or bleeding, erythropoiesis-stimulating agents (ESAs) are effective in low-risk, transfusion-dependent patients with low endogenous erythropoietin levels. Granulocyte colony-stimulating factor (G-CSF) may be used in neutropenia patients to reduce infection risk.(Franco L et al,2018)

In cases of Low-Risk MDS ,Immunosuppressive Therapy: Antithymocyte globulin (ATG) with or without cyclosporine is considered in younger patients with hypo-cellular marrow or HLA-DR15 positivity. Hypo-methylating Agents: Generally reserved for

higher-risk or ESA-refractory low-risk patients. (Fenaux *et al.*, 2010; Malcovati *et al.*, 2013).

in High-Risk MDS , allogeneic Hematopoietic Stem Cell Transplant (HSCT),the only potentially curative option. (Brodsky RA.2014)

Routine complete blood counts every 4–8 weeks, bone marrow evaluation to assess disease progression or response to therapy, monitoring for complications such as AML transformation, iron overload, infections, and bleeding.

1.1 Case Presentation

A 14-year-old previously healthy male presented with dizziness and generalized fatigue. There was no history of chronic illness, recent infection, drug exposure, or toxin exposure. On examination, the patient appeared pale without jaundice. He was afebrile, and no palpable lymph nodes were detected. Cardiovascular and respiratory examinations were unremarkable. Abdominal examination showed no hepatosplenomegaly or tenderness. Neurological examination revealed normal cranial nerves but decreased motor power (3/5). No ecchymosis were observed.

1.2 Ethic Approval

Ethical approval for reporting this clinical case was obtained in February 2025 . Written informed consent was obtained from patient's parents for publication of clinical details , laboratory data, and follow up outcomes. The all identifying information has been removed to ensure patient confidentiality .

1.3 Materials and Methods

Clinical information, laboratory results, imaging findings, and treatment outcomes were extracted directly from the hematology department and by the attending hematologist.

Laboratory investigations need included a complete blood count, reticulocyte count, liver function tests, Renal blood test , peripheral blood smear, Viral Serological (HBV,HCV,CMV) coagulation profile (INR , APTT) Serum electrolytes (Na, K, Cl), Hemoglobin electrophoresis,

Bone marrow biopsy, Imaging studies, including abdominal ultrasound, neck and axillary ultrasound, and chest X-ray.

Investigations

Initial laboratory findings were significant for pancytopenia: hemoglobin 7.4 g/dL, WBC $4.8 \times 10^9/L$, and platelets $16 \times 10^9/L$. MCV was 95 fL and MCHC 37 g/dL. Ferritin level was 65 ng/mL, LDH 235 U/L, and CRP 0.6 mg/L. Liver and kidney function tests were within normal limits. Serum electrolytes (Na, K, Cl) were normal. Bilirubin levels were mildly elevated (total 1.6 mg/dL; direct 0.9 mg/dL; indirect 0.7 mg/dL). Coagulation profile was normal (INR 1.2, aPTT 12

seconds). Viral serologies for HBV, HCV, HIV, and CMV were negative. Hemoglobin electrophoresis was normal.

Imaging studies, including abdominal ultrasound, neck and axillary ultrasound, and chest X-ray, showed no abnormalities. Peripheral blood smear revealed normocytic hypochromic RBCs, leukopenia, thrombocytopenia, and overall pancytopenia without blasts.

Bone marrow biopsy demonstrated hypocellularity with rare megakaryocytes, erythroid precursors at 34%, granulocytic lineage at 50%, lymphocytes 14%, and blasts at 2%. These findings were consistent with low-risk myelodysplastic syndrome

Table 1: Initial Laboratory Findings

Parameter	Result
Hemoglobin	7.4 g/dL
WBC	$4.8 \times 10^9/L$
Platelets	$16 \times 10^9/L$
MCV	95 fL
MCHC	37 g/dL
Ferritin	65 ng/mL
LDH	235 U/L
Bilirubin	1.6 mg/dL
CRP	0.6 mg/L

2 Results

The patient was admitted to the intensive care unit (ICU) and received four units of packed red blood cells. Intravenous broad-spectrum antibiotics were initiated, along with oral prednisolone at 1 mg/kg/day. After stabilization, he was transferred to a specialized hematology center and started on cyclosporine (125 mg twice daily) and eltrombopag (100 mg daily). Monthly follow-up was arranged.

Follow-up and Outcomes

After three months of therapy, significant improvement was noted: hemoglobin increased to 11 g/dL, WBC to $4.5 \times 10^9/L$, and platelets to $50 \times 10^9/L$. A repeat bone marrow biopsy showed increased megakaryocytes, stable blasts at 2%, erythroid lineage at 45%, and granulocytes 20%. Based on clinical improvement, cyclosporine was tapered gradually while eltrombopag was continued.

At the most recent follow-up, after 2 months ,the patient had discontinued all medications for one month and remained stable with hemoglobin 13 g/dL, WBC $6 \times 10^9/L$, and platelets $200 \times 10^9/L$. Bone marrow

examination was reported as normal, indicating complete hematologic recovery.

Table 2 : CBC Comparison

Parameter	On Admission	3 Months	Final Follow-up (5 months)
Hemoglobin	7.4	11	13
WBC	4.8	4.5	6
Platelets	16	50	200

Table 3 : Bone Marrow Comparison

Parameter	Initial	After 3 Months
Megakaryocytes	Rare	Increased
Blasts	2%	2%
Erythroid Line	34%	45%
Leukocytes	14%	20%

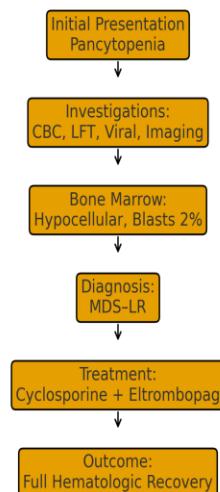
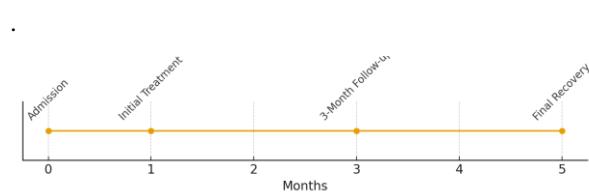


Figure 1: Diagnostic and Treatment Flowchart

3 Discussion

Pediatric MDS is rare and typically requires high clinical suspicion for diagnosis. This case highlights a classic presentation with severe pancytopenia but without Organomegaly, lymphadenopathy, or evidence of infection. Bone marrow biopsy was essential for diagnosis, showing hypocellularity and low blast percentage consistent with low-risk MDS.

Immunosuppressive therapy (IST), particularly with cyclosporine, has been shown to benefit selected MDS patients especially those with hypocellular bone marrow resembling aplastic anemia physiology. Eltrombopag, a thrombopoietin receptor agonist, has demonstrated synergistic effects with cyclosporine in promoting trilineage hematopoiesis. (Scheinberg P et al ,:2012). The rapid improvement observed in this patient aligns with reports showing improved hematologic parameters in pediatric patients undergoing IST(Danielle M et al ,:2017).

Early diagnosis and tailored therapy are crucial for preventing disease progression and reducing transfusion requirements. This case adds to the growing evidence supporting IST as an effective treatment modality in pediatric low-risk MDS.(Mikael A et al ,:2022) This should explore the significance of the results of the work, not repeat them.

4 Conclusions

This case demonstrates successful diagnosis and management of pediatric low-risk MDS using a combination of cyclosporine and eltrombopag, resulting in complete hematologic recovery. Early recognition, appropriate supportive care, and timely initiation of immunosuppressive therapy were essential to achieving remission

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