

Clinical Presentation and Treatment Outcomes of Pediatric Acute Promyelocytic Leukemia: A Study from a Developing Country

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Background and Aims

Acute Promyelocytic Leukemia (APL) constitutes **4–8%** of pediatric acute myeloid leukemia. Treatment with all-trans retinoic acid (ATRA) and arsenic trioxide (ATO) has improved survival to nearly **90%**. However, chemotherapy-related toxicities, particularly anthracycline-induced cardiotoxicity, remain a significant concern. Recent evidence supports chemotherapy-free protocols for low-risk APL and reduced anthracycline use for high-risk cases. This study evaluates the impact of this approach on morbidity and survival in pediatric APL.

Methods

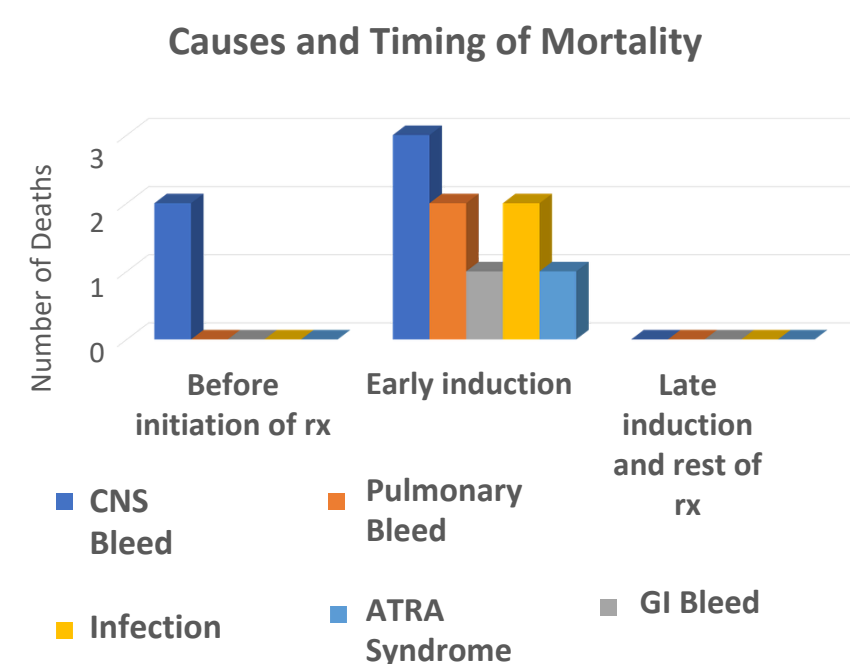
- **Design:** Retrospective study at the Indus Hospital and Health Network, January 2019 – December 2022.
- **Population:** Patients aged 1–16 years diagnosed with APL.
- **Data:** Demographics, risk stratification, treatment protocols, and outcomes from electronic medical records.
- **Analysis:** SPSS v26.0; Kaplan-Meier curves for survival; Chi-square or Fisher's exact tests for associations.

Results

- A total of **50 patients** were diagnosed (mean age 11.4 ± 3.4 years); 28 (56%) were male and 22 (44%) female.
- **34 patients (68%)** were high-risk ($WBC \geq 10,000/\mu L$).
- Of 38 patients eligible for post-consolidation molecular remission assessment, **36 (95%)** attained remission.
- Overall disease-free survival (DFS) was **60% (30/50)**, rising to **65% (30/46)** after excluding treatment abandonment.
- Kaplan-Meier OS was **81%** in standard-risk (SR) vs **50%** in high-risk (HR) patients; survival differed significantly by risk group ($p = 0.030$).

Clinical outcomes compared by risk group using Fisher's exact test			
Outcome Variable	Low-Risk (n = 16)	High-Risk (n = 34)	p-value
Post-Induction Morphologic Remission (n=32) n (%)	11/11 (100%)	21/21 (100%)	1.000
Post-Consolidation Molecular Remission (n=38) n (%)	15/15 (100%)	23/23 (100%)	1.000
Relapse, n (%)	2 (12.5%)	3 (8.8%)	0.650
Abandonment, n (%)	0 (0%)	4 (12%)	0.004
Mortality, n (%)	1 (6.3%)	10 (29.4%)	0.004

Demographic and clinical profile of the study cohort				
Variables	All patients (n=50)	Low-Risk (WCC < 10,000/ μ L, n=16)	High-Risk (WCC $\geq$ 10,000/ μ L, n=34)	p-value
Age (years), Median (IQR)	11 (8-14)	12 (8.25-16)	10 (7.75-14)	0.201a
Sex, n (%)				0.981b
Male	28 (56)	09 (56.3)	19 (55.9)	
Female	22 (44)	07 (43.7)	15 (44.1)	
WCC ($\times 10^9$ /L), Median (IQR)	18.50 (4.60–38.88)	3.1 (1.69–4.60)	29.0 (18–61.25)	0.000*a
Platelet count ($\times 10^9$ /L), n (%)				0.474b
<20	36 (72%)	13 (81.25%)	23 (67.6%)	
20–49	12 (24%)	03 (18.75%)	09 (26.4%)	
≥ 50	02 (04%)	00 (0%)	02 (5.80%)	
Fibrinogen#, n (%)				0.520c
Low (< 150 mg/dl)	16 (32.7%)	06 (37.5%)	10 (29.5%)	
Normal (≥ 150 mg/dl)	33 (67.3%)	09 (56.5%)	24 (70.5%)	
Detection of PML-RARA by FISH, n (%)	50 (100%)	16 (100%)	34 (100%)	0.322c



Conclusions

- The molecular remission rate of **95%** highlights the effectiveness of this approach.
- However, the high mortality in high-risk patients and an overall DFS of **60%** underscore the need for enhanced supportive care and tailored strategies to minimize and address complications.
- These findings emphasize the potential of **individualized, low-toxicity protocols** in pediatric APL, particularly in resource-limited settings.