Confirmation of diagnosis in LAD-I requires demonstration of absence of CD18 and associated alpha subunit of CD11a, CD11b, CD11c on flow cytometry. 4 of 6 patients had complete absence of the integrins. Differential diagnosis include Interleukin-1 receptor associated kinase deficiency, Hyper IgE syndrome and leukemoid reaction. The only corrective entity, especially, its course and outcome in resource limited countries.

Patients and methods: The case records of children who had been diagnosed to have AA since 2002 at our centre were reviewed. The diagnosis of AA was made in children with pancytopenia without organomegaly and confirmed by hypoeucellular bone marrow showing decreased expression of all three hematopoietic lineages. Clinical findings including dysmorphism, thumb abnormalities, congenital malformations and skin hyperpigmentation were noted down. Mitomycin C induced chromosomal instability to rule out Fanconi anaemia, Ham's test to look for paroxysmal nocturnal haemoglobinuria and chromosomal analysis to look for complex karyotypic abnormalities seen in myelodysplastic syndrome were documented in all patients.

After initial stabilization, the option of haematopoietic stem cell transplantation (HSCT) was offered to all those who had matched related donors. If not, immunosuppressive therapy was delivered within two months from diagnosis after the child was infection free. A combination of 40mg/kg/day of Horse derived antithymocyte globulin (ATGAM) and methyl-prednisolone at 2 mg/kg/day were started for the first 4 days through a central venous catheter. Oral cyclosporine and G-CSF were started on the fifth day and since 2015 Eltrombopag was added to the IST regimen. Revolode and GCSF were continued for about 8 to 12 weeks and cyclosporine from 6 to 12 months. All patient received Voriconazole and Acylcoylin prophyaxis till neutrophil recovery to 1000.

Results: A total of 109 children had been diagnosed as AA, of whom, 42 had acquired AA. The mean age of children with acquired AA was 8.16 years (2-15 yrs). Boys were twice more commonly affected (ratio M: F=2:1). Details are listed in table 1.

Of the 41, 21 underwent HSCT as they had fully matched related donor and 20 children received IST. Of the 20 children who received ATG, 10 children (70%) had complete remission in a median duration of 78 days. Two of the three non responders were treated with second IST and all three succumbed to the illness. One child developed acute myeloid leukaemia after achieving partial remission and was salvaged with HSCT, and 1 more non responder had an unrelated HSCT and is doing well. One child has suffered a relapse after a durable remission of over 18 months off immunosuppression. The 21 children who have been transplanted have an overall survival rate of 77%.

Conclusion: Immunosuppressive regimen including Horse ATGAM, Methyl prednisolone, Cyclosporine, GCSF and Eltrombopag along with adequate and meticulous supportive care including neutropenia care, pneumonia infection management and the use of irradiated and leukodepleted blood products results in a 70% response rate and should be offered to all children with aplastic anaemia with no matched family donor. The addition of Eltrombopag has resulted in early recovery of all three cell lines and must be added to the IST protocol in children.

RBC-1_V1.2 DISCORDANT BEATS – A PROSPECTIVE STUDY OF CARDIAC MRI TO ASSESS IRON OVERLOAD IN PATIENTS WITH THALASSAEMIA MAJOR

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Introduction: Advances in bone banking has ensured optimal delivery of monthly transfusion needed to save the lives of about 10,000 new thalassaemia children born each year in our country. The burden of iron chelation has now increased as these children survive into their second and third decade of life. Cardiac failure secondary to myocardial haemosiderosis is the most common cause of mortality in these patients. Our study has been done to evaluate the prevalence and severity of cardiac ironload in children with beta thalassemia major and the effectiveness of serum ferritin as a marker of iron overload.

Patients and Methods: We conducted a prospective study from the year 2015-2016 where we performed T2* cardiac magnetic resonance (CMR), serum ferritin levels and pulmonary artery pressures by echocardiography in 100 patients with transfusion dependent beta thalassaemia major. The study had been approved by IRB at the VHS centre and informed consent was obtained from all of these families. The median age of our patients was 14 (range 7 TO 33 years) with 51% of them being female. The mean serum ferritin level was 2520.78 ±223.454 ng/ml. High iron overload with a serum ferritin of over 2500 was seen in 37% of our patients. The 29% patient group that had less than 1000 ferritin were considered to be chelated optimally as per international guidelines. A cardiac T2* of over 20 milliseconds was taken as a marker of heart free of iron overload.

The mean cardiac MRI T2* was 12.73 ±2.436 milliseconds. About 70% of our patients had moderate myocardial iron overload (T2* 10-20 ms) and 24% had severe iron overload (T2* <10ms). The mean pulmonary arterial pressure was 35.96±8.3 mm of Hg. About 76% of the patients were on a single drug chelation therapy at the time of evaluation. Following the cardiac iron status they have been started on combination chelation therapy consisting of deferasirox and deferiprone.

We found no significant correlation (r=0.014, p=0.23) between the serum ferritin levels and myocardial ironload. The most significant finding was that 38 % (11/29) of our patients with serum ferritin < 1000 had severe cardiac iron load. A significant correlation could also be established between pulmonary arterial pressures and cardiac MRI T2* (r=0.2, p=0.0024).

Figure 1. No correlation between serum ferritin and cardiac ironload.
Conclusion: All patients with transfusion dependent thalassaemia major should be screened with a cardiac MRI for iron overload from the age of 7 years as serum ferritin done serially alone does not detect tissue iron. Should be screened with a cardiac MRI for iron overload from the age of 7 years. Multiple transfusions and induction chemotherapy respectively. Patient with Kala Azar, chronic Malaria infections and hypersplenism survived after appropriate treatment. Also hospital stay was prolonged for leukemias and Aplastic Anemia.

Conclusion: The etiology of pancytopenia varies widely ranging from transient marrow viral suppression, drugs etc to marrow infiltration by life-threatening malignancy. The severity of pancytopenia and the underlying pathology determine the management and prognosis of the patients. Hence such studies will help in planning the diagnostic and therapeutic approach in patients with pancytopenia and curtailing delays to reduce multiple transfusions, pre-treatment infections and overall morbidity and mortality associated with entity of Pancytopenia.

RBC-1_V1.5
ADVERSE DRUG EVENTS WITH IRON CHELATION DRUGS IN THALASSEMIC PATIENTS ATTENDING A DAY CARE THALASSEMIA CENTRE IN TERTIARY CARE GOVT. MEDICAL COLLEGE IN NORTH INDIA

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Introduction: The last three decades have witnessed profound changes in the management of patients with thalassemia major. Regular and periodic blood transfusion being the mainstay of treatment results in iron overload thus necessitating need for iron chelation. At present three iron chelators namely, Deferoxamine, Deferiprone and defrasirox are in use alone or in combinations. Since the iron chelators are required to be given for the lifetime, they also cause adverse events which are range from trivial to life threatening.

Aims: To identify and manage adverse drug events due to iron chelating drugs experienced by thalassemic patients.

Material and methods: This is an observational study done during 1st January to 30th June 2016 in Thalassemia Day Care Centre of SGSM hospital, Govt. Medical College Jammu. All the adverse events were recorded on a pretested proforma and were validated on Naranjo causality assessment scale.

Results: Of the 280 patients with the age ranging from 6 months to 53 years registered with Thalassemia Day Care Centre SGSM hospital Govt. Medical College Jammu only 180 attended regular fortnightly follow up clinics. Out of these 180 patients 168 are on Iron chelation drugs (129 on Deferiprone, 24 on Defrasirox, 8 on Deferiprone + defrasirox, 4 on Defrasirox and 3 on Deferiprone + Defrasirox) Out of 32 events observed during the course of study 21 (65.62%) were due to Deferiprone, 8 (25%) due to Deferasirox and 3 (9.38%) due to Deferasirox. Out of 21 events due to Deferiprone 12 (57.14%) were gastrointestinal symptoms, 5 (23.8%) arthropathy, 2 (9.5%) Neutropenia and 2 (9.5%) thrombocytopenia. All 8 (100%) events due to Deferasirox were gastrointestinal symptoms. 2 (66.66%) events with defrasirox were local reactions and 1 (33.33%) was encephalitis. On Naranjo Causality scale 5/32 was definite while as 24/32 was probable and 3/32 were possible. While 9 were mild reactions, 4 were severe reactions and 19 were moderate reactions.

Conclusion: Majority of adverse events were observed with Deferiprone followed by Deferasirox. Although least adverse events were observed with deferasirox, but adverse events with defrasirox were least severe.

Keywords: Adverse drug events, Iron chelation, Thalassemia

RBC-1_V1.6
ACQUIRED APLASTIC ANAEMIA IN CHILDREN – OPTIMAL OUTCOMES DEPEND ON OPTIMAL DELIVERY OF IMMUNOSUPPRESSIVE THERAPY

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