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Recent advances in the management of thalassemia

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Abstract:

Thalassemia mainly β-thalassemia is a clinically heterogenous group of inherited disorders caused by mutations in the β-globin gene, leading to a decreased or absent production of the β-globin chain. In this disease there is imbalance in the α/β-globin chain production, which results in variable grades of ineffective erythropoiesis, chronic haemolytic anaemia, compensatory haemopoietic expansion, hypercoagulability, and increased iron absorption. Approximately 1.5% of the global populations are carriers for a β-thalassaemia mutation, with traditionally higher prevalence in populations of Middle East, the Mediterranean region, and Southeast Asia. However, large scale migrations have recently made thalassemia distribution worldwide. Improved public health measures have prolonged life expectancy of affected individuals in low- and middle-income countries, making β-thalassemia now a significant global health problem.

Current guidelines have adopted a clinical classification of thalassemia based on the magnitude and frequency of transfusion requirements, reflecting the severity of the disease. Patients with transfusion-dependent thalassemia present with severe anaemia as early as 6 months of age and require life-long blood transfusion to survive. Whereas in non-transfusion-dependent thalassemia, patients usually maintain haemoglobin (Hb) levels between 7 and 10 g/dL and may require transfusions less frequently. Thalassemic patients gradually develop clinically significant iron overload, mainly as a consequence of erythron expansion and increased iron absorption driven by hepcidin suppression.

CONCLUSION

The last 50 years have witnessed dramatic improvements in thalassaemia understanding and patient care. These improvements have built a series of previously unimaginable therapeutic opportunities for patients with thalassaemia, with many more on the way. All of this was made possible by a synergy between the various fields of biological and clinical research, which have mutually reinforced one another to lead to shared success.

Having access to many therapeutic opportunities is undoubtedly beneficial for patients, but as opportunities have grown, the cost of optimal therapies has increased dramatically, and so has the demand for a better selection of the appropriate sequence of treatments in terms of cost/benefit ratio. When compared to HCT (the only other available curative option), gene therapy results in, on average, an additional 300,000–400,000 EUR/patient, justified by the high costs of the viral vector and preparation procedures. On the other hand, as competition between different suppliers grows and follow-up monitoring becomes less stringent, the whole procedure should become more affordable; nonetheless, requirements in terms of professional skills, quality efficacy, and regulatory compliance still make it an unattractive approach in low-income countries. In conclusion, an effort must be made to progress, consistent with resource availability and still crucially impacting the real opportunities for benefiting from these advances in the real world.

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Biography:

Dr Vijay Kumar Dahiya MBBS MD PEDIATRICS. He live in Jagadhri Town of District Yamunanagar in Haryana (INDIA). He retired in February 2022 as District Medical Chief (civil surgeon Yamunanagar) after 29 years of service in Health Deptt Govt of Haryana. He had an inclination towards writing start from his early student days. He remained editor and chief editor of college magazine ROHMEDCOL of Medical college Rohtak. Many of his articles got published in national and international journals of repute. He had written two books and 2 brochures of information till date. His first book titled "Thalassemia and its management" has been published by Lambert Academic Publishing and it has been translated in more than 10 international languages. His second book titled "Stress during examination and ways to cope it up" has been published by spotwrite publications recently.

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