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Immunotherapy for Childhood Aplastic Anemia in India: A Case for Universal Healthcare?

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Aplastic anemia in childhood is a difficult condition to treat, and Nair and colleagues are to be commended for their meticulous collection of single-center data over a 22-year period [1]. They evaluated the efficacy of immunosuppressive therapy (IST) with antithymocyte globulin (ATG) and cyclosporine (CSA) on 33 pediatric patients with acquired aplastic anemia in the Indian setting. Their results indicate that in India, with appropriate supportive care, pediatric aplastic anemia patients treated with IST can achieve response rates similar to North America and Europe, where the response rate is as high as 81% [2]. In addition, they confirm the experience of other authors, that ATG and CSA alone are the backbone of IST in treating childhood aplastic anemia, and that administration of G-CSF reduced infectious complications, but had no impact on survival or outcome [3].

Nair, *et al*. [1] have added valuable data on clinical outcomes on childhood aplastic anemia in India. With an estimated population incidence in India of less than 2 per million, such clinical series are hard to collect and analyze. As they acknowledge, some of their data need to be interpreted with caution: median follow-up was only 24 months, and we know that there are significant changes in expected survival over time. As previously emphasized, for clinical studies in India to provide useful data, long-term follow up is of paramount importance, and this is especially true given the known late effects in aplastic anemia patients treated with IST [4].

The data yields other interesting trends: compared to the 42 pediatric patients treated with IST in Toronto by Pongtanakul, *et al*. [2], although response rates were similar (88 *vs* 81%), there was a significant decrease in the percentage of patients with complete response (CR) (24% *vs* 62%) with an increase in partial response (PR) (64% *vs* 19%). This predominance of partial responders was seen also in another Indian series reported by Chandra, *et al*. [5], who raised the question of effective cyclosporine administration, given the expense and uncertain quality in the open market. The only other published series by George, *et al*. [6], had 60 children who received ALG while 10 received ATG, with response seen in only 43.5%, evenly divided between CR and PR. We know the nature of the immune globulin product makes a significant difference, for example equine-ATG has been shown to be superior to rabbit ALG in terms of long-term response [7].

Since there is significant variation in both ATG and CSA available in the Indian market, we can speculate whether Nair, *et al*. could ensure that patients at the Army Hospital received medications of better quality than those who had to purchase it on the open market, but there is no easy way to verify this hypothesis. In summary, the most important lesson we have learned is that within Indian pediatric hematology there is no lack of knowledge or skill, and equivalent results to any other country can be achieved. The key lies in ensuring supply of appropriate medication and adequate supportive care, and as the World Health Organization grapples with the control of non-communicable disease, this is the deep-seated issue we have to struggle with: how do we ensure that all children in India receive access to curative healthcare?

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