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The severity of acquired aplastic anemia (AA) and long-term prognosis: lessons from current results of immunosuppressive treatment (IST)

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Abstract

Objectives and Methods: We retrospectively analyzed the outcome of 65 patients (39 M and 26 F, median age 21) with moderate (MAA 22), severe (SAA 26) and very severe (VSAA 17) AA treated with ATG and CsA +/- Daclizumab (44) or with CsA alone (21) from October 1995 to May 2009.

Results: Twenty-two (34%) and 25 (38%) patients achieved CR and PR, respectively (according to strict response criteria of B. Camitta, 2000). The quality of response was higher in the VSAA group (CR/PR=11/1) than in the SAA (9/10, p=0.02) and MAA (2/14, p=0.001) groups.

There were 2 early and 7 late deaths. Four patients (8.5%) relapsed and 3 responded again after re-treatment with ATG and CsA. Late events included MDS/AML (n=2), rectal cancer (n=1) and hemolytic PNH (n=5). The 5-year overall survival was 82±6% without any difference between the MAA (81.6%), SAA (82.1%), and VSAA (82.4%). Surprisingly, there was a marked trend towards better failure-free survival (FFS) in more severe disease (32.6±12.6%, 45.2±11.8% and 68.8±11.7% in MAA, SAA, and VSAA respectively), which was also observed even when the CsA group alone was excluded from analysis.

The results of the telomere length measurement in PB cells by flow-FISH analysis in 35 patients gave a possible explanation for better quality of response and FFS in VSAA. The responders with VSAA have a less marked telomere loss than in MAA and SAA patients, probably due to lack of intrinsic stem cell defects and early intensive IST.

Conclusions: Modern IST provides a high rate of hematological response and long-term survival in more than 80% of AA patients. Nevertheless, different treatment-failure events remain a crucial problem. The severity of AA is a controversial prognostic factor. Our data indicates that more severe AA predicts a better long-term prognosis.

Keywords: aplastic anemia, severity, ATG, cyclosporine, response, telomere, prognosis