Aplastic anemia in the elderly: a nationwide survey on behalf of the French Reference Center for Aplastic Anemia

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Received: May 24, 2018. Accepted: September 24, 2018. Pre-published: September 27, 2018.

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SUPPLEMENTARY MATERIAL

Statistical Analysis

Results are expressed as median, first and third quartile [Q1; Q3] and minimum and maximum (min; max) for quantitative data, and counts and percentages for categorical data. Comparisons of patient characteristics by first-line treatment were performed using Fisher tests for categorical variables and Kruskal-Wallis tests for quantitative variables. Survival curve was obtained using the Kaplan Meier estimator.

The impacts of baseline characteristics and treatment on mortality were evaluated in univariate analysis using a Cox model with time-dependent variables. Multivariable analysis using adjusted Cox models for treatment line, disease severity and performance status was performed to assess adjusted treatment effects. To respect the rule of thumb of the number of variables per event, it was not possible to incorporate all significant variables used in univariate analysis into the model. Consequently, variable adjustment was driven by clinical knowledge. The degrees of significance provided correspond to partial Wald tests. Hazard ratio (HR) and 95% confidence intervals (CI95%) were estimated.

Impacts of characteristics at each new treatment line and of treatment on overall response were evaluated in univariate analysis using Fisher tests for categorical variables and Wilcoxon tests for quantitative variables. Multivariable analysis using logistic models adjusted for treatment line, disease severity and performance status was performed to assess adjusted treatment effects. The p-values provided correspond to partial Wald tests. Odds Ratio (OR) and 95% confidence intervals (CI95%) were estimated.

All tests were bilateral at 5%. All analyses were carried out using R 3.3.1 (R: A Language and Environment for Statistical Computing, R Core Team, R Foundation for Statistical Computing).

Supplementary Table 1: Impact of patient characteristics at initiation of each treatment line on overall response (n=181)

n(%) or median [IQR]	Failure (n=112)	CR + PR (n=69)	р
Treatment line (n°)	2 [1;3] (1-5)	1 [1;2] (1-5)	0.04
Male	63 (56%)	35 (51%)	0.54
Age (years)	70 [64.75;73] (60-89)	67 [62;72] (60-82)	0.17
Weight (kg)	71 [61.75;80] (45-110)	69 [61;78] (50-102)	0.43
Charlson comorbidity index score	2 [1;3] (0-6)	1 [0;2] (0-5)	0.088
Performance status	1 [1;2] (0-4)	1 [0;1] (0-3)	0.028
Blood count			
$PMN (x10^{9}/L)$	0.68 [0.34;1.2] (0-12.5)	0.93 [0.425;1.53] (0-7.4)	0.086
Lymphocytes (x10 ⁹ /L)	1.15 [0.71;1.5] (0.031-4.8)	1.07 [0.8;1.798] (0.01-2.99)	0.5
Hemoglobin (g/dL)	8.5 [7.45;9.2] (3-13.6)	8.3 [7.4;9.3] (4.4-12)	0.47
MCV (fL)	91 [83.25;100.8] (76-115)	92 [88;99] (79-113.4)	0.25
Reticulocytes (x10 ⁹ /L)	21.65 [6.85;41.25] (0.9-84)	20 [13;42] (0-203)	0.46
Platelets (x10 ⁹ /L)	13.5 [6;27] (2-82)	12.5 [6.75;21.75] (1-154)	0.57
AA severity			
Mild	41 (37%)	28 (41%)	
Severe	48 (43%)	29 (42%)	0.92
Very severe	19 (17%)	11 (16%)	
Unknown	4 (3%)	1 (1%)	

Supplementary Table 1: Impact of patient characteristics at the initiation of each treatment line on overall response (n=181)

Abbreviations: AA: Aplastic Anemia; CR: Complete Response; IQR: Inter-quartile range; MCV: Mean Corpuscular Volume; PMN: Polymorphonuclear Neutrophils; PR: Partial Response.