

Time without transfusion reliance: a novel patient-centric metric for new therapies in myelodysplastic syndromes

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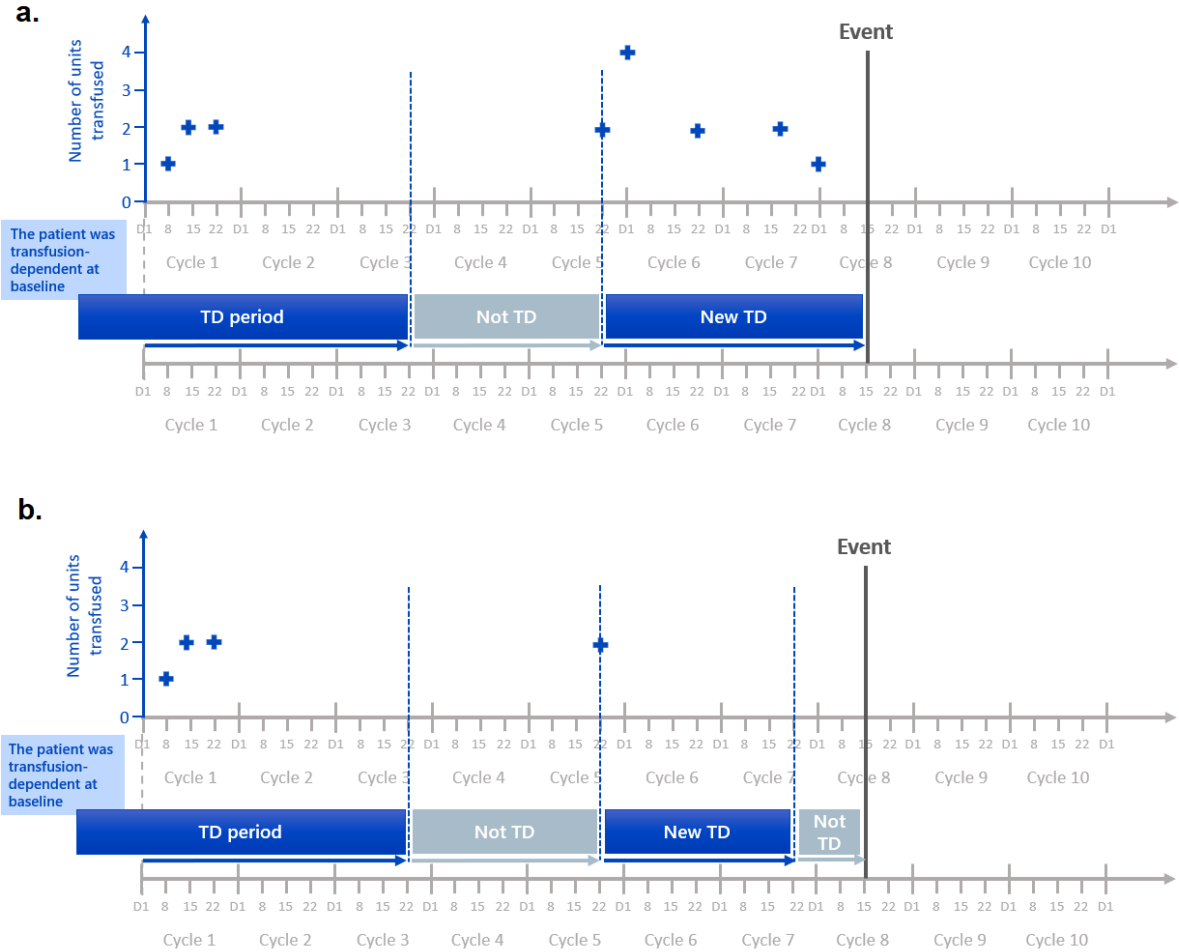
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Supplementary data



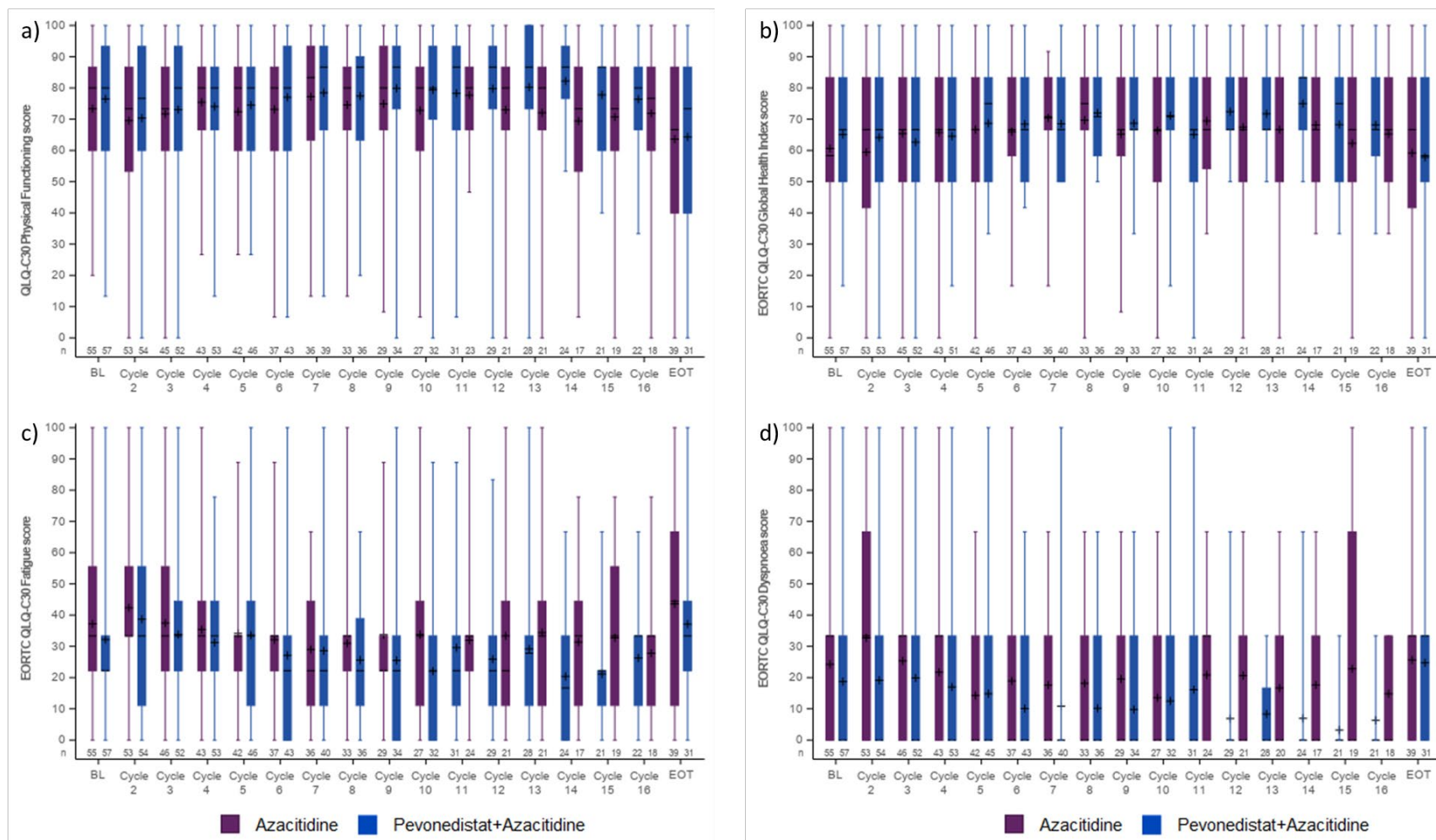
Supplemental figure 1: Illustration of decisions for calculation of transfusion dependence state duration: definition of transfusion dependence periods depending on the frequency of transfusions received by the patient with two scenarios

TD: transfusion dependence; D1: day 1.

The X-axis represents the time (in weeks and 28-day cycles); the Y-axis represents the number of blood units transfused; a blue cross represents the time at which the patient was transfused and the number of units transfused.

Scenario a presents patient experienced: 1) a 'TD period' of 11 weeks, starting from baseline to Cycle 3 Day 22; 2) a period of 8 weeks not reliant on transfusions, starting from Cycle 3 Day 22 to Cycle 5 Day 22; and 3) a TD period of 11 weeks, starting from Cycle 5 Day 22 to Cycle 8 Day 15; the TD state is therefore a period of 22 weeks.

Scenario b presents a patient experienced: 1) a 'TD period' of 11 weeks, from baseline to Cycle 3 Day 22; 2) a period of 8 weeks not reliant on transfusions, from Cycle 3 Day 22 to Cycle 5 Day 22; and 3) a TD period of 8 weeks, from Cycle 5 Day 22 to Cycle 7 Day 22; the TD state corresponds therefore to a period of 19 weeks.



Supplemental figure 2: No clear difference was observed in the distribution of key EORTC QLQ-C30 scores between arms from baseline at each available cycle over the treatment period in the PRO population (N=112) from P-2001 study

(a) EORTC QLQ-C30 Physical functioning score; (b) EORTC QLQ-C30 Global health/Quality of life score; (c) EORTC QLQ-C30 Fatigue score; (d) EORTC QLQ-C30 Dyspnea score.

BL: baseline visit; EORTC: European Organization for Research and Treatment of Cancer; EOT: end of treatment visit; PRO population: all patients with a patient-reported outcome (PRO) assessment at baseline and at least one post-baseline PRO assessment from the Intent-to-treat (ITT) population of P-2001 study (including higher-risk myelodysplastic syndromes/chronic myelomonocytic leukemia, and acute myeloid leukemia patients); QLQ-C30: Quality of Life Questionnaire-Core 30 items.