



PSDTA Neoplasie Mieloproliferative Croniche

Allegato 6 : Terapia di TE

A cura del Gruppo di Studio Sindromi Mieloproliferative
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Lo scopo della terapia nella TE è ridurre le complicanze trombotiche ed emorragiche. Per tutte le categorie di rischio si consiglia di monitorare e correggere i fattori di rischio cardiovascolare (fumo, diabete, dislipidemia, ipertensione, peso-BMI).

CRITERI DI RISPOSTA AL TRATTAMENTO

Figura 1. Criteri ELN di risposta alla terapia della TE (Barosi et al, *Blood* (2013) 121 (23): 4778–4781)

Response Grade	Response in ET
Complete response	1. Durable* resolution of disease-related signs** including palpable hepatosplenomegaly, large symptoms improvement and 2. Durable** peripheral blood count remission, defined as Platelet count $\leq 400 < 10^9/L$; WBC $< 10 \times 10^9/L$; absence of leukoerythroblastosis and 3. Without signs of progressive disease, and absence of any hemorrhagic or thrombotic event and 4. Bone marrow histological remission defined as disappearance of megakaryocyte hyperplasia and absence of $>$ grade 1 reticulin fibrosis
Partial response	1. Durable* resolution of disease-related signs including palpable hepatosplenomegaly, large symptoms improvement and 2. Durable* peripheral blood count remission, defined as platelet count $\leq 400 \times 10^9/L$, WBC count $< 10 \times 10^9/L$, absence of leukoerythroblastosis and 3. Without signs progressive disease, and absence of any hemorrhagic or thrombotic event, and 4. Without bone marrow histological remission defined as persistence of megakaryocyte hyperplasia
No response	Any response that does not satisfy partial response
Progressive disease	Transformation into PV, post-ET myelofibrosis, myelodysplastic syndrome or acute leukemia

* Lasting at least 12 weeks

** "Disease-related symptoms" includono disturbi del microcircolo, prurito e cefalea

Figura 2. Criteri ELN di resistenza/intolleranza alla terapia con HU nella TE (Barosi G et al. *Br J Haematol.* 2010; 148:961-963)

Resistance	Need for phlebotomy (Hct $< 45\%$) Platelets $> 400 \times 10^9/L$ and WBC $> 10 \times 10^9/L$ No reduction of spleen by 50% No reduction of spleen symptoms	➔	After > 3 months at MTD or 2 g/day
Intolerance	Cytopenias (any) - ANC $< 1.0 \times 10^9/L$ - Hb < 10 g/DL - Plt $< 100 \times 10^9/L$ Leg ulcers GI toxicity Fevers Mucocutaneous toxicity Skin cancers	➔	At lowest dose to achieve either a PR or CR by ELN criteria