Clinical outcomes of diagnosis and on-time treatment of acquired thrombotic thrombocytopenic purpura

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Introduction

- Acquired Thrombotic Thrombocytopenic Purpura (aTTP) is an autoimmune disease
- Its incidence ranges from 1 to 4 cases per million worldwide
- aTTP is an important cause of microangiopathic hemolytic anemia and thrombocytopenia which can lead to multiple organ damage.
- It is important to provide timely diagnose and management.
- Effective Therapies can significantly alter prognosis.

Objective

- To compare diagnosis and treatment initiation in the first 24 hours (early treatment) versus absence of diagnosis or treatment (no treatment) or late treatment initiation up to 5 days (late treatment) in terms of clinical outcomes

Methods

- A partitioned survival model was developed to evaluate 2 scenarios: 1) early treatment versus no treatment; 2) early treatment versus late treatment.
- The analysis modelled a hypothetical cohort of 100 patients with an aTTP episode.
- The outcomes in this study correspond to mortality rates within 30 days, platelet normalization within 10 days, number of patients alive without further exacerbations and without relapses.
- Clinical data for health outcomes was obtained from published literature (**Table 1**)

Results

- Early treatment vs no treatment (**Table 2**):
  - Early treatment reduces mortality and improves platelet normalization
- Early treatment vs late treatment (**Figure 1**):
  - Earlier treatment produces better results vs late treatment
  - Every additional day of treatment delay produces
    - Additional deaths (ranging from +17.9 to +32.6)
    - Less patients with platelet normalization at 10 days (ranging –42.6 to –18.8)
    - Less patients free of exacerbation (from -22.5 to 13.9) or relapses (from –16.1 to –9.8)

Table 1. Main Model Parameters

Population Characteristics

Variable	Value	Source
Woman (%)	84,1%	Milan Registry [2]
Hematocrit (%)	11,6%	Oklahoma Registry [2]

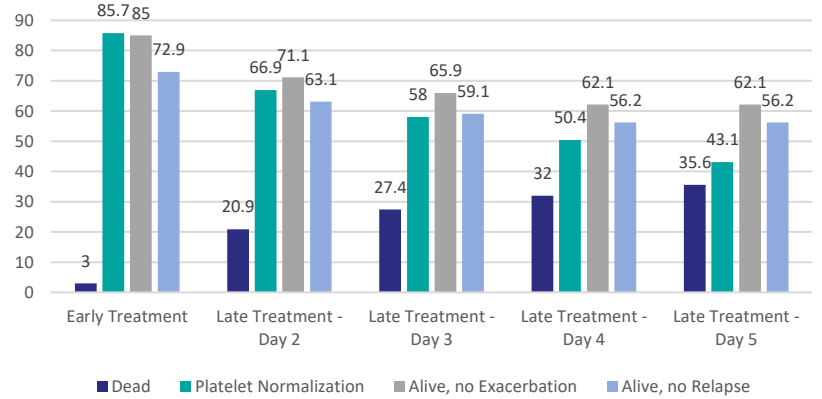
Effectiveness Parameters

Variable	Value	Source
Survival curve for patients with PEX	Weibull – α: 245.96 β: 0.70	Peigne et al [3]
Survival curve for patients without PEX	Weibull – α: 16.56 β: 0.50	Peigne et al [3]
PEX platelet normalization curve	Weibull – α: 5.67 β: 1.25	Scully et al [4]
Platelet normalization curve non – PEX	Weibull – α: 4.16β: 1.26	Scully et al [4]
Exacerbations – PEX	12,8%	Scully et al [4]
Relapses – PEX	25,6%	Scully et al [4]

Table 2. Model estimation, Early treatment vs no treatment

Consequences	Early Treatment	No treatment	Difference
Deaths (30 days)	3	68	-65
Platelet normalization - 10 days	86	0	86

Figure 1. Model estimation, Early treatment vs no treatment



Conclusion

- The early identification and treatment of patients with aTTP are associated with benefits in survival, exacerbations, and relapse free survival compared to no treatment or late treatment.
- Each day of treatment delay progressively worsens prognosis

References

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