

# EVALUATION OF COST AND EFFICACY OF ECULIZUMAB IN COMPLEMENT MEDIATED THROMBOTIC MICROANGIOPATHY IN THE CLINICAL SETTING

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## BACKGROUND AND IMPORTANCE

**Complement-mediated thrombotic microangiopathy (C-TMA)** is caused by complement disruption that leads to hemolysis and thrombocytopenia.

**Eculizumab** inhibits the C5b-9-complex formation by binding protein C5. It has been approved for C-TMA; nevertheless, studies on the effectiveness of Eculizumab in real-world conditions are scarce, even considering its high cost.

### AIM AND OBJECTIVES

To evaluate the real-world efficacy and cost of Eculizumab for C-TMA after 26 weeks of treatment.

## MATERIALS AND METHODS

- **Patients** diagnosed with C-TMA whose treatment with Eculizumab had been approved and lasted >26 weeks (W) were included.
- **Treatment:** Eculizumab 900mg weekly for 4 weeks, followed by Eculizumab 1,200 mg weekly.
- **Clinical variables** were obtained from electronic-health-records. Lab test were evaluated at and weeks 0, 12, 26 and 38 after initiation.
- **C-TMA remission** definition: lactate dehydrogenase (LDH) <upper limit of normal, platelets >150·10E<sup>9</sup>/L and <25% creatinine increase from baseline.

## RESULTS

- Six patients were included
  - 1 woman and 5 men
  - Median age 43 years (range 23-59)
  - Any patient had genetics related with complement alteration
  - One patient had a pulmonary transplant and one a renal transplant
- Two patients were in remission after 26W.
  - Hemoglobin and platelet count increased, LDH decreased.
  - LDH decreased in all patients, 3 patients had LDL > upper limit of normal after 38W
  - CH50 decreased in all patients and was undetectable for most patients within 12W (p=0.001).
  - Renal function was maintained or improved in 4/6 patients.
  - Two patients were in dialysis, one stopped.

	Weeks of treatment			
	0	12	26	38
<b>Hemoglobine (g/dL)</b>	11 (8.2-12.7)	12 (10.7-12.4)	12 (10.2-13,20)	13 (9.8-13.1)
<b>Platelets (10<sup>3</sup>/μL)</b>	206 (44-359)	189 (96-297)	233 (175-298)	291(175-305)
<b>LDH (UI/L)</b>	511(323-1787)	487 (288-1351)	491(313-642)	430 (266-642)
<b>Creatinine (mg/dl)</b>	4 (0,91-5,57)	2 (1.04-5.76)	2 (1.21-8.28)	1 (0.89-5,23)
<b>Reticulocytes (10<sup>9</sup>units/L)</b>	112 (90.8-190.3)	69 (20.3-101.2)	65 (44.1-85.4)	37 (25,7-115,1)
<b>C3 (mg/dL)</b>	106 (98.2-143)	1433 (89.6-101.2)	137 (96.7-176)	146 (104-171)
<b>C4 (mg/dL)</b>	31(28-35.1)	35 (24.8-41.3)	31(28.9-40.8)	42 (28.9-45.7)
<b>CH50 (UI/mL)</b>	59 (13.59-74.26)	<13	<13	<13
<b>sC5b9 (ng/mL)</b>	440 (249-1640)	1052 (162-1704)	493 (147-5398)	1251(169-5398)

- Eculizumab treatment
  - Duration 10(6.8-45.5) months
  - Two patients stopped because of resolution C-TMA
  - Estimated cost of 26 weeks treatment is 337,300 €
  - Median cost estimated/treatment was 160,458 € (118,055-640,870)

## CONCLUSIONS AND RELEVANCE

- ✓ Eculizumab was effective in C-TMA according to cellular and biochemical markers (platelets, LDH, creatinine); change in some parameters might not be detected because the small sample.
- ✓ Two patients out of 6 were in remission after 26W; the estimated cost for an additional C-TMA-remission was 1,011,900€.

