

# EFFICACY OF ECULIZUMAB IN ADULT PATIENTS RESISTANT TO PLASMA THERAPY WITH ATYPICAL HEMOLYTIC UREMIC SYNDROME

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## BACKGROUND

Eculizumab is an orphan drug for atypical Hemolytic Uremic Syndrome (aHUS). The disease is characterized by non-immune hemolytic anemia, thrombocytopenia, and renal impairment.

## PURPOSE

To assess the efficacy of eculizumab in adult patients, resistant to plasma therapy (PT), diagnosed with aHUS.

## MATERIAL AND METHODS

A **systematic review** of literature has been conducted focused on efficacy.

### Databases:

until September 2012  
MEDLINE  
EMBASE  
Cochrane Library  
Centre for Reviews and Dissemination

### Selection criteria:

Health technology assessment reports, meta-analyses, systematic reviews, the assessment report of the drug from the European Medicines Agency, randomized controlled trials (RCTs), controlled observational studies, and uncontrolled intervention studies evaluating efficacy of eculizumab in adult PT-resistant patients with aHUS were included.

Study selection, quality assessment, data extraction, and qualitative synthesis of the evaluated literature were independently undertaken by two researchers. Disagreements were resolved by consensus.

## RESULTS

Only one prospective uncontrolled intervention study was included.

The median follow-up of the study was 64 weeks (range: 2-90 weeks).

- ✓ **No deaths** were reported during the follow-up period.
- ✓ **87% of patients achieved a minimally important difference** of 0.06 in the **EuroQoL 5D** measurements.
- ✓ The **thrombotic microangiopathy (TMA) event-free status** (no PT session, new dialysis, and decrease in platelet count of > 25% from baseline for at least 12 weeks) was **achieved in 87%** of the patients.
- ✓ The **TMA intervention rate** (PT session and dialysis/patient/day) was **reduced from a median of 0.88 to 0 events/person/day**.
- ✓ **53% of patients achieved a sustained change in estimated glomerular filtration rate (eGFR)  $\geq 15$  mL/min/1.73 m<sup>2</sup>**.
- ✓ **76% of patients achieved a sustained 25% reduction from baseline in serum creatinine**.
- ✓ **65% of patients improved at least one chronic kidney disease (CKD) stage**.

## CONCLUSION

Eculizumab in the PT-resistant population improves quality of life and renal function, reduces the percentage of patients in dialysis and the necessity of PT. This systematic review could be use as a basis for developing recommendations for the use of eculizumab in this population.