Purpose: To determine whether Elizaria® is as effective and as well tolerated as Soliris® in patients with paroxysmal nocturnal hemoglobinuria.

Methods: 30 patients with PNH were enrolled into the phase IIIb clinical trial. All patients started receiving Elizaria® (n = 16) or Soliris® (n = 14). Baseline characteristics, including the number of weekly infusions per patient, were assessed. The study medication was administered in the morning of the first day of the treatment week, and patients received a weekly dose of 1,200 mg or 600 mg of eculizumab, respectively, for 26 weeks. The primary endpoint was the area under the curve (AUC) of LDH concentration, the secondary endpoints included the percentages of patients with a decrease of at least 30% in LDH concentration (D30%), the percentage of patients with one or more adverse drug reactions (ADRs), the median residence time (MRT) of eculizumab, and the half-life of eculizumab.

Results: The treatment groups were also comparable on all the secondary efficacy parameters (Table 1). Thirty patients have completed the study without significant protocol deviations (16 patients in Elizaria® group and 14 patients in Soliris® group).

The LDH AUC was highly variable between the patients but the means were not statistically different between the treatment groups at the end of the study (95% CI of 62,957.6 ± 46,066.5 U/L*day and 49,702.6 ± 26,182.1 U/L*day for Elizaria® and Soliris® groups, respectively).

The comparative PK parameters of eculizumab and MAC concentration have been demonstrated in the treatment groups in all studied time points. Thus, the median elimination half-life (1/2) of eculizumab amounted to (214.08 ± 93.57) ng/mL in Elizaria® and Soliris® groups, respectively (Figure 2).

The median residence time (MRT) of eculizumab was (25.96) µg/mL in Elizaria® group and to (94.13 ± 58.76) µg/mL in Soliris® group (p = 0.065). The median residence time of MAC amounted to (187.7 h (IQR 165.7) in Elizaria® group and to (257.3) h (IQR 240.9) in Soliris® group (p = 0.206). By the end of the study, the median NME values one time after the study product AUC were amounted to (77.0 ± 72.3) ng/mL and (94.13 ± 43.4) µg/mL in Elizaria® and Soliris® groups, respectively (Figure 3).

Both Elizaria® and Soliris® demonstrated the similar safety profile. Nineteen adverse drug reactions (16.7%) in patients were reported in the study (3 in Elizaria® patients, whereas 4 were reported in 2 Soliris® patients. ADRs were observed in the elizaria® group (219.08 ± 93.57) ng/mL and soliris® group (257.3) h (IQR 240.9) of eculizumab concentration (1/2) during the maintenance therapy period was the primary endpoint of the study. The secondary efficacy parameters were hemolysis, hemolytic anemia, and thrombotic microangiopathy (TMA). The PK profile, as well as the median NME and ADRs attack complex (ADRA) concentration were also assessed during the treatment.

Nocturnal Hemoglobinuria: Results of Comparative Analysis of Efficacy, Safety, and Pharmacological Data

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Disclosure: Eugene Zuev has nothing to disclose. Elena Gapchenko, MD holds institutional research grants from Sanofi Genzyme, Biogen, JSC GENERIUM, and Roche. Oksana Markova, MD has also nothing to disclose.