This English version is intended to be a reference material for the convenience of users. In the event of inconsistency between the Japanese original and this English translation, the former shall prevail.

Summary of Investigation Results

Eculizumab (genetical recombination)

December 8, 2020

Non-proprietary name

Eculizumab (genetical recombination)

Branded name (Marketing authorization holder)

Soliris for Intravenous Infusion 300 mg (Alexion Pharmaceuticals, Inc.)

Indications

· Reduction of haemolysis in paroxysmal nocturnal haemoglobinuria

· Inhibition of thrombotic microangiopathy in atypical haemolytic uremic syndrome

· Generalized myasthenia gravis (only for patients whose symptoms are difficult to control with high-dose intravenous immunoglobulin therapy or plasmapheresis)

· Prevention of relapse of neuromyelitis optica spectrum disorder (including neuromyelitis optica)

Summary of revisions

“Serious infection” should be added to the CLINICALLY SIGNIFICANT ADVERSE REACTIONS section.

Investigation results and background of the revision

As a result of the review of the recent partial change approval application for Ultomiris for Intravenous Infusion 300 mg (ravulizumab (genetical recombination)) to add atypical haemolytic uremic syndrome to the indications, it was decided to add a cautionary statement for infection to the Clinically Significant Adverse Reactions section in the package insert. In consideration of the fact that this drug targets the same epitope in complement protein C5 as Ultomiris, MHLW/PMDA in consultation with expert advisors

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considered the necessity of a cautionary statement and concluded that revision of the package insert for this drug was necessary.

Number of cases and patient mortalities reported in Japan during the previous 3 fiscal years

A total of 122 cases* involving infection have been reported to date.
A total of 11 patient mortalities have been reported to date.

*Reference information: In these cases, effects of the seriousness and susceptibility to infection of the primary disease and infection risk due to a concomitant drug(s) in the course of treatment could not be ruled out.

The expert advisors present at the Expert Discussion regarding the current investigation were nominated based on their conflict of interest declarations concerning the relevant products, pursuant to the “Rules for Convening Expert Discussions, etc., by the Pharmaceuticals and Medical Devices Agency” (PMDA Administrative Rule No. 20-8, dated December 25, 2008).