



Soliris Fact Sheet

Alexion's Soliris® (eculizumab) is approved for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH)¹, a progressive and life-threatening ultra-rare disease characterized by the excessive destruction of red blood cells (hemolysis).^{2,3} Soliris is the only treatment for PNH that reduces hemolysis.¹

Soliris is the first in a new class of therapies to inhibit terminal complement, a group of proteins involved in the body's immune response. Extensive clinical trials show that Soliris reduces hemolysis in patients with PNH, leading to an improvement in symptoms and a reduction in major health problems associated with this disease.^{7,8} Prior to approval of Soliris, there were no therapies specifically tested and approved for the treatment of PNH.

Soliris has been approved by the U.S. Food and Drug Administration (March 2007), the European Commission (June 2007), Health Canada (January 2009) and Australia's Therapeutic Goods Administration (February 2009). All four jurisdictions reviewed and approved their respective marketing applications for Soliris under their priority review or accelerated assessment procedures, and all four have designated Soliris as an orphan drug. Soliris is commercially available in more than 30 countries. The company has submitted a marketing application for Soliris in Japan and is working toward seeking regulatory approval in other countries in Asia and South America.

How Does Soliris Work?

Patients with PNH are missing a specific protein on the surface of their red blood cells that normally protects them from destruction by a component of the immune system called terminal complement. Soliris prevents hemolysis by selectively blocking terminal complement, thereby reducing symptoms.

Clinical Trial Results

Alexion's clinical studies of Soliris as a treatment for PNH involved nearly 200 patients, a substantial population given the rareness of the disease.^{4,5,9} Three multi-national clinical studies – TRIUMPH, SHEPHERD and E05-001 – demonstrated the safety and efficacy of Soliris, and were widely reported and discussed at major medical meetings and published in the *New England Journal of Medicine* and the journal *Blood*, both prestigious, peer-reviewed medical journals.⁴⁻⁶ The studies showed that patients who received Soliris experienced a number of benefits, including:

- Immediate and sustained reduction in chronic hemolysis in all patients^{4,5}
- Fewer thrombotic events (blood clots) as compared to the same time period prior to starting Soliris⁸
- Significant improvements in fatigue levels and overall quality of life^{4,5}
- Significant reduction in the need for transfusions⁵

Important Safety Information

Soliris is generally well tolerated. The most frequent adverse events observed in clinical studies were headache, nasopharyngitis (a runny nose), back pain and nausea. Treatment with Soliris should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established. The U.S. product label for Soliris also includes a boxed warning: "Soliris increases the risk of meningococcal infections. Vaccinate patients with a meningococcal vaccine at least two weeks prior to receiving the first dose of Soliris; revaccinate according to current medical guidelines for vaccine use. Monitor patients for early signs of meningococcal infections, evaluate immediately if infection is suspected, and treat with antibiotics if necessary." During clinical studies, two out of 196 vaccinated PNH patients treated with Soliris experienced a serious meningococcal infection. Prior to beginning Soliris therapy, all patients and their prescribing physicians are encouraged to enroll in the PNH Registry, which is part of a special risk-management program that involves initial and continuing education and long-term monitoring for detection of new safety findings.

Please see full prescribing information at www.soliris.net.

References

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