Eculizumab (Soliris®)

Summary of recommendations by Zorginstituut Nederland, 13 May 2016

Zorginstituut Nederland has re-assessed the medicine eculizumab (Soliris®) for the treatment of patients with paroxysmal nocturnal haemoglobinuria (PNH). Zorginstituut Nederland reached the following conclusion.

Based on necessity, effectiveness, cost-effectiveness and feasibility (the package criteria), eculizumab should no longer be reimbursed from the basic health insurance, unless there is clear insight into its cost-effectiveness (and in a case of unfavourable cost-effectiveness) transparency regarding how the price was determined.

**Background**

Zorginstituut Nederland has re-assessed eculizumab (Soliris®) for the treatment of patients with paroxysmal nocturnal haemoglobinuria (PNH). With the assistance of the Insured Package Advisory Committee (ACP) and the Scientific Advisory Board (WAR), the Zorginstituut has now completed this assessment.

The burden of disease involved in PNH is high (0.77 on a scale from 0 to 1). Eculizumab is the only treatment for patients with PNH. It is effective in treating transfusion-dependent PNH-patients and fulfils the 'established medical science and medical practice' criterion. It improves quality of life and there are indications that it can extend the life of a PNH patient considerably. Treatment with eculizumab costs 360,000 euro per patient per year. The life-long treatment of one PNH-patient costs about 15 million euro.

Zorginstituut Nederland finds the methodological quality of the pharmacoeconomic analysis submitted by the manufacturer is too low. Based on this analysis, the manufacturer arrived at a sum of approximately 400,000 euro per QALY (Quality-adjusted life-year). Due to insufficient substantiation by the manufacturer, the Zorginstituut regards this as a minimum. This minimum is already five times higher than the cost-effectiveness reference value that applies for this burden of disease, namely 80,000 euro per QALY. The Zorginstituut estimates the total costs to the pharmacy budget in 2017 for the indication PNH at 25 million euro.

Taking everything into consideration, we advise the Minister of Health, Welfare and Sport to cease the reimbursement of eculizumab for PNH patients at the expense of the basic health insurance, unless clear insight exists into its cost-effectiveness (and in the case of unfavourable cost-effectiveness) and transparency regarding how the price was determined. What weighed heavily for the Zorginstituut is that the manufacturer has not yet provided a realistic estimate of its cost-effectiveness and is not transparent about how the price of the drug was determined. A careful determination of the cost-effectiveness of eculizumab is necessary in order to continue reimbursing eculizumab at its current price from what is in principle a limited budget, as this

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inevitably puts pressure on other effective care and thus leads to health losses among patients who are effectively nameless. It is primarily the responsibility of manufacturers to be open about these matters so that Zorginstituut Nederland can formulate well-informed advice justifying - by way of an exception - a possibly higher reference value than the currently applicable 80,000 euro per QALY. This would also allow us to indicate what price reduction is necessary in order to fall within the limits of acceptability.

Zorginstituut Nederland also advises the Minister of Public Health, Welfare and Sport to enter into a dialogue with the manufacturer in order to negotiate an adequate cost-effectiveness analysis and insight into the price.

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The original text of this advice of Zorginstituut Nederland was in Dutch. Although great care was taken in translating the text from Dutch to English, the translation may nevertheless have resulted in discrepancies. Rights may only be derived on the basis of the Dutch version of the advice of Zorginstituut Nederland. Furthermore, Zorginstituut Nederland points out that only the summary of this report was translated. A proper understanding of all relevant considerations and facts would require familiarity with the Dutch version of this report, including all appendices.