

Short title (max. 90 characters)

One year of ODAP sutimlimab in cold agglutinin disease

Title

One year of Orphan Drug Access Protocol for the use of sutimlimab in cold agglutinin disease

Authors*

K. Verdeyen, S. van der Flier, S.J. Bernelot Moens, N. Reijnhout, T. Engel, C.E.M Hollak, J.M.I. Vos

Introduction

Cold agglutinin disease (CAD) is a rare chronic complement-mediated auto-immune hemolytic anemia with an estimated prevalence of approximately 200 persons in the Netherlands. Sutimlimab (Enjaymo®; Sanofi), a complement-C1s antibody, was shown effective to treat hemolysis in CAD in three clinical trials and received EU marketing authorization in November 2022. Sutimlimab is given long-term every 2 weeks intravenously. However, it remains unclear which patients will benefit most from this expensive treatment compared to currently available options such as rituximab or immunochemotherapy. Supported by Medicijn voor de Maatschappij, in a collaboration between academia, health insurance companies and pharma, a new method was developed: the Orphan Drug Access Protocol (ODAP, <https://medicijnvoordemaatschappij.nl/odap-orphan-drug-access-protocol>). The ODAP pilot aims for faster but controlled access to orphan drugs with limited evidence. The ODAP for sutimlimab started in august 2023.

Methods

A monthly indication committee discusses candidates using predefined indication criteria for starting treatment in phase 1. After 3 months, each case is discussed again, using predefined effectiveness criteria to decide if this patient can proceed to phase 2 (long term treatment). Essential clinical data including patient-reported-outcomes are collected in a central database and samples are biobanked for biomarker development. The ODAP will run for a minimum of 3 and a maximum of 5 years. The results, including a Health Technology Assessment (HTA), will be used in a joint assessment by health insurance companies (CieBAG) to decide on sutimlimab's position in the treatment of CAD patients in the Netherlands, and according suitability and conditions for reimbursement on a group level.

Results

Within the ODAP, sutimlimab became available < 10 months after EMA approval. So far, 8 patients were discussed in the indication committee of whom 6 patients were included in the ODAP. Three of these 6 received sutimlimab as post-trial-access. Five patients are currently still being treated with sutimlimab (median duration of sutimlimab in ODAP + range). One patient stopped after three months (phase 1) due to lack of effectiveness. Additional patients were pre-discussed informally but were not discussed formally in the committee meeting because they did not meet the inclusion criteria.

Conclusion

The ODAP approach led to fast yet protocolized post-approval access to Sutimlimab for CAD patients in the Netherlands. It is too early to draw conclusions about the results of the ODAP sutimlimab. We can conclude that the ODAP approach might be a suitable access route for novel drugs for orphan diseases, in this case CAD, enabling appropriate use. This approach required a collaboration between involved medical professionals, health insurance companies and pharma.

*** Affiliaties en potentiële belangenverstrengeling:**

Mw. drs. K. Verdeyen, arts-onderzoeker, afdeling Hematologie, Amsterdam UMC, locatie VUmc, Boelelaan 1117, 1081 HV Amsterdam. Belangenconflicten: Financiering ODAP-pilot door Zorgverzekeraars Nederland via Medicijn voor de Maatschappij, travel grant van Sanofi.

Mw. dr. S.J. Bernelot Moens, internist-hematoloog, afdeling Hematologie, Amsterdam UMC, locatie VUmc, Boelelaan 1117, 1081 HV Amsterdam. Belangenconflicten: geen

Dhr. dr. N. Reijnhout, Business Developer Medicijn voor de Maatschappij, afdeling Inwendige Geneeskunde, Amsterdam UMC, locatie AMC, Meibergdreef 9, 1105AZ Amsterdam Belangenconflicten: Medicijn voor de Maatschappij wordt gefinancierd door de Nationale PostcodeLoterij. De financiering voor ODAP komt van Zorgverzekeraars Nederland. Lid van de Ronde Tafel Weesgeneesmiddelen van Zorginstituut Nederland.

Dhr. Dr. T. Engel, Achmea-Zilveren Kruis, Belangenconflicten: geen

Mw. dr. S van der Flier, medisch Adviseur Intramurale Farmacie Coöperatie VGZ, Belangenconflicten: geen

Mw. prof. dr. Carla E.M. Hollak, hoogleraar Metabole ziekten, afdeling Inwendige Geneeskunde, Amsterdam UMC, locatie AMC, Meibergdreef 9, 1105AZ Amsterdam Belangenconflicten: betrokken bij klinische trials van Sanofi, Protalix en Idorsia. Financiële afspraken lopen via de AMC Medical research BV. Hoofd Medicijn voor de Maatschappij, gefinancierd door de Postcodeloterij; Lid van de Adviescommissie pakket, ronde tafel weesgeneesmiddelen en Horizonscangroep van het Zorginstituut.

Mw. dr. J.M.I. Vos, internist-hematoloog, afdeling Hematologie, Amsterdam UMC, locatie VUmc, Boelelaan 1117, 1081 HV Amsterdam. Belangenconflicten: consultancy en advisory boards voor Janssen en Sanofi, research support van Beigene en Abbvie/Genmab, en sprekersvergoeding van BMS, Sanofi en Amgen. Al deze honoraria zijn aan het instituut betaald en niet persoonlijk. Dr. Vos was lokale PI in het Amsterdam UMC van 2 klinische trials met sutimlimab.