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(54) Title **MODULATORS OF COMPLEMENT ACTIVITY**

(56) References

Cited:

WO-A-2005/023866

US-A1- 2015 011 474

WO-A1-2013/126006

WO-A2-2015/191951

US-A1- 2013 029 912

RICARDO ET AL.: 'Development of RA101348, a Potent Cyclic Peptide Inhibitor of C5 for Complement-Mediated Diseases.' 56TH ASH ANNUAL MEETING AND EXPOSITION. 17 December 2014, XP009500694

HOWARD J.F. ET AL.: "Clinical Effects of the Self-administered Subcutaneous Complement Inhibitor Zilucoplan in Patients With Moderate to Severe Generalized Myasthenia GravisResults of a Phase 2 Randomized, Double-Blind, Placebo-Controlled, Multicenter Clinical Trial", JAMA NEUROL., vol. 77, no. 5, 17 February 2020 (2020-02-17), pages 582-592,

C.M. Legendre ET AL: "Terminal Complement Inhibitor Eculizumab in Atypical Hemolytic-Uremic Syndrome", The New England Journal of Medicine, - NEJM -, vol. 368, no. 23, 6 June 2013 (2013-06-06) , pages 2169-2181, XP055688015, US ISSN: 0028-4793, DOI: 10.1056/NEJMoa1208981

BERGLUND M.M. & STRÖMBERG P.: "The clinical potential of Affibody-based inhibitors of C5 for therapeutic complement disruption.", EXPERT REV. PROTEOMICS, vol. 13, no. 3, 3 February 2016 (2016-02-03), pages 241-243,

HILLMEN PETER ET AL: "The complement inhibitor eculizumab in paroxysmal nocturnal hemoglobinuria", NEW ENGLAND JOURNAL OF MEDICINE, THE - NEJM, MASSACHUSETTS MEDICAL SOCIETY, US, vol. 355, no. 12, 21 September 2006 (2006-09-21), pages 1233-1243, XP002482088, ISSN: 1533-4406, DOI: 10.1056/NEJMOA061648

DATABASE GENBANK [Online] 26 September 2013 'hypothetical protein B655_1297

[Methanobacterium sp. Maddingley MBC34', XP055413419 Retrieved from NCBI Database accession no. EKQ53330

Enclosed is a translation of the patent claims in Norwegian. Please note that as per the Norwegian Patents Acts, section 66i the patent will receive protection in Norway only as far as there is agreement between the translation and the language of the application/patent granted at the EPO. In matters concerning the validity of the patent, language of the application/patent granted at the EPO will be used as the basis for the decision. The patent documents published by the EPO are available through Espacenet (<http://worldwide.espacenet.com>) or via the search engine on our website here: <https://search.patentstyret.no/>

Patentkrav

- 1.** Et polypeptid bestående av SEKV ID NR: 194 eller SEKV ID NR: 184 for bruk i en fremgangsmåte for behandling av en C5-komplement-relatert lidelse, fremgangsmåten omfatter administrering av polypeptidet i en dose på fra omtrent 0,1 mg/kg til omtrent 10 mg/kg hvori hemolyse hos individet er redusert med minst 50% i forhold til hemolysenivåer tidligere observert hos nevnte individ.
- 2.** Polypeptidet for bruk ifølge krav 1, hvori polypeptidet administreres til individet i en dose på fra ca. 0,3 mg/kg til ca. 3 mg/kg.
- 3.** Polypeptidet for bruk ifølge krav 2, hvori polypeptidet administreres til individet i en dose på ca. 0,3 mg/kg.
- 4.** Polypeptid for bruk ifølge hvilket som helst av kravene 1-3, hvor administreringen utføres daglig.
- 5.** Polypeptid for bruk ifølge krav 4, hvor administreringen utføres i syv dager.
- 6.** Polypeptidet for bruk ifølge hvilket som helst av kravene 1-5, hvori den C5-komplement-relaterte lidelsen er paroksysmal nattlig hemoglobinuri.
- 7.** Polypeptid for bruk ifølge krav 6, hvori nevnte individ tidligere har blitt behandlet med ECULIZUMAB.
- 8.** Polypeptidet for bruk ifølge krav 6, hvori nevnte individ også mottar behandling med ECULIZUMAB.
- 9.** Polypeptidet for bruk ifølge krav 7 eller 8, hvori behandling med ECULIZUMAB er ineffektiv.
- 10.** Polypeptid for bruk ifølge hvilket som helst av kravene 1-9, hvori hemolyse i individet er redusert med minst 90% i forhold til hemolysenivåer tidligere observert i nevnte individ.