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(56) References Cited:

US-A1- 2010 286 047
WO-A1-2012/006591
US-A1- 2010 099 603
WO-A1-2005/012354
US-A- 5 854 403

WO-A1-2009/156137
WO-A2-2009/086400
TURECEK PETER L ET AL: "Structure and function of a recombinant von Willebrand factor drug candidate", SEMINARS IN THROMBOSIS AND HEMOSTASIS,, vol. 36, no. 5, 1 July 2010 (2010-07-01), pages 510-521, XP009162279, ISSN: 1098-9064
VARADI KATALIN ET AL: "In Vivo Cleavage of Recombinant VWF Upon Intravenous Administration in Preclinical and Clinical Settings", BLOOD, AMERICAN SOCIETY OF HEMATOLOGY, US, vol. 118, no. 21, 1 November 2011 (2011-11-01), page 549, XP009162281, ISSN: 0006-4971 [retrieved on 2011-11-18]
SCHLOKAT U ET AL: "PRODUCTION OF HIGHLY HOMOGENEOUS AND STRUCTURALLY INTACT RECOMBINANT VON WILLEBRAND FACTOR MULTIMERS BY FURIN-MEDIATED PROPEPTIDE REMOVAL IN VITRO", BIOTECHNOLOGY AND APPLIED BIOCHEMISTRY, ACADEMIC PRESS, US, vol. 24, no. PART 03, 1 December 1996 (1996-12-01), pages 257-267, XP001097385, ISSN: 0885-4513
FISCHER B E ET AL: "Structural analysis of recombinant von Willebrand factor produced at industrial scale fermentation of transformed CHO cells co-expressing recombinant furin", FEBS LETTERS, vol. 375, 20 November 1995 (1995-11-20), pages 259-262, XP002078561, ISSN: 0014-5793
FISCHER B E: "Recombinint von Willenbrand factor: potential therapeutic use", JOURNAL OF THROMBOSIS AND THROMBOLYSIS, KLUWER ACADEMIC PUBLISHERS, DORDRECHT, NL, vol. 8, 1 January 1999 (1999-01-01), pages 197-205, XP002491372, ISSN: 0929-5305, DOI: 10.1023/A:1008906103637
TURECEK PETER L ET AL: "Biochemical and functional characterization of a serum-free rVWF drug candidate", BLOOD, AMERICAN SOCIETY OF HEMATOLOGY, US, vol. 108, no. 11, Part 1, 1 November 2006 (2006-11-01), page 303A, XP002524489, ISSN: 0006-4971

PATENTKRAV

1. Rekombinant Von Willebrand Factor (rVWF) for anvendelse i en fremgangsmåte for behandling av Von Willebrand Disease Type 3, hvor rVWF er en VWF multimer-sammensetning med høy molekylvekt omfattende minst 40% VWF-dekamerer eller multimerer av høyere orden.

2. rVWF for anvendelse ifølge krav 1, hvor rVWF modnes *in vitro* ved behandling med Furin.

3. rVWF for anvendelse ifølge krav 1 eller krav 2, hvor rVWF ikke er utsatt for ADAMTS13 under produksjon i cellekultur.

4. rVWF for anvendelse ifølge et hvilket som helst av kravene 1-3, hvor rVWF har en spesifikk aktivitet på 20 til 150 mU/mg pluss eller minus 10%, eventuelt på 30 til 120 mU/mg pluss eller minus 10%.

5. rVWF for anvendelse ifølge et hvilket som helst av kravene 1 - 4, hvor den høymolekylvekt VWF multimersammensetningen omfatter minst 50% VWF dekamerer eller høyere orden multimerer, mest foretrukket hvor den høymolekylvekten VWF multimersammensetningen omfatter minst 60% VWF dekamerer eller høyere ordens multimerer.