(12) **Translation of European patent specification**

(11) **NO/EP 4032979 B1**

NORWAY

(19) NO

(51) Int Cl.

C12N 9/64 (2006.01)
A61K 38/48 (2006.01)
A61P 7/04 (2006.01)

Norwegian Industrial Property Office

(45)	Translation Published	2024.08.19
(80)	Date of The European Patent Office Publication of the Granted Patent	2024.05.01
(86)	European Application Nr.	21200005.3
(86)	European Filing Date	2009.09.15
(87)	The European Application's Publication Date	2022.07.27
(30)	Priority	2008.09.15, IT, BO20080564 2009.05.06, IT, BO20090275
(84)	Designated Contracting States:	AT ; BE ; BG ; CH ; CY ; CZ ; DE ; DK ; EE ; ES ; FI ; FR ; GB ; GR ; HR ; HU ; IE ; IS ; IT ; LI ; LT ; LU ; LV ; MC ; MK ; MT ; NL ; NO ; PL ; PT ; RO ; SE ; SI ; SK ; SM ; TR
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(54) Title **FACTOR IX POLYPEPTIDE MUTANT, ITS USES AND A METHOD FOR ITS PRODUCTION**

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Patentkrav

1. Viral vektor for anvendelse i en genterapibehandling av alvorlig og/eller moderat hemofili B hos en human pasient, hvori den virale vektoren omfatter en nukleinsyre som koder for et modifisert FIX-polypeptid med minst den følgende sekvensen:

YNSGKLEEFV QGNLERECME EKCSFEEARE VFENTERTTE FWKQYVDGDQ
CESNPCLNGG SCKDDINSYE CWCPFGFEGK NCELDVTCNI KNGRCEQFCK
NSADNKVVCS CTEGYRLAEN QKSCEPAVPF PCGRVSVSQT SKLTRAEXVF
PDVDYVNSTE AETILDNITQ STQSFNDFTR VVGGEDAKPG QFPWQVVLNG
KVDAFCGGSI VNEKWIVTAA HCVETGVKIT VVAGEHNIEE TEHTEQKRNV
IRIIPHHNYN AAINKYNHDI ALLELDEPLV LNSYVTPICI ADKEYTNIFL
KFGSGYVSGW GRVFHKGRSA LVLQYLRVPL VDRATCLLST KFTIYNNMFC
AGFHEGGRDS CQGDSGGPHV TEVEGTSFLT GIISWGEECA MKGKYGIYTK
VSRYVNWIKE KTKLT,

hvori X representerer A.

2. Den virale vektoren for anvendelse ifølge krav 1, hvori nukleinsyren koder for et modifisert FIX-polypeptid med følgende sekvens:

MQRVNMIMAE SPGLITICLL GYLLSAECTV FLDHENANKI LNRPKRYNSG
KLEEFVQGNL ERECMEEKCS FEEAREVFEN TERTTEFWKQ YVDGDQCESN
PCLNGGSCKD DINSYECWCP FGFEGKNCEL DVTCNIKNGR CEQFCKNSAD
NKVVCSCTEG YRLAENQKSC EPAVPFPCGR VSVSQTSKLT RAEXVFPDVD

YVNSTEAETI LDNITQSTQS FNDFTRVVGG EDAKPGQFPW QVVLNGKVDA
FCGGSIVNEK WIVTAAHCVE TGVKITVVAG EHNIEETEHT EQKRNVIRII
PHHNYNAAIN KYNHDIALLE LDEPLVLNSY VTPICIADKE YTNIFLKFGS
GYVSGWGRVF HKGRSALVLQ YLRVPLVDRA TCLLSTKFTI YNNMFCAGFH
EGGRDSCQGD SGGPHVTEVE GTSFLTGIIS WGEECAMKGK YGIYTKVSRY
VNWIKEKTKL T,

hvori X representerer A.

3. Den virale vektoren for anvendelse ifølge krav 1 eller 2, hvori den virale vektoren er adenoassosiert virus.

4. Den virale vektoren for anvendelse ifølge et hvilket som helst av kravene 1–3, hvori det modifiserte FIX-polypeptidet utviser 8–9 ganger økt funksjonell aktivitet sammenlignet med FIX-villtype.