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(62)	Divided application	EP2490533, 2010.10.05
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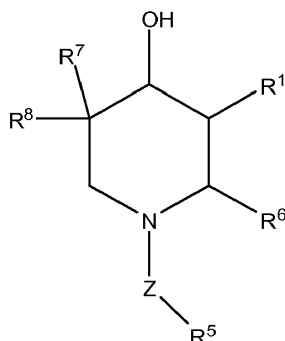
(54)	Title	NOVEL COMPOSITIONS FOR PREVENTING AND/OR TREATING LYSOSOMAL STORAGE DISORDERS
(56)	References Cited:	US-A1- 2008 009 516, US-A1- 2004 082 641, EP-A1- 1 860 101 HIDEKAZU OUCHI ET AL: "A New Route to Diverse 1-Azasugars from N -Boc-5-hydroxy-3-piperidine as a Common Building Block", THE JOURNAL OF ORGANIC CHEMISTRY, vol. 70, no. 13, June 2005 (2005-06), pages 5207-5214, XP055059837, ISSN: 0022-3263, DOI: 10.1021/jo050519j SUN, HONGBIN ET AL: "A new asymmetric route to substituted piperidines: synthesis of N-alkyl-3,4-dihydroxy-5-alkylpiperidines", TETRAHEDRON LETTERS , 41(16), 2801-2804 CODEN: TELEAY; ISSN: 0040-4039 DOI - DOI:10.1016/S0040-4039(00)00267-7, 2000, XP002695554, VANESSA FAUGEROUX ET AL: "C-Alkyl 5-membered ring imino sugars as new potent cytotoxic glucosylceramide synthase inhibitors", ORGANIC & BIOMOLECULAR CHEMISTRY, vol. 4, no. 24, 2 November 2006 (2006-11-02), page 4437, XP055059841, ISSN: 1477-0520, DOI: 10.1039/b613460b

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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. Forbindelse med formel I:



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hvor:

R^1 er $C(R^2)(R^3)(R^4)$;

R^2 er hydrogen, -OH eller halogen;

R^3 er hydrogen, -OH, halogen eller C_{1-8} alkyl;

10 R^4 er halogen, C_{1-8} alkyl, aryl eller alkylsykloalkyl;

R^3 og R^4 kan forenes sammen med karbonatomet som de er bundet til for å danne en sykloalkylring, som eventuelt kan substitueres med ett eller flere halogenatomer;

R^6 er hydrogen, C_{1-8} alkyl, arylalkyl eller alkylaryl;

Z er valgfri, når til stede er Z $-(CH_2)_{1-8}-$, $-C(=O)-$, $-S(=O)_2NH-$, $-S(=O)_2-$, $-C(=S)NH-$,

15 $-S(=O)_2-CH_2-$, $C(=O)-NH-$, $-S(=O)_2-NR^9-$, $-C(=O)C_{1-8}$ alkylen eller $-C(=O)CH(NH_2)CH_2-$;

R^9 er hydrogen eller C_{1-8} alkyl;

R^5 er hydrogen, C_{1-8} alkyl, aryl, C_{1-8} alkenyl, arylalkyl, alkylaryl eller aminoarylalkyl;

R^7 er -OH eller halogen; og

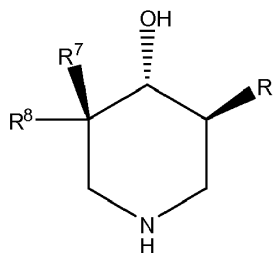
R^8 er hydrogen, halogen eller C_{1-8} alkyl,

20 forutsatt at R^2 og R^3 ikke begge kan være hydrogen når R^4 er et halogen, og når Z ikke er til stede, er R^7 -OH, og R^5 , R^6 og R^8 er hydrogen;

eller et farmasøytisk akseptabelt salt derav;

for anvendelse for å forebygge og/eller behandle en lysosomal lagringsforstyrrelse hos en pasient som er i risiko for å utvikle eller få samme diagnose.

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2. Forbindelse med formel III:

hvor:

R¹ er C(R²)(R³)(R⁴);

5 R² er hydrogen, -OH eller halogen;

R³ er hydrogen, -OH, halogen eller CH₃;

R⁴ er halogen, -CH₃, fenyl, fluorfenyl, metylfenyl, sykloheksylmetyl, hvori, når R⁴ er et halogen, kan ikke både R² og R³ være hydrogen;

10 R³ og R⁴ kan forenes sammen med karbonatomet som de er bundet til for å danne en sykloalkylring, som eventuelt kan substitueres med ett eller flere halogenatomer;

R⁷ er -OH eller halogen; og

R⁸ er hydrogen, halogen eller -CH₃,

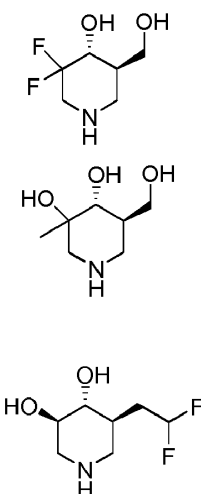
forutsatt at R² og R³ ikke begge kan være hydrogen når R⁴ er et halogen, R⁷ er -OH og R⁸ er hydrogen;

15 eller et farmasøytisk akseptabelt salt derav;

for anvendelse for å forebygge og/eller behandle en lysosomal lagringsforstyrrelse hos en pasient som er i risiko for å utvikle eller få samme diagnose.

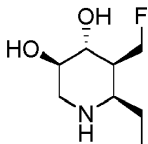
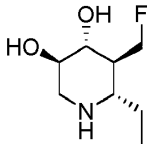
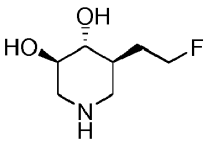
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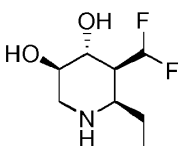
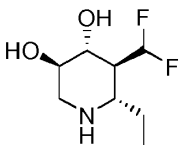


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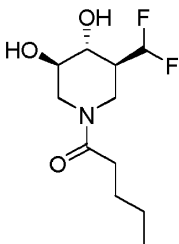
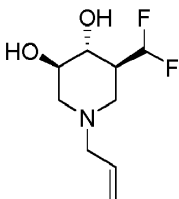
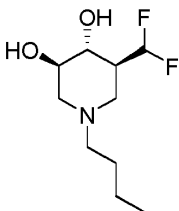
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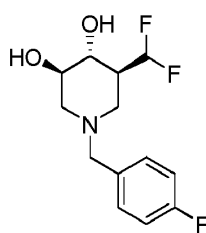
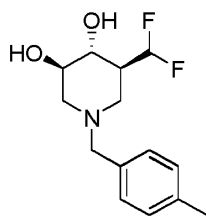
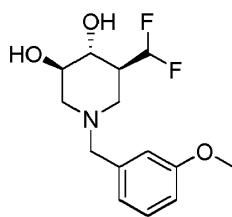


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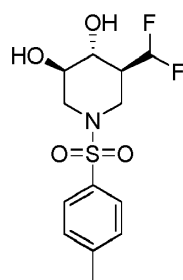
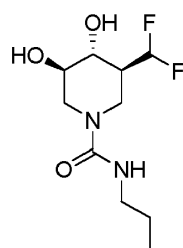


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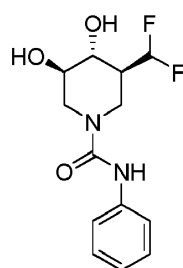
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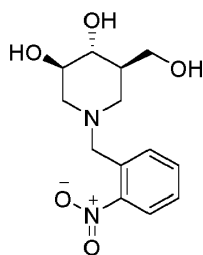
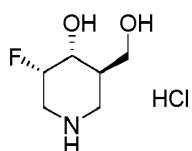
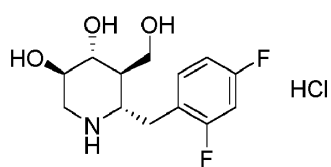
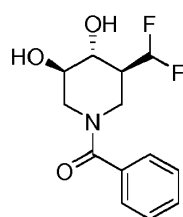
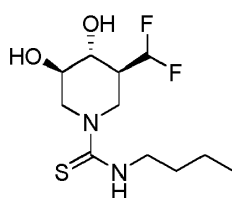
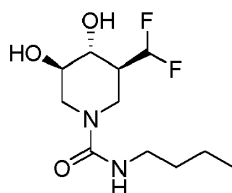
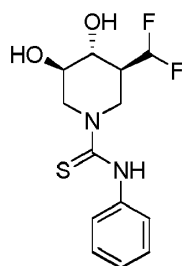
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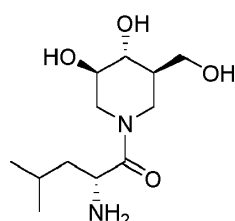
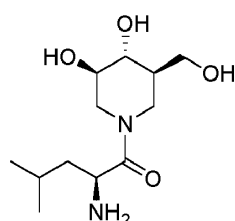
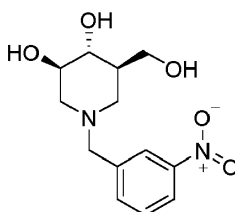
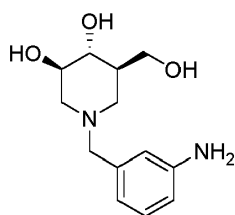
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10 eller et farmasøytisk akseptabelt salt derav eller en hvilken som helst kombinasjon av to eller flere derav; for anvendelse for å forebygge og/eller behandle en lysosomal lagringsforstyrrelse hos en pasient som er i risiko for å utvikle eller få samme diagnose.

15 **4.** Forbindelse for anvendelse ifølge krav 1 som videre omfatter administrering av en effektiv mengde av minst ett annet terapeutisk middel.

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5. Forbindelse for anvendelse ifølge ethvert av de foregående kravene, hvori den lysosomale lagringssykdommen er Niemann-Pick-sykdom.

20 **6.** Forbindelse for anvendelse ifølge ethvert av kravene 1 til 4, hvori den lysosomale lagringssykdommen er Gauchers sykdom.

7. Forbindelse for anvendelse ifølge ethvert av de foregående kravene, i kombinasjon med en effektiv mengde av minst ett annet terapeutisk middel.

8. Forbindelse for anvendelse ifølge krav 7, hvori minst ett annet terapeutisk middel er
5 imiglucerase eller 1,5-(butylimino)-1,5-dideoksy-D-glucitol.