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

(11) **NO/EP 2885288 B1**

(19) NO

NORWAY

(51) Int Cl.

C07D 401/14 (2006.01)
A61K 31/501 (2006.01)
A61P 21/00 (2006.01)
C07D 401/12 (2006.01)
C07D 403/14 (2006.01)
C07D 405/14 (2006.01)
C07D 409/14 (2006.01)
C07D 413/14 (2006.01)
C07D 417/14 (2006.01)
C07D 471/04 (2006.01)
C07D 471/08 (2006.01)
C07D 471/14 (2006.01)
C07D 487/04 (2006.01)
C07D 487/10 (2006.01)

Norwegian Industrial Property Office

(45)	Translation Published	2022.02.21
(80)	Date of The European Patent Office Publication of the Granted Patent	2021.10.06
(86)	European Application Nr.	13752768.5
(86)	European Filing Date	2013.08.13
(87)	The European Application's Publication Date	2015.06.24
(30)	Priority	2012.08.13, US, 201261682448 P
(84)	Designated Contracting States:	AL ; 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 ; RS ; SE ; SI ; SK ; SM ; TR
	Designated Extension States:	BA
(73)	Proprietor	Novartis AG, Lichtstrasse 35, 4056 Basel, Sveits
(72)	Inventor	CHEUNG, Atwood, Novartis Institutes for BioMedical Research Inc.250 Massachusetts Avenue, Cambridge, Massachusetts 02139, USA CHIN, Donovan Noel, Novartis Institutes for BioMedical Research Inc.100 Technology Square, Cambridge, Massachusetts 02139, USA DALES, Natalie, Novartis Institutes for BioMedical Research Inc.250 Massachusetts Avenue, Cambridge, Massachusetts 02139, USA FAZAL, Aleem, Novartis Institutes for BioMedical Research Inc.250 Massachusetts Avenue, Cambridge, Massachusetts 02139, USA HURLEY, Timothy Brian, Novartis Institutes for BioMedical Research Inc.250 Massachusetts Avenue, Cambridge, Massachusetts 02139, USA KERRIGAN, John, Novartis Institutes for BioMedical Research Inc.250 Massachusetts Avenue, Cambridge, Massachusetts 02139, USA O'BRIEN, Gary, Novartis Institutes for BioMedical Research Inc.250 Massachusetts Avenue, Cambridge, Massachusetts 02139, USA

SHU, Lei, Novartis Institutes for BioMedical Research Inc.250 Massachusetts Avenue, Cambridge, Massachusetts 02139, USA
SUN, Robert, 11 Lamplight Circle, Natick, MA 01760, USA
SUNG, Moo, Novartis Institutes for BioMedical Research Inc.250 Massachusetts Avenue, Cambridge, Massachusetts 02139, USA

(74) Agent or Attorney ZACCO NORWAY AS, Postboks 488, 0213 OSLO, Norge

---

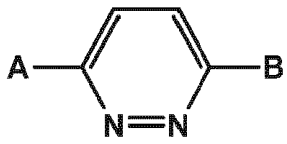
(54) Title **1,4-DISUBSTITUTED PYRIDAZINE ANALOGS AND METHODS FOR TREATING SMN-DEFICIENCY-RELATED CONDITIONS**

(56) References Cited:
WO-A1-2011/130515
WO-A1-2004/043960
WO-A2-2007/133561
EP-A2- 2 014 656
WO-A1-2011/133882
WO-A2-2007/003604
WO-A2-2007/137030
None
DATABASE REGISTRY [Online] CHEMICAL ABSTRACTS SERVICE, COLUMBUS, OHIO, US; XP002716119, Database accession no. 1330184-80-9
AKIO OHSAWA, YOSHIHITO ABE, HIROSHI IGETA: "Cross-Coupling Reaction of Chloropyridazines and Grignard Reagents with Nickel-phosphine Complexes: Alkylation and Arylation of Pyridazines", CHEMICAL AND PHARMACEUTICAL BULLETIN, vol. 26, no. 8, 1978, pages 2550-2554, XP002716118,
DATABASE CA [Online] CHEMICAL ABSTRACTS SERVICE, COLUMBUS, OHIO, US; PETRENKO, O. P. ET AL: "Effect of an intramolecular hydrogen bond on the rate of nucleophilic substitution in a series of haloazines", XP002716255, retrieved from STN Database accession no. 1981:191211

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 eller salt derav ifølge formel (I)

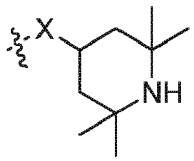


(I)

hvor

A er 2-hydroksyfenyl som er substituert med 0, 1, 2 eller 3 substituenter uavhengig valgt blant C_1-C_4-alkyl, halo-C_1-C_4-alkyl, C_1-C_4-alkoksy, hydroksy, cyano, halogen, amino, mono- og di-C_1-C_4-alkylamino, heteroaryl og C_1-C_4-alkyl substituert med hydroksy eller amino, hvilken heteroaryl har 5 eller 6 ringatomer, 1 eller 2 ringheteroatomer valgt blant N, O og S og substituert med 0, 1, eller 2 substituenter uavhengig valgt blant C_1-C_4-alkyl, mono- og di-C_1-C_4-alkylamino, hydroksy-C_1-C_4-alkylamino, hydroksy-C_1-C_4-alkyl, 4-7-leddet heterosyklus-C_1-C_4-alkyl, amino-C_1-C_4-alkyl og mono- og di-C_1-C_4-alkylamino-C_1-C_4-alkyl; og

B er



,

hvor X er O eller N(Me); eller

en forbindelse eller et salt derav, valgt fra gruppen bestående av

2-(6-(2,2,6,6-tetrametylpiperidin-4-ylamino)-pyridazin-3-yl)fenol;

N-allyl-3-hydroksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)benzamid;

5-(4-amino-1*H*-pyrazol-1-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

5-(3-amino-pyrazol-1-yl)-2-{6-[metyl-(2,2,6,6-tetrametyl-piperidin-4-yl)-amino]-pyridazin-3-yl}-fenol;

5-(5-amino-1H-pyrazol-1-yl)-2-(6-(metyl-(2,2,6,6-tetrametyl-piperidin-4-yl)amino)pyridazin-3-yl)fenol;

2-{6-[(2-hydroksy-etyl)-(2,2,6,6-tetrametyl-piperidin-4-yl)-amino]-pyridazin-3-yl}-5-pyrazol-1-yl-fenol;

5-(1H-pyrazol-4-yl)-2-(6-((2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-3-(trifluormetoksy)fenol;

2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1-metyl-1H-pyrazol-4-yl)-3-(trifluormetoksy)fenol;

2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1H-pyrazol-4-yl)-3-(trifluormetoksy)fenol;

4-(3-hydroksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(trifluormetoksy)fenyl)-1-metylpyridin-2(1H)-on;

3-metoksy-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(5,6,7,8-tetrahydroimidazo[1,2-a]pyridin-3-yl)fenol;

5-(1-syklopentyl-1H-pyrazol-4-yl)-3-metoksy-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

3',5-dimetoksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-[1,1'-bifenyl]-3-ol;

3-(benzyloksy)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(5-metyloksazol-2-yl)fenol;

3-(syklopropylmetoksy)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)-pyridazin-3-yl)-5-(5-metyloksazol-2-yl)fenol;

5-(1H-pyrazol-1-yl)-2-(6-((2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

3-hydroksy-4-(6-((2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)benzonitril;

2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-4-(4,5,6,7-tetrahydropyrazolo[1,5-a]pyridin-3-yl)fenol;

4-(syklopent-1-en-1-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

4-(4-hydroksy-3-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenyl)pyridin-2-ol;

4-(4-hydroksy-3-(6-((2,2,6,6-tetrametylpiperidin-4-yl)oksy)pyridazin-3-yl)fenyl)-1-metylpyridin-2(1H)-on;

4-(4-hydroksy-3-(6-((2,2,6,6-tetrametylpiperidin-4-yl)oksy)pyridazin-3-yl)fenyl)pyridin-2-ol;

4-hydroksy-3-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-N-((1-metyl-1H-pyrazol-4-yl)metyl)benzamid;

3-fluor-5-(2-metoksypyridin-4-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenolhydrokloridsalt;

4-(3-fluor-5-hydroksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenyl)pyridin-2(1H)-on-hydrokloridsalt;

4-(3-fluor-5-hydroksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenyl)-1-metylpyridin-2(1H)-on-hydrokloridsalt;

5-(3-fluor-5-hydroksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenyl)-1-metylpyridin-2(1H)-on-hydrokloridsalt;

5-(5-metoksypyridin-3-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

5-(3-hydroksy-4-(6-metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenyl)pyridin-2-ol;

4-(3-hydroksy-4-(6-metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenyl)pyridin-2-ol;

5-(6-metoksypyridin-3-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

5-(3-hydroksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenyl)-3-(trifluormetyl)pyridin-2-ol;

5-(3-hydroksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenyl)-1-metylpyridin-2(1H)-on;

4-(3-hydroksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenyl)-1-metylpyridin-2(1H)-on;

5-(2-metoksypyridin-4-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

4-(3-hydroksy-4-(6-((2,2,6,6-tetrametylpiperidin-4-yl)oksy)pyridazin-3-yl)fenyl)pyridin-2-ol;

4-(3-hydroksy-4-(6-((2,2,6,6-tetrametylpiperidin-4-yl)oksy)pyridazin-3-yl)fenyl)-1-metylpyridin-2(1H)-on;

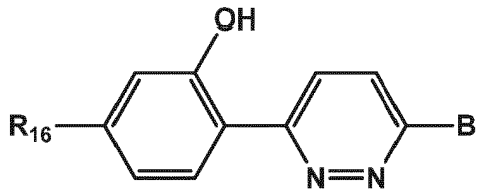
5-(3-hydroksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenyl)pyridin-3-ol;

1-syklopropyl-4-(3-hydroksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenyl)pyridin-2(1H)-on;

5-(syklopent-1-en-1-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

5-(3,6-dihydro-2H-pyran-4-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;
2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(4-nitro-1H-imidazol-2-yl)fenol; og
1-(3-hydroksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenyl)-1H-pyrazol-4-karboksamid.

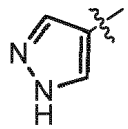
2. Forbindelse ifølge krav 1 eller et salt derav, hvilken forbindelse betegnes med formel (III):



(III)

hvor R_{16} er en 5-leddet heteroaryl som har ett ringnitrogenatom og 0 eller 1 ytterligere ringheteroatom valgt blant N, O og S, hvor heteroarylen er valgfritt substituert med C_1-C_4-alkyl.

3. Forbindelse eller salt derav ifølge krav 2, hvor R_{16} er:



.

4. Forbindelse eller salt derav ifølge krav 1, valgt fra gruppen bestående av
2-(6-(metyl-(2,2,6,6-tetra-metylpiperidin-4-yl)amino)-pyridazin-3-yl)fenol;
4-hydroksy-3-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)benzonitril;
2-{6-[metyl-(2,2,6,6-tetrametyl-piperidin-4-yl)-amino]-pyridazin-3-yl}-4-trifluormetyl-fenol;
2-fluor-6-{6-[metyl-(2,2,6,6-tetrametyl-piperidin-4-yl)-amino]-pyridazin-3-yl}-fenol;
3,5-dimetoksy-2-{6-[metyl-(2,2,6,6-tetrametyl-piperidin-4-yl)-amino]-pyridazin-3-yl}-fenol;
4,5-dimetoksy-2-{6-[metyl-(2,2,6,6-tetrametyl-piperidin-4-yl)-amino]-pyridazin-3-yl}-fenol;
5-metoksy-2-{6-[metyl-(2,2,6,6-tetrametyl-piperidin-4-yl)-amino]-pyridazin-3-yl}-fenol;
4,5-difluor-2-{6-[metyl-(2,2,6,6-tetrametyl-piperidin-4-yl)-amino]-pyridazin-3-yl}-fenol;

5-fluor-2-{6-[metyl-(2,2,6,6-tetrametyl-piperidin-4-yl)-amino]-pyridazin-3-yl}-fenol;
3-hydroksy-4-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)benzonitril;
2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1H-pyrazol-1-yl)fenol;
5-(5-metyl-oksazol-2-yl)-2-{6-[metyl-(2,2,6,6-tetrametyl-piperidin-4-yl)-amino]-pyridazin-3-yl}-fenol;
5-((4-hydroksymetyl)-1*H*-pyrazol-1-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;
5-(1H-imidazol-1-yl)-2-(6-(metyl(2,2,6,6-tetrametyl-piperidin-4-yl)amino)pyridazin-3-yl)fenol;
2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1-(2-morfolino-etyl)-1H-pyrazol-4-yl)fenol;
2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1-methyl-1H-pyrazol-4-yl)fenol;
2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-4-(1H-pyrazol-1-yl)fenol;
5-pyrazol-1-yl-2-[6-(2,2,6,6-tetrametyl-piperidin-4-yloksy)-pyridazin-3-yl]-fenol;
5-(1H-pyrazol-4-yl)-2-(6-((2,2,6,6-tetrametylpiperidin-4-yl)oksy)pyridazin-3-yl)fenol;
2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1H-pyrazol-4-yl)benzen-1,3-diol;
3-metoksy-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1H-pyrazol-4-yl)fenol;
3-metoksy-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1-metyl-1H-pyrazol-4-yl)fenol;
3-metoksy-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(pyridin-3-yl)fenol;
3-etoksy-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(5-metyloksazol-2-yl)fenol;
5-klor-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;
2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-4-(1H-pyrazol-4-yl)fenol;
2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-4-(1H-pyrazol-3-yl)fenol;

4-klor-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1H-pyrazol-4-yl)fenol;

4-fluor-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1H-pyrazol-4-yl)fenol;

5-fluor-4-(1H-imidazol-4-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

5-fluor-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-4-(1H-pyrazol-4-yl)fenol;

5-fluor-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-4-(1H-pyrazol-5-yl)fenol;

4-(4-(hydroksymetyl)-1H-pyrazol-1-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

3-fluor-5-(1H-pyrazol-4-yl)-2-(6-((2,2,6,6-tetrametylpiperidin-4-yl)oksy)pyridazin-3-yl)fenolhydrokloridsalt;

5-klor-3-fluor-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenolhydrokloridsalt;

3-fluor-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1H-pyrazol-4-yl)fenolhydrokloridsalt;

3-fluor-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1-metyl-1H-pyrazol-4-yl)fenolhydrokloridsalt;

5-(6-(dimetylamino)pyridin-3-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(pyrimidin-5-yl)fenol;

2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(2-metylpyridin-4-yl)fenol;

5-(1H-imidazol-2-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

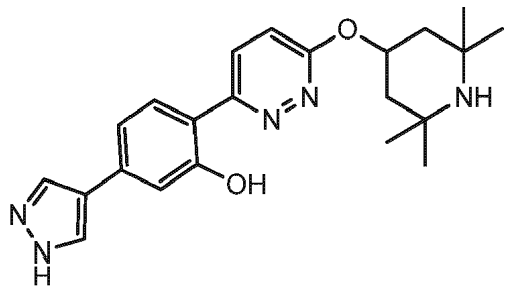
5-(1H-imidazol-4-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol;

2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(4-metyl-1H-imidazol-2-yl)fenol;

2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1-metyl-1H-imidazol-4-yl)fenol;

2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1-metyl-1H-imidazol-5-yl)fenol;
2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(2-metyl-1H-imidazol-4-yl)fenol; og
5-(1,2-dimetyl-1H-imidazol-4-yl)-2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)fenol.

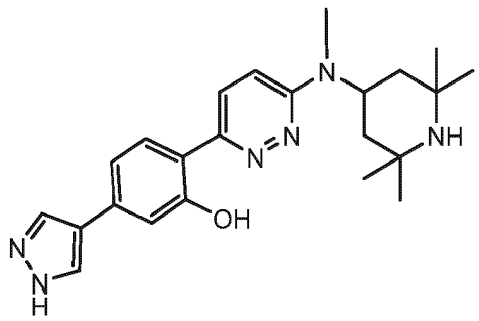
5. Forbindelse ifølge krav 1, hvor forbindelsen er 5-(1H-pyrazol-4-yl)-2-(6-((2,2,6,6-tetrametylpiperidin-4-yl)oksy)pyridazin-3-yl)fenol som har formelen:



eller et salt derav.

6. Forbindelse ifølge krav 5, hvor forbindelsen er 5-(1H-pyrazol-4-yl)-2-(6-((2,2,6,6-tetrametylpiperidin-4-yl)oksy)pyridazin-3-yl)fenolhydrokloridsalt.

7. Forbindelse ifølge krav 1, hvor forbindelsen er 2-(6-(metyl(2,2,6,6-tetrametylpiperidin-4-yl)amino)pyridazin-3-yl)-5-(1H-pyrazol-4-yl)fenol som har formelen:



,

eller et salt derav.

8. Farmasøytisk sammensetning omfattende en terapeutisk virksom mengde av en forbindelse ifølge et hvilket som helst av kravene 1 til 7 eller et farmasøytisk aksepterbart salt derav og én eller flere farmasøytisk aksepterbare bærere.

9. Kombinasjon omfattende en terapeutisk virksom mengde av en forbindelse ifølge et hvilket som helst av kravene 1 til 7 eller et farmasøytisk aksepterbart salt derav og ett eller flere terapeutisk aktive ko-midler.

10. Forbindelse ifølge et hvilket som helst av kravene 1 til 7 eller et farmasøytisk aksepterbart salt derav, for anvendelse som et medikament.

11. Forbindelse ifølge et hvilket som helst av kravene 1 til 7 eller et farmasøytisk aksepterbart salt derav, for anvendelse i behandlingen av en tilstand relatert til SMN-mangel.

12. Forbindelse eller farmasøytisk aksepterbart salt derav for anvendelse ifølge krav 11, hvor den til SMN-mangel relaterte tilstand er spinal muskelatrofi.

13. Forbindelse eller farmasøytisk aksepterbart salt derav for anvendelse ifølge krav 12, hvor den spinale muskelatrofi er av type I (Werdnig-Hoffmanns syndrom).

14. Forbindelse eller farmasøytisk aksepterbart salt derav for anvendelse ifølge krav 12, hvor den spinale muskelatrofi er av type II (intermediær, kronisk form).

15. Forbindelse eller farmasøytisk aksepterbart salt derav for anvendelse ifølge krav 12, hvor den spinale muskelatrofi er av type III (Kugelberg-Welanders syndrom eller juvenil spinal muskelatrofi).

16. Forbindelse eller farmasøytisk aksepterbart salt derav for anvendelse ifølge krav 12, hvor den spinale muskelatrofi er av type IV med debut i voksen alder.