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(54)	Title	COMPOSITIONS AND METHODS FOR THE TREATMENT OF LYSOSOMAL STORAGE DISORDERS AND DISORDERS CHARACTERIZED BY LYSOSOMAL DYSFUNCTION
(56)	References Cited:	WO-A2-2010/148253 WO-A1-2014/200705 WO-A1-2017/136922 US-A1- 2014 336 145 BARTH et al.: "Gaucher's Disease and Cancer: a Sphingolipid Perspective", Crit Rev Oncog, vol. 18, no. 3, 1 January 2014 (2014-01-01), pages 221-234, XP055198776, DOI: 10.1615/CritRevOncog.2013005814 DAVIDSON et al.: "Chronic Cyclodextrin Treatment of Murine Niemann-Pick C Disease Ameliorates Neuronal Cholesterol and Glycosphingolipid Storage and Disease Progression", PLoS ONE, vol. 4, no. e6951, 11 September 2009 (2009-09-11), pages 1-15, XP055144507, JIANG et al.: "Synthesis and Evaluation of Trehalose-Based Compounds as Novel Inhibitors of Cancer Cell Migration and Invasion", Chem Biol Drug Des, vol. 86, 30 April 2015 (2015-04-30), pages 1017-1029, XP055303314, WADA et al.: "Novel autophagy inducers lentztrehaloses A, B and C", The Journal of Antibiotics, vol. 68, 11 March 2015 (2015-03-11), pages 521-529, XP055433916,

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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/>

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Patentkrav

- 5 **1.** Sammensetning omfattende en terapeutisk effektiv mengde trehalose, for anvendelse i behandlingen av juvenil nevronal ceroid-lipofuscinose hos et individ med behov for dette.
- 2.** Sammensetning for anvendelse ifølge krav 1, hvori sammensetningen er ment å administreres parenteralt, foretrukket intravenøst.
- 10 **3.** Sammensetning for anvendelse ifølge krav 1 eller 2, hvori sammensetningen er en vandig løsning av trehalose.
- 4.** Sammensetning for anvendelse ifølge hvilke som helst av kravene 1 til 3, hvori den administreres oralt eller parenteralt i en per administrasjonsdose på
15 mellom 0,1 g/kg til 1 g/kg trehalose.
- 5.** Sammensetning for anvendelse ifølge hvilke som helst av kravene 1 til 4, ytterligere omfattende en trehalaseinhibitor.
- 20 **6.** Sammensetning for anvendelse ifølge krav 5, hvori trehalaseinhibitoren er valgt i gruppen bestående av: validoksyamin A, validamycin A, trehazolin, 1-tiatrehazolin, suidatrestin, salbostatin, MDL 26537, casuarina-6-O-a-D-glukopyranosid, miglustat og 86kD-proteinet fra den amerikanske kakerlakken.
- 25 **7.** Sammensetning for anvendelse ifølge krav 5, hvori trehalaseinhibitoren er miglustat.
- 8.** Sammensetning for anvendelse ifølge krav 7, hvori miglustat administreres i et doseringsområde fra 30 til 100 mg/kg, foretrukket fra 100 til 300 mg/kg og
30 mer foretrukket fra 100 til 150 mg/kg.
- 9.** Sammensetning for anvendelse ifølge hvilke som helst av kravene 1 til 8, hvori trehalosen er en trehaloseanalog valgt fra lentztrehalose A, lentztrehalose B, lentztrehalose C.