



(12) Translation of
European patent specification

(11) NO/EP 3292875 B1

NORWAY

(19) NO
(51) Int Cl.
A61K 48/00 (2006.01)
A61P 3/00 (2006.01)
A61P 3/10 (2006.01)

Norwegian Industrial Property Office

(45)	Translation Published	2020.10.05
(80)	Date of The European Patent Office Publication of the Granted Patent	2020.05.27
(86)	European Application Nr.	17184780.9
(86)	European Filing Date	2013.06.19
(87)	The European Application's Publication Date	2018.03.14
(30)	Priority	2012.06.19, US, 201213527350
(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
(73)	Proprietor	University of Florida Research Foundation, Inc., 223 Grinter Hall, Gainesville, FL 32611, USA
(72)	Inventor	Byrne, Barry, John, 123 Nw 23rd Street, Gainesville, FL 32608, USA Falk, Darin, J., 1600 Sw Archer Road, Gainesville, FL 32610-3003, USA Pacak, Christina, 300 Longwood Avenue, Boston, MA 02115, USA Deruisseau, Lara, Robert, 7810 Karakul LN, Fayetteville, NY 13066-2504, USA Mah, Cathryn, 5 Shelby, Irvine, CA 92620, USA Fuller, David, D., 9421 Sw 28th Road, Gainesville, FL 32608, USA
(74)	Agent or Attorney	ZACCO NORWAY AS, Postboks 2003 Vika, 0125 OSLO, Norge

(54)	Title	COMPOSITIONS AND METHODS FOR TREATING DISEASES
(56)	References Cited:	WO-A2-2008/103993 US-A1- 2012 082 653 WO-A1-2010/132847 WO-A1-2009/043936 DAVID A. WEINSTEIN ET AL: "Adeno-Associated Virus-Mediated Correction of a Canine Model of Glycogen Storage Disease Type Ia", HUMAN GENE THERAPY, vol. 21, no. 7, 1 July 2010 (2010-07-01), pages 903-910, XP055256119, US ISSN: 1043-0342, DOI: 10.1089/hum.2009.157 PACAK CHRISTINA A ET AL: "Tissue specific promoters improve specificity of AAV9 mediated transgene expression following intra-vascular gene delivery in neonatal mice", GENETIC VACCINES AND THERAPY, BIOMED CENTRAL, LONDON, GB, vol. 6, no. 1, 23 September 2008 (2008-09-23), page 13, XP021044007, ISSN: 1479-0556, DOI: 10.1186/1479-0556-6-13

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/>

EP3292875

1

Patentkrav

- 5 **1.** Sammensetning for anvendelse i behandling av Pompes sykdom, hvori sammensetningen omfatter rAAV2/8-vektor, hvori rAAV2/8-vektoren omfatter et heterolog nukleinsyremolekyl som koder for sur α -glukosidase, hvori den heterologe nukleinsyren er operativt forbundet med en desminpromotor, et myocyttspesifikt forsterker-faktor-2-forsterkerelement og et MyoD-forsterkerelement, hvori sammensetningen administreres til et subjekt med behov derav via intramuskulær, intratorokal, intraspinal, intratekal, intracisternal eller
- 10 intravenøs injeksjon.
- 2.** Sammensetningen for anvendelse ifølge krav 1, hvori subjektet ytterligere administreres en acetylkolinesterasehemmer.
- 15 **3.** Sammensetningen for anvendelse ifølge krav 1 eller 2, hvori individet er et menneske.