



Non-confidential summary: NWP007 as a therapeutic vaccine against progressive multifocal leucoencephalopathy (PML)

(Status: October 1, 2016)

Introduction	NEUWAY Pharma GmbH, Bonn, Germany, is focusing on the preclinical and clinical development of innovative therapeutics for treatment of orphan brain diseases based on its proprietary CNS drug delivery platform. The company also intends to partner its drug delivery technology for application to proprietary compounds of pharmaceutical companies to exploit their therapeutic use in the field of CNS indications. In addition, NEUWAY initiated the development of a vaccine - NWP007 - to treat a rare but frequently fatal neurological disease called progressive multifocal leucoencephalopathy (PML). The project is now at a stage where NEUWAY seeks a partner for the further clinical development and global commercialization.
Description of the Investigational Medicinal Product: NWP007	NWP007 is an engineered protein capsule (EPC) to be developed as a therapeutic vaccine against PML. It is derived from the major capsid protein VP1 of the human John Cunningham virus (JCV). JCV is a ubiquitous virus that exclusively infects humans. About 50-90% of adults are infected with JCV without clinical symptoms associated with the infection. However, JCV may cause PML in immunocompromised patients, which is usually combined with a very high viral load in the cerebrospinal fluid (CSF). PML is a rare, but potentially fatal demyelinating disease of the central nervous system (CNS) that usually occurs under immunosuppression. It is most frequently observed in patients suffering from HIV/AIDS or CD4 lymphocytopenia and in patients treated with monoclonal antibodies used in the treatment of multiple sclerosis (MS) and auto-immune conditions, such as rheumatoid arthritis (RA). Vaccination of PML patients with NWP007 is thought to produce a broad immune response that enables to fight the virus - including the mutant virus.
Market exclusivity	<p>The use of NWP007 as therapeutic or prophylactic vaccine in PML is subject matter of the patent application PCT/EP2012/064445 (document can be downloaded using the link provided below). If granted, patent term is until July 23 / 2032.</p> <p>https://dataspace.ssp-europe.eu/#/public/shares-downloads/w3jasocBxJ8iSm0YArns13yDQiMF6pMB</p> <p>The indication "Treatment of PML" qualifies for orphan designation, which may additionally provide a market exclusivity of 7 years (US) and 10 years (EU / Japan), respectively, after marketing approval.</p> <p>In addition, there is data protection of 12 years (US – biological) and 10 years (EU/Japan), respectively.</p>
Therapeutic indication(s)	<p>Use of NWP007 as therapeutic vaccine to treat PML, which is the current focus of development.</p> <p>Use of NWP007 as vaccine to prevent PML in patients with a high risk to develop PML.</p>
Current development status	<p><u>Preclinical</u>: Initial non-GLP tolerability studies in mice and primates demonstrated that NWP007 is generally well tolerated. According to the advice by the competent Authority in Germany one additional GLP toxicity study in mice testing the vaccination scheme will be required prior to initiation of clinical development.</p>



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	<p><u>CMC</u>: Manufacturing process has been established. MSA & Service Contract has been executed with Cobra Biologics, UK. Tech-transfer to Cobra for the upstream process has been initiated 10/2016. No further upscaling required.</p> <p><u>Clinical</u>: Initial results are available from compassionate use in three PML patients with CD4 lymphopenia in combination with topical imiquimod (adjuvant) and IL7. In all three patients vaccination stopped progression of the disease, significantly reduced CSF JCV viral load and induced a humoral and cellular immune response. All three patients were still alive 4.5 (2x) and 5.5 years after vaccination, resp. (status 6/2016). Detailed results are provided in the papers below.</p> <p>1. Ray U, Cinque P, Gerevini S, Longo V, Lazzarin A, Schippling S, Martin M, Buck CB, Pastrana DV. JC Polyomavirus Mutants Escape Antibody-Mediated Neutralization. Science Transl Med 2015; Vol. 7, Issue 306, pp. 306ra151.</p> <p>2. Sospedra M, Schippling AS, Yousef S, Jelcic, Bofill-Mas S, Planas R, et al. Treating progressive multifocal leukoencephalopathy with Interleukin 7 and vaccination with JC virus capsid protein VP1. Clinical Infectious Diseases 2014; 59(11):1588-92.</p> <p>A comprehensive study outline is available under CDA for pivotal study to treat PML (therapeutic vaccination).</p> <p><u>Regulatory</u>: As an outcome of a preclinical / clinical scientific advice meeting with the competent Authority in Germany (Paul-Ehrlich-Institute) it was concluded that the preclinical and preliminary clinical data available so far would be sufficient to allow initiation of clinical development with NWP007 unless a planned sub-chronic GLP toxicology study in mice to test the vaccination scheme would elicit safety signals prohibiting its use in humans.</p>
Next development milestones	Development of GMP process (T0) and - together with a partner - GLP toxicity study in mice (~9 months after T0) and initiation of clinical phase I/II (~17 months after T0)
Regulatory strategy	Treatment of PML qualifies for fast track designation (US) as well as for accelerated assessment /approval of BLA (US, EU, J).
Competitive profile	Very high unmet medical need: So far, there is no treatment option available to treat or prevent PML.
Market size / potential	Treatment of PML: Estimated incidence of patients with PML is ~800 in the EU and ~500 in the US with increasing tendency. Depending on ultimate study results and achievable reimbursement, peak sales in €millions may be in the order of magnitude of high double or even triple digit range. Hence, with a limited investment into the remaining clinical development of NWP007 this project provides an exciting opportunity for a very high ROI.
NEUWAY Pharma's partnering interest	NEUWAY Pharma seeks a partner for the further global development and commercialization of NWP007. NEUWAY Pharma would be prepared to continue development of this compound up to clinical phase I/II under (co-) funding by and in collaboration with a partner in the indication "Treatment of PML".

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