

## PRESS RELEASE



### **NOXXON's Emapticap Pegol Study Selected for Late Breaking Clinical Trials Symposium during ERA-EDTA Conference**

*Full clinical data presentation for anti-CCL2 /MCP-1 Spiegelmer® emapticap pegol (NOX-E36)*

**Berlin, Germany - 2 June 2014** - NOXXON Pharma AG announced that Prof. Hermann Haller, Director of the Department of Nephrology and Hypertension at Hannover Medical School, was invited in his role as the principal investigator to present the full data of NOXXON's phase IIa clinical trial with emapticap pegol (NOX-E36), for the treatment of diabetic nephropathy during the 2014 congress of the European Renal Association and the European Dialysis and Transplant Association.

The talk entitled "CCL2 inhibition with emapticap pegol (NOX-E36) in type 2 diabetic patients with albuminuria" was given during the first symposium of late breaking clinical trials on Sunday, June 1<sup>st</sup>. The ERA-EDTA congress is Europe's premier nephrology conference, including physiology, clinical nephrology, dialysis and transplantation.

Emapticap pegol (NOX-E36) is a Spiegelmer® that binds and inhibits the chemokine CCL2 (MCP-1). Based on pre-clinical work it is believed that the neutralization of this chemokine will prevent infiltration of pro-inflammatory cells into the kidney, thereby allowing existing inflammation to resolve over time. The expected downstream effects include preservation of podocyte numbers, as well as conservation of renal structure and function. The objective of the study was to determine the renoprotective and antidiabetic potential of emapticap pegol in type 2 diabetic patients with albuminuria.

The exploratory trial was laid out as a randomized, double blind, placebo-controlled phase IIa study and enrolled 75 albuminuric type 2 diabetics on a stable standard of care regimen which mandatorily included RAS\* blockade. Emapticap was administered subcutaneously at 0.5 mg/kg twice weekly for 12 weeks, followed by a treatment-free observation phase of 12 weeks.

In the Full Analysis Set/ Intent to Treat (ITT) population, strong trends were seen for reduction of protein in the urine as measured by the albumin to creatinine ratio (ACR), as well as for glycated hemoglobin (HbA1c) in blood. These reductions became statistically significant in a subgroup of 49 patients believed by NOXXON to be most relevant for future studies in this indication (see table below). This was defined as the Primary Efficacy Analysis Set (PEAS). The PEAS dataset excludes those patients with major protocol violations, those treated with dual RAS\* blockade which is now widely considered contraindicated, and those presenting with concomitant hematuria and leukocyturia suggestive of a kidney pathology different

from diabetic nephropathy. Emapticap pegol was generally safe and well tolerated in this study.

Data Set	Number subjects (active + placebo)	Parameter & Time Point Values presented as emapticap vs. placebo							
		ACR day 85 (end of therapy)		ACR day 141 (peak effect: 2 months off therapy)		HbA1c d85 (end of therapy)		HbA1c d113 (peak effect: 1 month off therapy)	
		% change	p-value	% change	p-value	% change	p-value	% change	p-value
ITT dataset	50 + 25	-15%	0.221	-26%	0.064	-4%	0.146	-6%	0.026
PEAS dataset	33 + 16	-32%	0.014	-39%	0.010	-5%	0.096	-7%	0.036

Importantly, the effect on ACR was not accompanied by relevant hemodynamic changes which is consistent with a novel mechanism of action and differentiates emapticap pegol from approved drugs.

Prof. Haller concludes: “The inhibition of the CCL2:CCR2 axis with emapticap pegol is well tolerated and reduces ACR and HbA1c in type 2 diabetics with albuminuria. The maintenance of the effects even after cessation of treatment suggests that CCL2 blockade interferes with the underlying pathophysiology. Emapticap pegol may hence be the first disease-modifying drug for this indication.”

\* Renin Angiotensin System

- Ends -

## Notes for editors:

### About NOXXON Pharma AG

NOXXON Pharma is a biopharmaceutical company pioneering the development of a new class of proprietary therapeutics called Spiegelmers. Spiegelmers are chemically synthesized L-stereoisomer oligonucleotide aptamers, a non-immunogenic alternative to antibodies. NOXXON has a diversified portfolio of clinical-stage Spiegelmer® therapeutics:

- Emapticap pegol (NOX-E36), an anti-CCL2/MCP-1 (C-C chemokine ligand 2 / Monocyte Chemoattractant Protein-1) Spiegelmer®, has completed a Phase IIa study in patients with type 2 diabetes with albuminuria. CCL2 is a pro-inflammatory chemokine involved in the recruitment of immune cells to inflamed tissues.
- Olaptased pegol (NOX-A12), an anti-CXCL12/SDF-1 (CXC chemokine ligand 12 / Stromal Cell-Derived Factor-1) Spiegelmer®, is currently in Phase IIa studies in two hematological cancers, multiple myeloma (MM) and chronic lymphocytic leukemia (CLL). CXCL12 is a chemokine mediator of tumor invasion, metastasis, and resistance to therapy.
- Lexaptepid pegol (NOX-H94), an anti-hepcidin Spiegelmer®, has completed a Phase IIa pilot study in cancer patients with anemia and will soon begin a study in EPO-hyporesponsive dialysis patients. Heparin is the key regulator of

iron metabolism and responsible for the iron restriction leading to anemia of chronic disease.

The Spiegelmer<sup>®</sup> platform provides the company with powerful and unique discovery capabilities, which have generated a number of additional leads under preclinical investigation. Located in Berlin, Germany, NOXXON is a well-financed mature biotech company with a strong syndicate of international investors, and approximately 60 employees.

**For more information, please visit: [www.noxxon.com](http://www.noxxon.com)**

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