



***First Data from NOXXON's NOX-H94 (Lexaptepid Pegol) Clinical Trial in ESA-Hyporesponsive Dialysis Patients Reported at ERA-EDTA Conference***

**Berlin, Germany and Boston, USA - May 29, 2015** - NOXXON Pharma announced that results from Part 1 of its two-part Phase 2a trial of the anti-hepcidin Spiegelmer® NOX-H94 in erythropoiesis-stimulating agent (ESA)-hyporesponsive dialysis patients were reported at the 52<sup>nd</sup> annual meeting of the European Renal Association – European Dialysis and Transplant Association (ERA-EDTA) conference in London today. This trial is investigating the tolerability, pharmacokinetics, iron mobilization effects and efficacy of NOX-H94 in the treatment of ESA-hyporesponsive anemia.

Approximately 10% of the anemic dialysis population in the US is hyporesponsive to treatment with ESAs, and thus these patients end up receiving higher doses of these agents to treat their anemia. As a result, the 10% of patients that are ESA-hyporesponders consume approximately 40% of all erythropoietin, a commonly used ESA, administered in the dialysis population. One factor believed to contribute to ESA-hyporesponsiveness is hepcidin, a peptide hormone increased by inflammation that is a key regulator of iron in the human body. Heparin shuts off iron supply to erythropoietic tissue in the bone marrow and blocks iron absorption from dietary sources, thereby preventing incorporation of iron into the oxygen carrying protein, hemoglobin. NOX-H94 offers the potential for a more effective treatment of this population by binding and neutralizing hepcidin.

Nine patients were enrolled in Part 1 of the trial in which the single-blind cross-over design called for each patient to receive sequentially one administration each of placebo and NOX-H94. Treatment with NOX-H94 was generally well tolerated and all adverse events observed were considered unrelated to treatment. The results showed that no adjustment of the dosing will be required in dialysis patients for the second part of the clinical trial and that the expected effect of increased serum iron levels was seen following NOX-H94 administration.

As a result of the data seen in Part 1 of this trial, NOXXON has elected to initiate Part 2. Dosing of the first of 24 patients in Part 2 has already begun, half of whom will receive NOX-H94, and the other half placebo, twice per week for four weeks. Patients that respond with a pre-defined increase of hemoglobin will be considered responders. The company plans to announce top-line data in the fourth quarter of 2015.

Matthias Baumann, NOXXON's Chief Medical Officer said: "Following these encouraging results from the Part 1 of this Phase 2a study and assuming that the results of Part 2 are positive as well, we anticipate initiating a randomized, double-blind Phase 2b clinical trial evaluating NOX-H94 versus placebo in anemic ESA-

hypo-responsive dialysis patients which would be statistically powered for hemoglobin correction and would also look at the ability of NOX-H94 to reduce the use of ESAs.”

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## Notes for Editors:

### About NOXXON Pharma

NOXXON Pharma is a clinical-stage biopharmaceutical company developing Spiegelmers, a novel class of proprietary nucleic acid-based therapeutic agents built on a backbone of mirror-image nucleotides. Spiegelmers are chemically synthesized L-stereoisomer oligonucleotide aptamers, an immunologically passive alternative to antibodies. NOXXON has a diversified portfolio of clinical-stage Spiegelmer® therapeutics:

- NOX-H94 (lexaptapid pegol) is a Spiegelmer® that binds and neutralizes the human peptide hormone hepcidin. Hepcidin regulates iron metabolism and is a key mediator of iron restriction in anemia of chronic disease. Hepcidin blocks iron inside cellular stores via its interaction with ferroportin, the iron-exporting protein expressed on the surface of iron storage cells, such as macrophages and liver cells. Phase 1 trials demonstrated that NOX-H94 is capable of inhibiting hepcidin in humans, resulting in higher serum iron concentrations relative to subjects receiving placebo and have also shown clinical proof-of-concept in patients with cancer-related anemia. A Phase 2a trial studying the effects of NOX-H94 in a ESA-hypo-responsive population is currently underway.
- NOX-A12 (olaptased pegol) binds and inhibits the human Chemokine CXCL12 also named SDF-1, inhibiting signaling via its two receptors (CXCR4 and CXCR7). Two 28-patient Phase 2a studies of NOX-A12 in combination with chemotherapy have completed the on-treatment phase in patients with relapsed/refractory Multiple Myeloma (MM) and Chronic Lymphocytic Leukemia (CLL). Data from both studies suggest improved efficacy versus relevant historical studies. NOXXON also plans to investigate the use of NOX-A12 in glioblastoma and has received orphan drug designation for glioblastoma in conjunction with radiotherapy in the United States and glioma in Europe.
- NOX-E36 (emapticap pegol) binds and inhibits the human Chemokine CCL2 also referred to as MCP-1 (C-C Chemokine Ligand 2 / Monocyte Chemoattractant Protein 1), which is being developed for the treatment of complications arising from diabetes and specifically diabetic nephropathy. One preclinical study has shown that inhibition of this chemokine led to reduced infiltration of inflammatory cells into the kidney. NOX-E36 has completed a Phase 2a exploratory study, which included 76 patients presenting with Type 2 diabetes with proteinuria on top of standard of care. Promising results were obtained on two key parameters: protein in the urine, or proteinuria, as measured by the albumin to creatinine ratio (ACR), and glycated hemoglobin (HbA1c) in blood. Importantly, the effect on ACR was not accompanied by relevant hemodynamic changes which is consistent with a novel mechanism of action. Further, the maintenance of the effects even after cessation of treatment suggests that CCL2 blockade interferes with the

underlying pathophysiology. These results suggest that NOX-E36 may be the first therapeutic agent with disease-modifying potential in this indication.

The Spiegelmer® platform provides the company with powerful and unique discovery capabilities, which have generated a number of additional candidates in discovery stage research. Located in Berlin, Germany, and Boston, USA, NOXXON has currently 53 employees.

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