

PRESS RELEASE



## **Anti-Hepcidin Spiegelmer<sup>®</sup> Lexaptetid Pegol Increases Hemoglobin in Subset of Anemic Patients in Pilot Phase IIa Study**

*Potential Diagnostic Markers Identified to Predict Response*

**Berlin, Germany - 8 April 2014** - NOXXON Pharma announced that data from a Phase IIa pilot study in anemic cancer patients treated with lexaptetid pegol (NOX-H94) will be presented today at the annual meeting of the American Association for Cancer Research (AACR) in San Diego, CA.

Lexaptetid pegol is a Spiegelmer<sup>®</sup> that binds and neutralizes hepcidin, a peptide hormone that negatively regulates serum iron levels. High hepcidin levels, commonly found in patients with cancer or in dialysis patients, lead to iron restriction, also known as functional iron deficiency: a condition in which iron is blocked inside its cellular stores and is thus unavailable for hemoglobin synthesis. This condition, over time, results in anemia of chronic disease.

The objective of this single-arm, open label, multi-center study was to evaluate the efficacy, pharmacokinetics, safety and tolerability of treatment with lexaptetid pegol. Twelve cancer patients with anemia (hemoglobin < 10 g/dL) were treated for four weeks with twice weekly intravenous infusions of lexaptetid pegol. This treatment period was followed by a one month observation period. No treatment with ESAs (erythropoiesis stimulating agents) such as EPO (erythropoietin) or iron products was allowed during the study period.

The results showed significant increases in hemoglobin levels (>1 g/dL) in 5 of 12 (42%) patients in response to lexaptetid pegol monotherapy. Increased hemoglobin was maintained throughout the follow-up period. For reference, the combination of EPO and intravenous iron in a similar population resulted in a response in 65% of patients<sup>i</sup>. Lexaptetid pegol responders showed increases of both red cell and reticulocyte hemoglobin and a decrease in soluble transferrin receptor levels. This latter marker is an indicator of increased iron demand, and was identified as one of two promising diagnostic predictors along with reticulocyte hemoglobin content.

Following this positive pilot study, NOXXON plans to initiate soon a new study with lexaptetid pegol in EPO-hyporesponsive dialysis patients.

The title and contributors to the poster presentation at AACR are as follows:

Tuesday, Apr 08, 2014, 8:00 AM -12:00 PM, Hall A-E, Abstract 3847, Poster 8

**The anti-hepcidin Spiegelmer<sup>®</sup> lexaptetid pegol (NOX-H94) as treatment of anemia of chronic disease in patients with multiple myeloma, low grade lymphoma, and CLL: A phase II pilot study**

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Members of NOXXON's drug development team will be at the AACR conference to explain the mode of action and clinical potential of this innovative drug candidate.

- 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 successfully completed a Phase IIa study in patients with type 2 diabetes with albuminuria, achieving proof-of-concept. CCL2 is a pro-inflammatory chemokine involved in the recruitment of immune cells to inflamed tissues.
- Olaptosed 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® 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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<sup>i</sup> Ludwig H, *et al.* *European Journal of Cancer*, Vol. **45**(9), 1603-1615, 2009.