



**NOXXON presents Updates on Phase II Studies with Olaptosed Pegol (NOX-A12) in CLL and MM and Lexaptetid Pegol (NOX-H94) in Anemic Cancer Patients**

*Four posters on NOXXON compounds at the European Hematology Association (EHA) Congress*

**Berlin, Germany - 13 June 2014** - NOXXON Pharma presented updated interim data sets from two independent clinical Phase IIa studies of the anti-CXCL12/SDF-1 Spiegelmer<sup>®</sup> olaptosed pegol (NOX-A12) and the outcome of a Phase IIa pilot study on the anti-hepcidin Spiegelmer<sup>®</sup> lexaptetid pegol (NOX-H94) for treating anemia of chronic diseases at the 19<sup>th</sup> Congress of the European Hematology Association (EHA) in Milan, Italy, from 12-15 June 2014.

In the first study, olaptosed pegol was administered to 28 relapsed chronic lymphocytic leukemia (CLL) patients in combination with bendamustine and rituximab (BR). Olaptosed pegol treatment resulted in an effective and prolonged mobilization of CLL cells into the peripheral blood. This mobilization reflects olaptosed pegol's ability to block tumor-microenvironment interactions, which is thought to increase tumor cell sensitivity to killing by chemotherapeutic agents. To date, 20 patients have been evaluated for response at the end of therapy, with 4 patients (20%) achieving a complete response (CR) and 13 patients (65%) achieving a partial response, the overall response rate (ORR) was 85%. Compared to historical data, olaptosed pegol compares favorably to BR baseline therapy with regards to overall response rate and shows an increased rate of complete responses. Moreover, the evaluation of baseline characteristics indicated that relapsed / refractory patients were enrolled into the study, which underlines the clinical relevance of the observed effects and warrants further development of this anti-CXCL12 Spiegelmer<sup>®</sup> in CLL.

In a second study in 28 relapsed multiple myeloma (MM) patients, olaptosed pegol was combined with Velcade<sup>®</sup>/bortezomib and dexamethasone (VD). Olaptosed pegol demonstrated effective and long-lasting mobilization of plasma cells into the peripheral blood. Data from 20 patients showed an overall response rate (ORR) of 70% with 6 patients achieving very good partial responses (VGPR) and 8 achieving a partial response, which compares favorably with published data on therapy with VD. Importantly, treatment with olaptosed pegol was not associated with additional toxicity on top of VD. Considering that the study population included patients retreated with bortezomib and a marked proportion of patients with unfavorable cytogenetics, these results support further development of olaptosed pegol in MM.

An additional preclinical study on olaptosed pegol suggests that targeting CXCL12 might prevent multiple myeloma (MM) cell dissemination to distant bone marrow niches and transition from MGUS (micrometastatic-stage) to active-MM (macrometastatic-stage).

The results of a pilot trial of lexaptepid pegol monotherapy in anemic cancer patients showed pharmacodynamic responses (ferritin decreases) in 10 of 12 patients and hemoglobin increases of 1g/dL or more in 5 of 12 patients supporting the concept of hepcidin inhibition as valuable treatment of anemia of cancer. Increased hepcidin levels, commonly found in anemic patients with cancer or on dialysis, lead to iron restriction, also known as functional iron deficiency: a condition in which iron is blocked in its cellular stores and is thus unavailable for hemoglobin synthesis.

The titles and contributors for the four above mentioned poster presentations at EHA are as follows:

Friday, 13 June 2014, 17:45 - 19:00, Poster Area (NW – Level 0), Abstract P244:

**INTERIM RESULTS FROM A PHASE IIA STUDY OF THE ANTI-CXCL12 SPIEGELMER® OLAPTESED PEGOL (NOX-A12) IN COMBINATION WITH BENDAMUSTINE/RITUXIMAB IN PATIENTS WITH CHRONIC LYMPHOCYTIC LEUKEMIA**

*Michael Steurer, Lydia Scarfò, Marco Montillo, Ann Janssens, Francesca Mauro, Livio Trentin, Josef Thaler, Sonja Burgstaller, Anna Kruschinski, Thomas Dümmler, Kai Riecke, Paolo Ghia, Federico Caligaris-Cappio, Marco Gobbi*

Friday, 13 June 2014, 17:45 - 19:00, Poster Area (NW – Level 0), Abstract P376:

**INTERIM RESULTS FROM A PHASE IIA STUDY OF THE ANTI-CXCL12 SPIEGELMER® OLAPTESED PEGOL (NOX-A12) IN COMBINATION WITH BORTEZOMIB AND DEXAMETHASONE IN PATIENTS WITH MULTIPLE MYELOMA**

*Heinz Ludwig, Katja Weisel, Maria T. Petrucci, Xavier Leleu, Anna M. Cafro, Martin Kropff, Richard Greil, Niklas Zojer, Thomas Dümmler, Anna Kruschinski, Kai Riecke, Robin Foa, Ibrahim Yakoub-Agha, Monika Engelhardt*

Saturday, 14 June 2014, 17:45 - 19:00, Poster Area (NW – Level 0), Abstract P1172:

**THE ANTI-HEPCIDIN SPIEGELMER® LEXAPTEPID 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**

*Pencho Georgiev, Mihaela Lazaroiu, Luminita Ocroteala, Janet Grudeva-Popova, Emanuil Gheorghita, Mariana Vasilica, Sanda Popescu, Andrei Cucuianu, Luciana Summo, Stéphanie Vauléon, Stefan Zöllner, Frank Schwoebel, Kai Riecke, Heinz Ludwig*

Saturday, 14 June 2014, 16:15 - 17:30, Room Gold (SW - Level 2), Abstract S700:

**IN VIVO TARGETING OF STROMAL-DERIVED FACTOR-1 AS A STRATEGY TO PREVENT MYELOMA CELL BONE TO BONE DISSEMINATION**

*Aldo Roccaro, Antonio Sacco, Michele Moschetta, Yuji Mishima, Patricia Maiso, Silvia Lonardi, Dirk Zboralski, Anna Kruschinski, Giuseppe Rossi, Irene Ghobrial*

Members of NOXXON's drug development team and collaboration partners will be at the EHA conference to explain the mode of action and clinical potential of these innovative drug candidates.

- 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<sup>®</sup> therapeutics:

- Emapticap pegol (NOX-E36), an anti-CCL2/MCP-1 (C-C chemokine ligand 2 / Monocyte Chemoattractant Protein-1) Spiegelmer<sup>®</sup>, has completed a Phase IIa proof-of concept study in patients with type 2 diabetes with albuminuria and a Phase IIb study is in the planning stages. 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<sup>®</sup>, is currently tested in Phase IIa studies in two hematological cancers, multiple myeloma (MM) and chronic lymphocytic leukemia (CLL). CXCL12 is a chemokine known to be involved in tumor invasion, metastasis, and resistance to therapy.
- Lexaptepid pegol (NOX-H94), an anti-hepcidin Spiegelmer<sup>®</sup>, has completed a Phase IIa pilot study in cancer patients with anemia and has started screening for 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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