PRESS RELEASE October 1, 2015

# Clinical Study of T-Guard™ in the Treatment of Graft versus Host Disease (GVHD) Extends to Germany

Nijmegen, the Netherlands, October 1, 2015 - **Xenikos B.V. today announced the extension of its ongoing T-Guard Phase 1/2 study to Germany after approval from the Paul Ehrlich Institute on 31 July, 2015.** T-Guard™ **is Xenikos' lead compound that is being developed for treatment of Graft versus Host Disease (GVHD), a frequent and potentially life-threatening complication of bone marrow and blood stem cell transplantation. The Phase 1/2 trial, which began in The Netherlands in March 2014, will be extended to the University Hospital of Muenster, in Germany**.

T-Guard is a combination of two toxin-loaded anti-T-cell antibodies that shows promise as a therapeutic tool for safely and swiftly resetting the body's immune system in T cell mediated diseases. T-Guard is currently being developed for the treatment of therapy resistant acute Graft versus Host Disease (GVHD), a feared and potentially life-threatening complication of hematopoietic stem cell transplantation. There are presently no registered therapies for acute GVHD patients, who have failed standard first-line corticosteroid therapy and the prognosis for these patients is very poor. A Phase 1/2 clinical trial to explore the safety and efficacy of T-Guard was started in March 2014 at the Radboud University Medical Centre in Nijmegen, the Netherlands. Approval from the competent German Authority, the Paul Ehrlich Institute, means that the study can now be extended to Germany, adding the University Hospital Muenster as an additional transplant centre for patients with severe steroid-refractory acute GVHD. The participation of this recognized institute will considerably speed up the inclusion rate. The targeted inclusion of twenty evaluable patients is foreseen around the end of this year.

The University Hospital of Muenster, in Muenster, Germany, is one of the largest hospital-complexes for specialized medical care in northern Germany. Research closely linked to patient-care has contributed largely to the prominent reputation of the University Clinic as a global centre of excellence. The T-Guard study will be managed by a team of dedicated specialists, nurses, and data analysts, led by Professor Matthias Stelljes, Head of the adult Bone Marrow Transplantation Program at the University Hospital and Principal Investigator of the study. Professor Matthias Stelljes is a leading specialist in hematology with a focus on allogeneic stem cell transplantation.

*"Global research efforts into GVHD have, until now, been minimally effective, and bearing in mind the currently dismal prognosis for patients with this condition, I have great interest in participating in this study,' said Professor Matthias Stelljes.*

The primary endpoint of the Dutch/German study will be the overall response rate at Day 28 (the standard primary endpoint of acute GVHD trials). Secondary endpoints include: six months overall survival and the safety and tolerability of T-Guard. Final trial outcomes are expected during Q1 2016. In the meantime, preparations have been started for an active-controlled follow up study for direct comparison of T-Guard to best Standard of Care which might serve as a pivotal study for obtaining early market access in the form of accelerated approval (US) and conditional marketing authorization (EU).

*"With the approval of the Paul Ehrlich Institute and the participation of the Muenster transplant centre we have taken another major step forward in bringing T-Guard to patients to rectify the current lack of registered treatment options for GVHD,"* said Ypke van Oosterhout, Chief Executive Officer of Xenikos. *"We anticipate that the results will strengthen the evidence of T-Guards' promise in the treatment of severe acute GVHD."*

***Notes to Editors:***

**About Graft versus Host Disease**
Graft versus Host Disease (GVHD) is a common complication following allogeneic (donor-derived) blood stem cell transplantation. Transplantation of allogeneic blood stem cells is a widely accepted procedure to restore normal blood cell production (hematopoiesis) in patients treated for blood- or lymphatic cancers, or otherwise suffering from defective blood formation, or immunity. For it to be successful, the blood stem cell graft must contain a minimum number of donor-derived T cells (immune cells), which are beneficial in fighting any residual cancer cells. However, sometimes they can attack the normal tissues of the patient, causing Graft versus Host Disease (GVHD). Approximately 25% of blood stem cell transplant patients develop severe acute GVHD that does not respond adequately to standard first-line therapy. In the last twenty years, there has been a steady increase in the number of allogeneic blood stem cell transplants performed annually in the EU and the US, mainly driven by a sharp increase of unrelated donor (high risk) transplantation (1) (2). This trend is expected to continue. There are currently no registered treatment options, for GVHD patients, who have failed standard corticosteroid therapy. The prognosis without treatment is very poor. All these factors combined emphasize the importance of finding new, effective treatment options for GVHD.

**About T-Guard™**
T-Guard is currently under development by Xenikos B.V. for treatment of certain life-threatening immune conditions, such as transplant-related rejection, Graft versus Host Disease (GVHD), acute solid-organ rejection and several severe autoimmune diseases. It consists of a combination of two toxin-loaded anti-T-cell antibodies, and shows promise as a therapeutic tool for safely and swiftly resetting the body's immune system in T cell mediated diseases. Once injected into the body, T-Guard specifically identifies and eliminates adult T cells, with a strong preference for the activated ones. The particular combination of immunotoxins used to construct T-Guard provides a unique blend of synergistic efficacy, narrow specificity and multiple, gentle mechanisms of action. Its action is unparalleled by any immunosuppressive product currently available commercially. T-Guard is not only very effective in killing activated T cells, but also acts through mechanisms associated with minimal side effects (via apoptosis). Its targeted action leaves patients less vulnerable to opportunistic infections as compared to currently available treatment options.

The safety and efficacy of the new medicine were first evaluated in 2001 in a clinical pilot study at the Radboud University Medical Centre in Nijmegen, the Netherlands. T-Guard was granted EU Orphan Drug Designation in 2005 and US Orphan Drug Designation in September 2013.

**About Xenikos B.V.**
Xenikos B.V. strives to develop new, innovative immunotherapy medicines to help restore patients' health and save lives. It is developing new medicines, based on the action of conjugated antibodies that enables patients suffering serious immune diseases, or rejection after transplantation, to reset their immune system quickly and efficiently. Further information is available at www.xenikos.com

**About the University Hospital of Muenster.**
With more than 7.500 highly qualified employees and a capacity of over 1.500 beds, the University Hospital of Muenster (UKM) is one of the largest hospital-complexes for specialised medical care in northern Germany. Approximately 420.000 patients per year receive high-level inpatient or outpatient treatment in 33 clinics and polyclinics, thanks to its sophisticated facilities and global leading specialists in medicine and research. For more information visit: http://internationalpatients.klinikum.uni-muenster.de

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**References:**
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*(2) Pasquini MC, Zhu X. Current uses and outcomes of hematopoietic stem cell transplantation: 2014 CIBMTR Summary Slides. Available at: www.cibmtr.org.*



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