**Press Release**

# TxCell announces start of phase IIb clinical trial with Ovasave® for refractory Crohn's disease

- ***TxCell undertaking one of largest ever controlled studies for a personalized T cell immunotherapy product***
- ***Top line results of this study are expected end 2016/early 2017***

**Valbonne, France, December 4, 2014 –** TxCell SA (FR0010127662 – TXCL), a biotechnology company developing novel and innovative, cost-effective personalized T cell immunotherapies using antigen specific regulatory T-cells (Ag-Tregs) for severe chronic inflammatory and autoimmune diseases, announces today that it has enrolled the first patient in its phase IIb clinical trial of its lead product Ovasave in refractory Crohn's disease. TxCell has received approval for this multi-center, multinational trial from the regulatory authorities in all six European countries in which the trial will be performed. Top line results of this study are expected at the end of 2016 to early 2017.

The phase IIb study, named CATS29 (Crohn's And Treg Study) includes 32 study sites in 6 countries (Austria, Belgium, France, Germany, Italy and United Kingdom). The trial has been designed to include 160 severe refractory Crohn's disease patients.

The primary objective of the CATS29 study is the evaluation of the response rate for a single intravenous injection of $1.10^6$ cells dose of Ovasave compared to placebo 6 weeks post administration. Response is defined as a decrease ≥ 100 points in the Crohn's Disease Activity Index (CDAI), the gold standard regulatory measure of response in Crohn's disease.

"TxCell anticipates that the CATS29 study with Ovasave in refractory Crohn's disease will confirm the exciting data from our first study CATS1, in this population and prepare the ground for the final phase of clinical development," said Dr Miguel Forte, Sr. VP Clinical

Development and Regulatory Affairs. "Ovasave's targeted, multi-target, multi-mechanism activity together with the personalized nature of the product, offers the chance of real innovation for refractory Crohn's disease patients who currently have no treatment options. There are currently over 100,000 such patients per year in Europe and in the US alone[1]."

"The management of refractory Crohn's disease poses a major challenge today," said Dr. Severine Vermeire, Professor of Gastroenterology, University Hospital Leuven and Principal Investigator of the CATS29 study. "We urgently need new approaches like Ovasave to reduce the heavy burden of this disease on both patients and health systems. All the clinical centers look forward to participating in this important study for the field."

The CATS29 study is a multicentre, randomised, double-blinded, placebo-controlled, multi-dose and multi-injection, 4 parallel groups study. It will evaluate the efficacy and the safety of Ovasave (ovalbumin-specific autologous Treg cells (Ova-Treg)) in patients with active refractory Crohn's disease. Patients will receive, double-blinded, two intravenous (iv) injections 8 weeks apart of either $1.10^4$, $1.10^6$, or $1.10^7$ cells of Ovasave or placebo. Patients will then receive either an open-label treatment with 2 additional iv injections of $1.10^6$ cells of Ovasave or a safety follow-up with no injection. Finally, there will be an extended safety follow-up for all patients.

"The launch of the phase IIb CATS29 study on schedule with our lead product Ovasave is another key milestone for the company," said Damian Marron, CEO, TxCell. "We are well on track to rapidly move this innovative therapeutic approach through the development process with our partner Ferring International Center, so that it can become available to sufferers of refractory Crohn's disease. This achievement is central to TxCell's strategy of targeting orphan/niche indications with poor or no treatment options.."

**About Crohn's disease**

Crohn's disease is an inflammatory bowel disease that can affect the gastrointestinal tract from mouth to anus as well as present some extra-intestinal manifestations. The disease generally begins during adolescence and often affects young adults. It is a chronic

[1] PharMetrics Analysis, September 2008

relapsing and remitting disease manifested mainly by abdominal pain, diarrhea and weight loss, together with systemic symptoms. The cause of Crohn's disease is still unknown, though an interaction between a genetic predisposition and environmental factors is believed to be in the origin of the pathology. The incidence is about 6 to 15 cases per 100,000 and the prevalence of 50 to 200 cases per 100,000[2]. Patient's quality of life is often significantly impaired with Crohn's disease. There is currently no curative therapy.

**About Ovasave**

Ovasave (Ova-Treg cells), TxCell's lead product, is a personalized T cell immunotherapy product, based on the properties of autologous ovalbumin-specific regulatory T lymphocytes. A first clinical study (CATS1) has been completed with Ovasave in refractory Crohn's disease patients and has reported positive clinical efficacy and good tolerability. A phase IIb (CATS29) multicentre, randomised, double-blinded, placebo-controlled, multi-dose and multi-injection, 4 parallel groups study is ongoing to evaluate the efficacy and the safety of Ovasave. Top line results of this study are expected end 2016/early 2017.

After administration, Ova-Treg cells home to the site of inflammation where they are activated by the specific antigen. The Ova-Tregs then act by locally releasing immune suppressive factors, cell-cell contacts and cytotoxic activity to treat the inflammation. Ovasave is classified an Advanced Therapy Medicinal Product (ATMP), by the European Medicines Agency (EMA).

Ovasave is developed in partnership with the Swiss company Ferring International Center. TxCell has granted Ferring an option to acquire an exclusive worldwide license for the development, manufacture and marketing of Ovasave for the treatment of Inflammatory Bowel Diseases (IBD), including Crohn's disease and ulcerative colitis. The deal is potentially worth up to EUR 76 million plus royalties subject to the achievement of milestones.

**About TxCell**

TxCell is developing innovative, cost-effective, personalized T cell immunotherapies for the treatment of severe chronic inflammatory diseases with high medical need. TxCell uses a unique and proprietary ASTrIA technology platform based on the properties of

[2] Gastroenterology. Volume 140, Issue 6, Pages 1785-1794.e4, May 2011. Epidemiology and Natural History of Inflammatory Bowel Diseases. Cosnes et all

autologous antigen-specific regulatory T lymphocytes (Ag-Tregs). The company has completed a phase I/IIa study of its lead product candidate, Ovasave® in refractory Crohn's disease patients and has reported good tolerability and positive clinical efficacy. The company has initiated a phase IIb study in the same patient population. TxCell has a strategic partnership for Ovasave with the Swiss company Ferring International Center. Listed on Euronext-Paris, TxCell, a spin-off of Inserm (France's National Institute for Health and Medical Research) is located in the Sophia Antipolis technology park, Nice, France. The company has 55 employees based at its headquarters and at its manufacturing site in Besançon.

For more information, please visit www.txcell.com

**Practical Information about TxCell shares**:

**ISIN code** FR0010127662

**Ticker code** TXCL

**Disclaimer:**

This press release contains certain forward-looking statements. Although the company believes its expectations are based on reasonable assumptions, these forward-looking statements are subject to numerous risks and uncertainties, which could cause actual results to differ materially from those anticipated.

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