



Press release

TxCell collaboration presents new proof-of-concept transplantation results with proprietary second-generation CAR-Tregs

- **Results presented at ESOT 2017 in Barcelona**
- **Significant effect on mice survival vs. control (p=0.0182) in GvHD model**
- **Confirm potential of CAR-Treg technology**

Valbonne, France, September 26, 2017, 6:00pm CEST – TxCell SA (FR0010127662 – TXCL), a developer of cellular immunotherapies based on regulatory T cells (Tregs) for inflammation, autoimmunity and transplantation, today announces details on the new proof-of-concept preclinical data presented at the 18th Congress of the European Society for Organ Transplantation (ESOT), held in Barcelona, Spain, on September 24-27, 2017. The oral presentation made by Prof. Megan Levings describes positive data obtained with proprietary second-generation CAR-Tregs (HLA-A2 CAR-Tregs) in a graft-versus-host disease (GvHD) preclinical model.

In this study, investigators used the same preclinical GvHD model as was used to evaluate the first-generation murine candidate, which was described in the *J. Clin. Invest.* 2016 publication¹. In this model, the second-generation humanized candidate showed similar efficacy as the first-generation murine candidate in preventing GvHD. Similarly to the murine candidate, the humanized candidate showed a significant effect on mice survival vs. control (p=0.0182).

These proprietary second-generation HLA-A2 CAR-Tregs have a humanized scFv² which is expected to minimize the potential for immunogenic clearance of CAR-Tregs cells, a problem known with CAR-T cells bearing a murine scFv.

The humanized candidate was shown to have higher target specificity compared to the murine candidate. It also appeared that the humanization had no impact on the Treg phenotype nor on Treg functions, such as suppressive effect and cytokine release.

“Developing a second-generation humanized version of our HLA-A2 CAR-Treg candidate is a major step in the TxCell-UBC collaboration. It brings us closer to entering clinical studies with this program,” said Megan Levings, PhD, Professor, Department of Surgery, University of British Columbia (UBC) and Head, Childhood Diseases Research Theme, BC Children's Hospital in Vancouver, Canada. *“These new positive proof-of-concept preclinical results confirm the potential of this CAR-Treg technology to address unmet medical needs in transplant rejection.*

¹ MacDonald KG, Hoeppli RE, Huang Q, Gillies J, Luciani DS, Orban PC, Broady R, Levings MK. Alloantigen-specific regulatory T cells generated with a chimeric antigen receptor. *J Clin Invest.* 2016, 126(4):1413-1424.

² The single-chain variable fragment (scFv) is the extracellular portion of the CAR, which is responsible for specific targeting of CAR-Treg cells to the diseased tissue of interest. The scFv is typically derived from a monoclonal antibody: the heavy chain variable region and the light chain variable region of said antibody are linked to one another by a peptide to give a single chain, which binds to the desired antigen.

Both TxCell and UBC are looking forward to continuing to collaborate and present complementary data as we progress.”

“These new proof-of-concept data demonstrates the progress TxCell has made since the start of our collaboration with UBC less than 12 months ago,” said François Meyer, PhD, Chairman of the Board and Head of Research at TxCell. *“The TxCell/UBC collaboration now has a promising proprietary humanized CAR-Treg candidate and we remain on track to initiate a first-in-man study in transplanted patients.”*

TxCell and UBC started collaborating on the development of a CAR-Treg-based cellular immunotherapy for the prevention of graft rejection in the context of solid organ transplantation in October 2016. This program is scheduled to start its first-in-man study by the end of 2018, pending appropriate funding.

Presentations details

- **Title:** *Alloantigen-specific regulatory T-cells generated with a chimeric antigen receptor.*
- **Speaker:** Megan Levings, PhD, Professor, Department of Surgery, University of British Columbia (UBC) and Head, Childhood Diseases Research Theme, BC Children's Hospital in Vancouver, Canada.
- **Event:** 18th Congress of the European Society for Organ Transplantation (ESOT), September 24-27, 2017, Barcelona, Spain.
- **Presentation date & time:** September 26, 2017, 11.50 am CEST.

About TxCell – www.txcell.com

TxCell is a biotechnology company that develops platforms for innovative, personalized T cell immunotherapies for the treatment of severe inflammatory and autoimmune diseases with high unmet medical need. TxCell is targeting a range of autoimmune diseases (both T-cell and B-cell-mediated) including multiple sclerosis, lupus nephritis and bullous pemphigoid, as well as transplant rejection.

TxCell’s cellular immunotherapies are based on regulatory T lymphocytes (Tregs). Tregs are a T cell population discovered in the 1990’s for which anti-inflammatory properties have been demonstrated. Contrary to conventional approaches based on non-specific polyclonal Tregs, TxCell is exclusively developing engineered antigen-specific Tregs, where the antigen specificity is brought by a Chimeric Antigen Receptor (CAR) (CAR-Treg cells).

Based in Sophia-Antipolis, France, TxCell is listed on Euronext Paris and currently has 46 employees.

Next TxCell events

Scientific and medical conferences

Sept 24-27	ESOT 2017 (European Society for Organ Transplantation)	Barcelona (SP)
Sept 26	Journée « Bioproduction des immunothérapies en France »	Paris (FR)
Oct 9-11	Final conference on the COST ‘A FACTT’ project	Barcelona (SP)
Oct 17-20	ESGCT 2017 (European Society of Gene & Cell Therapy)	Berlin (DE)

Financial and business conferences

Sept 26-27	Annual Biotech in Europe Investor Forum (Sachs Associates)	Basel (CH)
Oct 4-5	Large & Midcap Event Paris	Paris (FR)
Oct 4-5	Cell & Gene Meeting on the Mesa	La Jolla (US)
Nov 6-9	BIO-Europe	Berlin (DE)
Nov 9	5 th Annual European Advanced Therapies Investor Day	London (UK)
Nov 14	Inv€\$tival Showcase	London (UK)
Nov 15-16	Jefferies 2017 Global Healthcare Conference	London (UK)
Nov 23-24	Actionaria	Paris (FR)

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Forward-Looking Statements

This press release contains certain forward-looking statements relating to the business of TxCell, which shall not be considered *per se* as historical facts, including TxCell's ability to develop, market, commercialize and achieve market acceptance for specific products, estimates for future performance and estimates regarding anticipated operating losses, future revenues, capital requirements, needs for additional financing. In addition, even if the actual results or development of TxCell are consistent with the forward-looking statements contained in this press release, those results or developments of TxCell may not be indicative of their in the future.

In some cases, you can identify forward-looking statements by words such as "could," "should," "may," "expects," "anticipates," "believes," "intends," "estimates," "aims," "targets," or similar words. Although the management of TxCell believes that these forward-looking statements are reasonably made, they are based largely on the current expectations of TxCell as of the date of this press release and are subject to a number of known and unknown risks and uncertainties and other factors that may cause actual results, performance or achievements to be materially different from any future results, performance or achievement expressed or implied by these forward-looking statements. In particular, the expectations of TxCell could be affected by, among other things, uncertainties involved in the development of the Company's products, which may not succeed, or in the delivery of TxCell's products marketing authorizations by the relevant regulatory authorities and, in general, any factor that could affect TxCell capacity to commercialize the products it develops, as well as, any other risk and uncertainties developed or identified in any public documents filed by TxCell with the AMF, included those listed in chapter 4 "Risk factors" of the 2016 *document de référence* (registration document) approved by the AMF on April 26, 2017 under number R.17-024. In light of these risks and uncertainties, there can be no assurance that the forward-looking statements made in this press release will in fact be realized. Notwithstanding the compliance with article 223-1 of the General Regulation of the AMF (the information disclosed must be "accurate, precise and fairly presented"), TxCell is providing the information in these materials as of this press release, and disclaims any intention or obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events, or otherwise.