

## TiGenix Launches Global Phase III Trial for Cx601

**Leuven (BELGIUM) – June 13, 2017, 07:00h CEST – TiGenix NV (Euronext Brussels and NASDAQ: TIG), an advanced biopharmaceutical company focused on exploiting the anti-inflammatory properties of allogeneic, or donor-derived, stem cells to develop novel therapies for serious medical conditions, today announces that it has hosted its first European investigator meeting, which formally launches the global pivotal phase III clinical trial for Cx601 for the treatment of complex perianal fistulas in patients with Crohn's disease. Similar investigator meetings are planned to take place in Europe (EU), Israel, the United States and Canada from the fourth quarter of 2017. The global trial is designed to support a future regulatory filing for Cx601 in the U.S.**

The first investigator meeting, held on June 8 and June 9 in Rome, Italy, brought together more than 60 leading gastroenterologists, colorectal surgeons and study co-ordinators from 30 confirmed clinical trial sites across Belgium, Czech Republic, Italy, Poland and Spain. Presentations were given by national and regional study co-ordinators, including the principal EU/Israel gastroenterologist study co-ordinator, Professor Julián Panés, Head of the Inflammatory Bowel Disease Unit at the Hospital Clinic of Barcelona (Spain) and President of the European Crohn's and Colitis Organization (ECCO), and the EU/Israel surgeon study coordinator, Dr. Damián García Olmo, Chief of the Department of Surgery at Fundación Jiménez Díaz University Hospital, Universidad Autónoma de Madrid (Spain).

The global pivotal Phase III trial is a randomized, double-blind, placebo-controlled study designed to confirm the efficacy and safety of a single administration of Cx601 for the treatment of complex perianal fistulas in Crohn's disease patients. The trial design is similar to the European Phase III ADMIRE-CD trial for Cx601 with an identical primary endpoint. In January 2017, the U.S. Food and Drug Administration (FDA) agreed to the design of the protocol for the global Phase III trial, and confirmed that a future U.S. Biologics License Application (BLA) could be filed based on the study results at week 24, instead of week 52, from a broader patient population than the initial Special Protocol Assessment (SPA) formally endorsed in August 2015. With these adjustments, the trial should benefit from an expedited recruitment process, leading to shorter timelines, an earlier filing, and the possibility of an earlier approval in the U.S.

"Investigator meetings are critical to the success of any clinical trial. The attendance and support from leading experts in the GI field confirms the strong interest from the medical community in our global phase III trial and, ultimately, for Cx601 as a potential new treatment for patients," said Dr. Marie-Paule Richard, Chief Medical Officer at TiGenix.

"Following my participation as Principal Investigator for the European ADMIRE-CD trial, which successfully met its primary endpoint and safety and efficacy profile, I am excited to participate in this global trial as study coordinator for Europe and Israel," said Professor Julian Panés. "We are eager to continue building on the evidence gained from the ADMIRE-CD trial and, ultimately, to offer patients a durable cure for their complex perianal fistulas, which remain a severe and debilitating complication of Crohn's disease."

### For more information

**Claudia D'Augusta**  
Chief Financial Officer  
T: +34 91 804 92 64  
[claudia.daugusta@tigenix.com](mailto:claudia.daugusta@tigenix.com)

## **About TiGenix**

TiGenix NV (Euronext Brussels and NASDAQ: TIG) is an advanced biopharmaceutical company developing novel therapies for serious medical conditions by exploiting the anti-inflammatory properties of allogeneic, or donor-derived, expanded stem cells.

TiGenix' lead product, Cx601, has successfully completed a European Phase III clinical trial for the treatment of complex perianal fistulas - a severe, debilitating complication of Crohn's disease. Cx601 has been filed for regulatory approval in Europe and a global Phase III trial intended to support a future U.S. Biologic License Application (BLA) is expected to start in 2017. TiGenix has entered into a licensing agreement with Takeda, a global pharmaceutical company active in gastroenterology, under which Takeda acquired the exclusive right to develop and commercialize Cx601 for complex perianal fistulas outside the U.S. TiGenix' second adipose-derived product, Cx611, is undergoing a Phase I/II trial in severe sepsis – a major cause of mortality in the developed world. Finally, AlloCSC-01, targeting acute ischemic heart disease, has demonstrated positive results in a Phase I/II trial in acute myocardial infarction (AMI). TiGenix is headquartered in Leuven (Belgium) and has operations in Madrid (Spain). For more information, please visit <http://www.tigenix.com>.

## **About Cx601**

Cx601 is a local administration of allogeneic (or donor derived) expanded adipose-derived stem cells (eASCs) for the treatment of complex perianal fistulas in Crohn's disease patients that have previously failed conventional therapy. Crohn's disease is a chronic inflammatory disease of the intestine and complex perianal fistulas are a severe and debilitating complication for which there is currently no effective treatment. Cx601 was granted orphan drug designation by the European Commission in 2009. TiGenix completed a European Phase III clinical trial (ADMIRE-CD) in August 2015 in which both the primary endpoint and the safety and efficacy profile were met, with patients receiving Cx601 showing a 44% greater probability of achieving combined remission compared to control (placebo). A follow-up analysis was completed at 52 weeks and 104 weeks post-treatment, confirming the sustained efficacy and safety profile of the product. The 24-week results of the Phase III ADMIRE-CD trial were published in *The Lancet* in July 2016. Based on the positive 24 weeks Phase III study results, TiGenix submitted a Marketing Authorization Application to the European Medicines Agency (EMA) and a decision is expected in 2017. A global Phase III clinical trial intended to support a future U.S. Biologic License Application (BLA) is expected to start in 2017, based on a trial protocol that has been agreed with the Food and Drug Administration (FDA) through a special protocol assessment procedure (SPA). In July 2016, TiGenix entered into a licensing agreement with Takeda, a global pharmaceutical company active in gastroenterology, under which Takeda acquired exclusive rights to develop and commercialize Cx601 for complex perianal fistulas in Crohn's patients outside of the U.S.

## **Forward-looking information**

This press release may contain forward-looking statements and estimates with respect to the anticipated future performance of TiGenix and the market in which it operates. Certain of these statements, forecasts and estimates can be recognised by the use of words such as, without limitation, "believes", "anticipates", "expects", "intends", "plans", "seeks", "estimates", "may", "will" and "continue" and similar expressions. They include all matters that are not historical facts. Such statements, forecasts and estimates are based on various assumptions and assessments of known and unknown risks, uncertainties and other factors, which were deemed reasonable when made but may or may not prove to be correct. Actual events are difficult to predict and may depend upon factors that are beyond the Company's control. Therefore, actual results, the financial condition, performance or achievements of TiGenix, or industry results, may turn out to be materially different from any future results, performance or achievements expressed or implied by such statements, forecasts and estimates. Given these uncertainties, no representations are made as to the accuracy or fairness of such forward-looking statements, forecasts and estimates. Furthermore, forward-

*looking statements, forecasts and estimates only speak as of the date of the publication of this press release. TiGenix disclaims any obligation to update any such forward-looking statement, forecast or estimates to reflect any change in the Company's expectations with regard thereto, or any change in events, conditions or circumstances on which any such statement, forecast or estimate is based, except to the extent required by Belgian law.*