PRESSE RELEASE

LEUKOTAC® takes next step in paediatric acute steroid-resistant GvHD

- **ELSALYS BIOTECH plans to submit to the European Medicines Agency before summer 2018 a Paediatric Investigation Plan (PIP) proposing to conduct a prospective clinical study in the paediatric population**
- **The management of acute steroid-resistant graft versus host disease (SR-aGvHD0F[1]), the indication for LEUKOTAC®, is even more critical in paediatric patients than in adults as the limitations of therapeutic options are even worse**

Lyon, FRANCE, 05 April 2018, ELSALYS BIOTECH, a new player in immuno-oncology, met paediatric transplant physicians at the 44th EBMT congress that took place in Lisbon, Portugal, from 18 to 21 March. The Company presented the progress made on LEUKOTAC® (inolimomab), its monoclonal antibody that has demonstrated promising clinical results in the treatment of SR-aGvHD and addressed in particular the objectives and challenges of the upcoming paediatric investigation plan.

Retrospective analysis ongoing in about 150 children treated with LEUKOTAC®

In the context of the early access programme (Temporary Use Authorisation[2]) granted by the French medicine agency (ANSM), over 250 children suffering from SR-aGvHD were treated with LEUKOTAC® in compassionate use, mainly between 2005 and 2015. This antibody is well known by the French paediatric physicians specialised in bone marrow transplantation.
Efficacy and safety data of LEUKOTAC® were collected retrospectively among 147 patients and are being analysed. In a heterogeneous paediatric population – in terms of age and origin of the disease conducting to bone marrow transplantation – the main objectives of this analysis are to identify the optimal dosing, the rate of response as well as the duration of the response together with the overall impact on survival after treatment of this population with LEUKOTAC®. The data may allow a more precise identification of the patient typologies who are most likely to benefit from treatment and will be used to design the prospective clinical study in the paediatric population that will later be conducted within the frame of a Paediatric Investigation Plan.

Paediatric Investigation Plan to be filed before summer

The planned study will be multicentric in Europe, even North America. It is expected to involve approximately 60 to 80 patients in a non-randomised study (without control arm, in other words all patients will receive LEUKOTAC®).

«The medical need represented by steroid-resistant aGvHD among children under the age of 18 *is major and critical. The therapeutic solutions in this population are very limited, also coming from clinical trials given that treatments are always validated in adults before in children. The paediatric physicians who used LEUKOTAC® say that since the product has no longer been available they have had difficulties and they support making the product available again at the earliest opportunity,»* explains **Dr. David LIENS, Chief Medical Officer, ELSALYS BIOTECH.**

1 Steroid-Resistant acute Graft-versus-Host Disease.
2 French procedure allowing physicians to treat sick patients with medicines not yet available on the market.

«We are preparing a paediatric investigation plan that proposes to conduct a prospective clinical study in children with the support of several French and European clinicians. This plan, which is an obligation under European regulation, will be submitted before summer to the European Medicines Agency that is expected to welcome this proposal since it is not common to propose studies in these populations. It is an ambitious timing but it is equal to the challenges faced by young patients in the absence of any therapeutic solution,» adds **Dr. Catherine MATHIS, Chief Operations Officer, ELSALYS BIOTECH.**

Watch the interview (in French) with Professor Jean-Hugues Dalle,
head of paediatric haematopoietic stem cell transplantation, Robert-Debré University Hospital, Paris, who presents the challenge of SR-aGvHD in paediatrics

About LEUKOTAC®

LEUKOTAC® (inolimomab) is an immunotherapy monoclonal antibody that targets the interleukin-2 receptor (IL-2), a chemical molecule named cytokine that contributes to the development and proliferation of some white blood cells including T-cells responsible for aGvHD. By linking specifically to the α chain of the receptor (CD25), LEUKOTAC® prevents IL-2 from binding on the surface of the donor's over-active T-cells which blocks their multiplication.

The efficacy of LEUKOTAC® in steroid-resistant aGvHD lies mainly in its specificity and its preferential affinity to the CD25 receptor found on the surface of T-lymphocytes.

About steroid-resistant aGvHD

Formerly called bone marrow transplant, Hematopoietic Stem Cell Transplantation (HSCT) is the last therapeutic option for patients with certain blood cancers or severe immunodeficiency. In practice, the treatment is designed to replace the diseased blood cells of the patient with the hematopoietic stem cells of a matching donor (allograft).

Once grafted, these stem cells will produce new healthy and functional blood cells, including white blood cells that will allow patients to bridge their immune deficiency or to eliminate surviving cancer cells.

If this technique has made considerable progress in 60 years, half of transplant recipients are still victims of complications: side effects of conditioning pretreatment (that aims to prevent transplant rejection), long-term susceptibility to infections and GvHD. In the latter case, the donor's over-active T-cells «turn against» the patient's tissues: mucous membranes, skin, gastro-intestinal tract, liver and lungs. The acute form appears just after the transplant, the chronic form occurring several months later (preceded or not by an aGvHD).

Affecting between 30 to 55% of patients, GvHD is the main complication of transplantation. To halt this "autoimmune disease", physicians combine corticosteroids with other immunosuppressive agents. The fact remains that some 30 to 50% of aGvHD gradually become resistant to these first-line treatments. To date clinicians do not have any standard of treatment for these patients for whom there is a strong unmet medical need. Thus in Europe, 4,000 children and adults die each year from their aGvHD.

About ELSALYS BIOTECH

ELSALYS BIOTECH is a clinical stage immuno-oncology company that designs and develops a new generation of therapeutic antibodies that target tumors and their immune and/or vascular microenvironment. By modulating the action of immune cells (immunomodulator antibodies) or by blocking the mechanisms that promote tumor growth (targeted antibodies), ELSALYS BIOTECH intends to offer new options to patients for whom therapy is no longer an option.

To convert these novel targets into drug candidates, the Company relies on a world-class academic network, a team and an R&D platform that encompasses targets sourcing to clinical development and the commercialization of monoclonal antibodies derived from these targets. Today ELSALYS BIOTECH is conducting 5 proprietary development programs including LEUKOTAC® (inolimomab), an immunotherapy antibody that has recently demonstrated its clinical superiority in Phase 3 in an orphan disease with very poor prognosis: steroid-resistant acute Graft-versus-Host Disease. Bringing LEUKOTAC® to the market could lead ELSALYS BIOTECH to build, by acquisitions, a portfolio of commercial immunotherapy antibodies against rare diseases.

Founded in 2013, ELSALYS BIOTECH is located in the heart of the European cluster LYON BIOPOLE. Its founding shareholders are TRANSGENE and SOFIMAC INNOVATION, joined in 2015 by the INSTITUT MERIEUX EUROPE and CREDIT AGRICOLE CREATION.

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Contacts

ELSALYS BIOTECH
Dr. Christine GUILLEN
CEO and Co-founder
+33(0)6 37 69 75 15
guillen@elsalysbiotech.com

PRESSE
ATCG PARTNERS
Marie PUVIEUX (France)
+33 (0)6 10 54 36 72
Céline VOISIN (UK/US)
+33 (0)6 62 12 53 39
presse@atcg-partners.com