ElsaLys Biotech announces submission of Biologics License Application to FDA for LEUKOTAC® (inolimomab) for the treatment of graft-versus-host disease in adult patients

- **The Biologics License Application (BLA) will be reviewed under the FDA's Real-Time Oncology Review pilot program (RTOR program)**

Lyon, FRANCE, July 23, 2020, **ElsaLys Biotech**, a clinical stage company, owned by Mediolanum Farmaceutici Spa, announced today the U.S. Food and Drug Administration (FDA) agreement to start the LEUKOTAC® (inolimomab) submission process for a Biologics License Application (BLA) for the treatment of Steroid-Refractory acute graft-versus-host disease (aGvHD), grade II-IV adult patients.
The Biologics License Application (BLA) will be reviewed under the FDA's Real-Time Oncology Review pilot program (RTOR program), an initiative of the FDA's Oncology Center of Excellence. The RTOR program aims to explore a more efficient review process to ensure that safe and effective treatments are available to patients as early as possible, while maintaining and improving review quality by the FDA.

"This is an important milestone for ELSALYS BIOTECH as we are one step closer towards potentially bringing inolimomab to patients, responding to an increasing health need," **said Dr. Christine GUILLEN, CEO and co-founder of ElsaLys Biotech.** *" I want to thank the team for their dedication in working to address the FDA's findings over the past few months. We look forward to working with the FDA throughout its review process."*

Inolimomab has shown a robust and long-lasting response Rate in steroid-refractory acute graft versus host disease (SR-aGvHD) in a randomized multicentre controlled parallel-group Phase 3 study (INO-107 - EUDRACT 2007-005009-24). In addition, Inolimomab demonstrated a clear advantage in long-term survival compared to control group treated with ATG (Anti-Thymo Globulin). Affecting 30 to 55% of patients, aGvHD is the main complication of Hematopoietic Stem Cell Transplantation (HSCT) and is the major cause of morbidity and mortality in this setting. Around half of the patients with aGvHD do not respond to initial steroid treatment and are left with few therapeutic options.

Since December 24, 2019, the French National Agency for the Medicines and Health Products Safety (ANSM) granted a cohort ATU for inolimomab in France in this indication (also including pediatric patients).

"*The increasing number of hematopoietic stem cell transplantation worldwide has triggered an increasing need for a large variety of new approved drugs addressing the acute Graft versus Host Disease and especially its Steroid-Resistant form that tend have a very poor outcome*" **said Dr. David LIENS, Chief Medical Officer, ElsaLys Biotech**, concluding that "*Robust clinical data including a positive safety profile, obtained from over 1400 treated individuals, positions inolimomab as a meaningful therapeutic alternative for THE treatment of steroid refractory aGvHD*."

In June 2020, FDA accepted the application for Leukotac under the RTOR pilot program which is a more efficient review process to ensure that safe and effective treatments are available to patients as early as possible.

RTOR program is granted BY FDA to therapies that have the potential to demonstrate substantial improvements over available therapy.

This allows the FDA to review much of the data earlier, before the applicant formally submits the complete application.

Additional information about RTOR can be found at: https://www.fda.gov/about-fda/oncology-center-excellence/real-time-oncology-review-pilot-program.

The next step in the BLA Submission process to the FDA will be the Chemistry, Manufacturing and Controls (CMC) pre-BLA meeting in September 2020.

About inolimomab (LEUKOTAC®)

Inolimomab (LEUKOTAC®) 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), inolimomab prevents IL-2 from binding on the surface of the donor's over-active T-cells which blocks their multiplication.

The efficacy of inolimomab in 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 50% 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 are resistant or dependant to these first-line treatments. To date limited therapeutic options are available for these patients with no standard treatment approved so far in Europe and only one in the US.

About ELSALYS BIOTECH

ELSALYS BIOTECH is a clinical stage immuno-oncology company which designs and develops a new generation of therapeutic antibodies targeting tumors and their immune and/or vascular microenvironment.

To convert these novel targets into drug candidates, the Company is currently conducting 3 proprietary development programs including Inolimomab (LEUKOTAC®), 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 and that is currently benefitting of cohort ATU in France for the treatment of graft-versus-host disease, corticosteroid-resistant or corticosteroid-dependent, with grade II-IV.

Founded in 2013, ELSALYS BIOTECH is located in the heart of the European cluster LYON BIOPOLE. As announced in May 2020, the company is from now owned by Mediolanum Farmaceutici Spa.

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