# PRESS RELEASE

**AB2 Bio's Tadekinig alfa therapy receives Breakthrough Therapy Designation from the US Food and Drug Administration (FDA) for the treatment of monogenic, Interleukin-18 (IL-18) associated autoinflammatory conditions with ongoing systemic inflammation.**

**In addition, AB2 Bio receives Orphan Drug Designation from the FDA for the treatment of Haemophagocytic Lymphohistiocytosis (HLH).**

- **Breakthrough Therapy Designation (BTD) recognises the major potential therapeutic benefits of Tadekinig alfa (IL-18BP) in the treatment of severe and potentially fatal autoinflammatory conditions**
- **FDA BTD and ODD status supports the accelerated development of Tadekinig alfa**

**Lausanne (Switzerland), April 3, 2017**. AB2 Bio Ltd, a clinical-stage Swiss biotech company, specialized in developing innovative therapies for the treatment of severe systemic autoinflammatory diseases, today announced that the US Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation to Tadekinig alfa in the treatment of monogenic, IL-18 associated autoinflammatory conditions with ongoing systemic inflammation.

In addition, the FDA has also granted Orphan Drug Designation (ODD) to Tadekinig alfa for the treatment of Haemophagocytic Lymphohistiocytosis (HLH).

Dr. Andrew Sleight, CEO of AB2 Bio Ltd, commented: "The Breakthrough Therapy Designation underscores the potential of Tadekinig alfa to provide much-needed treatment options to patients suffering from these life-threatening diseases. We look forward to working closely with the FDA in order to bring this potentially life-saving medicine to critically ill patients."

Dr. Andrew Sleight, CEO of AB2 Bio Ltd, continued: "We are also very pleased that, following on from the granting of ODD by the European Medicines Agency (EMA) in November 2016, the FDA also granted ODD to Tadekinig alfa for the treatment of Haemophagocytic Lymphohistiocytosis (HLH)."

**Breakthrough Therapy Designation and Orphan Drug Designation for Tadekinig alfa**

Following on from the extraordinary case report presented at the Annual Meeting of the American College of Rheumatology in November 2015, in which AB2 Bio treated a critically ill baby girl suffering from a genetic mutation of the NLRC4 gene with IL-18BP under a compassionate use basis, AB2 Bio is currently initiating a pivotal Phase III clinical trial in patients affected by the same condition.

NLRC4 mutations are monogenic and IL-18 associated autoinflammatory diseases and are classified as a sub-group of HLH diseases. The BTD covers this condition together with conditions such as XIAP deficiencies. It is intended to expedite the development and review timelines of potential new medicines to treat serious or life-threatening diseases, where preliminary clinical evidence shows that the medicine may provide substantial improvement over existing treatments.

**About Breakthrough Therapy Designation**

The FDA's Breakthrough Therapy designation is intended to expedite the development and review of a drug candidate that is planned for use in the treatment of a serious or life-threatening disease or condition when preliminary clinical evidence indicates that the drug may demonstrate a substantial improvement over existing therapies on one or more clinically significant endpoints. The benefits of

Breakthrough Therapy Designation include eligibility for rolling review, priority review and more intensive and interactive dialogue with FDA's senior managers for an efficient drug development program.

**About Orphan Drug Designation**

To receive Orphan Drug Designation from the FDA, a medicinal product must be intended for the treatment of a disease or disorder affecting fewer than 200,000 individuals in the U.S. Orphan Drug Designation provides certain incentives including protocol assistance and seven years market exclusivity in the U.S. upon marketing approval for the designated indication.

**About Haemophagocytic Lymphohistiocytosis (HLH)**

HLH and related disorders are potentially life-threatening conditions of severe systemic autoinflammation. People with HLH usually develop symptoms within the first months or years of life which may include fever, pancytopenia, coagulopathy, and hemophagocytosis. These disorders can be either inherited (genetic) or secondary to other conditions such as cancer. HLH is caused by an overactivation of macrophages. A key aspect of Tadekinig alfa's therapeutic profile is that it specifically targets the core of HLH by controlling macrophages and preventing them from becoming overactivated.

**About Interleukin-18 Binding Protein (IL-18BP), a safe and transformative potential treatment in severe autoinflammatory diseases**

While the time-limited inflammatory response is a natural mechanism intended to limit harm to the body, dysregulated and persistent inflammatory processes are the basis of several chronic inflammatory and autoimmune diseases. IL-18BP is a human protein with a high affinity for IL-18, a major inflammatory cytokine. In healthy people, there is a large excess of naturally occurring IL-18BP keeping levels of free IL-18 low. However, in patients with certain inflammatory diseases, the IL-18/IL-18BP balance is disrupted, resulting in high levels of free and active IL-18, which in turn leads to pathological inflammation. Administration of AB2 Bio's exogenous recombinant human IL-18BP restores the IL-18/IL-18BP balance, removing free IL-18 and thereby reducing inflammation. AB2 Bio has developed the first and unique proprietary assay detecting free IL-18 allowing the identification of clinical entities that are driven by free IL-18. Extensive Phase I, Ib and II clinical trial results have demonstrated that IL-18BP is very well tolerated and has an excellent safety profile.

**About AB2 Bio Ltd**

AB2 Bio Ltd, located on the Innovation Park at the École polytechnique fédérale de Lausanne (EPFL), is specialised in the development of treatments against autoinflammatory diseases. The Swiss clinical-stage biotech company is developing drugs that will not only treat the symptoms but particularly target the underlying causes of inflammation-based diseases.

AB2 Bio is initiating a pivotal Phase III clinical trial in patients carrying mutations of the NLRC4 gene or the XIAP gene. Also, AB2 Bio has just completed a Phase II clinical trial in Adult onset Still's disease and is currently analysing the clinical data. Please find further information on www.ab2bio.com.

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