AB2 Bio expands Phase 3 trial of Tadekinig alfa in monogenic HLH

Lausanne (Switzerland), August 25, 2021 — AB2 Bio Ltd, a Swiss advanced clinical-stage biotech company, specialized in developing innovative therapies for the treatment of severe systemic autoinflammatory diseases including rare diseases with high unmet medical needs, announces the expansion of its Phase 3 trial of Tadekinig alfa in the orphan disease IL-18 driven monogenic Hemophagocytic Lymphohistiocytosis (HLH).

Recruitment to the pivotal Phase 3 clinical trial, which aims to assess the efficacy and safety of Tadekinig alfa in patients with monogenic, interleukin-18 driven autoinflammation caused by NLRC4-MAS mutation or XIAP deficiency, has been opened up to adults in addition to children and is currently recruiting in the U.S., Canada and Germany.

Monogenic HLH is a rare, life-threatening disease characterized by hyperinflammation due to an overactivated immune system. There are currently no drugs specifically approved to treat IL-18 driven monogenic HLH and severe inflammatory conditions; disability and death are common outcomes.

Dr. Michael Soldan, CEO of AB2 Bio commented: "We are pleased to expand this Phase 3 trial of Tadekinig alfa to adults and thus offering participation in this trial to a wider patient population. With a manufacturing agreement in place with WuXi Biologics, we are now focused on completing enrolment in the Phase 3 trial and expect results in second half 2022. In parallel, we are preparing to file for marketing authorization in the U.S. and the European Union to bring this product as soon as possible to patients suffering from monogenic HLH associated with elevated IL-18 levels, a devastating condition for which there are currently no approved treatment options."

Previous clinical research indicates that Tadekinig alfa binds free IL-18 and may help manage severe systemic disease manifestation. Eligible patients with either NLRC4-MAS mutation or XIAP deficiency can be identified by a genetic diagnosis. Patients with a XIAP deficiency and persistent disease manifestations after an unsuccessful stem-cell transplantation are also allowed for recruitment. Once enrolled, patients will be administered Tadekinig alfa for 18 weeks (single-arm open-label phase) in addition to the standard of care, followed by 16 weeks of randomized withdrawal.

For more on AB2 Bio's clinical programs, click here.

About Hemophagocytic Lymphohistiocytosis (HLH)

HLH can be either monogenic (with inherited or spontaneous mutations) or multifactorial (secondary to other conditions like rheumatic diseases, cancer or infections).

People with monogenic HLH usually develop symptoms within the first months or years of life. Symptoms may include fever, enlarged liver or spleen, cytopenia (decreased number of blood cells), coagulopathies, and neurological abnormalities. The long-term outlook (prognosis) of monogenic forms without treatment is poor, with a median survival of less than 2 months to 6 months after diagnosis. NLRC4 mutations and XIAP deficiency are part of monogenic HLH. Single point mutations in the NLRC4 gene have been identified which give rise to severe, life-threatening systemic inflammation associated with extremely high levels of IL-18, the therapeutic target of Tadekinig alfa.

About Tadekinig alfa

Tadekinig alfa is a recombinant interleukin-18 binding protein (r-hIL-18BP), that binds with high affinity to IL-18, a major inflammatory mediator, and is administered subcutaneously. The IL-18/IL-18BP balance is disrupted in patients with inflammatory diseases, resulting in high levels of free IL-18 ("active form), which leads to pathological inflammation. Administration of Tadekinig alfa restores the IL-18/IL-18BP balance, by removing excess free IL-18 and thereby reducing inflammation.

Tadekinig alfa is also Phase 3-ready for Still's Disease. It has been granted Orphan Drug Designation by the US Food and Drug Administration (FDA) for the treatment of HLH and Still's disease, including Adult-onset Still's Disease and systemic Juvenile Idiopathic Arthritis, and by the European Medicines Agency (EMA) for the treatment of HLH. In addition, the FDA has been granted Breakthrough Therapy Designation for the treatment of monogenic, IL-18 associated autoinflammatory conditions with ongoing systemic inflammation, and Rare Pediatric Disease Designation for the treatment of primary HLH.

About AB2 Bio

AB2 Bio Ltd (www.ab2bio.com) is a private advanced clinical-stage biotech company located in the Innovation Park at the Ecole Polytechnique Fédérale de Lausanne (EPFL), Switzerland. AB2 Bio is focused on developing best in class innovative therapies, which will not only treat the symptoms but also target the underlying causes of inflammation-based diseases with large unmet medical needs.