ABFERO ANNOUNCES PK STUDY RESULTS FOR SP-420 IN TRANSFUSIONAL IRON OVERLOAD -- PROCEEDS WITH PHASE 1-2 TRIAL OF IRON CHELATOR

Boston, MA, 10/06/2020 – <u>AbFero Pharmaceuticals, Inc.</u>, a privately-held clinical stage pharmaceutical company dedicated to treating diseases of iron overload, today announced it completed analysis of a 12-patient normal volunteer pharmacokinetic (PK) trial of its lead iron chelator, SP-420. The PK data confirmed that the doses of SP-420 planned for the company's upcoming Phase 1-2 trial in patients with transfusional iron overload (TIO) should provide sufficient drug exposure for the desired therapeutic effect.

The Phase 1-2 trial is a 12-month, open-label, dose-escalation design in patients with TIO due to myelodysplastic syndrome (MDS) or myelofibrosis (MFS). The trial design is of sufficient size and duration to establish SP-420 safety and efficacy in reducing liver iron content (LIC) caused by chronic red blood cell transfusions. The trial is expected to provide key data in 2021.

"This is an important mile-marker for SP-420," said AbFero CEO <u>Thomas X. Neenan</u>. "It brings AbFero's lead iron chelator one step closer to the patients who need it the most."

Additionally, AbFero anticipates conducting a Phase 3 pivotal trial in patients with MDS, MFS, β-thalassemia and sickle cell disease using the FDA-approvable endpoint of LIC as measured by MRI.

Kevin H.M. Kuo, MD, University of Toronto hematologist and clinical researcher from University Health Network, who was not involved in the study, commented: "All of the currently approved iron chelators have significant toxicity and side-effect profiles, which are major barriers in establishing good adherence. This represents a promising direction in the treatment of iron overload, which remains an area of largely unmet need."

AbFero believes SP-420 has utility in additional iron-overload conditions, including other rare anemias that require transfusion, non-transfusion dependent thalassemia, and genetic hemochromatosis (in those unwilling to undergo phlebotomy) and may pursue clinical studies in these areas as follow-on indications.

About AbFero

AbFero Pharmaceuticals, Inc. is a privately-held clinical stage pharmaceutical company dedicated to treating diseases of iron overload. Our therapeutic platform addresses transfusional iron overload (TIO) and iron accumulation associated with retinal and neurologic diseases including age-related macular degeneration (AMD), Parkinson's disease, and traumatic brain injury (TBI). AbFero has completed three clinical trials with the company's lead iron chelator, SP-420. AbFero Pharmaceuticals, Inc., is based in Boston, Massachusetts, and our subsidiary, AbFero, Ltd., is located in Harwell, UK. For more information, visit: https://www.abferopharmaceuticals.com.

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