

Sanofi's efdoralprin alfa met all primary and key secondary endpoints in alpha-1 antitrypsin deficiency emphysema phase 2 study

- Efdoralprin alfa demonstrated superiority in a head-to-head study versus a standard of care plasma-derived therapy
- Results reinforce the potential of efdoralprin alfa to be the first restorative recombinant therapy that normalizes and maintains functional AAT levels
- Phase 2 data support both three-week and four-week dosing regimens for efdoralprin alfa - a potentially significant improvement in convenience - compared to a plasma-derived therapy dosed weekly

Paris, October 22, 2025. Positive results from the global ElevAATe phase 2 study (clinical study identifier: [NCT05856331](#)) showed that efdoralprin alfa (SAR447537, formerly known as INBRX-101), met all primary and key secondary endpoints when dosed every three weeks (Q3W) or four weeks (Q4W) in adults with alpha-1 antitrypsin deficiency (AATD) emphysema, a rare disease. Efdoralprin alfa is an investigational recombinant human alpha-1 antitrypsin (AAT)-Fc fusion protein. It demonstrated a statistically significant greater mean increase in functional AAT levels within normal range as measured by trough concentrations at steady state compared to those receiving weekly plasma-derived augmentation therapy at week 32 [$p < 0.0001$]. The study also met key secondary endpoints, demonstrating superior mean increase in fAAT average concentration as well as higher percentage of days above the lower limit of the normal range for both Q3W and Q4W dosing.

The recombinant efdoralprin alfa was well tolerated with a similar adverse event profile to plasma-derived therapy. Additional safety follow-up will be assessed in the ElevAATe OLE phase 2 study (clinical study identifier: [NCT05897424](#)).

*"These data demonstrate that efdoralprin alfa achieved consistently higher normal functional AAT levels, with less frequent dosing, compared to a current standard of care," said **Christopher Corsico**, Global Head of Development at Sanofi. "This is particularly meaningful as maintaining protective protein levels is the cornerstone of pulmonary management of AATD and currently available treatments require weekly therapeutic infusions. The ElevAATe results represent the potential for efdoralprin alfa to be a restorative recombinant therapeutic option for the AATD community, reinforcing our commitment to develop treatments for both rare and respiratory conditions with great unmet medical need."*

*"AATD is a debilitating condition that can be challenging to treat," said **Igor Barjaktarevic**, MD, PhD, Associate Professor, David Geffen School of Medicine at UCLA and primary investigator on the ElevAATe phase 2 study. "Achieving and maintaining normal AAT levels with less frequent dosing and with complete independence from blood donation programs would be a welcome change to the current treatment experience for people living with AATD. With the current standard of care, patients reach but do not maintain normal protein levels between the infusions, leaving a remaining unmet need. I'm encouraged by the ElevAATe trial results and what efdoralprin alfa could mean for the AATD community."*

Efdoralprin alfa was previously granted fast track and orphan drug designation by the US Food and Drug Administration (FDA) for the treatment of AATD emphysema. Efdoralprin alfa is currently under clinical investigation, and its safety and efficacy have not been evaluated by any regulatory authority. Sanofi plans to present the data at a forthcoming medical meeting and engage with global regulatory authorities on the appropriate next steps.

About AATD

AATD is a rare, inherited disorder characterized by low levels or absence of AAT, a protein produced by the liver that protects the lungs from inflammation and damage. The disease causes progressive deterioration of the tissue of the lungs and liver. Without adequate AAT levels, affected individuals often experience lung damage and develop COPD, including emphysema, and in severe forms of the disease, patients can sometimes require lung transplantation. Plasma-derived therapies were introduced in 1987 to treat the condition but since then, no new therapies have been introduced. About 235,000 people worldwide live with AATD, with nearly 100,000 people in the US, but about 90% of individuals with AATD are likely undiagnosed.

About efdoralprin alfa

Efdoralprin alfa (SAR447537, formerly known as INBRX-101) is a recombinant human AAT-Fc fusion protein being investigated as a restorative therapy in adults with AATD emphysema, with Q3W or Q4W dosing. The investigational treatment is being studied to restore functional AAT levels to the normal range and inhibit neutrophil elastase, an enzyme that can cause lung tissue damage in patients with AATD. Efdoralprin alfa was granted fast track designation and orphan drug designation by the FDA for the treatment of AATD emphysema.

About ElevAATe

The ElevAATe phase 2 study was a double-blind, randomized study evaluating efdoralprin alfa versus a standard of care plasma-derived augmentation therapy in patients with AATD emphysema. Ninety-seven patients were randomized 2:2:1 to receive efdoralprin alfa every three weeks or every four weeks, or plasma-derived augmentation therapy once weekly. The primary endpoint was the mean change in average fAAT concentrations as measured from baseline to average serum trough fAAT concentrations at steady state in patients treated with efdoralprin alfa Q3W or Q4W compared with weekly plasma-derived augmentation therapy, following a treatment period of up to 32 weeks. Key secondary endpoints included mean change in serum fAAT concentration from baseline to fAAT average concentrations at steady state and percentage of days that steady-state functional AAT levels were above the lower limit of the normal range.

About Sanofi

Sanofi is an R&D driven, AI-powered biopharma company committed to improving people's lives and delivering compelling growth. We apply our deep understanding of the immune system to invent medicines and vaccines that treat and protect millions of people around the world, with an innovative pipeline that could benefit millions more. Our team is guided by one purpose: we chase the miracles of science to improve people's lives; this inspires us to drive progress and deliver positive impact for our people and the communities we serve, by addressing the most urgent healthcare, environmental, and societal challenges of our time.

Sanofi is listed on Euronext: SAN and NASDAQ: SNY

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