

ATS: phase 2 data demonstrate the superiority of efdoralprin alfa over a standard-of-care augmentation therapy in achieving higher fAAT levels in AATD

- Data from the head-to-head study show efdoralprin alfa normalized and maintained functional AAT levels in people living with alpha-1 antitrypsin deficiency
- Efdoralprin alfa is a recombinant protein distinct from plasma-derived therapy, which has been the standard-of-care for nearly 40 years

Paris, May 18, 2026. Data from the global ElevAATe phase 2 study (clinical study identifier: [NCT05856331](#)) demonstrated superiority of investigational efdoralprin alfa over standard-of-care therapy in achieving and maintaining normalized functional alpha-1 antitrypsin (fAAT) levels in adult patients with alpha-1 antitrypsin deficiency (AATD)-related emphysema. These results are being presented today at the 2026 American Thoracic Society (ATS) International Conference in Orlando, FL, US.

Efdoralprin alfa, dosed every three weeks (Q3W), achieved mean increases in fAAT trough levels more than three times greater than plasma-derived protein (pdAAT) dosed weekly (Q1W), meeting the primary endpoint ($p < 0.0001$). All key secondary endpoints in the study were also met ($p < 0.0001$), highlighting the potential for efdoralprin alfa to be the first therapy to sustain normal fAAT levels for patients and do so with less frequent dosing. In patients dosed Q3W, fAAT levels remained above the normal threshold (23.8 μM) for 100% of days during the 32-week study compared to 41% of days in patients on a standard-of-care augmentation therapy.

AATD is an underdiagnosed, rare genetic condition that can cause considerable respiratory illness and is characterized by low levels or absence of AAT, a protein designed to protect the lungs from damaging inflammation. Without adequate fAAT levels, patients often experience progressive deterioration of the lung tissue and may develop emphysema, the most common form of chronic obstructive pulmonary disease (COPD), accounting for up to 72% of deaths in people with AATD. An estimated 90% of individuals with AATD are believed to be undiagnosed.

*"AATD presents a persistent clinical challenge. Widespread lack of awareness of the condition as a genetic cause of some forms of COPD leaves many patients under-served. Without treatment to address the underlying AAT protein deficiency, these patients are unable to maintain protective protein levels and become vulnerable to progressive lung disease," said **Igor Barjaktarevic**, MD, PhD, Associate Professor, David Geffen School of Medicine at UCLA, Los Angeles, CA, US and principal investigator on the ElevAATe phase 2 study. "The ElevAATE data suggest efdoralprin alfa, through its mechanism of action, may be able to restore normal AAT levels and keep patients in that range for longer than the standard-of-care therapy, helping to address an unmet need of this disease with a restorative recombinant approach."*

The ElevAATe phase 2 study

The ElevAATe phase 2 study was a double-blind, randomized study evaluating efdoralprin alfa versus a standard-of-care augmentation therapy in patients with AATD-related emphysema. Ninety-seven patients were randomized 2:2:1 to receive efdoralprin alfa every three or four weeks, or plasma-derived augmentation therapy once weekly.

The following results are being shared today:

	Efdoralprin alfa Q3W	Efdoralprin alfa Q4W	Plasma-derived augmentation therapy Q1W
Primary endpoint			
Mean change in average serum fAAT trough concentrations from baseline to steady state at week 32*	24.1µM [22.8µM, 25.3µM] p<0.0001	16.8µM [15.5µM, 18.1µM] p<0.0001	7.6µM [6.0µM, 9.3µM]
Key secondary endpoints			
Mean change in serum fAAT average concentrations from baseline to steady state at week 32*	32.9µM [31.7µM, 34.2µM] p<0.0001	26.0µM [24.6µM, 27.3µM] p<0.0001	17.9µM [16.2µM, 19.6µM]
Percentage of days that steady state fAAT levels were above the lower limit of the normal range at week 32**	100% p<0.0001	89.3% p<0.0001	40.8%

*Least square mean change; [95% confidence interval].

** Normal range for functional AAT = 23.8-42.4 uM.

Q4W: every four weeks.

Efdoralprin alfa was well tolerated with a safety profile comparable to pdAAT, with no participants experiencing treatment-emergent adverse events (TEAEs) leading to permanent discontinuation of study intervention. The most common TEAEs in the efdoralprin alfa Q3W, efdoralprin alfa Q4W and pdAAT therapy arms were COPD exacerbations (34.1%, 42.1% and 44.4%, respectively), headache (19.5%, 13.2% and 11.1%, respectively), and COVID-19 infection (17.1%, 2.6% and 16.7%, respectively). Notably, the incidence of grade ≥ 2 COPD exacerbations, which were captured as an adverse event of special interest in the ElevAATe study, were numerically lower for the efdoralprin alfa Q3W arm (26.8%) versus the efdoralprin alfa Q4W (42.1%) and pdAAT (44.4%) arms. Efdoralprin alfa anti-drug antibodies were detected in two participants and were transient and non-neutralizing.

*"The data demonstrate efdoralprin alfa, a restorative recombinant therapy designed to achieve a longer half-life than standard of care augmentation therapy, has the potential to raise and sustain fAAT levels within normal range with less frequent dosing. This could represent an important advancement in the treatment of AATD-related emphysema, offering patients new hope in a disease state where innovation has been limited over the past 40 years," said **Christopher Corsico**, Global Head of Development at Sanofi.*

Sanofi is engaging with global regulatory authorities on the appropriate next steps for efdoralprin alfa. Efdoralprin alfa was granted fast track designation and orphan drug designation in the US and orphan designation in the EU. Additional long-term safety and efficacy outcomes are being evaluated in the ElevAATe OLE phase 2 study (clinical study identifier: [NCT05897424](#)).**Error! Bookmark not defined.**

Efdoralprin alfa is currently in clinical development, and its safety and efficacy have not been evaluated by any regulatory authority.

About efdoralprin alfa

Efdoralprin alfa is a recombinant human AAT-Fc fusion protein being investigated as a restorative therapy in adults with AATD emphysema, with Q3W or Q4W dosing. This investigational treatment, designed to achieve a longer half-life than plasma-derived augmentation therapy, is

being studied to restore and maintain fAAT levels to the normal range and inhibit neutrophil elastase and other proteases that can cause lung tissue damage in patients with AATD.

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 treatment approaches have become available to patients. 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 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.

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