# FDA grants priority review to Roche's Esbriet (pirfenidone) for unclassifiable interstitial lung disease

Basel, 21 January 2021 - Roche (SIX: RO, ROG; OTCQX: RHHBY) announced today that the US Food and Drug Administration (FDA) has accepted the company's supplemental New Drug Application (sNDA) and granted Priority Review for Esbriet® (pirfenidone) for the treatment of unclassifiable interstitial lung disease (UILD). The FDA is expected to make a decision on approval by May 2021.

"Since its US approval, Esbriet has become a standard of care for people living with idiopathic pulmonary fibrosis. However, significant unmet need remains in fibrotic lung diseases, including unclassifiable interstitial lung disease (UILD)," said Levi Garraway, M.D., Ph.D., Roche's Chief Medical Officer and Head of Global Product Development. "We are working closely with the FDA in hopes of offering Esbriet to people with UILD, a rare and debilitating disease."

The sNDA is based on results from a pivotal, 24-week Phase II trial, which was the first randomised controlled study specifically designed and conducted solely in people with UILD.[1] The data were presented as a late-breaking abstract at the 2019 European Respiratory Society's annual meeting and simultaneously published in *The Lancet Respiratory Medicine*.[2]

In 2020, the FDA granted Orphan Drug Designation and Breakthrough Therapy Designation to Esbriet for UILD.

**About Unclassifiable Interstitial Lung Disease**
Interstitial lung disease (ILD) broadly describes a diverse group of more than 200 types of rare pulmonary diseases. ILDs share similar features, including cough and shortness of breath. However, each ILD has different causes, treatment approaches, and outlooks.[3] Approximately 1 in 10 people living with ILD cannot be given a definitive diagnosis, even after a thorough investigation, and in these cases, they are categorised as having unclassifiable interstitial lung disease (UILD).[4,5]

**About the Pivotal Study**[1]
This international, multicentre, double-blind, randomised, placebo-controlled Phase II trial at 70 centres included patients (aged ≥18-85 years) with progressive fibrosing UILD, a percent predicted forced vital capacity (FVC) of 45 percent or higher and percent predicted carbon monoxide diffusing capacity (DLco) of 30 percent or higher, more than 10 percent fibrosis on high-resolution CT, and a high-resolution CT from the previous 12 months.

The primary endpoint was mean change in FVC from baseline over 24 weeks, measured by daily home spirometry. Key secondary endpoints included change in FVC measured by site spirometry; proportion of patients who had a more than 5 percent or more than 10 percent absolute or relative decline in percent

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predicted FVC measured by clinic-based spirometry; change in percent predicted DLco; change in 6-minute walk distance (6MWD); and several patient reported outcomes; all of which were compared with baseline.

The planned statistical model to estimate mean FVC decline could not be applied due to a number of physiologically implausible outlier results and short observation time for a few patients. Over 24 weeks, predicted median change in FVC measured by home spirometry was -87.7 mL (Q1-Q3 -338.1 to 148.6) in the Esbriet group versus -157.1 mL (–370.9 to 70.1) in the placebo group. Over 24 weeks, predicted mean change in FVC measured by site spirometry was lower in patients given Esbriet than placebo (treatment difference 95.3 mL, p=0.002). Results for DLco and 6MWD generally trended in favour of Esbriet treatment. Adverse event reporting reflected the known safety profile of Esbriet. The most common treatment-related and treatment-emergent adverse events in the Phase II UILD study were gastrointestinal disorders (47% in the Esbriet group vs. 26% in the placebo group), photosensitivity (8% vs. 2%), rash (10% vs. 7%), dizziness 8% vs. 3%), weight decreased (8% vs. 1%) and fatigue (13% vs. 10%). Esbriet treatment was associated with less loss to lung function and exercise capacity compared with placebo over 24 weeks. The results of this study suggest that patients with UILD may benefit from Esbriet therapy.

**About Esbriet**
Esbriet is an oral medicine approved for the treatment of idiopathic pulmonary fibrosis (IPF) and is available in more than 60 countries worldwide. Esbriet was FDA-approved for use in people with IPF in October 2014.[6]

**Esbriet U.S. Indication**
Esbriet is a prescription medicine used to treat people with a lung disease called idiopathic pulmonary fibrosis (IPF).

It is not known if Esbriet is safe and effective in children.

**About Roche**
Roche is a global pioneer in pharmaceuticals and diagnostics focused on advancing science to improve people's lives. The combined strengths of pharmaceuticals and diagnostics under one roof have made Roche the leader in personalised healthcare – a strategy that aims to fit the right treatment to each patient in the best way possible.

Roche is the world's largest biotech company, with truly differentiated medicines in oncology, immunology, infectious diseases, ophthalmology and diseases of the central nervous system. Roche is also the world leader in in vitro diagnostics and tissue-based cancer diagnostics, and a frontrunner in diabetes management.

Founded in 1896, Roche continues to search for better ways to prevent, diagnose and treat diseases and make a sustainable contribution to society. The company also aims to improve patient access to medical innovations by working with all relevant stakeholders. More than thirty medicines developed by Roche are included in the World Health Organization Model Lists of Essential Medicines, among them life-saving antibiotics, antimalarials and cancer medicines. Moreover, for the twelfth consecutive year, Roche has been recognised as one of the most sustainable companies in the Pharmaceuticals Industry by the Dow Jones

Sustainability Indices (DJSI).

The Roche Group, headquartered in Basel, Switzerland, is active in over 100 countries and in 2019 employed about 98,000 people worldwide. In 2019, Roche invested CHF 11.7 billion in R&D and posted sales of CHF 61.5 billion. Genentech, in the United States, is a wholly owned member of the Roche Group. Roche is the majority shareholder in Chugai Pharmaceutical, Japan. For more information, please visit www.roche.com.



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