NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Pirfenidone for treating idiopathic pulmonary fibrosis (review of TA282)

Final scope

Remit/appraisal objective

To appraise the clinical and cost effectiveness of pirfenidone within its marketing authorisation for treating idiopathic pulmonary fibrosis.

Background

Idiopathic pulmonary fibrosis (IPF) is a chronic, progressive lung disease in which scarring (fibrosis) occurs. The cause of IPF is unknown although it is thought to be related to an abnormal immune response. It is a difficult disease to diagnose and requires a multidisciplinary team. Most people with IPF experience symptoms of breathlessness, which may initially be only on exertion. Cough, with or without sputum, is a common symptom. Over time, these symptoms are associated with a decline in lung function, reduced quality of life and death.

The rate of disease progression can vary greatly in people with IPF. The median survival for people with IPF in the UK is approximately 3 years from the time of diagnosis, but is generally longer for people with mild-to-moderate IPF. Prognosis is difficult to estimate at the time of diagnosis and may only become apparent after a period of careful follow-up. Although there are challenges in assessing the severity of the condition, it is widely accepted that severe idiopathic pulmonary fibrosis is defined as forced vital capacity (FVC) less than 50% predicted and a diffusing capacity for carbon monoxide less than 35% predicted. Therefore, mild-to-moderate idiopathic pulmonary fibrosis could be assumed to include a FVC greater than or equal to 50% predicted and a diffusing capacity for carbon monoxide greater than or equal to 35%.

The incidence of IPF is approximately 8 to 9 per 100,000 person-years, which equates to more than 5000 new diagnoses each year in the UK. The incidence is higher in men than women, and increases with age (median age of presentation is 70 years).

The aim of treatment is to manage the symptoms and slow progression. NICE clinical guideline 163 on the diagnosis and management of suspected idiopathic pulmonary fibrosis recommends that best supportive care (including symptom relief, managing co-morbidities, withdrawing therapies suspected to be ineffective or causing harm, and end of life care) should be offered to people from diagnosis and be tailored according to disease severity, rate of progression and the person's preference. If pharmacological treatment is considered appropriate, NICE technology appraisal guidance 282 recommends pirfenidone if a person's forced vital capacity (FVC) is between

50% and 80% of their expected value. Preliminary guidance from an ongoing NICE technology appraisal recommends nintedanib as an option for treating idiopathic pulmonary fibrosis, in people with a percent predicted FVC of 50–80%. Both technology appraisals recommend that treatment should be stopped if there is evidence of disease progression (a decline in per cent predicted FVC of 10% or more within any 12 month period). Lung transplantation is an option if there are no contraindications.

NICE is reviewing the technology appraisal guidance 282 following publication of the ASCEND study, which showed that people with a predicted FVC greater than 80% could potentially benefit from treatment with pirfenidone.

The technology

Pirfenidone (Esbriet, Roche) is an oral immunosuppressant that is thought to have anti-inflammatory and antifibrotic effects. Pirfenidone has a marketing authorisation in the UK for treating mild to moderate IPF in adults.

Intervention(s)	Pirfenidone
Population(s)	Adults with mild to moderate idiopathic pulmonary fibrosis
Comparators	• Best supportive care • Nintedanib (only for people with a percent predicted FVC of 50–80%, subject to ongoing NICE appraisal)
Outcomes	The outcome measures to be considered include: • pulmonary function parameters • physical function • exacerbation rate • progression-free survival • mortality • adverse effects of treatment • health-related quality of life.

Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective. The availability of any patient access schemes for the intervention or comparator technologies should be taken into account.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. If evidence allows, subgroup analysis by disease severity, defined by FVC (such as above and below or 80% FVC) and/or diffusing capacity for carbon monoxide, will be considered.
Related NICE recommendations and NICE Pathways	**Related Technology Appraisals**: Technology Appraisal No. 282, April 2013, 'Pirfenidone for treating idiopathic pulmonary fibrosis'. Technology Appraisal in preparation, 'Nintedanib for treating idiopathic pulmonary fibrosis [ID752]', Anticipated publication date: January 2016. **Related Guidelines:** Clinical Guideline No.163, July 2013, 'Idiopathic pulmonary fibrosis: The diagnosis and management of suspected idiopathic pulmonary fibrosis'. Review Proposal Date June 2015. **Related Quality Standards:** Quality Standard No. 79, January 2015, 'Idiopathic pulmonary fibrosis'. http://www.nice.org.uk/guidance/qs79 **Related NICE Pathways:** NICE pathway: Idiopathic pulmonary fibrosis, Pathway created June 2013. http://pathways.nice.org.uk/pathways/idiopathic-pulmonary-fibrosis

Related National Policy	National Service Frameworks: Older People Department of Health, November 2013, 'NHS Outcomes Framework 2014-2015'.