7.20 PIRFENIDONE 267 mg capsule, 270 Esbriet®, Roche Products Pty Ltd

1 Purpose of Application

1.1 The minor re-submission requested a Section 100 or Section 85, Authority Required listing for pirfenidone for the treatment of idiopathic pulmonary fibrosis.

1.2 This was a re-submission which sought to address the following concerns raised by the PBAC for the November 2015 submission: (1) whether the inclusion of a stopping rule would improve the cost-effectiveness of pirfendione; (2) the appropriateness of the overall survival (OS) extrapolation associated with best supportive care (BSC); (3) the appropriateness of the time horizon for the economic evaluation; (4) the appropriateness of the utility weight applied for the progression health state; and (5) the appropriateness of incorporating an assumed price reduction after the expiry of the five-year data exclusivity period into the economic model and financial estimates.

2 Requested listing

2.1 No requested listing was provided as part of the minor re-submission. It was assumed the sponsor is requesting the same restriction as that from November 2015.

2.2 The Secretariat suggested restriction is below. Additions are italicised and deletions are in strikethrough.

Name, Restriction, Manner of administration and form	Max. Qty	№.of Rpts	Dispensed Price for Max. Qty	Proprietary Name and Manufacturer
PIRFENIDONE Capsule 267 mg, 270	1	5	Public hospital: $[redacted] (effective price: $[redacted]) Private hospital: $[redacted] (effective price: $[redacted])	Esbriet® RO

Category / Program	~~Section 100 – Highly Specialised Drugs Program~~ GENERAL – General Schedule (Code GE)
Prescriber type:	☐Dental ☒Medical Practitioners ☐Nurse practitioners ☐Optometrists ☐Midwives
Episodicity:	-
Severity:	-
Condition:	Idiopathic pulmonary fibrosis
PBS Indication:	Idiopathic pulmonary fibrosis
Treatment phase:	Initial treatment
Restriction Level / Method:	☐Restricted benefit ☒Authority Required - In Writing ☐Authority Required - Telephone ☐Authority Required – Emergency ☐Authority Required - Electronic ☐Streamlined
Treatment criteria:	*Patient must be treated by a respiratory physician or specialist physician experienced in the management of patients with idiopathic pulmonary fibrosis.*
Clinical criteria: *(To be finalised)*	*Patient must have confirmed diagnosis of idiopathic pulmonary fibrosis;* *AND* *Patient must have chest high resolution computed tomography (HRCT) with surgical lung biopsy consistent with the diagnosis of idiopathic pulmonary fibrosis; OR* *Patient must have chest high resolution computed tomography (HRCT) without surgical lung biopsy consistent with the diagnosis of idiopathic pulmonary fibrosis* *AND* *Patient must have percent predicted Forced Vital Capacity (FVC) equal or greater than 50%* *AND* *Patient must have percent predicted carbon monoxide diffusing capacity (DL_{CO}) equal to or greater than 30%.*
Population criteria:	*Patient must be aged 40 years or older.*
Prescriber Instruction 1 *(To be finalised)* **Prescriber Instruction 2**	*Consultation with a multidisciplinary team is necessary in the diagnosis of idiopathic pulmonary fibrosis (IPF). The multidisciplinary team may comprise of at least a pulmonologist, radiologist and where required, pathologist.* *Authority applications for initial treatment must be made in writing and must include:* *(a) a completed authority prescription form; and* *(b) a completed IPF Initial PBS authority application form which includes:* *(i) a copy of the high-resolution computed tomographic scan with or without surgical lung biopsy results confirming the diagnosis of IPF* *(ii) a copy of the respiratory function test results showing Forced Vital Capacity (FVC) equal to or greater than 50%* *AND* *(iii) a copy of the percent predicted carbon monoxide diffusing capacity (DL_{CO}) equal or greater than 30%.*

Administrative Advice 1	*No applications for increased maximum quantities will be authorised.* *No applications for increased repeats will be authorised.*
Administrative Advice 2	*Any queries concerning the arrangements to prescribe may be directed to the Department of Human Services on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday).* *Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Department of Human Services website at www.humanservices.gov.au* *Applications for authority to prescribe should be forwarded to:* *Department of Human Services* *Prior Written Approval of Complex Drugs* *Reply Paid 9826* *HOBART TAS 7001*

Category / Program	~~*Section 100 – Highly Specialised Drugs Program (Community Access)*~~ GENERAL – General Schedule (Code GE)
Prescriber type:	☐Dental ☒Medical Practitioners ☐Nurse practitioners ☐Optometrists ☐Midwives
Episodicity:	-
Severity:	-
Condition:	Idiopathic pulmonary fibrosis
PBS Indication:	Idiopathic pulmonary fibrosis
Treatment phase:	Continuing treatment
Restriction Level / Method:	☐Restricted benefit ☒Authority Required - In Writing ☒Authority Required - Telephone ☐Authority Required – Emergency ☐Authority Required - Electronic ☐Streamlined
Treatment criteria:	*Must be treated by a respiratory physician or specialist physician experienced in the management of patients with idiopathic pulmonary fibrosis.*
Clinical criteria: *(To be finalised)*	*Patient must have previously received PBS subsidised treatment with this drug.*
Population criteria:	*Patient must be aged 40 years or older.*
Administrative Advice 1	*No applications for increased maximum quantities will be authorised.* *No applications for increased repeats will be authorised.*
Administrative Advice 2	*Any queries concerning the arrangements to prescribe may be directed to the Department of Human Services on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday).* *Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Department of Human Services website at www.humanservices.gov.au* *Applications for authority to prescribe should be forwarded to:* *Department of Human Services* *Prior Written Approval of Complex Drugs* *Reply Paid 9826* *HOBART TAS 7001*

Category / Program	~~*Section 100 – Highly Specialised Drugs Program (Community Access)*~~ GENERAL – General Schedule (Code GE)
Prescriber type:	☐Dental ☒Medical Practitioners ☐Nurse practitioners ☐Optometrists ☐Midwives
Episodicity:	-
Severity:	-
Condition:	Idiopathic pulmonary fibrosis
PBS Indication:	Idiopathic pulmonary fibrosis
Treatment phase:	*Initial PBS-subsidised treatment* (grandfather patient)
Restriction Level / Method:	☐Restricted benefit ☒Authority Required - In Writing ☒Authority Required - Telephone ☐Authority Required – Emergency ☐Authority Required - Electronic ☐Streamlined
Treatment criteria:	*Must be treated by a respiratory physician or specialist physician experienced in the management of patients with idiopathic pulmonary fibrosis.*
Clinical criteria *(To be finalised)*	*Patient must have received non-PBS subsidised treatment with this drug prior to [listing date]*
Population criteria:	Patient must be aged 40 years or older.
Administrative Advice 1 **Administrative Advice 2**	*No applications for increased maximum quantities will be authorised.* *No applications for increased repeats will be authorised.* *Any queries concerning the arrangements to prescribe may be directed to the Department of Human Services on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. EST Monday to Friday).* *Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Department of Human Services website at www.humanservices.gov.au* *Applications for authority to prescribe should be forwarded to:* *Department of Human Services* *Prior Written Approval of Complex Drugs* *Reply Paid 9826* *HOBART TAS 7001*

2.3 The Pre-PBAC response accepted the Secretariat suggested restriction with the following change to prescriber instruction 2 (suggested additions are bolded and deletions are in strikethrough).

Authority applications for initial treatment must be made in writing and must include:
(a) a completed authority prescription form; and
(b) a completed IPF Initial PBS authority application form which includes:
*(i) a copy of the high-resolution computed tomographic scan **results** with or without surgical lung biopsy results confirming the diagnosis of IPF*
(ii) a copy of the respiratory function test results showing Forced Vital Capacity (FVC) equal to or greater than 50% AND
(iii) percent predicted carbon monoxide diffusing capacity (DLCO) equal to or greater than 30%.

2.4 In November 2015, the PBAC advised that a future resubmission should explore the cost effectiveness of a stopping rule in restricting use of pirfenidone to patients likely to receive the most benefit. The re-submission states that the proposed [redacted]% rebate

would improve the cost-effectiveness of pirfenidone, and thus a stopping rule would not be required. Therefore, no stopping rule has been included in the requested restriction.

For more detail on PBAC's view, see section 5 "PBAC outcome"

3 Background and current submission

3.1 Pirfenidone sought TGA approval under TGA-PBAC parallel process. It was approved for registration on 25 February 2016 with the indication of idiopathic pulmonary fibrosis (IPF).

3.2 The Pre-PBAC response noted the approved indication was in line with requested PBS indication.

3.3 Pirfenidone was previously considered by the PBAC in November 2015. The following table provides a summary of the previous submission and the current re-submission.

Table 1: Summary of the previous submission and current re-submission

Component	Pirfenidone November 2015 submission	Current re-submission
Requested PBS listing	Idiopathic pulmonary fibrosis, where the patient must have confirmed diagnosis of IPF as assessed by a multidisciplinary team, a predicted Forced vital capacity (FVC)) ≥50%, and a predicted carbon monoxide diffusing capacity (DLCO) ≥ 30%. **PBAC Comment (5.12 PBAC Public Summary Document (PSD) November 2015, pirfenidone, paragraph 7.4):** "The PBAC agreed that a one-off requirement for diagnosis by the multidisciplinary team for initiating therapy would be appropriate." **PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 7.5):** "PBAC considered a future resubmission should explore the likelihood of such a [stopping] rule proving effective by exploring the numbers of patients who may discontinue treatment based on the clinical trial evidence and the proposed model."	Although not explicitly stated, the Secretariat assumed this to be unchanged from the November 2015 submission.
Requested price	Ex-manufacturer price: $[redacted] Proposed rebate on ex-manufacturer price: [redacted]% Effective ex-manufacturer price: $[redacted] S100 HSD fees and mark-ups: $0 (public), $46.93 (private) Split public/private setting: 79.9% (public), 20.1% (private) Weighted DPMQ/pack: $[redacted]	Ex-manufacturer price: $[redacted] Proposed rebate on ex-manufacturer price: [redacted]% Effective ex-manufacturer price: $[redacted] S100 HSD fees and mark-ups: unchanged from November 2015 Split public/private setting: Unchanged from November 2015 Weighted DPMQ/pack: $[redacted]
Main comparator	Best supportive care. **PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 7.6):** "The PBAC confirmed that best supportive care was the	Unchanged from November 2015 submission.

Component	Pirfenidone November 2015 submission	Current re-submission
	appropriate main comparator for pirfenidone for IPF. Another novel agent for the treatment of IPF, nintedanib, was considered at the same November 2015 PBAC meeting and although not currently PBS listed, nintedanib was considered to be a relevant secondary comparator. The PBAC noted its concern that, should both drugs become PBS subsidised in the future, the risk of concomitant use would need to be addressed."	
Clinical evidence	3 trials: ASCEND (n=555), CAPACITY-2 (n=435) and CAPACITY-1 (n=344). **PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 7.7):** "The PBAC noted the submission presented three head-to-head trials comparing pirfenidone to placebo: ASCEND (n=555), CAPACITY-2 (n=435) and CAPACITY-1 (n=344). The Pre-PBAC response (p1) reiterated that the pooled analysis of mortality from ASCEND, CAPACITY-2 and CAPACITY-1 was pre-specified in the statistical analysis plan for ASCEND which was finalised before unblinding."	The re-submission presented additional supportive data from Nathan 2015. *The submission claimed this evidence supported the assertion that patients continue to benefit from pirfenidone treatment following an FVC decline ≥10%.*
Key effectiveness data	**PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 7.8 and 7.11):** On the basis of direct evidence in comparison to placebo presented by the submission, pirfenidone was associated with: • Approximately a [redacted]% reduction in absolute change in FVC%Pred from baseline to week 52. • No significant difference for overall survival, as reported in the vital status-end of study analysis, which was considered by the FDA cross discipline team leader review for pirfenidone to be most representative of the efficacy of a drug in terms of disease modification/survival. Interpretation of the indirect comparison with nintedanib is difficult given the differences in the trial populations and the outcomes. The network meta-analysis from Loveman et al 2015 suggests a trend to better overall survival for pirfenidone (OR = 1.39, 95% CI: 0.70, 2.82), but a superior benefit in slowing FVC decline for nintedanib (OR = 0.67, 95% CI: 0.51, 0.88) and a trend to better prevention of exacerbations with nintedanib (no OR provided but only nintedanib had a superior result to placebo). However, PBAC considered both drugs are likely to be similarly clinically effective.	Unchanged from November 2015 submission.
Key safety data	The incidence of photosensitivity reaction, rash, stomach discomfort, dyspepsia, dysgeusia, nausea, anorexia, decreased appetite, weight decrease, asthenia, insomnia, dizziness, gastro-oesophageal reflux disease, fatigue and diarrhoea were significant higher in pirfenidone treated patients. Incidence of IPF and peripheral oedema was significantly lower in the pirfenidone treatment group when compared to placebo.	Unchanged from November 2015 submission.

Component	Pirfenidone November 2015 submission	Current re-submission
	PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 7.10): "PBAC also noted pirfenidone was associated with statistically significantly higher instances of adverse events in several items across system organ classes. The highest relative differences were observed in the skin and subcutaneous tissue disorders (photosensitivity reaction, rash) and gastrointestinal disorders (stomach discomfort, dyspepsia). Also a significant increase was observed in gastro-oesophageal reflux disease, a known co-morbidity of IPF. A statistically significant reduction in peripheral oedema as observed."	
Clinical claim	Superior efficacy and acceptable safety profile compared with placebo. **PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 6.25-6):** The ESC considered that pirfenidone is superior in efficacy to placebo and has an inferior safety profile. The PBAC agreed with the ESC's conclusion.	Although not explicitly stated, it appears to be unchanged from November 2015 submission.
Economic evaluation	•Cost-utility model with cost/QALY $75,000 - $105,000 (this included the submission's anticipated [redacted]% price reduction after 5 years due to generic competition). **PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 7.18):** "Overall, the PBAC did not consider that the model could be relied upon as a basis for considering the cost-effectiveness of pirfenidone. It recommended that the base case of a modelled economic evaluation in any re-submission should: •omit potential price reductions associated with any multi-brand competition •examine the consequences of including a continuation rule reflecting or modified from that proposed in other countries •reduce the time horizon to 10 years •revise the extrapolation methods and the post-progression utility so that they no longer overestimate the incremental QALYs gained.	•Cost-utility model with cost/QALY $45,000 - $75,000 (10 year time horizon); $45,000 - $75,000 (16 year time horizon). *The minor re-submission respecified the base case ICER through the following:* *•removing the assumed price reduction following expiry of data exclusivity;* *•increasing the proposed rebate from [redacted]% to [redacted]%; and* *•providing the option of a 10 year or 16 year time horizon (the previous model only included the 10 year time horizon).*
Number of patients	Less than 10,000 in Year 1 increasing to less than 10,000 in Year 5.	Unchanged from November 2015 submission.
Estimated cost to PBS	$30 – $60 million in Year 1 increasing to more than $100 million in Year 5.	$30 - $60 million in Year 1 increasing to $60 - $100 million in Year 5.
PBAC decision	Reject **PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 7.1):** "The PBAC decided not to recommend pirfenidone for PBS listing for idiopathic pulmonary fibrosis (IPF) on the basis of unacceptably high and uncertain cost-effectiveness."	-

Source: Compiled during the minor overview

For more detail on PBAC's view, see section 5 "PBAC outcome"

4 Consideration of the evidence

Sponsor hearing

4.1 There was no hearing for this item as it was a minor submission.

Consumer comments

4.2 The PBAC noted and welcomed the input from individuals (1) and organisations (1) via the Consumer Comments facility on the PBS website. The comments noted that there are only two medicines for idiopathic pulmonary fibrosis, pirfenidone and nintedanib; for the patients who cannot tolerate nintedanib, pirfenidone is the only option available.

Clinical trials

4.3 As a minor submission, no new clinical trials were presented in the minor re-submission.

4.4 The minor submission addressed the matters of concern to the PBAC as per the following table.

Table 2: Outstanding matters of concern to the PBAC

Matters of concern	How the minor re-submission addresses it
Inclusion of a continuation rule **PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 7.5):**"PBAC considered a future re-submission should explore the likelihood of such a rule proving effective by exploring the numbers of patients who may discontinue treatment based on the clinical trial evidence and the proposed model."	The minor re-submission asserts that the application of any stopping rule for IPF patients treated with pirfenidone is not evidenced based, and that the stopping criteria is unjustifiable as progression with treatment does not constitute treatment failure. The minor re-submission references the Nathan (2015) data which examines outcomes (≥10% decline in FVC or death; death; >0% to <10% decline in FVC; and no further decline in FVC) between patients treated with pirfenidone or placebo following a FVC decline ≥10%. The submission asserts that this data shows that continued pirfenidone treatment significantly reduced the risk of death and increased disease stabilisation compared with placebo patients. *The sponsor has not made any attempt to explore the number of patients who may discontinue treatment based on the clinical trial evidence and the proposed model.*
OS extrapolation associated with BSC **PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 7.16):** "The PBAC was particularly concerned that the model was sensitive to the use of this historical data [Strand] to extrapolate OS in the BSC arm and that the Strand data may have underestimated BSC compared with BSC in the trials, therefore over-estimating the benefit from pirfenidone"	The minor re-submission made reference to patient level data from two different registries enrolling patients with IPF (Lung Fibrosis Clinic at the University of Edinburgh (UK) and the Inova Fairfax Hospital in Virginia (USA)) and compared the patient demographics and disease characteristics from the registries with the Strand data and the pooled BSC arms of the ASCEND and CAPACITY trials . The submission also presented a Kaplan-Meier estimate of the comparison between strand and the two registries.

Matters of concern	How the minor re-submission addresses it
Time horizon for the economic evaluation **PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 7.15):** "The 16 year time horizon was considered optimistic in this patient population, and the PBAC noted…a substantial number of patients remained alive at 10 years".	The minor re-submission disagreed with the PBAC's view that the 16 year time horizon is overly optimistic, stating that [redacted]% of patients were still alive at that point. However, the minor re-submission has presented the 10 year and 16 year time horizons in parallel to enable a comparison between the two.
Progression health state utility weight **PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 7.17):** "The PBAC noted the utility values sourced from the literature appeared reasonable, however, given the age of the proposed eligible PBS population they may overstate the utility associated with progression".	The minor re-submission represented data from Section C of the 2015 Major submission for pirfenidone and reiterated its' claim that the utility value is representative of patients with progressive IPF in the proposed PBS population. The minor-resubmission further stated that the economic evaluation is only somewhat sensitive to the utility weight, and that the sensitivity is reduced further if the time horizon is reduced to the PBAC's preference of 10 years.
Price reduction following expiry of five-year data exclusivity period **PBAC Comment (5.12 PBAC PSD November 2015, pirfenidone, paragraph 7.12):** "The PBAC did not consider it appropriate to estimate the cost-effectiveness of a medicine for initial PBS listing by including potential future price reductions due to generic competition. This would not be consistent with previous considerations of ICERs and would create major additional uncertainty in the consideration of cost-effectiveness".	The minor re-submission has not included any future price reductions due to generic competition. The proposed rebate, however, has been increased from [redacted]% to [redacted]%. *This has substantial impact on the ICER and financial estimates, as explored above.*

Economic analysis

4.5 The November 2015 major submission presented a cost-effectiveness analysis against best supportive care. The minor re-submission recalculated the base case ICER by:

- removing the assumed price reduction following expiry of data exclusivity;
- increasing the proposed rebate from [redacted]% to [redacted]%; and
- providing the option of a 10 year or 16 year time horizon (the previous model only included the 10 year time horizon).

4.6 The re-submission presented a revised ICER of $45,000/QALY – $75,000/QALY using a 10 year time horizon and $45,000/QALY -$75,000/QALY using a 16 year time horizon. The respecified base case ICER was verified by the Secretariat.

Drug cost/patient/year: $[redacted]

4.7 The minor re-submission estimated patients would receive [redacted] scripts per year. Using the weighted DPMQ of $[redacted], the drug cost per patient per year was estimated to be $[redacted]. As no stopping rule has been included, it is likely that patients will receive treatment for the rest of their life following commencement on pirfenidone.

Estimated PBS usage & financial implications

4.8 The minor re-submission's revised financial implications estimated a net cost to the PBS of $60 - $100 million in Year 5 of listing, with a total net cost to the PBS of more than $100 million over the first 5 years of listing. This is summarised in the table below with the expected patient and prescription numbers. Revised costs from the previous November 2015 submission have been shaded in grey.

Table 3: Estimated use and financial implications

	Year 1	Year 2	Year 3	Year 4	Year 5
Incident IPF population					
Patients treated with pirfenidone					
Pirfenidone packs					
Net costs to PBS/RPBS*	$	$	$	$	$
Net costs to MBS	$	$	$	$	$
Net savings to state and territory governments	$	$	$	$	$
Overall net cost to government*	**$**	**$**	**$**	**$**	**$**

Abbreviations: IPF = idiopathic pulmonary fibrosis
*inclusive of proposed % rebate
Source: Updated Financial Cost to PBS Spreadsheet

For more detail on PBAC's view, see section 5 "PBAC outcome"

5 PBAC Outcome

5.1 The PBAC did not recommend the PBS listing of pirfenidone for the treatment of idiopathic pulmonary fibrosis on the basis of unacceptably high cost effectiveness, in the context of total cost and uncertain utilisation.

5.2 The PBAC noted that the minor re-submission did not explore the number of patients who may discontinue treatment based on the clinical trial evidence and the proposed model, stating that progression with treatment does not constitute treatment failure. The minor re-submission argued that, following a FVC decline ≥10% with pirfenidone, continued treatment significantly reduced the risk of death and increased disease stabilisation compared to placebo patients who continued treatment with placebo following a FVC decline ≥10%. Noting this, the PBAC considered that implementing a stopping rule would be difficult in clinical practice.

5.3 The PBAC noted that no new data had been presented in the submission. The PBAC recalled that it had considered pirfenidone to be superior in efficacy to placebo with an inferior safety profile.

5.4 The PBAC recalled its concern from November 2015 that the use of historical data to extrapolate OS in the modelled BSC arm may have underestimated survival compared with the BSC arm in the trials. The PBAC noted the minor re-submission presented Kaplan-Meier curves comparing the survival in two IPF registries with the historical data. The PBAC considered that the survival demonstrated in the historical data was acceptably similar to that of the registries.

5.5 The PBAC noted that the submission applied a % rebate to the monthly cost of

pirfenidone, which had a substantial impact on the cost-effectiveness and financial estimates.

5.6 The PBAC recalled its consideration from November 2015 that a 16 year time horizon would be optimistic, and noted that the minor re-submission evaluated both a 10 and 16 year time horizon within the economic model (including the [redacted]% rebate). The 10 year time horizon yielded an ICER of $45,000/QALY - $75,000/QALY, and the 16 year time horizon yielded an ICER of $45,000/QALY - $75,000/QALY.

5.7 The PBAC recalled its key concerns in November 2015 regarding the derivation of the pirfenidone treated population (potentially higher IPF incidence rate; limiting calculation of prevalent population to IPF patients in the year prior to listing) and hospitalisations are likely to result in underestimated net costs to the government. The evaluation also noted additional factors (application of ABS population projections; potential duplication of deaths in the pirfenidone treatment continuation rates) that were likely to further contribute to this underestimate. The PBAC considered that the minor resubmission did not sufficiently address these issues, and noted the potential for the absence of a stopping rule to increase the utilisation estimates.

5.8 The PBAC considered that the most plausible ICER using the current adjustments to the model was $45,000/QALY - $75,000/QALY. The PBAC considered that the minor resubmission had not adequately established the cost-effectiveness of pirfenidone, particularly in the context of the high total cost and remaining concerns about utilisation. The PBAC considered that the [redacted]% rebate offered in the minor resubmission did not sufficiently address these concerns.

5.9 The PBAC noted that this submission was eligible for an Independent Review.

Outcome:
Rejected

6 Context for Decision

The PBAC helps decide whether and, if so, how medicines should be subsidised in Australia. It considers submissions in this context. A PBAC decision not to recommend listing or not to recommend changing a listing does not represent a final PBAC view about the merits of the medicine. A company can resubmit to the PBAC or seek independent review of the PBAC decision.

7 Sponsor's Comment

Roche is disappointed with this outcome and, given the high unmet need, continues to work with the PBAC to provide access at the earliest opportunity to pirfenidone for patients with idiopathic pulmonary fibrosis.