

5.18 DEFERASIROX

Tablet, film coated, 90 mg, tablet, film coated, 180 mg, tablet, film coated, 360 mg
Jadenu[®],
Novartis

1 Purpose of Application

- 1.1 The minor submission requested the Section 100 – Highly Specialised Drugs Program PBS listing of a new film coated tablet formulation of deferasirox (90 mg, 180 mg, 360 mg) for patients with chronic iron overload due to disorders of haemopoiesis.

2 Requested listing

- 2.1 The submission requested that deferasirox film coated tablets be listed under the same restriction criteria as deferasirox dispersible tablets.
- 2.2 Deferasirox dispersible tablets are PBS listed for initial and continuing treatment for patients with chronic iron overload who:
- are transfusion dependent and do not have a malignant disorder of erythropoiesis, OR
 - are not transfusion dependent and have thalassemia, OR
 - are red blood cell transfusion dependent and have a serum ferritin level of greater than 1,000 micrograms/L; have a malignant disorder of haemopoiesis; and have a median life expectancy exceeding 5 years.
- 2.3 All initial listings are Authority Required (telephone) and the continuing listings are Authority Required (Streamlined).
- 2.4 Initial prescriptions for malignant disorders of haemopoiesis and all continuing prescriptions have a maximum of two repeats. Initial prescriptions for transfusion dependent iron overload and thalassemia have a maximum of five repeats.

Name, Restriction, Manner of administration and form	Max. Qty	No. of Rpts	DPMQ Public/Private	Proprietary Name and Manufacturer
DEFERASIROX				
Film coated tablet 90 mg, 30	180	2 / 5	\$ [REDACTED] / \$ [REDACTED]	Jadenu [®]
Film coated tablet 180 mg, 30	180	2 / 5	\$ [REDACTED] / \$ [REDACTED]	Novartis
Film coated tablet 360 mg, 30	180	2 / 5	\$ [REDACTED] / \$ [REDACTED]	

- 2.5 A Special Pricing Arrangement, in the form of a rebate, is applied to deferasirox dispersible tablets. It was proposed that the same arrangements would apply to the film coated tablets.

- 2.6 The pre-PBAC response requested that patients currently treated with deferasirox dispersible tablets be eligible for continuing treatment with deferasirox film coated tablets.

For more detail on PBAC's view, see section 6 PBAC outcome.

3 Background

- 3.1 This was the first submission for deferasirox film coated tablets.
- 3.2 Deferasirox dispersible tablets were recommended for PBS listing in July 2006 for the treatment of chronic iron overload secondary to transfusion dependent anaemias.
- 3.3 At the July 2015 meeting the PBAC recommended revising the PBS listing for deferasirox dispersible tablets to include the following populations: patients with transfusion dependent non-malignant disorders of erythropoiesis; patients with non-transfusion dependent thalassaemia; and patients with transfusion dependent malignant disorders of haemopoiesis with a median life expectancy greater than five years.

TGA status

- 3.4 Deferasirox film coated tablets were registered by the TGA in March 2018 for:
- “The treatment of chronic iron overload due to blood transfusions (transfusional haemosiderosis) in adults and paediatric patients 6 years and older. Jadenu® is also indicated for the treatment of chronic iron overload in paediatric patients aged 2 to 5 years who are unable to take desferrioxamine therapy or in whom desferrioxamine has proven ineffective. Jadenu® is also indicated for the treatment of chronic iron overload in patients with non-transfusion-dependent thalassemia syndromes aged 10 years and older.”
- 3.5 The TGA indication for the film coated tablets is the same as deferasirox dispersible tablets.
- 3.6 In their consideration of the TGA application for the film coated tablets the TGA Delegate considered that the film coated tablet formulation was biocomparable to the dispersible tablet formulation. The Delegate noted that the film coated tablets had a higher bioavailability than the dispersible tablets; therefore, the formulations could not be considered bioequivalent. Although the area under the curve (AUC) of the film coated tablet was equivalent to that of the dispersible tablet using the reference bioequivalence criteria of 80% to 125%, the peak concentration (C_{max}) of the film coated tablet was 30% higher than that of the dispersible tablet, with the 90% confidence interval (CI) being out of the reference range. The PBAC considered that the film coated tablets were biocomparable to the dispersible tablet formulation.
- 3.7 The TGA Delegate was of the view that the overall benefit/risk balance for the film coated tablet formulation of deferasirox was positive.

3.8 The table below summarises the differences between the current dispersible tablet and the new film coated tablet.

Table 1: Key differences between the deferasirox dispersible tablets and the film coated tablets

	Dispersible tablets	Film coated tablets
Strength	125 mg; 250 mg; 500mg	90 mg; 180 mg; 360 mg
Administration	Once daily on an empty stomach, ≥ 30 minutes before a meal; Dispersed in water or orange or apple juice; Consume suspension, resuspend residue and consume; Do not chew or swallow whole.	Once daily on an empty stomach or with a light meal (< 7% fat; approx. 250 calories); Swallow whole or crush and mix with soft food, e.g. yoghurt or apple sauce.
Starting dose	TDIO: 20 mg/kg/day NTDT: 10 mg/kg/day	TDIO: 14 mg/kg/day NTDT: 7 mg/kg/day
Titration increments	TDIO: 5-10 mg/kg/day NTDT: 5-10 mg/kg/day	TDIO: 3.5-7 mg/kg/day NTDT: 3.5-7 mg/kg/day
Maximum dose	TDIO: 40 mg/kg/day NTDT: 20 mg/kg/day	TDIO: 28 mg/kg/day NTDT: 14 mg/kg/day

Source: Table 1, p6 of the submission

NTDT = non-transfusion-dependent thalassemia; TDIO = transfusion-dependent iron overload

For more detail on PBAC's view, see section 6 PBAC outcome.

4 Comparator

4.1 The minor submission nominated deferasirox dispersible tablets as the comparator. The PBAC considered that the comparator was appropriate.

For more detail on PBAC's view, see section 6 PBAC outcome.

5 Consideration of the evidence

Sponsor hearing

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

Consumer comments

5.2 The PBAC noted and welcomed the input from individuals (6) and health care professionals (2) via the Consumer Comments facility on the PBS website. The comments described a range of benefits of treatment with deferasirox film coated tablets including improved compliance, fewer side effects and increased treatment options.

Pharmacokinetic data

5.3 Pharmacokinetic data was presented from three Phase I studies, for which references were not provided. They are described in the table below.

Table 2: Description of the Phase I pharmacokinetic studies

Study ID	Study description	Study design	Status
F2101	Pilot bioavailability	3 tablet formulations; 1,500 mg dose for dispersible and film coated tablets; N = 20	Completed
F2102	Pivotal pharmacokinetic comparability study with film coated tablets	1,500 mg dispersible tablet versus 1,080 mg film coated tablet, using BE criteria; N = 32	Completed
F2103	Food effect with film coated tablet	Fasting versus low-fat diet versus high-fat diet at 1,200 mg dose; N = 25	Completed

Source: Table 5, p12 of the submission
BE = bioequivalence

- 5.4 The submission claimed that Study F2101 demonstrated that a single film coated 1,500 mg dose of deferasirox had 36% to 38% greater bioavailability than a single dispersible 1,500 mg dose. Subsequent studies adopted strength-adjusted formulations (e.g. 360 mg film coated tablets to match 500 mg dispersible tablets).
- 5.5 The submission claimed that Study F2102 demonstrated comparable bioavailability in terms of AUC when comparing a single film coated 1,080 mg (3 x 360 mg) dose with a single dispersible 1,500 mg (3 x 500 mg) dose. AUC_{last} and AUC_{inf} had geometric mean ratios of 1.00 (90% CI: 0.93, 1.08) and 0.98 (90% CI: 0.92, 1.06) respectively. AUC data met the prospectively defined bioequivalence criteria, with the 90% CIs lying within 80% and 125%. Single film coated 1,080 mg doses yielded peak deferasirox concentrations (C_{max}) that were 30% higher than single dispersible 1,500 mg doses. The geometric mean ratio was 1.30 (90% CI: 1.20, 1.40). This did not meet the bioequivalence criteria.
- 5.6 The TGA Delegate’s Overview agreed that the film coated tablets had a higher bioavailability than the dispersible tablets and that the 360 mg film coated tablet formulation was equivalent to the 500 mg dispersible tablet formulation with respect to the mean AUC.
- 5.7 The TGA evaluator noted that the C_{max} values of the film coated tablet were within the range of historical C_{max} values observed with the dispersible tablet formulation. The submission stated that although the film coated tablets could not be considered bioequivalent to the dispersible tablets as they did not meet the pre-specified C_{max} limits, no evidence of clinically relevant effects of an increase in C_{max} were found in a large exposure-response analysis. This was noted by the PBAC.

Clinical trials

- 5.8 The TGA Delegate noted that “There are no efficacy studies conducted specifically with the film coated tablet formulation. This application cross-refers to the existing efficacy data generated with the dispersible tablet formulation for which the film coated tablet has been demonstrated to be biocomparable”.
- 5.9 The minor submission presented data from the following randomised safety trial.

Table 3: Randomised trial presented in the re-submission

Trial ID	Protocol title/Publication title	Publication citation
Direct randomised trial		
ECLIPSE	A randomized, open-label, multicentre, two-arm, phase II study to investigate the benefits of an improved deferasirox formulation (film-coated tablet) Taher AT, Origa R, Perrotta S, <i>et al.</i> New film-coated tablet formulation of deferasirox is well tolerated in patients with thalassemia or lower-risk MDS: Results of the randomized, phase II ECLIPSE study.	7 July 2016 <i>Am J Hematol.</i> 2017; 92: 420-428.

Source: Table 5, p12 of the submission
MDS = myelodysplastic syndrome

- 5.10 The ECLIPSE trial was a randomised, open-label, Phase II study that evaluated the safety of the film coated and dispersible tablet formulations in patients with either transfusion-dependent thalassemia (TDT) or Revised International Prognostic Scoring System (IPSS-R) very low-, low-, or intermediate-risk myelodysplastic syndrome (MDS).
- 5.11 Iron chelation therapy naïve or pre-treated patients were included in the trial if they were aged 10 years or older, required treatment with deferasirox dispersible tablet doses of ≥ 30 mg/kg/day (TDT) or ≥ 20 mg/kg/day (MDS), had a transfusion history of ≥ 20 mg packed red blood cell units, anticipated transfusion requirements of ≥ 8 units/year during the study and serum ferritin $> 1,000$ ng/mL at screening.
- 5.12 The primary outcome was overall safety, measured by frequency and severity of adverse events and changes in laboratory values from baseline to 24 weeks. Secondary endpoints included gastrointestinal adverse events, treatment compliance, patient satisfaction, palatability, patient reported gastrointestinal outcomes and pharmacokinetics.
- 5.13 The baseline demographics of the 173 trial patients are presented below.

Table 4: Baseline characteristics of ECLIPSE trial patients

	Dispersible tablets	Film coated tablets
N	86	87
Mean age, years (SD)	35.1 (18.6)	34.6 (20.0)
Male, n (%)	39 (45.3%)	46 (52.9%)
Race, n (%):		
Caucasian	61 (70.9%)	62 (71.3%)
Asian	20 (23.3%)	16 (18.4%)
Other	5 (5.8%)	9 (10.3%)
Previous chelation, n (%)	77 (89.5%)	79 (90.8%)
Prior deferasirox, n (%)	68 (79.1%)	71 (81.6%)

Source: Table 6, pp15-16 of the submission
SD = standard deviation

- 5.14 Iron chelation naïve patients received a starting dose of 20 mg/kg/day for the dispersible tablets and 14 mg/kg/day for the film coated tablets, with dose adjustments possible every four weeks. Previously treated patients started on a dispersible or film coated tablet dose equivalent; dose adjustments were possible every three months.

5.15 Patient exposure is summarised in the table below.

Table 5: Patient exposure in the ECLIPSE trial

	Dispersible tablets	Film coated tablets
Exposure:		
Mean, days (SD)	154.5 (44.7)	163.2 (27.8)
Median, days (range)	168.0 (2, 224)	169.0 (30, 239)
Dose:		
Mean, mg/kg/day (SD)	27.5 (7.7)	20.8 (5.4)
Median, mg/kg/day (range)	29.2 (8.5, 40.9)	20.6 (7.2, 29.1)

Source: Table 7, pp16-17 of the submission
SD = standard deviation

5.16 Pharmacokinetic results were consistent with those observed in study F2102, with overall exposure of the film coated tablet comparable to the dispersible tablet (as measured by AUC) and higher absorption for the film coated tablet (as measured by C_{max}).

5.17 No efficacy data for the deferasirox film coated tablet were presented in the submission.

Comparative harms

5.18 A summary of adverse events and the most commonly reported adverse events from the ECLIPSE trial are presented below.

Table 6: Summary of adverse events from the ECLIPSE trial

	Dispersible tablets (N = 86)	Film coated tablets (N = 87)
AEs	77 (89.5%)	78 (89.7%)
SAEs	13 (15.1%)	16 (18.4%)
AEs leading to study drug discontinuation	6 (7.0%)	5 (5.7%)
AEs requiring dose adjustment or/and interruption	40 (46.5%)	32 (36.8%)
AEs requiring additional therapy	51 (59.3%)	52 (59.8%)
Adverse events of special interest	32 (37.2%)	35 (40.2%)
All deaths	0	1 (1.1%)

Source: Table 8, p18 of the submission

AE = adverse event; SAE = serious adverse event

Table 7: Most common AEs (> 10% in any group) from the ECLIPSE trial

	Dispersible tablets (N = 86)		Film coated tablets (N = 87)	
	All AEs, n (%)	Severe AEs, n (%)	All AEs, n (%)	Severe AEs, n (%)
Total	77 (89.5%)	22 (25.6%)	78 (89.7%)	17 (19.5%)
Diarrhoea	30 (34.9%)	6 (7.0%)	29 (33.3%)	1 (1.1%)
Nausea	23 (26.7%)	2 (2.3%)	24 (27.6%)	1 (1.1%)
Abdominal pain	23 (26.7%)	4 (4.7%)	23 (26.4%)	2 (2.3%)
Increased UPCR (> 0.5)	11 (12.8%)	2 (2.3%)	18 (20.7%)	0
Vomiting	19 (22.1%)	1 (1.2%)	15 (17.2%)	0
Abdominal pain upper	6 (7.0%)	1 (1.2%)	10 (11.5%)	0
Constipation	13 (15.1%)	2 (2.3%)	7 (8.0%)	0
Headache	12 (14.0%)	2 (2.3%)	5 (5.7%)	0

Source: Table 9, p18 of the submission

AE = adverse event; UPCR = urine protein to creatinine ratio

- 5.19 Overall, the safety profiles of the dispersible tablet and the film coated tablet were similar.
- 5.20 The most commonly reported adverse events in both arms of the trial were diarrhoea, nausea, abdominal pain and vomiting.
- 5.21 The rate of gastrointestinal adverse events was similar between the dispersible and film coated tablet arms (61.6% versus 58.6% respectively). More patients treated with the dispersible tablet experienced severe gastrointestinal adverse events (12.8% versus 4.6%).
- 5.22 In the pre-PBAC response it was noted that the exposure adjusted incidence of gastrointestinal adverse events was lower in the film coated tablet group (137 per 100 patient years) compared to the dispersible tablet group (153 per 100 patient years). The pre-PBAC response stated that the rates of adverse events need to be considered in terms of the increased time of exposure for patients using the film coated tablets.
- 5.23 A greater proportion of patients in the film coated tablet arm experienced any renal disorder (34.5%) compared to in the dispersible tablet arm (26.7%). Renal adverse events were considered to be dose-dependent.
- 5.24 The TGA Delegate noted that:
- “study F2201 demonstrated a higher proportion of renal adverse events for the film coated tablet formulation (34.5%) compared to the dispersible tablet formulation (26.7%). A subsequent pharmacokinetic/pharmacodynamic analysis indicated that renal functional changes are more closely associated with AUC than with C_{max} ; and,
 - “although the clinical evaluator concludes that no apparent new safety signal emerged for the new formulation of deferasirox, the long term safety of the new formulation needs ongoing monitoring, including renal safety, gastrointestinal events, and all events identified as adverse events of special interest in previous studies.”

Clinical claim

- 5.25 The submission claimed that deferasirox film coated tablets were non-inferior to deferasirox dispersible tablets.
- 5.26 The submission stated that the dispersible tablet and the film coated tablet had a comparable pharmacokinetic profile. The pre-specified limits to be considered bioequivalent were not met as the C_{max} for the film coated tablet was higher; however, a large exposure-response analysis concluded no clinically relevant effects of an increase in C_{max} .
- 5.27 The PBAC considered that the claim of non-inferiority for deferasirox film coated tablets compared to deferasirox dispersible tablets was reasonable.

Economic analysis

- 5.28 The submission calculated prices for the film coated tablet using a cost-minimisation analysis assuming direct replacement of the dispersible tablet formulation at the low, medium, and high strengths, with the corresponding low, medium, or high strength of the film coated tablets.
- 5.29 The published prices of the film coated presentations (90 mg, 180 mg, 360 mg) were identical to the dispersible tablet presentations (125 mg, 250 mg, 500 mg), adjusted to reflect the 30 tablet pack size of the film coated tablet and are presented in the proposed PBS listing above.

Estimated PBS usage & financial implications

- 5.30 The minor submission estimated there to be no financial implications to the PBS as the submission expects deferasirox film coated tablets to only substitute for deferasirox dispersible tablets and both drugs have the same price per tablet. The PBAC noted that there may be a small financial impact of the new listing as a result of a different pack size, which will reduce the total number of scripts and patient co-payments.

For more detail on PBAC's view, see section 6 PBAC outcome.

6 PBAC Outcome

- 6.1 The PBAC recommended the Section 100 – Highly Specialised Drugs Program listing of deferasirox film coated tablets for the treatment of chronic iron overload due to disorders of haemopoiesis. The PBAC recommended the listing on a cost minimisation basis with deferasirox dispersible tablets. The PBAC's recommendation for listing was based on, among other matters, its assessment that deferasirox film coated tablets, while not strictly bioequivalent, were biocomparable to deferasirox dispersible tablets.
- 6.2 The PBAC noted the input provided by individuals and health care professionals describing improvements in compliance and a reduction in side effects as potential benefits of listing the deferasirox film coated tablets.
- 6.3 The PBAC considered, based on pharmacokinetic evidence presented in the minor submission and to the TGA, that deferasirox film coated tablets were biocomparable to deferasirox dispersible tablets. The PBAC noted that the pre-specified limits to be considered bioequivalent were not met as the C_{max} for the film coated tablet was higher; however, a large exposure-response analysis concluded no clinically relevant effects of an increase in C_{max} .
- 6.4 The PBAC was satisfied that, although the film coated tablets had a higher bioavailability than the dispersible tablets, the 360 mg film coated tablet formulation was equivalent to the 500 mg dispersible tablet formulation with respect to the mean AUC.

- 6.5 Based on the results of the ECLIPSE trial, the PBAC considered that the safety profile of the film coated tablets was similar to that of the dispersible tablets.
- 6.6 The PBAC considered that deferasirox film coated tablets were non-inferior to deferasirox dispersible tablets, and therefore a cost-minimisation approach against the dispersible tablets was appropriate.
- 6.7 The PBAC advised that the equi-effective doses of deferasirox were:
- 360 mg film coated = 500 mg dispersible;
 - 180 mg film coated = 250 mg dispersible; and
 - 90 mg film coated = 125 mg dispersible.
- 6.8 The PBAC accepted that addition of the film coated tablets to the PBS would result in a small financial implication to the PBS, due to a difference in pack sizes.
- 6.9 The PBAC considered that the proposed restriction, which was identical to that for deferasirox dispersible tablets, was consistent with the TGA-approved indication.
- 6.10 The PBAC noted that all initial listings for deferasirox dispersible tablets are Authority Required (Telephone), with continuing listings Authority Required (Streamlined). The PBAC considered that the same restriction levels were appropriate for deferasirox film coated tablets.
- 6.11 The PBAC considered that it would be appropriate for patients currently treated with deferasirox dispersible tablets to be eligible for continuing treatment with deferasirox film coated tablets. The PBAC recommended that the clinical criteria section of the deferasirox film coated tablets continuing restrictions include “Patient must have previously received PBS-subsidised treatment with deferasirox for this condition.”
- 6.12 The PBAC noted that flow-on changes would be required to the deferasirox dispersible tablets continuing restrictions with the clinical criteria amended to state “Patient must have previously received PBS-subsidised treatment with deferasirox for this condition.”
- 6.13 The PBAC noted that the sponsor has requested that the Special Pricing Arrangement (SPA) that currently applies to the deferasirox dispersible tablets is extended to include the film coated tablets. The PBAC further noted that SPA are given effect through a deed made under Section 85E of the National Health Act 1953 between the Minister (or his delegate) and the responsible person. The PBAC recalled that deferasirox was recommended for listing on a cost effectiveness basis versus the comparator desferrioxamine (Deferasirox Public Summary Document, July 2006).
- 6.14 The PBAC advised that deferasirox film coated tablets are not suitable for prescribing by nurse practitioners.
- 6.15 The PBAC recommended that the Early Supply Rule should apply.

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6.16 The PBAC noted that this submission is not eligible for an Independent Review as it received a positive recommendation.

Outcome:

Recommended

7 Recommended listing

7.1 Add new item:

Chronic iron overload in patients with transfusion-dependent malignant disorders of haemopoiesis and a median life expectancy exceeding five years: Public and Private (Initial):

Name, Restriction, Manner of administration and form	Max. qty packs	Max. qty units	No. of repeats	Proprietary Name and Manufacturer
DEFERASIROX				
film coated tablet 90 mg, 30	6	180	2	Jadenu® Novartis
film coated tablet 180 mg, 30	6	180	2	
film coated tablet 360 mg, 30	6	180	2	
Category / Program:	Section 100 – Highly Specialised Drugs Program (Public) Section 100 – Highly Specialised Drugs Program (Private)			
Prescriber type:	<input type="checkbox"/> Dental <input checked="" type="checkbox"/> Medical Practitioners <input type="checkbox"/> Nurse practitioners <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives			
Condition:	Chronic iron overload			
PBS indication:	Chronic iron overload			
Treatment phase:	Initial treatment			
Restriction level / Method:	<input checked="" type="checkbox"/> Authority Required – Telephone			
Clinical criteria:	Patient must be red blood cell transfusion dependent, AND Patient must have a serum ferritin level of greater than 1000 microgram/L, AND Patient must have a malignant disorder of haemopoiesis, AND Patient must have a median life expectancy exceeding five years.			
Administrative Advice:	<p>Note A patient's median life expectancy is determined by the severity of their underlying disease.</p> <p>Note Patients with underlying myelodysplastic syndrome are considered to have a median life expectancy exceeding five years if they are classified as: - low risk according to the International Prognostic Scoring System (IPSS); or - very low and low risk according to the Revised International Prognostic Scoring System (IPSS-R); or - very low and low risk according to the WHO classification based Prognostic Scoring System (WPSS).</p> <p>Note Patients with underlying myelofibrosis have a median life expectancy exceeding five years if they are classified as: - low or intermediate risk according to the International Prognostic Scoring System (IPSS); or - low or intermediate-1 risk according to Dynamic International Prognostic Scoring System (DIPSS).</p> <p>Note Special Pricing Arrangements apply.</p>			

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Chronic iron overload in patients with transfusion-dependent malignant disorders of haemopoiesis and a median life expectancy exceeding five years: Public and Private (Continuing):

Name, Restriction, Manner of administration and form	Max. qty packs	Max. qty units	No. of repeats	Proprietary Name and Manufacturer
DEFERASIROX				
film coated tablet 90 mg, 30	6	180	2	Jadenu® Novartis
film coated tablet 180 mg, 30	6	180	2	
film coated tablet 360 mg, 30	6	180	2	
Category / Program:	Section 100 – Highly Specialised Drugs Program (Public) Section 100 – Highly Specialised Drugs Program (Private)			
Prescriber type:	<input type="checkbox"/> Dental <input checked="" type="checkbox"/> Medical Practitioners <input type="checkbox"/> Nurse practitioners <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives			
Condition:	Chronic iron overload			
PBS indication:	Chronic iron overload			
Treatment phase:	Continuing treatment			
Restriction level / Method:	<input checked="" type="checkbox"/> Streamlined (Public and Private hospital)			
Clinical criteria:	Patient must be red blood cell transfusion dependent, AND Patient must have a malignant disorder of haemopoiesis; AND <i>Patient must have previously received PBS-subsidised treatment with deferasirox for this condition.</i>			
Administrative Advice:	<u>Note</u> Interruption of treatment should be considered if serum ferritin levels fall consistently below 500 microgram/mL.			
Administrative Advice:	<u>Note</u> Special Pricing Arrangements apply.			

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Chronic iron overload in patients with transfusion dependent non-malignant disorders of erythropoiesis: Public and Private (Initial):

Name, Restriction, Manner of administration and form	Max. qty packs	Max. qty units	No. of repeats	Proprietary Name and Manufacturer
DEFERASIROX				
film coated tablet 90 mg, 30	6	180	5	Jadenu® Novartis
film coated tablet 180 mg, 30	6	180	5	
film coated tablet 360 mg, 30	6	180	5	
Category / Program:	Section 100 – Highly Specialised Drugs Program (Public) Section 100 – Highly Specialised Drugs Program (Private)			
Prescriber type:	<input type="checkbox"/> Dental <input checked="" type="checkbox"/> Medical Practitioners <input type="checkbox"/> Nurse practitioners <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives			
Condition:	Chronic iron overload			
PBS indication:	Chronic iron overload			
Treatment phase:	Initial treatment			
Restriction level / Method:	<input checked="" type="checkbox"/> Authority Required – Telephone			
Clinical criteria:	Patient must be transfusion dependent, AND Patient must not have a malignant disorder of erythropoiesis.			
Administrative advice:	<u>Note</u> Special Pricing Arrangements apply.			

Chronic iron overload in patients with transfusion dependent non-malignant disorders of erythropoiesis: Public and Private (Continuing):

Name, Restriction, Manner of administration and form	Max. qty packs	Max. qty units	No. of repeats	Proprietary Name and Manufacturer
DEFERASIROX				
film coated tablet 90 mg, 30	6	180	2	Jadenu® Novartis
film coated tablet 180 mg, 30	6	180	2	
film coated tablet 360 mg, 30	6	180	2	
Category / Program:	Section 100 – Highly Specialised Drugs Program (Public) Section 100 – Highly Specialised Drugs Program (Private)			
Prescriber type:	<input type="checkbox"/> Dental <input checked="" type="checkbox"/> Medical Practitioners <input type="checkbox"/> Nurse practitioners <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives			
Condition:	Chronic iron overload			
PBS indication:	Chronic iron overload			
Treatment phase:	Continuing treatment			
Restriction level / Method:	<input checked="" type="checkbox"/> Streamlined (Public and Private hospital)			
Clinical criteria:	Patient must be transfusion dependent, AND Patient must not have a malignant disorder of erythropoiesis, AND <i>Patient must have previously received PBS-subsidised treatment with deferasirox for this condition.</i>			
Administrative advice:	<u>Note</u> Special Pricing Arrangements apply.			

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Chronic iron overload in patients with non-transfusion dependent thalassaemia: Public and Private (Initial):

Name, Restriction, Manner of administration and form	Max. qty packs	Max. qty units	No. of repeats	Proprietary Name and Manufacturer	
DEFERASIROX					
film coated tablet 90 mg, 30	6	180	5	Jadenu®	Novartis
film coated tablet 180 mg, 30	6	180	5		
film coated tablet 360 mg, 30	6	180	5		
Category / Program:	Section 100 – Highly Specialised Drugs Program (Public) Section 100 – Highly Specialised Drugs Program (Private)				
Prescriber type:	<input type="checkbox"/> Dental <input checked="" type="checkbox"/> Medical Practitioners <input type="checkbox"/> Nurse practitioners <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives				
Condition:	Chronic iron overload				
PBS indication:	Chronic iron overload				
Treatment phase:	Initial treatment				
Restriction level / Method:	<input checked="" type="checkbox"/> Authority Required – Telephone				
Clinical criteria:	Patient must not be transfusion dependent, AND The condition must be thalassaemia.				
Administrative advice:	<u>Note</u> Special Pricing Arrangements apply.				

Chronic iron overload in patients with non-transfusion dependent thalassaemia: Public and Private (Continuing):

Name, Restriction, Manner of administration and form	Max. qty packs	Max. qty units	No. of repeats	Proprietary Name and Manufacturer	
DEFERASIROX					
film coated tablet 90 mg, 30	6	180	2	Jadenu®	Novartis
film coated tablet 180 mg, 30	6	180	2		
film coated tablet 360 mg, 30	6	180	2		
Category / Program:	Section 100 – Highly Specialised Drugs Program (Public) Section 100 – Highly Specialised Drugs Program (Private)				
Prescriber type:	<input type="checkbox"/> Dental <input checked="" type="checkbox"/> Medical Practitioners <input type="checkbox"/> Nurse practitioners <input type="checkbox"/> Optometrists <input type="checkbox"/> Midwives				
Condition:	Chronic iron overload				
PBS indication:	Chronic iron overload				
Treatment phase:	Continuing treatment				
Restriction level / Method:	<input checked="" type="checkbox"/> Streamlined (Public and Private hospital)				
Clinical criteria:	Patient must not be transfusion dependent, AND The condition must be thalassaemia, AND <i>Patient must have previously received PBS-subsidised treatment with deferasirox for this condition.</i>				
Administrative Advice:	<u>Note</u> Special Pricing Arrangements apply.				

8 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.

9 Sponsor's Comment

Novartis is pleased with the PBAC recommendation. This is an important outcome for patients with chronic iron overload.