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7.03 ODEVIXIBAT,
Capsule 200 micrograms,
Capsule 400 micrograms,
Capsule 600 micrograms,
Capsule 1,200 micrograms,
Bylvay®,
Ipsen PTY LTD.

1 Purpose

1.1 The early re-entry resubmission requested PBS listing of odevixibat for the treatment of cholestatic pruritus in Alagille syndrome (ALGS).

1.2 The resubmission was based on the PBAC decision to not recommend odevixibat for this indication at its March 2026 meeting (odevixibat Public Summary Document (PSD), March 2026 PBAC meeting). A summary of the key matters that were to be addressed in the resubmission is provided in Table 1.

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Table 1: Summary of key matters to be addressed

Matter of concern	Response	Addressed?
Revision of restrictions		
Provide a revised restriction criteria that incorporated the following: • Age agnostic. • No requirement for genetic testing for initiation of therapy. • Written authority for initial and grandfather prescriptions, and telephone authority for continuing prescriptions. • Continuing restriction to include requirement for improvement in pruritis score and initial restriction to require demonstration of response by 3 (rather than 6) cumulative months of treatment. • 2 repeats for initial treatment listing to allow for 3 months' initial treatment, as per PFIC. 5 repeats for continuing. • Dose reductions should be allowed in initial and continuing restrictions and patients should be allowed to continue on the lower dose providing they demonstrate and maintain an adequate clinical response (via the ObSRO score). • No stopping rule is required providing patients continue to maintain a positive response. Assessment of response should occur for each continuing prescription. • Restrictions to specify where recommencement of treatment can occur if treatment is ceased for reasons other than lack of response. (para 7.5)	The resubmission stated that all proposed changes to the restriction have been accepted.	Partially addressed (see paragraphs 3.3 - 3.5**Error! Reference source not found.**)
Revised cost per patient per year		
Propose a cost per patient per year no higher than recommended for PFIC (in the order of $[redacted]) assuming a dose of 120 mcg/day and the same patient weight (i.e., 45 kg).	Cost per patient per year ranged from $[redacted] (for a patient 6 months of age) to $[redacted] (for an adult). The resubmission stated the weighted average cost per patient per year was $[redacted] (based on an average patient weight of 19.8 kg).	Not addressed.
Revision of financial estimates		
Financial estimates should be updated to use more conservative, lower-end utilisation estimates, separated into paediatric and adult patients and incorporate the updated price.	The resubmission stated that financial estimates have been updated as requested. The resubmission stated that, combined with a 16.7% price reduction, the net financial impact of listing odevixibat was reduced by 69% *(*using estimates provide in resubmission; 52% using corrected estimates) compared to the March 2026 submission.	Addressed.
Propose an RSA		
The PBAC considered that an RSA would be necessary to mitigate any outstanding uncertainty regarding the eligible number of patients and utilisation.	The resubmission considered an RSA was not required	Not addressed

Source: compiled from resubmission, and March 2026 Odevixibat minutes
ALGS, Alagille syndrome; RSA, risk sharing arrangement,

2 Background

2.1 The resubmission was made under the TGA/Pharmaceutical Benefits Advisory Committee (PBAC) Parallel Process and assessed via the Comparable Overseas Regulator (COR) pathway. The TGA Delegate's Overview (provided prior to PBAC consideration) proposed approval of odevixibat "for the treatment of cholestatic pruritus in Alagille syndrome (ALGS) in patients aged 6 months or older."

2.2 Odevixibat was TGA registered in March 2025 for the treatment of Progressive Familial Intrahepatic Cholestasis (PFIC) in patients aged six months or older. Odevixibat was recommended by the PBAC for the treatment of PFIC at the July 2025 PBAC meeting and listed on the PBS on 1 January 2026. A comparison of the current resubmission with the July 2025 submission is presented in Attachment 1.

2.3 The PICO from the previous submission is presented below.

Table 2: Key components of the clinical issue addressed by the submission (as stated in the submission)

Component	Description
Population	Patients with cholestatic pruritus in Alagille Syndrome (ALGS).
Intervention	Odevixibat 120 µg/kg administered orally once daily in the morning. Dose reduction to 40 µg/kg/day may be considered if tolerability issues occur in the absence of other causes.
Comparator	Standard of care including biliary diversion surgery and liver transplant
Outcomes	Pruritus response, serum bile acid (sBA) response, sleep parameters, growth parameters, quality of life and safety.
Clinical claim	Superiority in terms of efficacy (pruritus response, sBA response and sleep parameters). Non-inferiority in terms of safety.

Source: Table 1, odevixibat PSD, March 2026 PBAC meeting

3 Requested listing

MEDICINAL PRODUCT medicinal product pack	Max. qty packs	Max. qty units	№. of Rpts	DPMQ[a]	Available brands
Odevixibat 200 micrograms capsules bottle, 30	12	360	2 initial / 5 continuing	Published: $67,122.93 Effective: $[redacted]	Bylvay
Odevixibat 400 micrograms capsules bottle, 30	6	180	2 initial / 5 continuing	Published: $67,122.93 Effective: $[redacted]	Bylvay
Odevixibat 600 micrograms capsules bottle, 30	12	360	2 initial / 5 continuing	Published: $201,042.96 Effective: $[redacted]	Bylvay
Odevixibat 1,200 micrograms capsules bottle, 30	6	180	2 initial / 5 continuing	Published: $201,042.96 Effective: $[redacted]	Bylvay

[a] DPMQ from financial estimates spreadsheet provided with resubmission

Category / Program: Section 85 – General Schedule
Restriction type: ☒Restricted benefit ☒Authority Required (in writing only via post/HPOS upload or Online PBS Authorities system)
Indication: The treatment of cholestatic pruritus in Alagille syndrome (ALGS)
Treatment Phase: Initial[1]

Clinical criteria: Patient must not have received prior PBS-subsidised treatment with this drug for this condition AND Patient must have a clinical diagnosis of ALGS AND The patient must have elevated serum bile acids at treatment initiation with this drug AND Patient must be experiencing itch at treatment initiation with this drug, with other factors causing pruritus excluded through both:(i) physical examination and (ii) patient history AND Patient must have had an average pruritus score of ≥2 on the Prucision™ ObsRO Pruritus Scale assessed on three occasions over a one-week period prior to treatment initiation with this drug.
Treatment criteria: Must be treated by a specialist experienced in the management of ALGS, who is either a: (i) gastroenterologist, (ii) hepatologist
Prescribing Instructions: The authority application must be made via the Online PBS Authorities System, or in writing via HPOS form upload or mail and must include: (1) details of the pruritus score using the Prucision™ ObsRO Pruritus scale (2) details of serum bile acids (date and micromol/L)
Prescribing Instructions: If the application is submitted through HPOS form upload or mail, it must include: (i) details of the proposed prescription; and (ii) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).
Prescribing Instructions: Prescriber must exclude any other causes of pruritus which include any of the following: (i) drug related (e.g., opioid-related pruritus) (ii) drug hypersensitivity or adverse effect; contact dermatitis; allergy (iii) differential diagnoses (e.g., xerosis; infestations; iron deficiency; chronic kidney disease; polycythaemia vera/leukemia/lymphoma; hypothyroidism; uncontrolled diabetes, eczema, scabies).
Prescribing Instructions: At the time of the authority application, the prescriber should request the appropriate strength(s) and quantity based on the patients' weight, according to the dosing schedule in the TGA approved Product Information to provide sufficient drug for one month's supply with 5 repeats. A separate authority approval is required for each strength requested
Prescribing Instructions: All diagnostic reports/tools must be documented in the patient's medical records.
Prescribing Instructions: Eligible patients must not be recommencing treatment directly through this treatment phase
Patients who have demonstrated an adequate clinical response and have ceased treatment for reasons other than a lack of response may recommence treatment through the continuing treatment phase.
Administrative Advice:
The Prucision™ ObsRO Pruritus Scale is available as a downloadable document at: Prucision ™ ObsRO Pruritus Scale

Source: resubmission.

Note: The resubmission also proposed a grandfather restriction. Not presented here for brevity.

Continuing:

Category / Program: Section 85 – General Schedule

Restriction type: ☒Restricted benefit ☒Authority Required (telephone/online PBS Authorities system)
Indication: The treatment of cholestatic pruritus in Alagille syndrome (ALGS)
Treatment Phase: Continuing
Clinical criteria: Patient must have previously received PBS-subsidised treatment with this drug for this condition AND Patient must have demonstrated an adequate clinical response to the most recent course of treatment
Treatment criteria: Must be treated by a specialist experienced in the management of ALGS, who is either a: (i) gastroenterologist, (ii) hepatologist OR Must be treated by a medical practitioner under the supervision of a specialist experienced in the management of ALGS
Prescribing Instructions: An adequate clinical response under this treatment phase is defined as an average ≥1 grade improvement in pruritus score, versus baseline measurement, on the Prucision™ ObsRO Pruritus Scale, assessed on 3 occasions over a one-week period prior to assessment of response.
Prescribing Instructions: Dose reduction to 40 µg/kg/day may be considered if tolerability issues occur in the absence of other causes. Once tolerability issues stabilize, increase to 120 µg/kg/day
Administrative Advice:
The Prucision™ ObsRO Pruritus Scale is available as a downloadable document at: Prucision ™ ObsRO Pruritus Scale
Administrative Advice:
Patients who have demonstrated an adequate clinical response and have ceased treatment for reasons other than a lack of response may recommence treatment through the continuing treatment phase.

3.1 The proposed effective manufacturer price (EMP) per 30 capsule pack was $[redacted] for 200 mcg, $[redacted] for 400 mcg, $[redacted] for 600 mcg and $[redacted] for 1,200 mcg. These prices are approximately [redacted]% lower than proposed in the March 2026 submission.

3.2 The resubmission proposed separate initial, grandfather and continuing treatment phase restrictions, however the Secretariat advised that grandfather patients may be eligible under the initial treatment phase. The Secretariat recommended referring to "Ileal bile acid transporter (iBAT) inhibitors" rather than "this drug", to accommodate all grandfather patients within the initial treatment phase including any patients previously treated with maralixibat. The Secretariat also recommended capturing administrative advice in an overarching administrative note, including that each subsequent course of continuing treatment requires reassessment of response or disease maintenance at 6 monthly intervals, aligning with the provision of five repeats under the continuing treatment phase. The Pre-PBAC response accepted all changes proposed by the secretariat.

3.3 The PBS listing proposed in the resubmission addressed several of the PBAC's previous recommendations (paragraph 7.5 odevixibat PSD, March 2026 PBAC meeting), including that the restriction:

- is age agnostic;
- does not require genetic testing;

- requires written authority for initial and grandfather prescriptions and telephone authority for continuing prescriptions;
- continuing restriction includes requirement for improvement in pruritis score, and initial restriction requires demonstration of response by 3 cumulative months of treatment;
- 2 repeats for initial treatment and 5 repeats for continuing; and
- no stopping rule providing patients maintain a positive response.

3.4 The resubmission partially addressed previous PBAC advice regarding dose reductions. The resubmission proposed allowing dose reduction in the continuing treatment restriction only; however the PBAC had previously advised that dose reductions should also be permitted in the initial treatment phase and patients should be allowed to continue on the lower dose providing they demonstrate and maintain an adequate clinical response (via the ObSRO score) (paragraph 7.5 odevixibat PSD, March 2026 PBAC meeting). The Pre-PBAC response accepted changes proposed by the Secretariat to enable dose reductions in the initial treatment phase.

3.5 The resubmission partially addressed previous PBAC advice regarding retreatment by permitting retreatment only within the continuing treatment phase; however, the PBAC previously advised that retreatment under the initial treatment restriction would be appropriate if less than 3 months of treatment has been received. The Secretariat recommended that patients recommencing treatment under the initial restriction should restart and complete a full 3-month initial treatment period, rather than resuming only the remaining portion of their prior supply (i.e. not a balance of supply). The Pre-PBAC response accepted changes proposed by the Secretariat.

For more detail on PBAC's view, see section 7 PBAC outcome.

4 Consideration of the evidence

Sponsor hearing

4.1 There was no hearing for this item.

Consumer inputs

4.2 The PBAC noted that no consumer inputs were received for this item but recalled the input received for its March 2026 consideration (paragraph 7.2, odevixibat PSD, March 2026 PBAC meeting).

Clinical claim

4.3 No additional clinical data was presented in the resubmission.

4.4 The PBAC previously considered the claim of superior comparative effectiveness compared to placebo (as a proxy for best supportive care) was reasonable (paragraph

7.7, odevixibat PSD, March 2026 PBAC meeting) and the claim of non-inferior comparative safety was reasonable (paragraph 7.8, odevixibat PSD, March 2026 PBAC meeting).

4.5 The PBAC recalled that some limited evidence was presented previously that odevixibat may improve native liver survival in PFIC (paragraph 7.11, odevixibat PSD, July 2024 PBAC meeting) but similar evidence was not presented for ALGS.

Early Access Program

4.6 The resubmission provided information on the Early Access Program (EAP). The EAP commenced in August 2022 and has enrolled < 500 patients as of April 2026. The resubmission stated the access criteria is consistent with that of the pivotal clinical trial: (i) patients aged ≥ 6 months with confirmed diagnosis of ALGS (ii) patient must have cholestatic pruritus documented by the physician using the Clinical Global Impression of Symptoms (CGIS) and (iii) other treatment options have been exhausted (inclusion in clinical trial and viable alternative therapies).

4.7 The resubmission stated that 17 patients had been previously enrolled in an EAP for maralixibat. These patients have been included in the pool of grandfathered patients in the financial estimates. No information was provided regarding the age or weight of these patients. Maralixibat is an IBAT inhibitor approved by the FDA in September 2021 for the treatment of cholestatic pruritus in ALGS patients aged ≥1 year old. The May 2026 EAP Report lodged with the resubmission stated that maralixibat has not progressed to TGA submission, and it is understood the maralixibat EAP was closed to new patients at the end of 2024, but there is no evidence that access to maralixibat for previously treated patients has ceased.

Cost per patient per year

4.8 The PBAC previously considered that odevixibat would be used primarily for the symptomatic management of cholestatic pruritus in ALGS, noting that odevixibat is not considered to be disease-modifying in ALGS and its impact on underlying liver disease progression or long-term outcomes is unclear (see paragraph 7.3, odevixibat PSD, March 2026 PBAC meeting).

4.9 At the March 2026 meeting, the PBAC recalled that it had previously considered odevixibat would be cost effective (for PFIC) with a cost per patient per year in the order of $[redacted] but it remained highly uncertain if a similar cost per patient per year would be cost-effective for ALGS, noting the lack of clinical data on long term outcomes (see paragraph 7.9, odevixibat PSD, March 2026 PBAC meeting). Some limited evidence on long term outcomes was available for PFIC (see Table 8, odevixibat PSD, July 2025 PBAC meeting). However, the PBAC previously considered that in the context of the small and vulnerable population with a high clinical need for effective treatments, odevixibat may be cost effective for ALGS with a cost per patient per year no higher than for PFIC (assuming the same patient weight) (paragraph 7.9, odevixibat PSD, March 2026 PBAC meeting).

4.10 To calculate the average cost per patient per year, the resubmission assumed three cohorts of patients would commence treatment:

- Incident paediatric patients, commencing treatment at 6 months of age, weighing 5.2 kg;
- Prevalent paediatric patients, commencing treatment at 5.2 years of age, weighing 16.01 kg; and
- Prevalent adult patients.

4.11 The cost per patient per year for the three cohorts at commencement of treatment is presented in Table 3. The weight at commencement was informed by the Global Alagille Alliance (GALA) registry data published in Huysentruyt, 2025[1]. It is unclear how applicable data collected between 2018 and 2023 from an international registry is to the Australian ALGS population. The patient weight at treatment commencement in the cost per patient calculation differs from that observed in the EAP (i.e., 5.2 kg vs 6.3 kg for the 3 patients commencing treatment at 6 months; 16.01 kg vs 19.5 kg for the 11 patients commencing treatment aged 1 to 15). The patient weight used in the cost per patient per year calculation was likely underestimated relative to Australian EAP experience, which may underestimate cost per patient per year.

4.12 The cost per patient per year calculation assumed the same price per mcg across all strengths of odevixibat. However, applying a price per mcg does not account that patients need to take whole capsules (i.e., doses should be a multiple of 200 mcg).

Table 3: Cost per patient per year calculation per ALGS patient population, at treatment commencement, assuming a cost of $[redacted] EMP per mcg

Patient population	Average age	Average weight	Daily dose (mcg/day)	Daily cost (EMP)	Cost / patient / year (EMP)
Incident paediatric patients	6 months	5.2 kg	624	$[redacted]	$[redacted]
Prevalent paediatric patients	5.2 years	16.01 kg	1,921	$[redacted]	$[redacted]
Prevalent adult patients	26 years	60 kg	7,200	$[redacted]	$[redacted]

Source: Table 11 of resubmission
EMP, Ex-Manufacturer Price

4.13 The resubmission calculated the average daily dose across a 6 year time horizon, assuming the two paediatric cohorts (the incident pool of patients and the prevalent pool of patients) increased in weight over time, informed by the GALA registry data.

4.14 The split across the cohorts was calculated based on the utilisation estimates presented in the resubmission with 35% incident paediatric patients, 61% prevalent paediatric patients and 5% prevalent adult patients.

[1] Huysentruyt K, Vandriel, S, Roelants, M et al. Condition-specific growth charts for children with Alagille Syndrome. *JAMA Network Open.* 2025;8(11):e2545294. doi:10.1001/jamanetworkopen.2025.45294

Table 4: Weighted average daily dose, across 6 years

	Weight over 6 years (kg)	Average dose per day, calculated over 6 years assuming 120 mcg/kg/ day	Number of patients, based on utilisation estimates (% of use)
Incident paediatric patients	Yr 1: 5.2 Yr 2: 8.9 Yr 3: 11.0 Yr 4: 12.8 Yr 5: 14.6 Yr 6: 16.5 Average: 11.50	1,380 mcg	[redacted][1] (35)
Prevalent paediatric patients	Yr 1: 16.01 Yr 2: 17.7 Yr 3: 19.9 Yr 4: 22.4 Yr 5: 24.7 Yr 6: 27.4 Average: 21.35	2,562 mcg	[redacted][1] (61)
Prevalent adult patients	60	7,200 mcg	[redacted][1] (5)
Weighted average dose per day	**2,375 mcg**		

Source: 3a.Scripts-proposed worksheet, Attachment 2 Odevixibat in ALGS UCM July 2026.xls and Table 10 of the resubmission

The redacted values correspond to the following ranges:

[1] *< 500*

4.15 The resubmission proposed using a weighted average daily dose of 2,375 mcg to calculate the cost per patient per year as presented in Table 5. The PBAC previously considered odevixibat may be considered cost effective with a cost per patient per year no higher than for PFIC assuming the same patient weight (as discussed in paragraph 4.8). The cost per patient per year using the same average patient weight as PFIC is presented in Table 5. To achieve the same cost per patient per year for ALGS assuming the same patient weight as used for PFIC as requested by PBAC (i.e., cost per patient per year of $[redacted] for a 45.1 kg patient) would require a cost per mcg of $[redacted] per mcg[2].

[2] Calculated as $[redacted]/(45.1 kg x 120 mcg/day x 365.25 days)

Table 5: PFIC and ALGS cost per patient using ALGS weighted average daily dose,

	PFIC - Average EAP Patient (basis of recommendation)	ALGS - Weighted average daily dose across 6 years, as calculated in resubmission	ALGS – based on same weight as PFIC patient (as requested by PBAC)
Average age	13 years old	7.2 years old	Approximately 15 years old-
Average weight	45.1 kg	19.8 kg[1]	45.1 kg
Dose per day[2]	40 mcg/kg	120 mcg/kg	120 mcg/kg
Daily dose	1,804 mcg	2,375 mcg	5,412 mcg
Price per mcg (AEMP)	$[redacted]	$[redacted]	$[redacted]
Cost per patient per year (EMP)	$[redacted][3]	$[redacted]	$[redacted]

Source: Table 13 of resubmission;

1. Calculated as 2,375/120. Incorrectly reported in resubmission as 22.6 kg.
2. The recommended odevixibat dosage for patients with ALGS is 120 µg/kg/day (with de-escalation to 40 µg/kg/day if tolerability issues) compared to 40 µg/kg/day (with escalation to 120 µg/kg/day in the case of inadequate response) for patients with PFIC.
3. Calculated as 45.1 x 40 x 0.306 x 365.25. Unclear why different from the cost per patient per year presented in resubmission ($[redacted])

4.16 The resubmission stated that while ALGS shares certain features with other inherited cholestatic disorders, it is clinically distinct from PFIC, particularly in its multisystem involvement, heterogeneity of presentation, and broader non-hepatic manifestations. The resubmission stated that ALGS presents at a much younger age than PFIC and is predominantly a paediatric disease. However, the evidence provided to support this is from the financial models for each indication, whereby the number of adult patients was estimated (for ALGS based on < 500/< 500 patients in the EAP being an adult, for PFIC as estimated by a patient group) rather than based on epidemiology of the disease.

4.17 The proposed cost per patient per year is higher than recommended for PFIC ($[redacted]) and substantially higher than based on the March 2026 PBAC advice ($[redacted]), and this was in the context of uncertainty regarding whether a similar cost per patient per year would be cost effective (as discussed in paragraph 4.9). Additionally, achievement of this cost per patient per year is dependent on an average daily dose of 2,375 mcg which is highly uncertain.

4.18 The PBAC noted that assuming the weight at commencement of treatment for the incident and prevalent paediatric populations was 50% higher than estimated, resulted in an average weight of 25.5 kg (calculated over 6 years) and an average daily dose of 3,066 mg, with a cost per patient per year of $[redacted]. The PBAC considered an average weight of 25.5 kg was more reasonable than the 19.8 kg proposed in the resubmission.

Estimated PBS usage & financial implications

4.19 The resubmission's financial estimates incorporated revised utilisation assumptions, including more conservative incidence estimates, restriction of prevalence to younger populations, and the use of EAP data to inform patient characteristics and dosing. In addition, the resubmission proposed a lower effective net price, representing a 16.7% reduction compared to the March 2026 submission, resulting in an overall reduction

in the estimated net financial impact of approximately 69% (based on financial estimates provided in the resubmission) compared with the March 2026 submission. Financial estimates were 52% lower, based on corrected estimates.

4.20 The key inputs for the financial estimates are presented in **Error! Reference source not found.**.

Table 6: Key inputs for financial estimates

Data	Value / Source	Comment
Eligible population		
Incident patients	Applied 1 in 50,000 live births. 6 to 7 patients per year.	Updated from 1 in 30,000 More conservative assumption applied as requested in March 2026.
Prevalent patient pool	Applied 1 in 100,000 prevalence rate to the ABS 2025 population 0 - 19 years of age. 69 to 73 patients per year.	The resubmission stated that prevalence was applied to the population aged 0 - 19 years of age, as requested in March 2026.
Grandfathered patients	[redacted][1] ([redacted][1] from odevixibat EAP and [redacted][1] from maralixibat). [redacted][1] GF patients assumed to be adults based on 7% of patients in the EAP being an adult ([redacted][1]/[redacted][1]).	Updated from [redacted][1] in March 2026. GF patients were subtracted from the prevalent pool of patients to avoid double counting
Cholestasis prevalence	92.1%	Unchanged.
No prior liver transplant	67.55%	The March 2026 PSD noted the proportion of prevalent patients with prior LT was not considered in the submission (Table 19, odevixibat PSD, March 2026 PBAC meeting). The resubmission has applied an eligibility criteria to the prevalent population to account for patients that have undergone prior LT (32.5%) based on the cumulative incidence of LT from the GALA cohort.
Eligibility – moderate to severe pruritis	Not included	March 2026 submission assumed 74% of patients would have moderate to severe pruritis. This eligibility criteria was removed in the resubmission due to the likelihood of overlap between cholestatic patients and patients with moderate to severe pruritus (as discussed in Table 19, odevixibat PSD, March 2026 PBAC meeting).
Treatment utilisation		
Uptake rate – incident patients	Initiating: [redacted]%	Unchanged
Uptake rate – prevalent patients	Yr 1: [redacted]%; Yr 2: [redacted]%; Yr 3: [redacted]%	Updated from Yr 1: [redacted]%; Yr 2: [redacted]%; Yr 3: [redacted]% Consistent with previous PBAC consideration that uptake in Year 1 in the prevalent population may be higher than proposed.
Continuation/ response rate	87.4%	Unchanged
Compliance	95.5%	Unchanged
Dosage:	Incident paediatric patients: 1,380 mcg per day.	As calculated in Table 4.

Data	Value / Source	Comment
	Paediatric patients: 2,562 mcg per day. Adult patient: 7,200 mcg per day.	
Scripts	Based on average capsules per day calculated using average doses for each population.	Amended from approach to estimating scripts applied in March 2026 submission. The calculation assumed patients would receive partial capsules and scripts, which is not reasonable, and would need to be corrected for whole capsules of available strengths.
Duration of treatment	Initiating: 3 months; Continuing: 69 months. (Total 72 months / 6 years).	The financial estimates assumed no patients discontinued treatment.

Source: Table 8 and Attachment 2 Odevixibat in ALGS UCM July 2026.xls
The redacted values correspond to the following ranges:
[1] < 500

4.21 The Secretariat identified a number of errors in the financial estimates provided with the resubmission:

- Incorrect linking to DPMQ in Cells H274 to H286 on 3c.Impact-proposed (eff) worksheet. For example, Cell H279 links to DPMQ for 400 mcg presentation, whereas it should link to the DPMQ for the 1,200 mcg presentation.
- Incorrect linking to average capsules per day in cell N122 on 3c.Impact-proposed (eff) worksheet (should be 365/L122*O270)
- Incorrect accounting for split of strengths. Cells H240 and H241 should be 50% reflecting 3 years of 400 mcg scripts and 3 years of 600 mcg scripts, rather than split by average capsules per day. Similarly, Cell H275 should be 33.3% (reflecting 2 years of 600 mcg scripts) and Cell 276 should be 66.7% (reflecting 4 years of 1,200 mcg scripts).

These errors were acknowledged in the Pre-PBAC response.

4.22 The resubmission applied a prevalence of ALGS of 1 in 100,000 to the Australian population 0 to 19 years of age with this population then split into paediatric and adult patients based on the proportional split in the EAP (i.e., 7% adult patients). The resubmission stated no additional adults were added into the patient pool, rather the 0-19 year old prevalent group was conservatively assumed to be sufficient to capture the remaining prevalent population outside of the grandfathered patients from the odevixibat and maralixibat EAPs.

4.23 The estimated use and financial impact of listing odevixibat (corrected for the errors identified above) is presented in Table 7.

Table 7: Estimated patient populations, March 2026 and July 2026 updates

	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
July 2026 resubmission						
Number of patients initiating treatment						
Incident patients	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
Prevalent patients						
- Paediatric	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
- Adult	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
Grandfathered patients						
- Paediatric	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
- Adult	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
Total initiating	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
Net cost to the PBS/ RPBS ($)	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]
Net cost to the PBS/ RPBS (as presented in resubmission) ($)	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]
March 2026 submission						
Number of patients initiating treatment						
Incident	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
Prevalent	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
Grandfathered patients	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
Total initiating	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
Net cost to the PBS/ RPBS ($)	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]

Source: Table 9 of the resubmission, Table 20 of odevixibat minutes, March 2026 PBAC meeting.
The redacted values correspond to the following ranges:
[1] *< 500*
[2] *$10 million to < $20 million*

4.24 The corrected net cost to the PBS/RPBS of listing odevixibat was estimated to be $10 million to < $20 million in Year 1, increasing to $10 million to < $20 million in Year 6, totalling $80 million to < $90 million over the first 6 years of listing.

4.25 The approach of using the average cost per patient per year calculated over 6 years (see Table 4) in the financial estimates will overestimate the costs in early years (when patient weight will be lower) and underestimate in later years (when patient weight will be higher).

4.26 The estimated number of patients commencing PBS-subsidised treatment with odevixibat is lower in the resubmission than the March 2026 submission (< 500vs < 500). This is largely due to the application of prevalence estimates to the population aged 0 to 19 years (rather than the total population) and removal of patients assumed to have had a prior liver transplant.

Financial management – Risk Sharing Arrangement

4.27 The PBAC previously considered that a risk sharing arrangement (RSA) would be necessary to mitigate any outstanding uncertainty regarding the eligible number of

patients and utilisation (paragraph 7.11, odevixibat PSD, March 2026 PBAC meeting). An RSA was not proposed in the resubmission. The resubmission and Pre-PBAC response stated that in the context of rare, genetic diseases such as ALGS, a financial cap is not necessary nor proportionate and may inadvertently undermine equity of access for a very small population with high unmet clinical need.

4.28 The resubmission noted that ALGS is a severe, inherited cholestatic disorder with a clearly defined genetic basis, and diagnosis requiring specialist paediatric hepatology supervision. Because of this, the treatable population is small and clinically validated. The resubmission stated there is an extremely low risk of use outside the indication, misdiagnosis, or widespread off-label use, and the proposed PBS restrictions already ensure appropriate use through tight initiation and continuation limits and specialist prescribing. Given these controls, the resubmission stated that any additional volume-based measure such as an expenditure cap does not add value, it simply duplicates safeguards inherent to the disease and its management.

4.29 The resubmission noted an RSA for odevixibat in PFIC was implemented due to material uncertainty surrounding real-world utilisation, driven primarily by unpredictable rates of dose escalation from 40 mcg per day to 120 mcg per day. In contrast, utilisation of odevixibat in ALGS is demonstrably more stable and predictable. The resubmission maintained that the key concerns identified in the initial submission have now been comprehensively addressed, and as a result, no significant residual uncertainty remains that would justify the need for an RSA in this indication.

4.30 The Secretariat considered that without a risk sharing arrangement there is a substantial risk of the use of odevixibat not being cost-effective. For example, if the average patient weight is higher than assumed, the average cost per patient will also be higher and likely not cost-effective.

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

5 PBAC Outcome

5.1 The PBAC recommended the listing of odevixibat for the treatment of cholestatic pruritus in Alagille syndrome (ALGS). The PBAC noted that ALGS is a rare condition and pruritus can have severe impacts on quality of life. The PBAC recalled that it previously considered the claim of superior comparative effectiveness compared to standard of care (SoC) to be reasonable, as there is evidence showing that odevixibat can reduce itching for some patients with ALGS, however long-term benefits remain uncertain. The PBAC noted the higher odevixibat dose when used in ALGS compared to PFIC, but accepted this was partly offset by the average patient weight being lower; however, the PBAC considered the average patient weight was underestimated in the resubmission. In the context of a higher prevalence for ALGS and limited evidence, regarding survival outcomes (i.e., native liver survival), the PBAC considered odevixibat would be cost-effective with a cost per patient per year in the order of $[redacted].

The PBAC considered that a risk sharing agreement (RSA) would be required to manage uncertainty regarding the average weight of the treated population.

5.2 The PBAC recalled input from consumers, healthcare professionals and organisations in relation to the March 2026 submission, describing the substantial burden of cholestatic pruritus in ALGS in this vulnerable population (paragraph 7.2 odevixibat PSD, March 2026 PBAC meeting).

5.3 The PBAC noted that the resubmission had addressed some of the issues identified at the March 2026 PBAC meeting (Table 1). However, the PBAC noted that the request for a revised cost per patient per year no higher than recommended for PFIC was not addressed as the resubmission assumed lower patient weight than that assumed for Progressive Familial Intrahepatic Cholestasis (PFIC) (paragraph 5.11) and the resubmission did not propose a risk sharing arrangement (RSA).

5.4 The PBAC noted that the PBS restriction proposed in the resubmission addressed several of its previous recommendations as outlined in paragraph 3.3. Additionally, the PBAC considered that the following would be appropriate:

- A combined initial and grandfather restriction (see paragraph 3.2).
- Dose reductions should be permitted in all treatment phases and patients should be allowed to continue on the lower dose providing they demonstrate and maintain an adequate clinical response (via the ObSRO score) (see paragraph 3.4).
- An overarching administrative note to capture retreatment can occur in both initial and continuing treatment phases. In the initial treatment phase, retreatment can occur for patients who did not complete a full 3-month of initial therapy (see paragraph 3.5).
- Continuing restriction to include requirement for improvement in pruritic score, and initial restriction to require demonstration of response by 3 cumulative months of treatment
- An overarching administrative note specifying that each subsequent course of continuing treatment requires reassessment of response or disease maintenance at 6-monthly intervals, aligning with the provision of five repeats under the continuing treatment phase (paragraph 3.2)

5.5 The PBAC noted the resubmission's claim that ALGS is clinically distinct from PFIC, particularly in its multisystem involvement, heterogeneity of presentation, broader non-hepatic manifestations, and that it is a predominantly a paediatric disease (see paragraph 4.16). However, the PBAC recalled that odevixibat would be used primarily for the symptomatic management of cholestatic pruritus, and that odevixibat is not considered to be disease-modifying in ALGS and evidence demonstrating improvements in quality-of-life outcomes (QoL) or survival with odevixibat was limited and uncertain (paragraph 7.3 odevixibat PSD, March 2026 PBAC meeting).

5.6 The PBAC recalled that it previously considered the proposed comparator, standard of care (SOC), including medicines used off-label (ursodeoxycholic acid, rifampicin and cholestyramine) and surgical procedures (biliary diversion surgery and liver transplant), was reasonable (paragraph 7.4 odevixibat PSD, March 2026 PBAC meeting).

5.7 The PBAC noted that no additional clinical data was presented in the resubmission, and recalled that submission relied on a single, small, 24 week randomised controlled trial (RCT, ASSERT n=52) of odevixibat vs placebo, supplemented by a 72 week single-arm open-label extension (ASSERT-EXT) (see paragraph 7.6 odevixibat PSD, March 2026 PBAC meeting).

5.8 The PBAC recalled that it previously considered that the claim of superior comparative effectiveness of odevixibat compared to placebo (as a proxy for SOC) was reasonable; however the lack of demonstrated QoL or survival benefit, and short comparative data limited confidence in the clinical relevance and duration of the observed treatment effects beyond the trial setting (see paragraph 7.7 odevixibat PSD, March 2026 PBAC meeting).

5.9 The PBAC recalled that it previously considered that the claim of non-inferior comparative safety was reasonable, while noting that the available evidence was limited by small patient numbers and the short duration of comparative follow-up (paragraph 7.8 odevixibat PSD, March 2026 PBAC meeting).

5.10 At its March 2026 meeting the PBAC advised that the cost per patient per year for odevixibat for ALGS should be no higher than recommended for odevixibat for PFIC assuming the same patient weight (45.1 kg). Because the recommended odevixibat dose for ALGS is three times higher than for PFIC, the cost per microgram for the ALGS indication needed to be one-third of that for the PFIC indication, to achieve the same cost per patient per year. The resubmission proposed a [redacted]% (rather than [redacted]%) lower price for ALGS , and assumed a lower patient weight of 19.8 kg, which resulted in a cost per patient per year of $[redacted].

5.11 The PBAC noted the assumed average weight in the resubmission (19.8 kg) was derived from ALGS specific growth charts from the Global Alagille Alliance (GALA) registry data but the weight of patients at the age of commencement in Australian patients appeared to be higher than in the GALA registry (see paragraph 4.11). The PBAC considered an average weight of 25.5 kg (as calculated in paragraph 4.18) would more reasonably reflect the average weight of Australian patients (over the 6 year time horizon), and that it would be reasonable to use this weight to calculate the cost per patient per year.

5.12 The PBAC recalled that the cost per patient per year recommended for the PFIC indication was approximately $[redacted]. However, in the context of accepting a differential patient weight across the ALGS and PFIC indications, the PBAC considered the cost per patient per year for the ALGS indication would need to be in the order of $[redacted] to be considered cost-effective. The lower cost per patient per year for ALGS takes into

account the higher disease prevalence and the more limited evidence demonstrating broader QoL, survival or long-term clinical benefits when compared to PFIC (see paragraphs 5.5 and 5.8). The PBAC noted this recommendation would result in a higher price per microgram than recommended in March 2026.

5.13 The PBAC considered that the estimated number of patients likely to be treated with odevixibat provided in the resubmission was reasonable. The PBAC considered that the utilisation and financial estimates would need to be updated to (i) correct the errors identified (see paragraph 4.20) (ii) revise the average patient weight as outlined above (i.e. 25.5 kg) (iii) apply the use of whole capsules (see paragraph 4.12), and (iv) apply the reduced, cost-effective price as per paragraph 5.11.

5.14 The PBAC noted the cost effectiveness of odevixibat is highly dependent on patient weight and considered that an RSA would be required to manage uncertainty regarding the average weight of the treated population. The PBAC considered that a [redacted]% rebate for use above agreed expenditure caps (consistent with the PFIC indication) would be appropriate.

5.15 The PBAC considered that odevixibat is not suitable for inclusion in the PBS medicines for prescribing by nurse practitioners.

5.16 The PBAC found that the criteria prescribed by the *National Health (Pharmaceuticals and Vaccines – Cost Recovery) Regulations 2022* for Pricing Pathway A were not met. Specifically, the PBAC found that in the circumstances of its recommendation for odevixibat:

a) The treatment is not expected to provide a substantial and clinically relevant improvement in efficacy compared to best supportive care, because the magnitude of benefit and long-term clinical outcomes were uncertain;

b) The treatment is expected to address a high and urgent unmet clinical need because there are other no treatments for ALGS listed on the PBS;

c) It was not necessary to make a finding in relation to whether it would be in the public interest for the subsequent pricing application to be progressed under Pricing Pathway A because one or more of the preceding tests had failed.

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

Outcome:
Recommended

6 Recommended listing

6.1 Amend existing listing as follows: add new restriction

Initial Treatment and Grandfather Arrangements

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
ODEVIXIBAT					
odevixibat 200 mcg capsule, 30	NEW	12	360	2	Bylvay
odevixibat 400 mcg capsule, 30	NEW	6	180	2	Bylvay
odevixibat 600 mcg capsule, 30	NEW	12	360	2	Bylvay
odevixibat 1200 mcg capsule, 30	NEW	6	180	2	Bylvay

Restriction Summary / Treatment of Concept

Category / Program: Section 85 - General Schedule

Prescriber type: ☒ Medical Practitioners

Restriction type: ☒ Authority Required (in writing via post/HPOS upload or Online PBS Authorities system)

Administrative Advice:

Demonstration of Response following Initial Treatment

- If an adequate clinical response is achieved following the initial treatment phase, odevixibat may be renewed under the continuing treatment phase at a dose of 40 to 120 micrograms/kg/day.
- If a patient fails to demonstrate an adequate clinical response after a cumulative 3 months of treatment with this drug during the initial treatment phase, they will no longer be eligible for further PBS-subsidised therapy for this condition.

Demonstration of Response following the first course of Continuing treatment

- If an adequate clinical response is achieved or maintained following the first course of continuing treatment, odevixibat can be renewed under the continuing treatment phase at a dose of 40 to 120 micrograms/kg/day.
- Patients who are unable to maintain an adequate clinical response with this drug during the continuing treatment phase, will no longer be eligible for further PBS-subsidised therapy for this condition.

Demonstration of Response for subsequent courses of Continuing treatment

- A patient must demonstrate or maintain an adequate clinical response to each course of continuing treatment (every 6 months) at a dose of 40 to 120 micrograms/kg/day, to continue receiving PBS-subsidised treatment.

Recommencing treatment

- For patients who have demonstrated a response to Initial Treatment:
  Patients who have demonstrated an adequate clinical response following 3 cumulative months of treatment and have ceased therapy for reasons other than a lack of response, may recommence through the **continuing treatment phase** at a dose of 40 to120 micrograms/kg/day.
- For patients who have not completed Initial Treatment:
  Patients who have been undergoing treatment for less than 3 cumulative months, may recommence through the **initial treatment phase** at a dose of 40 to 120 micrograms/kg/day. Patients must complete a complete 3-month initial treatment period when recommencing.

Administrative Advice:

See the following article for details of the Prucision™ ObsRO Pruritus Scale:
Gwaltney C, Ivanescu C, Karlsson L, Warholic N, Kjems L, Horn P. Validation of the PRUCISION instruments in paediatric patients with progressive familial intrahepatic cholestasis. Adv Ther. 2022;39(11):5105-5125
The Prucision™ ObsRO Pruritus Scale is available as a downloadable document at:
https://d2rkmuse97gwnh.cloudfront.net/a88aa6d6-3ca0-4362-a711-d53c45ae33ff/f5ea92bf-e36b-421c-a9fe-af77243f0e42/f5ea92bf-e36b-421c-a9fe-af77243f0e42_source__v.docx

Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).
Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au

		Applications for authorisation under this restriction should be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/hpos) Alternatively, applications for authority to prescribe can be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001
		Administrative advice: No increase in the maximum quantity or number of units may be authorised
		Administrative advice: No increase in the maximum number of repeats may be authorised
		Administrative Advice: Special Pricing Arrangements Apply
		Episodicity: n/a
		Severity: n/a
		Condition: Cholestatic pruritus in Alagille syndrome (ALGS)
		Indication: Cholestatic pruritus in Alagille syndrome (ALGS)
		Treatment Phase: Initial treatment
		Clinical criteria:
		Patient must not have received prior PBS-subsidised treatment with this drug for this condition; OR
		Patient must be recommencing treatment with this drug for this condition following a treatment break that occurred before completing 3 cumulative months of therapy under this restriction.
		AND
		Clinical criteria:
		Patient must have a clinical diagnosis of Alagille syndrome (ALGS)
		AND
		Clinical criteria:
		Patient must have/have had elevated serum bile acids at treatment initiation with an Ileal bile acid transporter (iBAT) inhibitor
		AND
		Clinical criteria:
		Patient must be/have been experiencing itch at treatment initiation with an iBAT inhibitor, with other factors causing pruritus excluded through both: (i) physical examination and (ii) patient history
		AND
		Clinical criteria:
		Patient must have had an average pruritus score of ≥2 on the Prucision™ ObsRO Pruritus Scale assessed on three occasions over a one-week period prior to treatment initiation with an iBAT inhibitor
		Treatment criteria:
		Must be treated by a specialist experienced in the management of Alagille syndrome (ALGS), who is either a: (i) gastroenterologist, (ii) hepatologist
		Prescribing Instructions: The authority application must be made via the Online PBS Authorities System, or in writing via HPOS form upload or mail and must include: 1) details of the pruritus score using the Prucision™ ObsRO Pruritus scale 2) details of serum bile acids (date and micromol/L)
		Prescribing Instructions: If the application is submitted through HPOS form upload or mail, it must include: (i) details of the proposed prescription; and (ii) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).
		Prescribing Instructions:

	Prescriber must exclude any other causes of pruritus which include any of the following: (i) drug related (e.g., opioid-related pruritus) (ii) drug hypersensitivity or adverse effect; contact dermatitis; allergy (iii) differential diagnoses (e.g., xerosis; infestations; iron deficiency; chronic kidney disease; polycythaemia vera/leukemia/lymphoma; hypothyroidism; uncontrolled diabetes, eczema, scabies).
	Prescribing Instructions: Ileal bile acid transporter (iBAT) inhibitors that will be accepted for the purposes of administering this restriction are odevixibat and maralixibat
	Prescribing Instructions: At the time of the authority application, the prescriber should request the appropriate strength(s) and quantity based on the patients' weight, according to the dosing schedule in the TGA approved Product Information to provide sufficient drug for one month's supply with 2 repeats. A separate authority approval is required for each strength requested
	Prescribing Instructions: An application for continuing treatment must occur following an assessment of response conducted within 3 cumulative months of therapy under this treatment phase. This will enable ongoing treatment for those who meet the continuing restriction criteria for PBS-subsidised treatment. If a patient fails to demonstrate a response to treatment with this drug for this condition following 3 cumulative months of treatment, they will no longer be eligible to receive further PBS-subsidised treatment.
	Prescribing Instructions: All diagnostic reports/tools must be documented in the patient's medical records.
	Prescribing Instructions: An adequate clinical response to initial treatment is defined as either: i) an average improvement of at least 1 grade in pruritus score from baseline on the Pruritus™ ObsRO Pruritus Scale, assessed on three occasions over a one-week period prior to response assessment; or ii) maintenance of an average of at least 1 grade improvement in pruritus score from baseline on the Pruritus™ ObsRO Pruritus Scale, assessed on three occasions over a one-week period prior to response assessment.
	Prescribing Instructions: Dose reduction to 40 micrograms/kg/day may be considered if tolerability issues occur in the absence of other causes. Once tolerability issues stabilise, patients should be treated at a dose of 40 to120 micrograms/kg/day.
	Prescribing Instructions: Patients may recommence treatment with this drug for this condition through the initial treatment phase at a dose of 40 to120 micrograms/kg/day, if demonstration of response was not assessed due to a treatment break occurring prior to the initial 3 cumulative months of treatment.
	Prescribing Instructions: Patients who have demonstrated an adequate clinical response following 3 cumulative months of initial treatment and have ceased treatment for reasons other than a lack of response, may recommence treatment through the continuing treatment phase.

Continuing Treatment

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
ODEVIXIBAT					
odevixibat 200 mcg capsule, 30	NEW	12	360	5	Bylvay
odevixibat 400 mcg capsule, 30	NEW	6	180	5	Bylvay
odevixibat 600 mcg capsule, 30	NEW	12	360	5	Bylvay
odevixibat 1200 mcg capsule, 30	NEW	6	180	5	Bylvay

Restriction Summary / Treatment of Concept	
	Category / Program: Section 85 - General Schedule
	Prescriber type: ☒ Medical Practitioners
	Restriction type: ☒ Authority Required (telephone/electronic)
	Administrative Advice: OVERARCHING ADMINISTRATIVE NOTE
	Administrative Advice: See the following article for details of the Prucision™ ObsRO Pruritus Scale:

		Gwaltney C, Ivanescu C, Karlsson L, Warholic N, Kjems L, Horn P. Validation of the PRUCISION instruments in paediatric patients with progressive familial intrahepatic cholestasis. Adv Ther. 2022;39(11):5105-5125 The Prucision™ ObsRO Pruritus Scale is available as a downloadable document at: https://d2rkmuse97gwnh.cloudfront.net/a88aa6d6-3ca0-4362-a711-d53c45ae33ff/f5ea92bf-e36b-421c-a9fe-af77243f0e42/f5ea92bf-e36b-421c-a9fe-af77243f0e42_source__v.docx
		Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).
		Administrative advice: No increase in the maximum quantity or number of units may be authorised
		Administrative advice: No increase in the maximum number of repeats may be authorised
		Administrative Advice: Special Pricing Arrangements Apply
		Episodicity: n/a
		Severity: n/a
		Condition: Cholestatic pruritus in Alagille syndrome (ALGS)
		Indication: Cholestatic pruritus in Alagille syndrome (ALGS)
		Treatment Phase: Continuing Treatment
		Clinical criteria:
		Patient must have previously received PBS-subsidised treatment with this drug for this condition
		AND
		Clinical criteria:
		Patient must have demonstrated/maintained an adequate clinical response to the most recent course of treatment
		Treatment criteria:
		Must be treated by a specialist experienced in the management of Alagille syndrome (ALGS), who is either: (i) gastroenterologist, (ii) hepatologist; OR
		Must be treated by a medical practitioner under the supervision of a specialist experienced in the management of Alagille syndrome (ALGS)
		Prescribing Instructions: An adequate clinical response to continuing treatment is defined as either: i) an average improvement of at least 1 grade in pruritus score from the most recent course of treatment on the Pruritus™ ObsRO Pruritus Scale, assessed on three occasions over a one-week period prior to response assessment; or ii) maintenance of an average of at least 1 grade improvement in pruritus score from baseline on the Pruritus™ ObsRO Pruritus Scale, assessed on three occasions over a one-week period prior to response assessment.
		Prescribing Instructions: Confirmation of eligibility for continuing treatment must be provided with the authority application and documented in the patient's medical records (include assessment of adequate clinical response to preceding supply).
		Prescribing Instructions: Dose reduction to 40 micrograms/kg/day may be considered if tolerability issues occur in the absence of other causes. Once tolerability issues stabilise, patients should be treated at a dose of 40 to120 micrograms/kg/day.
		Prescribing Instruction: Patients who have demonstrated an adequate clinical response and have ceased treatment for reasons other than a lack of response may recommence treatment through the continuing treatment phase.

These restrictions may be subject to further review. Should there be any changes made to the restriction the sponsor will be informed.

7 Context for Decision

The PBAC helps decide whether and, if so, how medicines should be subsidised through the Pharmaceutical Benefits Scheme (PBS) in Australia. It considers applications regarding the listing of medicines on the PBS and provides advice about other matters relating to the operation of the PBS in this context. A PBAC decision in relation to PBS listings does not necessarily represent a final PBAC view about the merits of the medicine or the circumstances in which it should be made available through the PBS. The PBAC welcomes applications containing new information at any time.

8 Sponsor's Comment

The sponsor had no comment.