Australian Government
Department of Health, Disability and Ageing

New arrangements for nitisinone (Orfadin®) for the treatment of hereditary tyrosinaemia type 1 (HT1) – Information for prescribers

July 2026

Overview

From 1 August 2026, nitisinone (Orfadin®) for hereditary tyrosinaemia type 1 (HT1) will be available through the Pharmaceutical Benefits Scheme (PBS) under the Section 100 Highly Specialised Drugs (HSD) Program, rather than the Life Saving Drugs Program (LSDP).

This follows a recommendation by the Pharmaceutical Benefits Advisory Committee (PBAC) at its November 2025 meeting.

What is changing?

From 1 August 2026, eligible patients will access nitisinone (Orfadin®) through the PBS instead of the LSDP. Prescribers will need to submit a completed PBS authority application for each patient starting treatment, transitioning from the LSDP or continuing PBS-subsidised treatment.

Authority applications for initial treatment and grandfather arrangements for patients transitioning from the LSDP can be submitted either electronically through the Online PBS Authorities system (OPA) or in writing. Applications submitted through OPA are assessed immediately. Written authority application forms may be submitted through the Health Professional Online Services (HPOS) form upload or sent by post. Applications uploaded through HPOS may take a few days to be assessed, while postal applications may take up to 2 weeks, depending on postal delivery times.

Authority applications for continuing treatment can be submitted electronically using OPA or by calling Services Australia on 1800 700 270 and are assessed immediately.

Any queries concerning prescribing arrangements may be directed to Services Australia on 1800 700 270. Prescribing information (including authority application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au.

Who can prescribe nitisinone (Orfadin®)?

Nitisinone (Orfadin®) can only be prescribed by a physician with expertise in the management of HT1 and patients must be treated in a centre with expertise in genetic metabolic disorders.

When does LSDP supply end?

For patients currently receiving nitisinone (Orfadin®) through the LSDP, LSDP supply will end on 1 November 2026. This gives existing patients 3 months, from 1 August 2026 to 31 October 2026, to transition to PBS supply.

Prescribers are encouraged to transition patients as soon as the PBS listing becomes available on 1 August 2026. This will ensure patients receive authority approval in time and avoid disruption to treatment.

The LSDP will not approve access to nitisinone (Orfadin®) for any new patients from 1 August 2026.

Will patient access to nitisinone (Orfadin®) be impacted by this new arrangement?

There should be no impact on eligible patients' access to continuing treatment, provided patients are transitioned to PBS supply before LSDP supply ends.

For existing patients currently receiving treatment through the LSDP

To ensure a smooth transition, prescribers should schedule an appointment with existing LSDP patients as soon as possible on or after 1 August 2026 to apply for a PBS prescription.

Until 31 October 2026, the LSDP will continue to support access for existing patients through the nominated authorised pharmacies. Once a patient has an approved PBS prescription, their LSDP supply will cease.

A PBS co-payment will apply, in line with other PBS medications.

For new patients who require access to nitisinone (Orfadin®) prior to 1 August 2026

Prior to 1 August 2026, treating physicians can apply for access through the LSDP for patients who meet the LSDP eligibility requirements.

For new patients who require access to nitisinone (Orfadin®) on or after 1 August 2026

From 1 August 2026, the LSDP will no longer accept applications for new patients to commence treatment with nitisinone (Orfadin®).

New patients will need to access nitisinone (Orfadin®) through the PBS. Prescribers should follow the PBS authority approval process outlined above and confirm that patients meet all the PBS eligibility criteria.

How often will I need to see my patient to renew their prescription?

Under the LSDP, medical practitioners must apply annually on behalf of patients to access nitisinone (Orfadin®). Under the PBS, the frequency of the prescription will be up to every 6 months depending on the treatment phase as set out in the PBS Schedule for nitisinone (Orfadin®).

Are the PBS eligibility criteria for nitisinone (Orfadin®) the same as the LSDP criteria?

The PBS eligibility criteria for access to nitisinone (Orfadin®) are similar to the LSDP eligibility criteria. The main differences are:

- No restrictions on the laboratory conducting urine and/or blood tests.
- Test results or diagnostic reports confirming a patient's initial and ongoing PBS eligibility need only be documented in the patient's medical records.

What information do medical practitioners have to provide when seeking the PBS authority approval?

Patients must have a confirmed diagnosis of HT1, based on the presence of succinylacetone in urine or blood.

Prescribers must ensure that patients meet the PBS eligibility criteria and document the diagnostic reports, including blood and/or urine test results, in the patient's medical records.

Prescribers are required to request the appropriate quantity and strength of nitisinone, based on the patient's weight, to provide up to 4 weeks of treatment according to the dosage regimen specified in the approved Therapeutic Goods Administration (TGA) Product Information (PI). Up to 5 repeats will be authorised, providing a total of 24 weeks of treatment per prescription. A separate authority prescription form must be completed for each strength requested.

The oral liquid formulation of nitisinone is only available through the PBS for patients under 18 years of age.

The PBS listing of nitisinone (Orfadin®) provides access through:

- an initial treatment phase for new patients, including those who commenced immediate/emergency non-PBS treatment with nitisinone in hospital, provided they meet the eligibility criteria;
- a grandfather arrangement for patients transitioning from LSDP-funded treatment;
- a continuing treatment phase for patients who meet the continuing treatment eligibility criteria and wish to continue nitisinone (Orfadin®) through the PBS.

The eligibility criteria are outlined below.

Initial treatment

Prescribers must assess and document the following in the patient's medical records:

- The patient must have a confirmed diagnosis of HT1 based on the presence of succinylacetone in either: (i) urine, (ii) blood; **and**
- The treatment must be in combination with dietary restriction of tyrosine and phenylalanine; **and**
- The patient must not be suffering from any other severe or life-threatening medical condition, including complications or sequelae of HT1, that might compromise the effectiveness of treatment with this drug or where the long-term prognosis is unlikely to be altered by treatment with this drug.

Patients transitioning from LSDP-funded treatment (grandfather arrangement)

Patients may access PBS-subsidised treatment under the grandfather arrangement.

Prescribers must assess and document that the following criteria are met:

- The patient must have demonstrated clinical improvement or stabilisation of the condition; **and**
- The patient must not have developed another life threatening/severe disease where long term prognosis is unlikely to be influenced by this drug; **and**
- The treatment must be in combination with dietary restriction of tyrosine and phenylalanine.

Can patients receive continuing treatment?

Patients can receive continuing treatment with nitisinone (Orfadin®) by qualifying under the continuing treatment restriction criteria, outlined below.

Continuing treatment

- The patient must have received prior PBS-subsidised treatment with this drug for this condition; **and**
- The patient must have demonstrated clinical improvement or stabilisation of the condition, the details of which must be kept with the patient's record; **and**
- The patient must not have developed another life threatening/severe disease where long term prognosis is unlikely to be influenced by nitisinone (Orfadin®); **and**
- The treatment must be in combination with dietary restriction of tyrosine and phenylalanine.

Liver function tests (LFTs), full blood count (FBC) and alpha-fetoprotein (AFP) results confirming eligibility for continuing treatment must also be documented in the patient's medical records.

- The PBS item codes for nitisinone presentations under S100 HSD Public (HB) and Private (HS) hospitals are as follows:
  - **Nitisinone 2 mg capsule:** 15482W (HB) and 15481T (HS);
  - **Nitisinone 5 mg capsule:** 15460Q (HB) and 15425W (HS);
  - **Nitisinone 10 mg capsule:** 15435J (HB) and 15467C (HS);
  - **Nitisinone 20 mg capsule:** 15501W (HB) and 15459P (HS);
  - **Nitisinone 4 mg/mL oral liquid:** 15426X (HB) and 15502X (HS).

From 1 August 2026, further details will be available in the PBS Schedule.

Is there a change to the cost for patients?

Yes. As with other PBS-subsidised medicines, patients are required to make a co-payment towards the cost of their treatment with nitisinone (Orfadin®).

In 2026, the co-payment is $25.00 or $7.70 for patients with a concession card, for each dispensing of a prescription.

Patient contributions for PBS prescriptions for nitisinone (Orfadin®) will count towards the patient's PBS Safety Net threshold.

Patients can seek further information from their local pharmacy, the PBS Information telephone line on 1800 020 613, the PBS website at www.pbs.gov.au or the Services Australia website at www.servicesaustralia.gov.au.

Will pharmacies stock these medicines?

Yes. PBS-subsidised nitisinone (Orfadin®) can be dispensed by community pharmacies, or alternatively by:

- public hospital pharmacies for eligible patients receiving treatment at or from public hospitals (as day admitted patients, non-admitted patients or patients on discharge); and
- private hospital pharmacies for eligible patients receiving treatment in, at or from private hospitals.

Patients should contact their chosen pharmacy to confirm availability and ongoing supply arrangements.

What information or support is available for my patients?

Patients can access a factsheet about the changes to accessing nitisinone (Orfadin®) through the PBS at www.health.gov.au/initiatives-and-programs/life-saving-drugs-program/for-patients.

Patients can also seek further advice from their pharmacist.