CONSULTATION

PHARMAC
Pharmaceutical Management Agency

7 August 2014

Deferasirox (Exjade)

PHARMAC is proposing to list deferasirox (Exjade) on the Pharmaceutical Schedule for patients with chronic transfusional iron overload due to congenital inherited anaemias.

- Deferasirox (Exjade) would be listed in Section B and Part II of Section H of the Pharmaceutical Schedule from 1 November 2014 at the following prices and subsidies (ex-manufacturer and excluding GST):

Chemical	Presentation	Brand	Strength	Pack size	Price and subsidy
Deferasirox	Tablet	Exjade	125 mg	28	$276.00
Deferasirox	Tablet	Exjade	250 mg	28	$552.00
Deferasirox	Tablet	Exjade	500 mg	28	$1,105.00

- A confidential rebate would apply to Exjade which would reduce the net price of the treatment.
- Exjade would have subsidy and delisting protection until 31 October 2017.
- Deferasirox would be listed subject to the following restrictions in Section B and Part II of Section H of the Pharmaceutical Schedule:

Section B

Special Authority for Subsidy
Initial application only from a haematologist. Approvals valid for 2 years for applications meeting the following criteria:
All of the following:

1. The patient has been diagnosed with chronic transfusional iron overload due to congenital inherited anaemia; and
2. Deferasirox is to be given at a daily dose not exceeding 40 mg/kg/day; and
3. Any of the following:
   - 3.1. Treatment with maximum tolerated doses of deferiprone monotherapy or deferiprone and desferrioxamine combination therapy have proven ineffective as measured by serum ferritin levels, liver or cardiac MRI T2*; or
   - 3.2. Treatment with deferiprone has resulted in severe persistent vomiting or diarrhoea; or
   - 3.3. Treatment with deferiprone has resulted in arthritis; or
   - 3.4. Treatment with deferiprone is contraindicated due to a history of agranulocytosis (defined as an absolute neutrophil count (ANC) of < 0.5 cells per µL) or recurrent episodes (greater than 2 episodes) of moderate neutropenia (ANC 0.5 - 1.0 cells per µL).

Renewal only from a haematologist. Approvals valid for 2 years for applications meeting the following criteria:
Either:

1. For the first renewal following 2 years of therapy, the treatment has been tolerated and has resulted in clinical improvement in all three parameters namely serum ferritin, cardiac MRI T2* and liver MRI T2* levels; or

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2. For subsequent renewals, the treatment has been tolerated and has resulted in clinical stability or continued improvement in all three parameters namely serum ferritin, cardiac MRI T2* and liver MRI T2* levels.

Part II of Section H

Initiation
Haematologist
Re-assessment required after 2 years
All of the following:

1. The patient has been diagnosed with chronic transfusional iron overload due to congenital inherited anaemia; and
2. Deferasirox is to be given at a daily dose not exceeding 40 mg/kg/day; and
3. Any of the following:
   - 3.1. Treatment with maximum tolerated doses of deferiprone monotherapy or deferiprone and desferrioxamine combination therapy have proven ineffective as measured by serum ferritin levels, liver or cardiac MRI T2*; or
   - 3.2. Treatment with deferiprone has resulted in severe persistent vomiting or diarrhoea; or
   - 3.3. Treatment with deferiprone has resulted in arthritis; or
   - 3.4. Treatment with deferiprone is contraindicated due to a history of agranulocytosis (defined as an absolute neutrophil count (ANC) of < 0.5 cells per µL) or recurrent episodes (greater than 2 episodes) of moderate neutropenia (ANC 0.5 - 1.0 cells per µL)

Continuation
Haematologist
Re-assessment required after 2 years
Either:

1. For the first renewal following 2 years of therapy, the treatment has been tolerated and has resulted in clinical improvement in all three parameters namely serum ferritin, cardiac MRI T2* and liver MRI T2* levels; or
2. For subsequent renewals, the treatment has been tolerated and has resulted in clinical stability or continued improvement in all three parameters namely serum ferritin, cardiac MRI T2* and liver MRI T2* levels.

About deferasirox

Deferasirox is an oral iron chelator (binder) used to remove excess iron from the body.

The listing of deferasirox for patients with iron overload due to congenital inherited anaemias would provide another treatment option for patients who have not responded to an alternative funded oral iron chelator, deferiprone, or who cannot take deferiprone due to contraindication or intolerance.

PTAC and the Cancer Treatments Subcommittee of PTAC (CaTSoP) have reviewed deferasirox on a number of occasions. Most recently, at its April 2011 review, CaTSoP recommended that deferasirox be funded with high priority for patients with chronic transfusional iron overload due to congenital inherited anaemia who have not responded to deferiprone therapy and for patients who are intolerant or contraindicated to deferiprone. PTAC recommended a small amendment to the funding criteria proposed by CaTSoP but otherwise accepted CaTSoP's recommendation.

The minutes for the relevant reviews can be found on the PHARMAC website through the following links:

- www.pharmac.govt.nz/2011/11/04/April%20CaTSoP%20Subcommittee%20web%20minutes.pdf
- www.pharmac.health.nz/assets/ptac-minutes-2011-08.pdf