Avatrombopag (immune thrombocytopenia)

Resolution of: 16 September 2021 Valid until: unlimited
Entry into force on: 16 September 2021
BAnz AT 14 10 2021 B3

Therapeutic indication (according to the marketing authorisation of 20 June 2019):

Doptelet is indicated for the treatment of severe thrombocytopenia in adult patients with chronic liver disease who are scheduled to undergo an invasive procedure.

Therapeutic indication (according to the marketing authorisation of 18 January 2021):

Doptelet is indicated for the treatment of primary chronic immune thrombocytopenia (ITP) in adult patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins).

Therapeutic indication of the resolution (resolution from the 16 September 2021):

Doptelet is indicated for the treatment of primary chronic immune thrombocytopenia (ITP) in adult patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins).

1. Additional benefit of the medicinal product in relation to the appropriate comparator therapy

Adult patients with primary chronic immune thrombocytopenia (ITP) who are refractory to other treatments (e.g. corticosteroids, immunoglobulins)

Appropriate comparator therapy:

Eltrombopag or romiplostim

Extent and probability of the additional benefit of avatrombopag compared to the appropriate comparator therapy:

An additional benefit is not proven.

Study results according to endpoints:

Adult patients with primary chronic immune thrombocytopenia (ITP) who are refractory to other treatments (e.g. corticosteroids, immunoglobulins)

No adequate data are available to allow an assessment of the additional benefit.

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ risk of bias	Summary
Mortality	n.a.	There are no assessable data.
Morbidity	n.a.	There are no assessable data.
Health-related quality of life	n.a.	There are no assessable data.
Side effects	n.a.	There are no assessable data.
Explanations: ↑: statistically significant and relevant positive effect with low/unclear reliability of data ↓: statistically significant and relevant negative effect with low/unclear reliability of data ↑↑: statistically significant and relevant positive effect with high reliability of data ↓↓: statistically significant and relevant negative effect with high reliability of data ↔ : no statistically significant or relevant difference ∅: There are no usable data for the benefit assessment. n.a.: not assessable		

2. Number of patients or demarcation of patient groups eligible for treatment

Adult patients with primary chronic immune thrombocytopenia (ITP) who are refractory to other treatments (e.g. corticosteroids, immunoglobulins)

approx. 4,260 – 10,830 patients

3. Requirements for a quality-assured application

The requirements in the product information are to be taken into account. The European Medicines Agency (EMA) provides the contents of the product information (summary of product characteristics, SmPC) for Doptelet® (active ingredient: avatrombopag) at the following publicly accessible link (last access: 11 June 2021):

https://www.ema.europa.eu/en/documents/product-information/doptelet-epar-product-information_en.pdf

Treatment with avatrombopag should be started and continuously monitored by doctors experienced in the treatment of haematological diseases.

4. Treatment costs

Annual treatment costs:

Adult patients with primary chronic immune thrombocytopenia (ITP) who are refractory to other treatments (e.g. corticosteroids, immunoglobulins)

Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Avatrombopag	€ 6,034.50 - 84,715.04
Appropriate comparator therapy:	
Eltrombopag	€ 8,297.73 - € 49,303.20
Romiplostim	€ 23,307.46 - 146,233.37

Costs after deduction of statutory rebates (LAUER-TAXE®) as last revised: 1 September 2021)

Costs for additionally required SHI services: not applicable