SUMMARY

ADAMTS13r

ADZYNMA 500 IU and 1,500 IU

powder and solvent for solution for injection

Inclusion on list

Adopted by the Transparency Committee on 15 January 2025

Summary of opinion

Favourable opinion for reimbursement as "enzyme replacement therapy (ERT) for the treatment of ADAMTS13 deficiency in children and adult patients with congenital thrombotic thrombocytopenic purpura (cTTP). ADZYNMA can be used for all age groups."

Transparency Committee's conclusions

Clinical Benefit

- cTTP is a disease that is spontaneously fatal without treatment, with onset in childhood in the majority of cases and a high mortality in the under 20s (56%). The clinical presentation is variable, and can involve several organs (brain and heart especially) with a significant impact on the quality of life of patients and their families.
- This is a curative and preventive medicinal product.
- The efficacy/adverse effects ratio is high.
- This is a first-line treatment in view of the therapies available.

Public health benefit

Considering:

- the seriousness of the disease, its incidence estimated to be 1 to 2 new cases per year, and with 80 to 90 patients currently diagnosed in France,
- the medical need partially met by plasma-based therapies given the limitations identified for this treatment,
- the expected response to the identified need given:
  - an expected additional impact on morbidity and mortality (need for longer-term data)
  - an expected additional benefit on quality of life and the organisation of care, with, in particular, the possibility of home administration of ADZYNMA (rADAMTS13) and of self-administration under the supervision of a healthcare professional, which may facilitate access to the treatment and reduce the number of hospitalisations, prescribed sick leave and absences from work or school, as well as anxiety. However, this potential impact remains to be confirmed in real world conditions, particularly in view of the need to implement a specific

community medicine-hospital pathway for patients in order to avoid delays in diagnosis and management of acute/subacute episodes in the event of laboratory workups performed in the community setting and to facilitate compliance with treatment.

ADZYNMA (rADAMTS13) is likely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of ADZYNMA 500 IU and 1,500 IU (rADAMTS13) is substantial in the MA indication.

The Committee issues a favourable opinion for inclusion of ADZYNMA 500 IU and 1,500 IU (rADAMTS13) in both the list of covered drugs for outpatients and the list of covered drugs for inpatients approved for use in the MA indication and at the MA dosages.

➔ **Recommended reimbursement rate for inclusion in the list of covered drugs for outpatients: 65%**

Clinical Added Value

Considering:

- comparative efficacy results versus the standard of care (plasma-based therapy) observed in the pivotal crossover study (281102):
  - no acute TTP episodes occurring during the periods of prophylactic treatment with rADAMTS13 versus one episode during the periods of prophylactic treatment with the standard of care (plasma-based therapy),
  - one confirmed episode, treated and resolved by rADAMTS13 in 3 days versus 2 confirmed acute TTP episodes, treated and resolved by the standard of care (plasma-based therapy) in 14.8 days and 1.5 days,
  - the lower incidence of subacute episodes and isolated clinical manifestations with rADAMTS13 versus standard of care (plasma-based therapy),
- the known limitations of the current standard of care (plasma-based therapy) in terms of efficacy, safety, quality of life, socioeconomic impact and accessibility,
- the efficacy results observed in the extension study (3002), supporting those of the pivotal study, with a single confirmed acute TTP episode occurring in a patient in the prophylactic treatment cohort out of 65 patients included in the cohort,
- the more favourable safety profile of rADAMTS13 compared to plasma-based therapy,
- the purely descriptive nature of the results of the two interim analyses, with no formalised statistical hypothesis, limiting interpretation of the comparative results,
- the lack of robust data demonstrating an additional impact on quality of life and the organisation of care, although this impact is expected,
- the absence of data on morbidity and mortality in terms of the incidence of complications,
- uncertainties with respect to the long-term safety, particularly concerning the development of anti-rADAMTS13 neutralising antibodies,

the Committee deems that ADZYNMA 500 IU and 1,500 IU (rADAMTS13) provides a moderate clinical added value (CAV III) in the current care pathway for congenital thrombotic thrombocytopenic purpura (cTTP) due to ADAMTS13 deficiency, which includes relevant comparators

(paragraph Erreur ! Source du renvoi introuvable.**) as prophylactic treatment and on demand in the event of an acute cTTP episode.**