SUMMARY

riociguat

ADEMPAS 0.5 mg, 1 mg, 1.5 mg, 2 mg and 2.5 mg

film-coated tablets

Indication extension

Original French opinion adopted by the Transparency Committee on 28 February 2024

The legally binding text is the original French opinion version

Summary of opinion

Favourable opinion for reimbursement in the treatment of PAH in children and adolescents from 6 years to less than 18 years of age and body weight ≥ 50 kg with WHO Functional Class (FC) II to III in combination with endothelin receptor antagonists.

Transparency Committee's conclusions

Clinical Benefit

- PAH is a rare, potentially life-threatening lung disease, characterised by an increase in pulmonary arterial pressures and potentially resulting in right heart failure. Asthenia, progressive exertional dyspnoea, chest pain, and fainting are the most common clinical signs.
- The proprietary medicinal product ADEMPAS (riociguat) is a symptomatic medicinal product.
- The efficacy of ADEMPAS (riociguat) is currently established solely on the basis of pharmacokinetic data in the paediatric population. The safety profile of riociguat in children and adolescents is known and is similar to that observed in adults. Thus, the efficacy/adverse effect ratio is moderate.
- There are therapeutic alternatives.
- It is a first-line treatment.

➔ Public health benefit

Considering:

- the seriousness of the disease and its very low prevalence;
- the partially met medical need among children with PAH;
- the lack of response to the identified need due to a lack of evidence of an impact on morbidity and mortality among treated children in the absence of comparative data;
- the lack of evidence of an impact on quality of life and on the organisation of care;

ADEMPAS (riociguat) is unlikely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of ADEMPAS (riociguat) film-coated tabletsis moderate in the treatment of PAH in children and adolescents less than 18 years of age and body weight ≥ 50 kg with WHO Functional Class (FC) II to III in combination with endothelin receptor antagonists.

The Committee issues a favourable opinion for inclusion of ADEMPAS (riociguat) film-coated tablets in both the list of covered drugs for outpatients and the list of covered drugs for in-patients approved for use in the abovementioned MA paediatric indication and at the MA dosages.

➔ Recommended reimbursement rate for inclusion in the list of covered drugs for outpatients: 30%.

Clinical Added Value

Considering:

- the results observed in the paediatric population compared to those in adults in a study assessing the pharmacokinetics of riociguat, suggesting the transposability to the paediatric population of the clinical results demonstrated in adults;
- the lack of evidence of clinical efficacy of ADEMPAS (riociguat), particularly in terms of morbidity and mortality, in children and adolescents with WHO Functional Class (FC) II to III PAH less than 18 years of age and body weight ≥ 50 kg, due to the absence of comparative data;
- the safety profile that appears to be similar to that of adults;

the Committee deems that ADEMPAS (riociguat) film-coated tablets provide no clinical added value (CAV V) compared to the relevant comparators (see paragraph 5.2) in the current care pathway in children and adolescents with WHO Functional Class (FC) II to III pulmonary arterial hypertension less than 18 years of age and body weight ≥ 50 kg.