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Acoziborole Winthrop (*acoziborole)*

A plain-language overview of Acoziborole Winthrop and why it received a positive opinion

What is Acoziborole Winthrop and what is it used for?

Acoziborole Winthrop is a medicine used to treat human African trypanosomiasis (sleeping sickness) caused by the parasite *Trypanosoma brucei gambiense* (g-HAT) in adults and in adolescents from 12 years of age weighing at least 40 kg. The medicine is intended for use outside the European Union (EU).

g-HAT is spread through the bite of tsetse flies infected with the parasite. In the first few days after infection, patients may have symptoms such as fever, headache and rash (first stage). Later, they may experience a more severe form of the disease which affects the nervous system, causing symptoms such as sleep disturbances and changes in behaviour (second stage).

Acoziborole Winthrop can be used to treat both first-stage and second-stage g-HAT, including severe second stage.

Acoziborole Winthrop contains the active substance acoziborole.

How is Acoziborole Winthrop used?

Acoziborole Winthrop should only be prescribed and administered by healthcare professionals experienced in the management and treatment of HAT.

Acoziborole Winthrop is available as tablets to be taken by mouth. Treatment consists of a single dose.

Arrangements for supply of the medicine will be the responsibility of national medicines regulators.

For more information about using Acoziborole Winthrop, see the package leaflet or contact your healthcare provider.

How does Acoziborole Winthrop work?

The exact way that Acoziborole Winthrop works is not fully understood. However, the active substance, acoziborole, is thought to bind to and block the activity of an enzyme (protein) that the parasite needs to produce essential proteins, causing the parasite to die.

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What benefits of Acoziborole Winthrop have been shown in studies?

A main study involving 208 adults and adolescents from 15 years of age with g-HAT found that a single dose of Acoziborole Winthrop was effective at treating the infection. The study, which was carried out in the Democratic Republic of Congo and Guinea, did not compare Acoziborole Winthrop with another medicine or placebo (a dummy treatment).

Eighteen months after a single dose of the medicine, treatment was successful in around 95% (159 out of 167) of people with second-stage disease, including severe second stage, and in 100% (41 out of 41) of people with first- and very early second-stage g-HAT. Treatment success was based on the patient being alive, with no clinical signs of g-HAT, no detectable parasites and a white blood cell count below a set limit.

Supportive data also showed that the medicine is expected to behave in the same way in adolescents aged 12 to 17 and weighing at least 40 kg as it does in adults.

Studies carried out with Acoziborole Winthrop are described in more detail in the medicine's assessment report.

What are the side effects and restrictions with Acoziborole Winthrop?

For the full list of side effects and restrictions with Acoziborole Winthrop, see the package leaflet.

The most common side effects with Acoziborole Winthrop (which may affect more than 1 in 10 people) include changes to the heart's electrical activity seen on an electrocardiogram (ECG). Other common side effects (which may affect up to 1 in 10 people) include headache, fever, asthenia (weakness), decreased appetite and tremor (shaking).

The medicine must not be used in people with a familial short QT syndrome (an abnormal electrical activity of the heart that affects its rhythm), nor must it be taken with nifedipine, a medicine used to treat heart conditions. Because Acoziborole Winthrop can affect the way the body processes some medicines, it must not be used with certain medicines used to treat HIV, hepatitis C, tuberculosis, parasite infections or fungal infections; these medicines should not be used for 3 months after taking Acoziborole Winthrop. It should also not be taken with the combination artemether and lumefantrine, which is used to treat malaria; this treatment should not be started within 1 month of taking Acoziborole Winthrop.

Why did Acoziborole Winthrop receive a positive opinion?

Current therapies for g-HAT involve multiple doses and may require intravenous infusions (drip into a vein) or intramuscular injections (injections into a muscle), as well as hospitalisation. They are selected based on disease stage, which is determined by a lumbar puncture (spinal tap). Acoziborole Winthrop is given as a single oral dose and has been shown to be effective at treating all stages of g-HAT, removing the need for lumbar puncture and hospitalisation. Although there are some risks associated with using this medicine, including changes to the heart's electrical activity and interactions with other medicines, recommendations on how to manage these risks have been adequately described in the product information.

The European Medicines Agency therefore decided that Acoziborole Winthrop's benefits are greater than its risks and issued a positive opinion.

What measures are being taken to ensure the safe and effective use of Acoziborole Winthrop?

The company that markets Acoziborole Winthrop will carry out a study to evaluate information on the real-world safety of the medicine during the 3-month period after treatment.

The company will also provide a patient card to remind patients and healthcare professionals that certain medicines must not be used together with Acoziborole Winthrop or for a certain period of time (up to 3 months) after treatment.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Acoziborole Winthrop have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Acoziborole Winthrop are continuously monitored. Suspected side effects reported with Acoziborole Winthrop are carefully evaluated and any necessary action taken to protect patients.

Other information about Acoziborole Winthrop

The European Medicines Agency gave a positive opinion for Acoziborole Winthrop on 24 February 2026.

The Agency assessed Acoziborole Winthrop as part of its cooperation with the World Health Organization, whereby the Agency evaluates medicines that are not intended for use in the EU but are needed to prevent or treat diseases of major public health importance around the world.

Further information on Acoziborole Winthrop, including the package leaflet and assessment report, can be found on the Agency's website: ema.europa.eu/opinion-medicine-use-outside-EU/human/acoziborole-winthrop.

This overview was last updated in 03-2026.