21 August 2026
EMADOC-628903358-142631
EMEA/H/C/005909

Withdrawal of application for the marketing authorisation of Isembyld (Apitegromab)

Scholar Rock Netherlands B.V. withdrew its application for a marketing authorisation of Isembyld for the treatment of 5q spinal muscular atrophy (SMA), a genetic disease that causes weakness and wasting of the muscles including the lung muscles.

The company withdrew the application on 13 August 2026.

What is Isembyld and what was it intended to be used for?

Isembyld has been developed as a medicine to be used in people aged 2 years and older for the treatment of 5q SMA, a condition which is linked to a defect on chromosome 5q.

Symptoms of 5q SMA usually start shortly after birth. Isembyld was to be used in patients who could not walk on their own without assistance and were already taking a medicine to increase levels of the SMN protein, which is important for muscle function, by acting on the *SMN2* gene.

Isembyld contains the active substance apitegromab and was to be available as an infusion (drip) given into a vein.

Isembyld was designated an 'orphan medicine' (a medicine used in rare diseases) on 14 December 2018 for the treatment of SMA. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu3182115

How does Isembyld work?

The active substance in Isembyld, apitegromab, is a type of protein which is designed to attach to inactive forms of myostatin, a protein that limits muscle growth. By preventing these inactive forms from becoming active, apitegromab reduces the effects of myostatin. This was expected to prevent muscle loss and maintain muscle mass.

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What did the company present to support its application?

The company presented findings from one main study involving 188 children aged 2 to 12 years of age with 5q SMA who could not walk on their own without assistance. In the study patients received treatment with either Isembyld or placebo (a dummy treatment) in addition to background treatment for 5q SMA (i.e. medicines containing nusinersen or risdiplam). The main measure of effectiveness was the change in the Hammersmith Functional Motor Scale Expanded (HFMSE), a scale used to assess movement abilities and monitor disease progression in people with SMA. The scale evaluates 33 motor activities and gives a total score ranging from 0 to 66, with higher scores indicating better physical function.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the European Medicines Agency had evaluated the information from the company and prepared questions for the company. After the Agency had assessed the company's responses to the last round of questions, there was still an unresolved issue.

What did the Agency recommend at that time?

Based on the review of the data and the company's response to the Agency's questions, at the time of the withdrawal, the Agency's provisional opinion was that Isembyld could not have been authorised for the treatment of 5q SMA. This is because the company had not yet provided confirmation that the manufacturing site used to make the medicine complied with EU good manufacturing practice (GMP) requirements.

Therefore, at the time of the withdrawal, the Agency's opinion was that the company had not provided enough data to support the application for Isembyld.

What were the reasons given by the company for withdrawing the application?

In its letter notifying the Agency of the withdrawal of the application, the company stated that the manufacturing site used to make the medicine had not demonstrated compliance with EU GMP requirements within the required time limit.

Does this withdrawal affect patients in clinical trials or compassionate use programmes?

The company informed the Agency that there are no consequences for patients in clinical trials or in compassionate use programmes using Isembyld.

If you are in a clinical trial or compassionate use programme and need more information about your treatment, speak with your clinical trial doctor.