



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Diacerein for the treatment of epidermolysis bullosa

On 8 November 2018, the Committee for Orphan Medicinal Products (COMP) adopted a negative opinion on the orphan designation application for diacerein for the treatment of epidermolysis bullosa. A negative decision was issued by the European Commission on 22 March 2019.

The sponsor applied for orphan designation on the basis of the seriousness and the rarity of the condition.

The negative opinion was based on the following reason:

- The sponsor failed to demonstrate that the medicine could successfully treat patients with epidermolysis bullosa. The sponsor presented evidence from the scientific literature showing that a different formulation of diacerein was effective at treating blisters in patients with the condition; however, it did not demonstrate that the proposed formulation would be equally effective.

Requests for designation as an orphan medicinal product are made for investigational products. Absence of orphan designation does not preclude the development of this product, including its use in clinical trials. A marketing authorisation can still be obtained if quality, safety and efficacy are demonstrated.

For more information:

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on [the EMA website](#).

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- [Orphanet](#), a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- [European Organisation for Rare Diseases \(EURORDIS\)](#), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

