



Company disagreement on medicines not validated as ultra-orphan

Appeal process

1. Request to reconsider validation outcome

If the company disagrees when SMC has not validated a medicine as ultra-orphan, the company may request that this is reconsidered. The company should provide a short statement to explain the basis of the disagreement within three months of being notified of the validation outcome.

2. Appeal process

The appeal panel will consist of three members of the SMC executive who did not participate in the initial validation, including the SMC Chair. The panel will review the proforma initially provided by the company to support ultra-orphan status, together with responses from clinical experts. No new data can be submitted as part of the appeal. Conflicts of interest will be handled in line with standard SMC arrangements.

3. Conclusion of appeal

The appeal panel will reach a consensus view on whether the medicine meets the ultra-orphan criteria. The company and NHS boards will be advised of the conclusion.

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