**Proforma: evidence to support SMC ultra-orphan status**

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| **Medicine (generic, proprietary name and formulation)** |  |
| **Submitting company** |  |
| **Indication**  |  |
| **Date supporting evidence for ultra-orphan status provided** |  |

Medicines for the treatment of extremely rare conditions must be validated as ultra-orphan prior to initial assessment and entry to the ultra-orphan pathway. Guidance on the ultra-orphan pathway is available on our [website](https://www.gov.scot/publications/ultra-orphan-medicine-pathways-guidance/).

All criteria listed below should be met to qualify as an ultra-orphan medicine. **Supporting evidence for each of the criteria must be provided and referenced appropriately**. Copies of all references should be sent to his.smcsubmissionportal@nhs.scot in a zipped file at the same time as returning the completed proforma.

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| **Criteria** | **Supporting evidence** |
| 1. **The condition has a prevalence of 1 in 50,000 or less in Scotland**
* *This criterion refers to the prevalence of the* ***condition*** *which may be broader than the population specified within the licensed indication.*
* *SMC considers the orphan condition reported in the Medicine and Healthcare products Regulatory Agency’s (MHRA) Orphan Register. This is typically a recognised distinct disease or syndrome.*
* *If the company submits the ultra-orphan proforma to SMC for validation in advance of the MHRA granting a Great Britain (GB) orphan marketing authorisation, then the relevant European Medicine Agency (EMA) orphan designated condition can be referenced.*
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| 1. **The medicine has a GB orphan marketing authorisation for the indication**
* *Provide GB orphan designation number and date*
* *If the company submits the ultra-orphan proforma to SMC for validation in advance of the MHRA granting a GB orphan marketing authorisation, then the relevant EMA orphan designation can be referenced.*
* *The final decision will be contingent on the MHRA granting a GB orphan marketing authorisation*
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| 1. **The condition is chronic and severely disabling**
* *Outline how the nature of the condition meets this description, for example:*
	+ *it is a persistent condition requiring ongoing management which may include medicines and/or other treatments or supportive care*
	+ *it limits the patient’s ability to undertake activities of daily living*
* *300 words maximum*
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| 1. **The condition requires highly specialised management**
* *Briefly outline how Scottish patients are managed at present*
* *If management is not within the context of a* [*nationally funded service*](https://www.nsd.scot.nhs.uk/services/specialised/index.html) *then indicate why it is considered as highly specialised*
* *300 words maximum*
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**References**

*List below*