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

|  |  |
| --- | --- |
| **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](mailto:his.smcsubmissionportal@nhs.scot) in a zipped file at the same time as returning the completed proforma.

|  |  |
| --- | --- |
| **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.* |  |
| 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* |  |
| 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* |  |
| 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* |  |

**References**

*List below*