

... about ultra-orphan medicines

- ◆ Ultra-orphan medicines are orphan products licensed for the treatment of diseases that have a UK prevalence of fewer than 1 in 50000 (i.e. a prevalence in Wales of approximately 60 cases).
- ◆ Consideration of orphan and ultra-orphan products for use in NHS Wales is based on the same clinical criteria as other products. However, ultra-orphan medicines are invariably expensive and cost-effectiveness measures almost always exceed conventional thresholds.
- ◆ Ultra-orphan medicines are reviewed in Wales on a case-by-case basis. For those shown not to be cost-effective, the All Wales Medicines Strategy Group makes additional considerations. In order of priority, these are:
 1. The degree of severity of the untreated disease, in terms of quality of life and survival.
 2. Whether the drug can reverse, as opposed to stabilise, the condition.
 3. The overall budget impact.
 4. Whether the drug may bridge a gap to a "definitive" therapy (e.g. gene therapy), which is currently in development.
 5. The innovative nature of the drug.

If an ultra-orphan medicine is approved for use, details of patient therapy should be entered into a registry for recording prospective measures of clinical outcome. A date may be set for reviewing additional clinical trial evidence or audit data.