

... about orphan medicines

- ◆ Orphan medicines are products that have not been "adopted" by the pharmaceutical industry because they are used for rare diseases with markets that yield little financial return on investment.
- ◆ Orphan diseases are defined by the European Committee for Orphan Medicinal Products as severe or life-threatening conditions that affect fewer than 5 in 10000 people.
- ◆ There are more than 5000 orphan diseases, of which approximately 80% are genetic. (Many of these diseases are considered 'very rare' and orphan medicines used for their treatment are termed 'ultra-orphan'.)

- ◆ European Union policy supports research and development of orphan medicines by providing incentives to pharmaceutical companies.

These incentives include market exclusivity for 10 years, reductions in license fees, assistance with marketing applications, direct access to the centralised procedure for marketing authorisation, and provision of specific research grants.

- ◆ Because of the limited markets for orphan, and in particular, ultra-orphan medicines, their costs are high, and this has led to recent controversy around availability and funding.
- ◆ Orphan medicines are approved for use in NHS Wales in the same manner as other medicines; however, the All Wales Medicines Strategy Group makes additional considerations for ultra-orphan medicines (see *Things to know about ultra-orphan medicines.*)