GENERAL PRINCIPLES OF RISK SHARING FOR MEDICINES

1. In February 2007 BCEs agreed that the general principles for considering a service or therapy for risk-sharing are:

- incidence is unpredictable and sporadic
- the effect is so financially significant that individual NHS Boards could be at financial risk
- clinical practice across Scotland is based on appropriate clinical evidence and/or national protocols, where available, so that services can demonstrate equity across Scotland (accepting there will always be local and individual patient needs)
- there are no unexplained differences in either clinical practice or costing methodology which unbalance the share of costs in one part of the country against another

2. Although various funding mechanisms were discussed, NHS Boards agreed that, as all other nationally designated services were funded via Arbuthnott, then this should remain the mechanism for funding risk-shared agreements as well.

Enzyme replacement therapy and orphan drugs

3. BCEs agreed that the risk-sharing scheme for enzyme replacement therapy (ERT) for lysosomal storage disorders should be extended to include orphan drugs and other high-cost therapies which are approved for use in Scotland by the Scottish Medicines Committee (SMC) and which fit the general principles of risk-sharing above.

4. BCEs agreed that orphan drugs and other high-cost therapies which receive EU licences should be considered for inclusion in the risk-sharing arrangements on the following basis:

- Only SMC-approved products should be added to the scheme
- That, prior to assessment by the SMC, decisions on treatment - and, therefore, responsibility for funding – are for individual NHS Boards on advice from their Director of Public Health
- If approved by the SMC, products should be added to the risk-share from the date of SMC approval

5. NHS Boards should have the opportunity to include other costs/therapies which fall outside the core principles but which are agreed by BCEs to be appropriate for inclusion in the risk share.

6. Proposals for any orphan or high cost therapies to be presented to NHS Boards in Oct/Nov each year should be sent to NSD by mid October each year.
Definition of orphan drugs

1. The Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMEA) defines an orphan drug as:

   "(for) the diagnosis, prevention or treatment of life-threatening or very serious conditions that affect not more than 5 in 10,000 persons in the European Union."

2. There are currently 31 licensed orphan drugs with a European licence. These fall broadly into three treatment categories:

   - pulmonary hypertension (already nationally-designated and funded)
   - rare cancers (e.g. Glivec)
   - inborn errors of metabolism

3. A full list of orphan drugs is available at:
   http://www.orpha.net/docs/List_of_orphan_drugs_Europe.pdf