rare voices
AUSTRALIA

Orphan Drugs Fact Sheet

1. What are Orphan Drugs?

The term “Orphan drugs” refers to drugs, vaccines or products that diagnose, prevent or treat people with rare diseases (i.e. diseases that occur in less than 1 in 2000 people in Australia). Pharmaceutical companies often will not develop or market orphan drugs because they usually generate only a small amount of income compared with the costs to develop the drugs. As a result orphan drugs are usually not profitable for pharmaceutical companies to develop. The lack of drugs to treat rare diseases means people with rare diseases are denied diagnosis and treatment. People with rare diseases have a right to drugs with the same quality, effect and safety as people with more common diseases. So the Therapeutic Goods Administration (TGA) developed the Orphan Drug Program.

2. The Orphan Drug Program in Australia.

The Orphan Drug Program in Australia began in 1998. The aim of the program is to encourage the availability of orphan drugs in Australia.
Before a drug can be made available in Australia, the drug must go through an evaluation process to get approval from the TGA. The evaluation process looks at the drug in terms of safety, quality and clinical efficacy (performance). There are costs involved in having the TGA evaluate drugs, except when the drug is an orphan drug, in which case there is no cost for the evaluation. This provides a major financial incentive to pharmaceutical companies, encouraging them to have orphan drugs evaluated by the TGA.

To be designated an orphan drug in Australia, the pharmaceutical company that developed the drug must prove to the Orphan Drug Program that the drug is for such a small patient population that the drug is not commercially viable. The Orphan Drug Program also reduces the amount of time it usually takes the TGA to evaluate a drug.

For a list of the Orphan Drugs available in Australia refer to the website:
http://www.tga.gov.au/industry/pm-orphan-drugs.htm

Once a drug is approved as an orphan drug it is easier for the drug to become available and in certain circumstances it can be accessed through the Life Saving Drugs Program.

3. Life Saving Drugs Program in Australia

Orphan Drugs are not usually available through the Pharmaceutical Benefits Scheme (PBS) or through Medicare. This means the drugs cost more for people to buy because, unlike drugs on the PBS, the Australian government does not cover some of the cost to consumers.
A special program, the Life Saving Drugs Program (LSDP), was developed to help people get access to extremely expensive drugs, not available through the PBS, particularly for inherited disorders of metabolism. The LSDP provides these drugs at no cost for eligible patients.
A group of experts, the Pharmaceutical Benefits Advisory Committee (PBAC), evaluates drugs to decide if they can be provided on the LSDP, using a set of criteria based on the effect in treating patients, the cost and evidence that they will significantly prolong the patient’s lifespan.

If the drug is approved under the LSDP the cost of the drug is funded, however, some administration costs may still need to be paid by the patient. Criteria for initiating, maintaining and evaluating continued use of the drug is managed by disease advisory committees (DACs) functioning under the administration of the LSDP. The DACs consist of specialist physicians with expertise in managing these rare inherited disorders of metabolism.

For further information on the LSDP and a list of the drugs included refer to the website:
http://www.health.gov.au/lsdp

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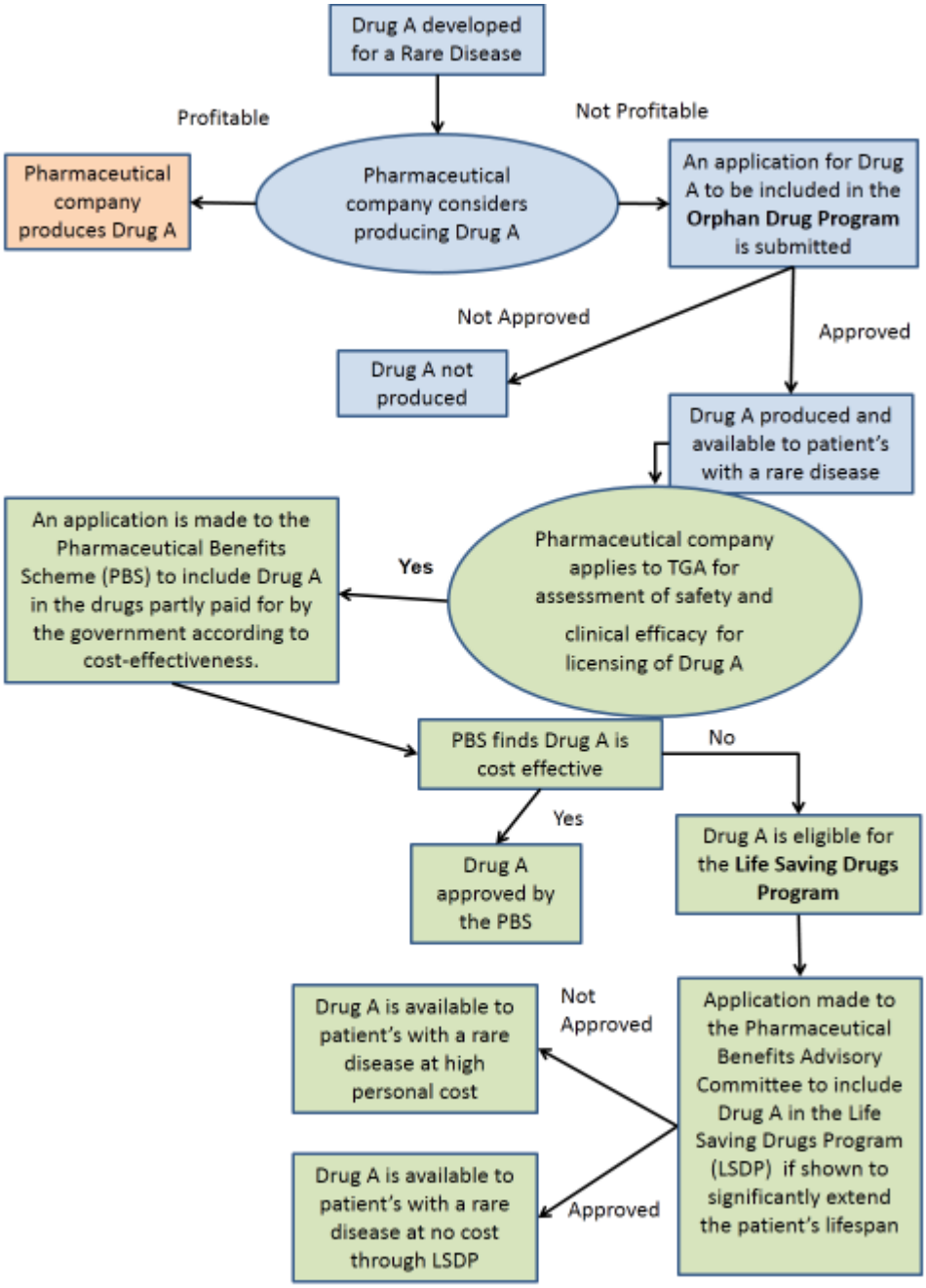
A Flow Chart is provided summarising how the Orphan Drug Program and Life Saving Drugs Program interrelate.



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