Factsheet: Orphan Drug Designation

WHAT IS AN ORPHAN DRUG?

Orphan drugs are medicinal products that are meant for the prevention, diagnosis, or treatment of rare diseases or orphan diseases. Rare or "orphan" diseases affect a small number of people globally, defined as less than 200,000 in the US and 10,000 in Australia. Their definition is dictated by the regulatory bodies around the world including the Therapeutic Goods Administration (TGA) in Australia, the U.S Food & Drug Administration (FDA), and the European Medicines Agency (EMA).

WHAT IS AN ORPHAN DRUG DESIGNATION?

An Orphan Drug Designation (ODD) is a financial incentive for pharmaceutical and biotech companies to develop and treat rare diseases. The benefits differ between different jurisdictions (see below) but typically include tax credits, waiving of drug application fees and post-approval market exclusivity.

Australia

In Australia, an orphan drug is a medicine, vaccine or in vivo diagnostic agent that meets the requirements of regulation 16J of the Therapeutic Goods Regulations 1990. The TGA flow chart guide determines eligibility.

USA

The Orphan Drug Act of 1983 is a law passed in the United States to facilitate development of orphan drugs and provides a special status or designation to a drug or biological product for treating a rare or orphan disease at the application of a sponsor. This status is known as orphan drug designation or orphan drug status.

UK & Europe

A medicine must be intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating; the prevalence of the condition in the EU must not be more than 5 in 10,000 or it must be unlikely that marketing of the medicine would generate sufficient returns to justify the investment needed for its development; no satisfactory method of diagnosis, prevention or treatment of the condition concerned can be authorised, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

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ORPHAN DRUG DESIGNATION ADVANTAGES

Australia

The TGA has an orphan drug program that waives application fees for new medicines to treat rare diseases. There is no fee for an orphan drug designation application.

USA

- Seven years of marketing exclusivity to sponsors of approved orphan products
- 25% federal tax credit for expenses incurred in conducting clinical research within the U.S
- Tax credits may be applied to prior year or applied over as many as 20 years to future taxes
- Waiver of Prescription Drug User Fee Act (PDUFA) fees for orphan drugs
- Ability to qualify to compete for research grants from the Office of Orphan Products Development (OOPD) to support clinical studies for orphan drugs
- Eligibility to receive regulatory assistance and guidance from the FDA in the design of an overall drug development plan.

UK & Europe

- Unlimited and reduced charge protocol assistance
- Access to the centralised authorisation procedure resulting in a single opinion and a single decision from the European Commission, valid in all EU Member States
- Ten years of protection from market competition with similar medicines with similar indications once they are approved.

NEUROTECH'S INTERNATIONAL ORPHAN DRUG PORTFOLIO

Neurotech is expanding its research and development (R&D) pipeline, focusing on rare paediatric orphan diseases and working closely with world-leading experts to treat neurological disorders including Autism Spectrum Disorder (ASD), Rett syndrome, PANS/PANDAS and cerebral palsy. NTI164 has been exclusively licenced by Neurotech International for neurological applications globally.

REFERENCES

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