Over 30 years after the Orphan Drug Act was passed, orphan drugs continue to be a lucrative market for pharma companies. Although orphan diseases affect small populations, these treatments address a high unmet need and also benefit from commercially attractive pricing structures and additional regulatory benefits.

**RARE DISEASE CLASSIFICATION VARIES BY COUNTRY**

- **United States**: fewer than 200,000 people in the US or less than 5 per 10,000 in individual communities
- **European Union**: fewer than 5 in 10,000 citizens
- **Canada**: has no official “orphan disease” status
- **Japan**: fewer than 50,000 prevalent cases (0.1% of population)
- **Australia**: does not define a rare disease in terms of the number of patients, but the drug must not be intended for use in more than 2,000 patients per year

**ORPHAN DISEASES ARE DISEASES THAT AFFECT FEWER THAN 200,000 PEOPLE**

- 80% of rare diseases are genetic
- 95% have no approved treatment
- 50% of those affected are children

**United States**: fewer than 200,000 people in the US or less than 5 per 10,000 in individual communities

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**Addition**

**ORPHAN DRUG DEVELOPMENT PROVIDES PHARMA COMPANIES WITH A VARIETY OF BENEFITS, BOTH R&D AND COMMERCIAL**

**R&D INCENTIVES**

- Tax credits
- R&D grants
- Waived FDA fees
- Shorter development
- Greater regulatory success

**COMMERCIALLY INCENTIVES**

- Favorable reimbursement
- Fewer hurdles to approval
- Longer exclusivity
- Lower marketing costs
- Faster uptake
- Premium pricing

**Favorable Incentives: Worth of the global orphan drug market**

29% of orphan drugs have annual sales greater than $1 Billion

15% of orphan drugs have indications for additional rare diseases

Sources: ISR Research, Forbes, NCBI, FDA, Thomson Reuters, DrugDiscovery.com