ORPHAN DRUG 101

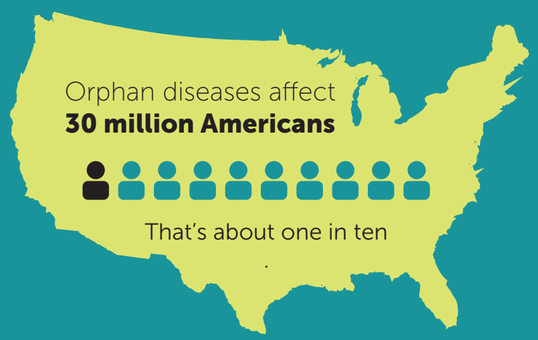
Over 30 years after the Orphan Drug Act was passed, orphan drugs continue to be a lucrative market for pharma companies. Although orphan diseases affect small populations, these treatments address a high unmet need and also benefit from commercially attractive pricing structures and additional regulatory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

80% of rare diseases are genetic

95% have no approved treatment

50% of those affected are children

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



7,000
Estimated number of rare diseases

6%
Spending on orphan drugs out of total pharmaceutical sales

1983
Year the US passed the Orphan Drug Act

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

Taiwan: prevalent in 1 out of 10,000 people

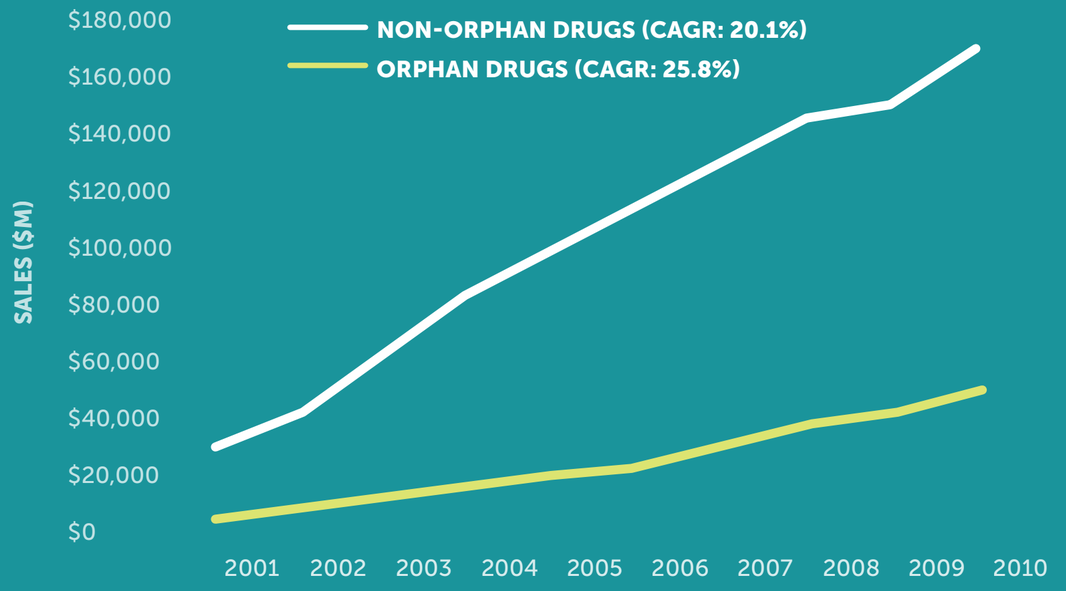
Japan: fewer than 50,000 prevalent cases (0.4% of population)

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

ADDITIONAL FDA REQUIREMENTS FOR ORPHAN DRUG APPROVAL

- ✓ Drugs and biologics only
- ✓ Intended for the safe and effective treatment, diagnosis, or prevention of rare diseases/disorders
- ✓ Rare diseases/disorders must affect fewer than 200,000 people in the U.S., or if the disease affects more than 200,000 persons, the treatment drug must not be expected to recover the costs of development and marketing.

GROWTH RATE OF ORPHAN VS. NON-ORPHAN DRUGS (2001-10)



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

R&D INCENTIVES

- $ **Tax credits**
- R&D **R&D grants**
- $ **Waived FDA fees**
- ⏳ **Shorter development**
- ✓ **Greater regulatory success**

COMMERCIAL INCENTIVES

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

$50 BILLION
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,