

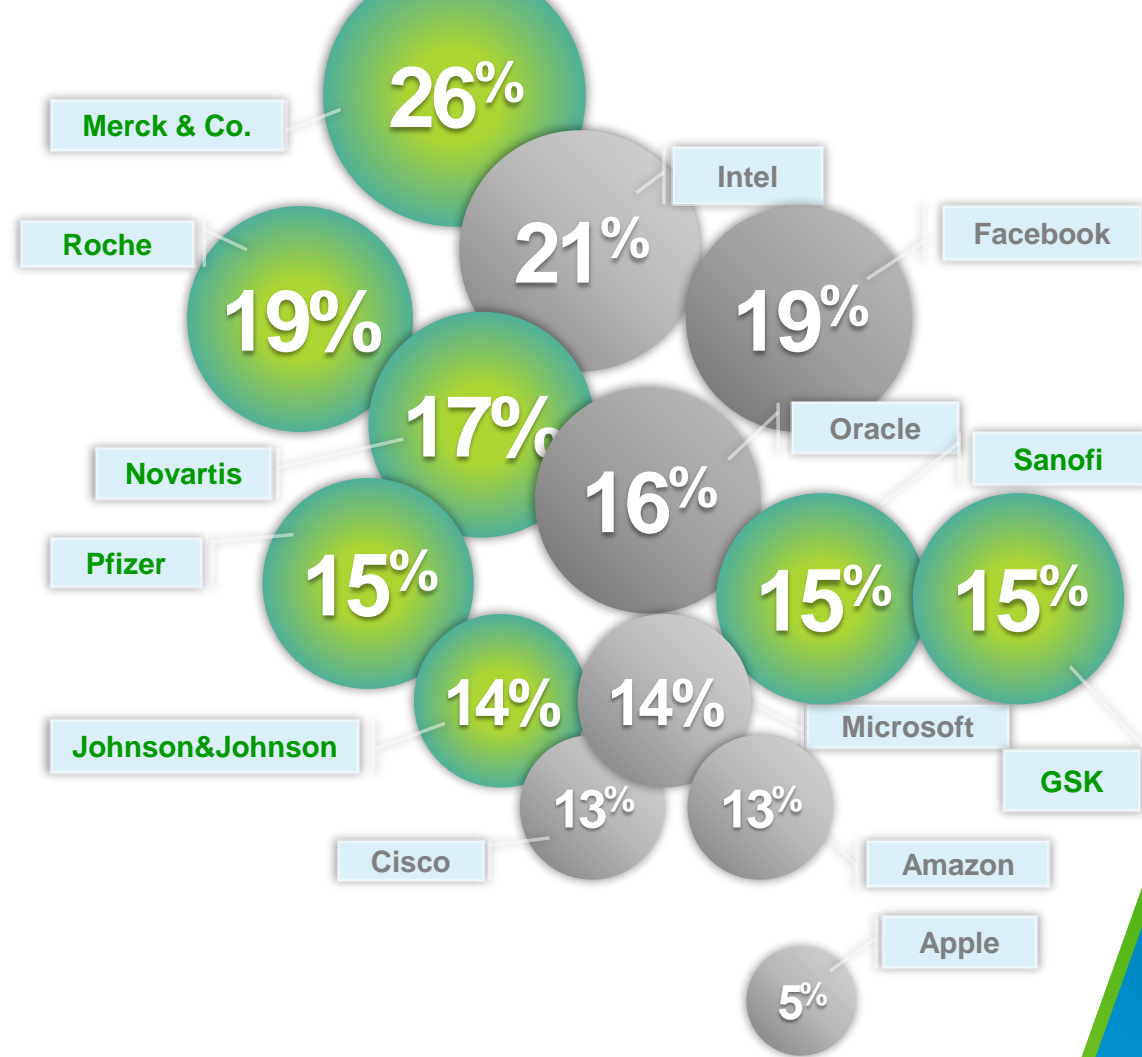
Orphan Drugs Development



Pharmaceutical Companies are Top Corporate R&D Spenders

R&D Intensity

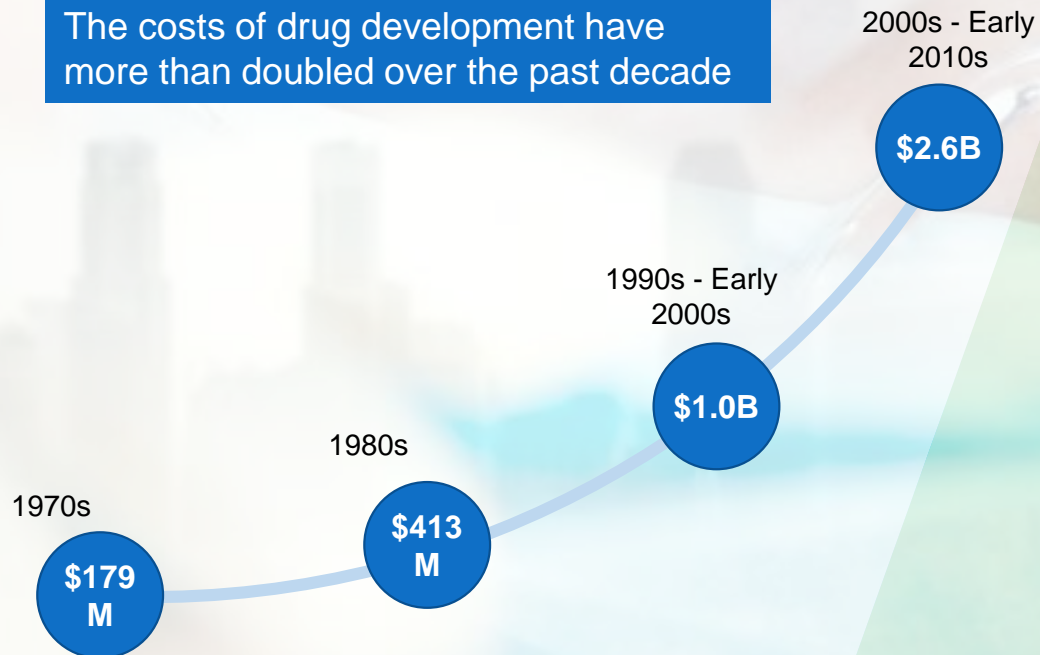
R&D expenditures as a percentage of Revenues



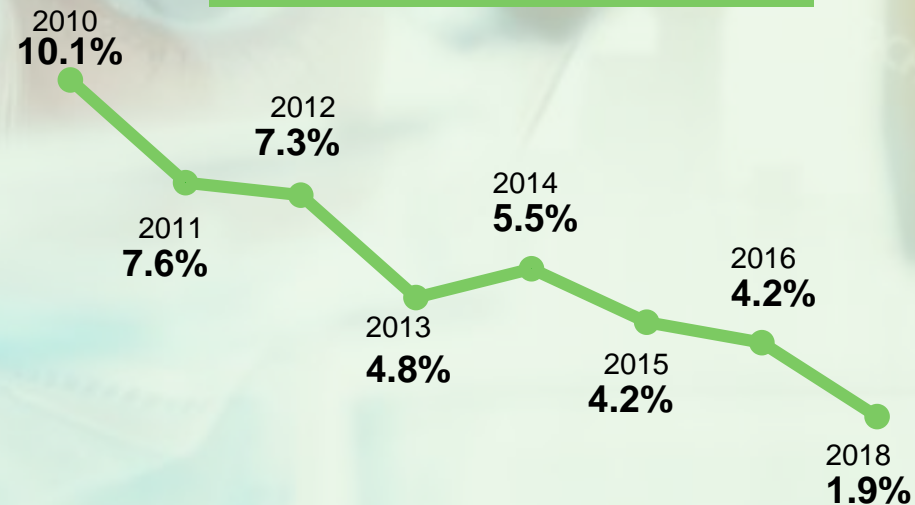
A Tough Road

Extremely Low Chances to Recoup R&D Investments

The costs of drug development have more than doubled over the past decade



R&D returns have fallen to the lowest level in nine years



An Era of Innovation

New Drugs for Rare Diseases



7,000

rare diseases
are known to
exist today



95%

have no approved
medicines



822

projects in development
are designated as
orphan drugs

A Tough Road for Orphan Drugs

Unique Challenges and Higher risks

Average development time (first patent filing to launch)
1999-2012

First-in-class
orphan drugs that
won FDA approval



15.1 years

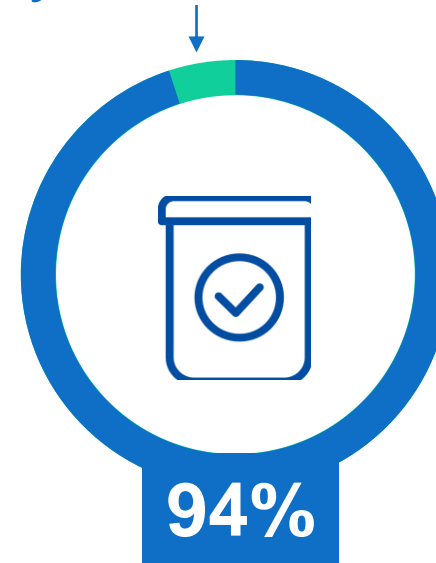
+18%

All new drugs



12.8 years

Only 6% success rate



94%
Unsuccessful
orphan drugs in
development