

Rare Disease Research: Shifting Paradigms

Traditional View	New Paradigm
Public health improvements require a focus on primarily common diseases and cancers	All cancers are rare cancers; personalized medicine is necessary to deal with the diversity of genomic profiles
Innovation spills over from common diseases to rare diseases	Innovation spills over from rare diseases to common diseases
Patients and researchers are too dispersed to reach a critical mass for fundraising or research	The internet has lowered the barriers to aggregating interested parties
Research costs are too high	High-throughput technologies are permitting genome-wide studies at tractable costs, outpacing Moore's Law
Profit opportunities are minimal in rare diseases	Rare disease therapies have become blockbusters, aided by orphan drug designations