

How Small Can A Disease State Be & Still Be Economically Attractive For Commercial Drug Development?



**It's Getting Smaller
Every Day.**

The Progressive Interest of Cancer Drug Discovery & Development by Companies



Drug discovery was highly empiric (natural products, alkylating agents).



Companies ignored the field in part because you could only sell a few doses to any one person because of cumulative toxicity and low incidence (HTN vs. lung cancer).



Single agents could be used in multiple cancers creating larger markets (e.g., CDDP in lung, ovarian, H&N, testicular, uterine, etc)



Drugs became more tolerable and could be used longer which increased sales (e.g., imatinib).



Premium pricing made small single indications attractive (e.g., Giladel).

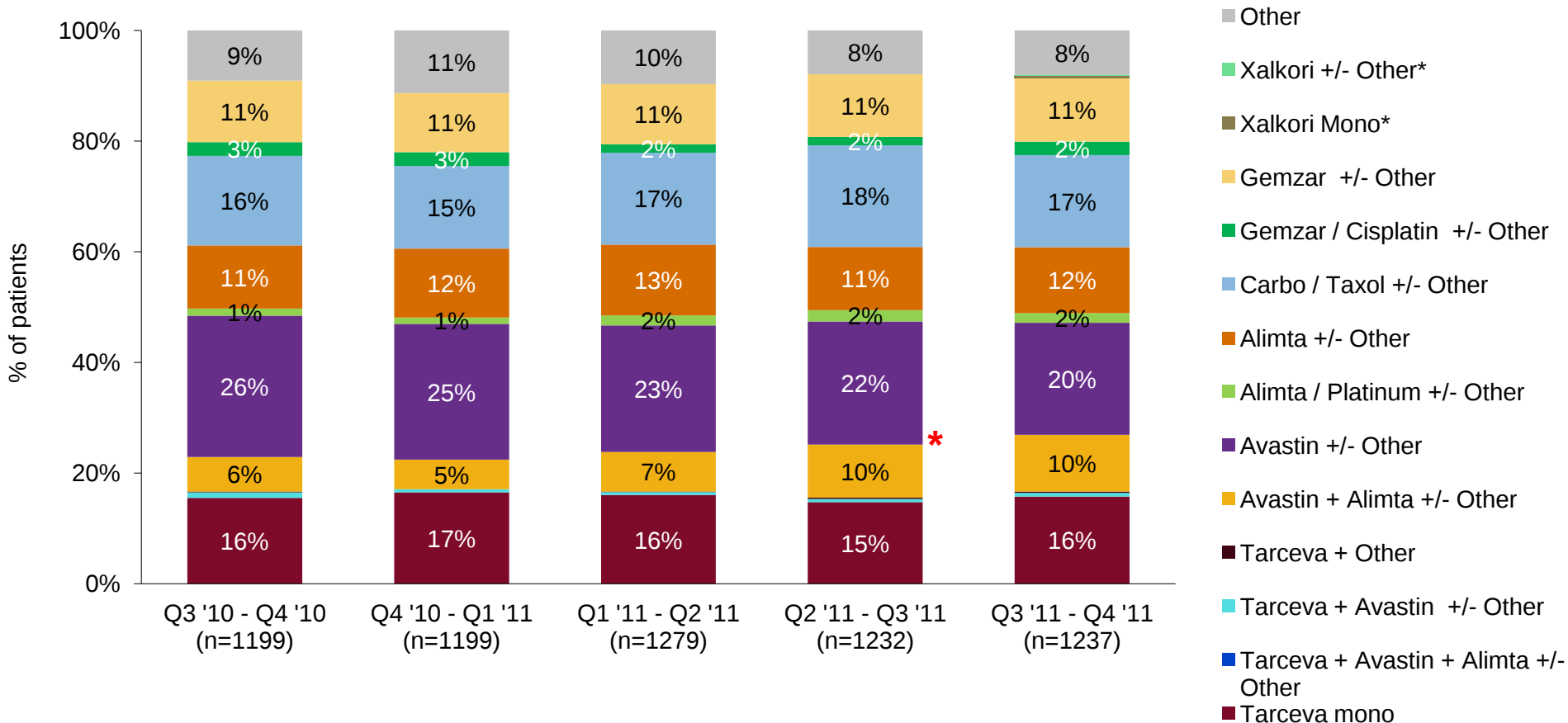


Scientific advances made rational drug discovery faster and cheaper. (molecular characterization of cancers, better SAR, high throughput screening, etc)



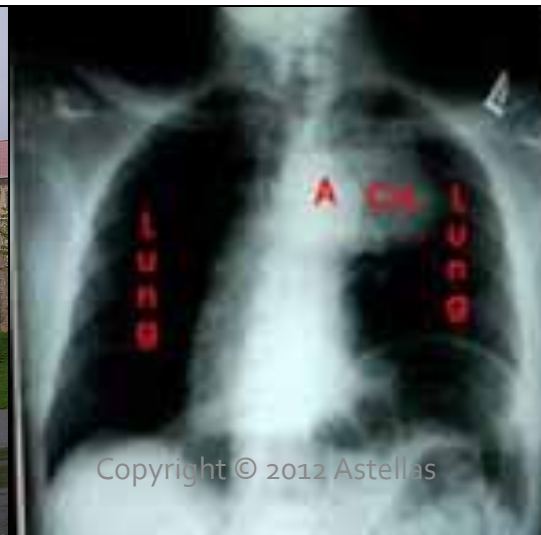
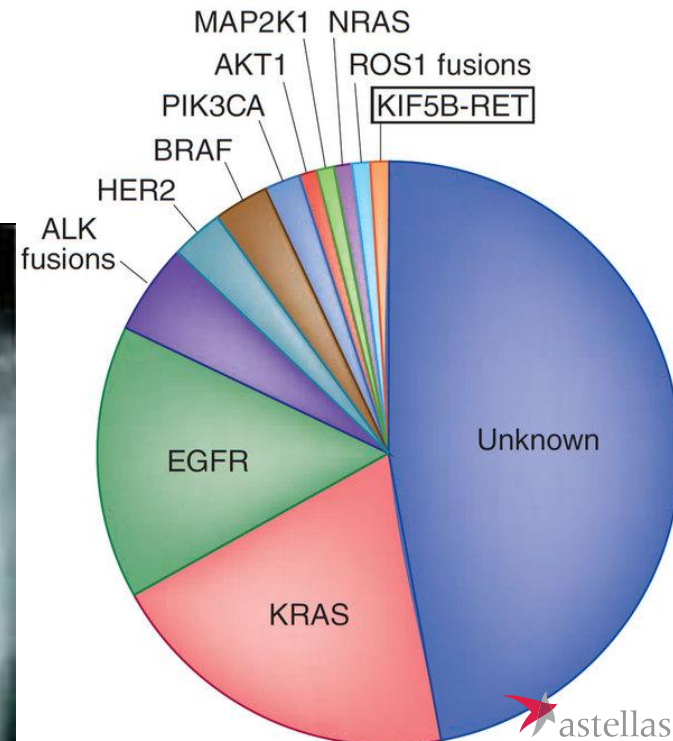
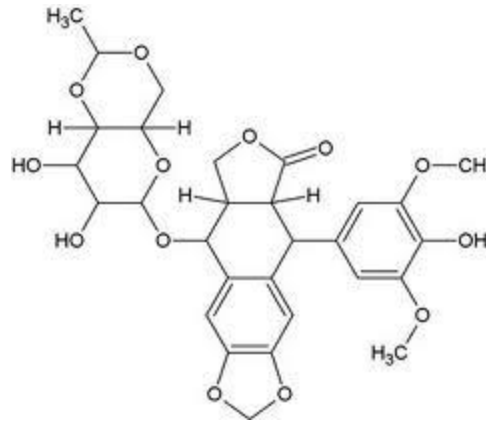
Small subsets of cancer become economically attractive (e.g., crizotinib)

Use of Medicines in First line Non Small Cell Lung Cancer

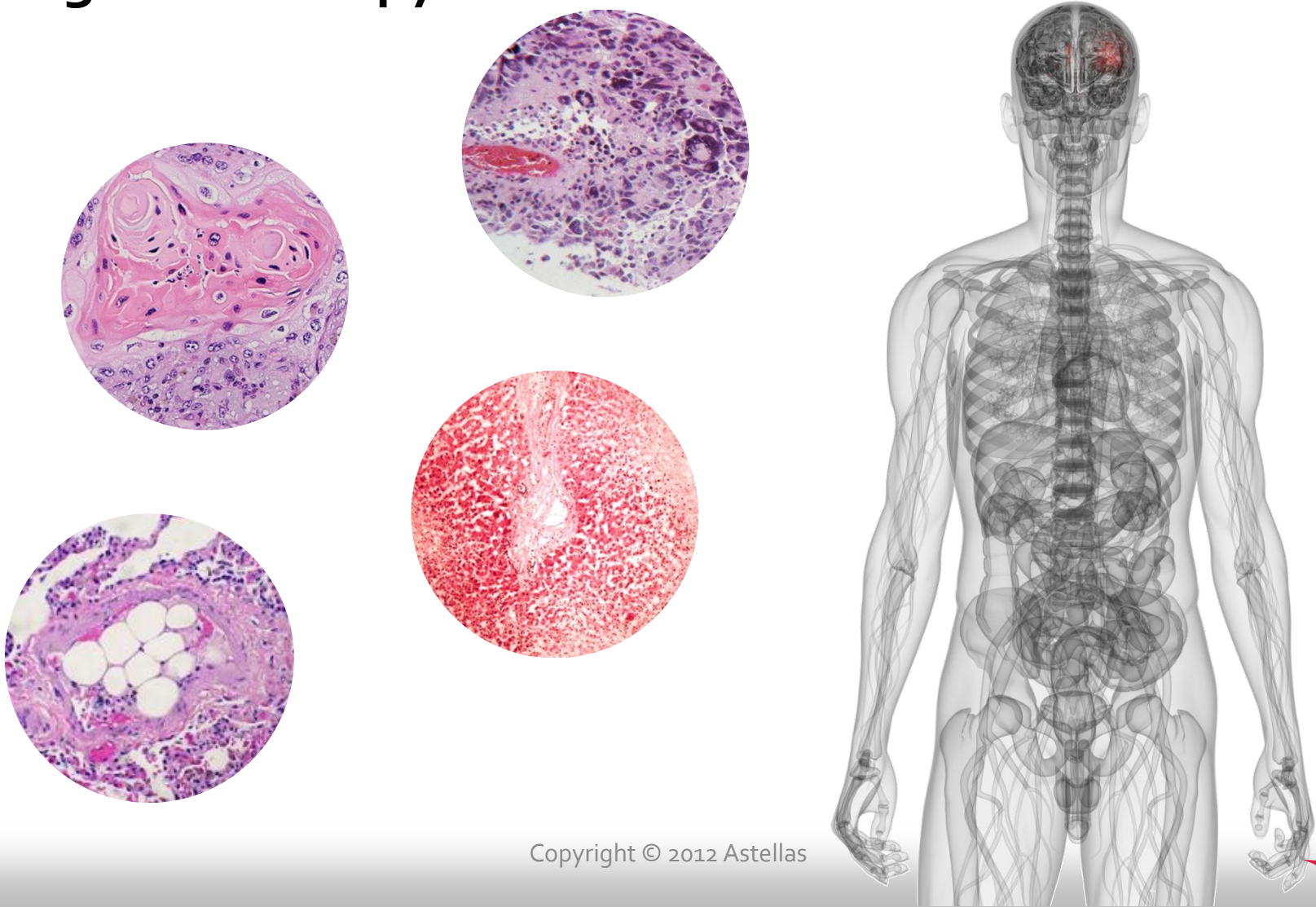


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A detailed botanical illustration of *Ranunculus repens* (Cuckoo-pint). The central figure shows the whole plant with its thick, horizontal, dark brown rhizome and several upright, green, deeply palmately lobed leaves. Two white flowers with yellow centers are shown on long pedicels. Surrounding the main plant are detailed anatomical drawings: a single petal (1), a single sepal (2), a stamen (3), a pistil (4), a cross-section of the fruit (5) showing six locules, and a single seed (6). The illustration is rendered in a classic scientific style with fine lines and naturalistic coloring.



Futurist's View: Patients Will Get A Series Of Targeted Therapies Based On Periodic Molecular Reassessment Of Their Cancer & Selection Of Next Targeted Therapy



What is Needed To Make This Economically Feasible for the *Entire* Biomedical Enterprise?



Cheaper, faster technology for molecular characterization



New drug development paradigm with smaller trials demonstrating big effects.



Systematic approach to pharmacovigilance.



Advances in regulatory science.



Effective drug use management



Change in cost structure

The Same Targeted Therapy May Find Use in Multiple Indications

- Lung
- CNS
- Lymphoma
- Renal
- Sarcoma



Most diseases are heterogeneous and will be segmented into their smaller components



Drug development will become a science of rare diseases

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Questions & Comments

