Clinical Trials: Facts and Trends

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What is a clinical trial?

- A clinical trial is a research study in human volunteers to answer specific health questions.

- Traditional classification:
  - Phase I (20-80 volunteers/patients):
    - initial assessment of safety, determine dosage
  - Phase II (100-300 patients): further confirmation of safety, initial data on efficacy
  - Phase III (1000-3000 patients, ): confirmation of safety and efficacy
  - Phase IV: post-marketing surveillance
How many clinical trials are there?

- ClinicalTrials.gov currently lists 155,790 studies with locations in all 50 states of the United States and in 185 countries.
- There are over 31,500 currently recruiting clinical trials registered on clinicaltrials.gov.
- There are over 8,000 new drugs in clinical development worldwide.
Where clinical trials are conducted?
Distribution of Clinical Trials by Therapeutic Area

- Oncology
- CNS
- Anti-infectives
- Cardiovascular
- Endocrine
- Respiratory
- Musculoskeletal
- GI
- Dermatology
- Hematology
- Genito-Urinary
- Other

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Cost and Duration of Clinical Trials

• Often referenced “cliché statistics” on the development of a blockbuster drug:
  – Takes 12 years
  – Costs 1 bln USD (including the cost of failures)

• Cost of clinical trials (supported by the data of US National Cancer Institute):
  – Takes 7-8 years to conduct Phase I-III clinical trials
    • + approval time of 1.5-2 years
  – Average cost of clinical trials is 56.3 mln USD
Per-Patient Cost of Clinical Trials

Phase I
Phase II
Phase III
Phase IV

0
5000
10000
15000
20000
25000
30000
Why Clinical Trials are so Costly?

- Compliance with extreme volume of increasingly complex regulation, proof of drug safety
- New drugs are often only marginally more effective than older ones which requires more and more patients to prove the benefits
- Overall rising cost of healthcare services
- Competition for patients – slow patient recruitment - costly delays
- Competition for investigators - high demand and rising cost of efficient sites
Effect of Economic Recession on Clinical Research

- Between 2008 and 2011 - a 17% decrease in the number of new clinical trials in the EU
- Big Pharma reducing clinical research funding
- BioTech and MedTech companies struggle to obtain VC and other forms of funding
- Funders focus on late-stage R&D pipeline candidates - negative effect on early stage trials
- In the last 5 years the cost of clinical trials has risen by 70% (despite the economic crisis)
Effect of Economic Recession on Clinical Research

Pfizer closes Kent plant as it trims research spending by $1bn a year

*BMJ* 2011; 342 doi:

http://dx.doi.org/10.1136/bmj.d771

*Published 4 February 2011*
2012-2013: Green Shoots of Recovery

- Venture capital investment in biotechnology increased by 20 percent in 2012
- Health and biotech funds rose by 22.4% in 2012
- New forms of funding (public-private partnerships)
- Signs of growth in the number of new clinical trials mainly in emerging locations
  - Poland: 7% growth in 2012
  - Ukraine: 15% growth in 2012
- Positive trend continues in 2013
Today’s Trends in Clinical Trials

• Globalization of clinical trials and outsourcing to emerging locations
  – Driven by cost and timelines and rising importance of emerging markets
  – Over 60% of patients in clinical trials submitted to EMA are not from European Union – predominantly from Eastern Europe and Asia
  – EU is planning to reform the European Directive on Clinical trials and stop the loss of clinical trials to emerging markets
  – Collaboration between regulators internationally
Today’s Trends in Clinical Trials

• New models of clinical trials
  – Virtual clinical trials
    • Use of modern technology for data collection, monitoring and analysis
    – Introduction of mobile health applications
    – Regulators have to introduce new standards
• Calls for more clinical data transparency
Today’s Trends in Clinical Trials

• Personalized Medicine and Companion Diagnostics
  – Variable responses to drugs are increasingly recognized
  – Proof of efficacy in smaller better defined subpopulations of patients
  – More flexible study design
  – Smaller and faster clinical trials, reduced cost

• Growing role of Companion Diagnostics
Today’s Trends in Clinical Trials

- Focus on unmet medical needs
  - New drugs must be better or cheaper, duplicates are not required
- Focus on cost-effectiveness;
- Increasing importance of payers/reimbursement
  - Companies have to prove that their new product is
    - Effective
    - Safe
    - Affordable
Thank you!

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