



New Approaches to Drug Development



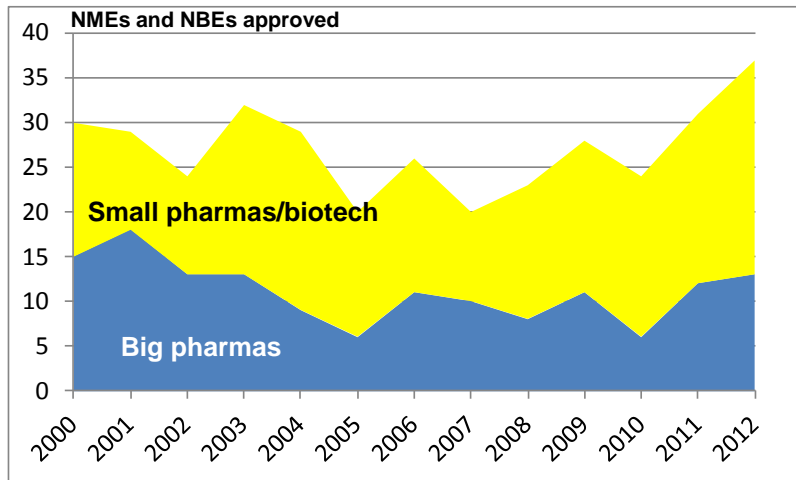
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InnoThink Center For Research in Biomedical Innovation
Washington, D.C., November 22, 2013

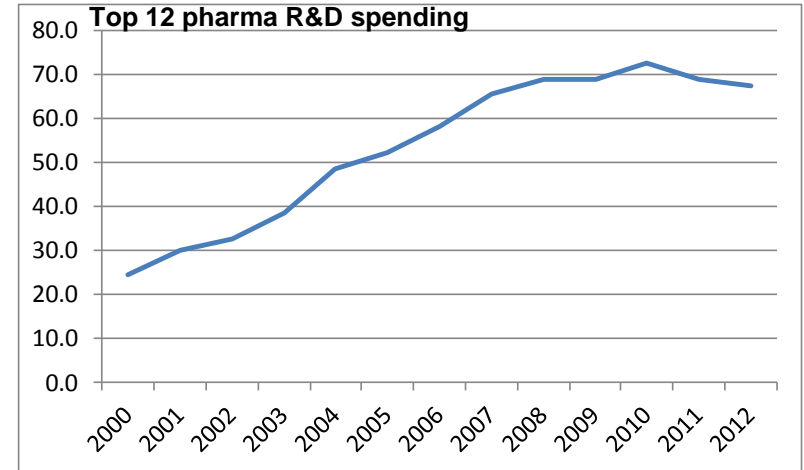
A broken model

“Many companies see **dwindling revenues** after years of lackluster R&D productivity”

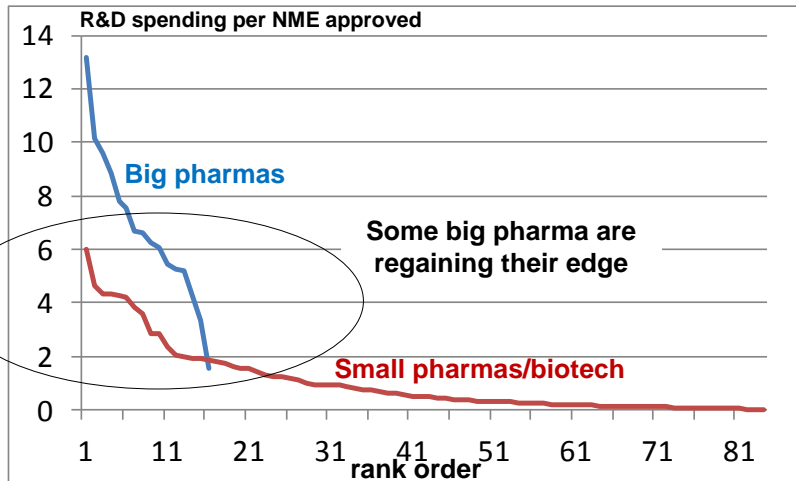
Big Pharma R&D Productivity Not Getting Better



Source: US FDA



Source: annual reports



Source: Matt Herper, Forbes, 8/11/13

- ~11 NME per year for top 12 pharma; only 2 or 3 will be blockbusters
- R&D spending (\$70bn) leveling out
- \$4 to \$12 bn R&D spend per NME
- But some big pharma are starting seeing their efforts pay off (Bayer, GSK, J&J, Roche, Novartis, Sanofi)

New **research models**

- **Drug rescue**
- **Drug repurposing**
- **Public-Private Partnerships**
 - **Structural Genomics Consortium**
 - **Neglected diseases (MMV, GAVI...)**
- **Micropharmas**
- **Observational data flow capture**
- **Crowdsourcing**
 - **OSDD.net**
 - **Transparency Life Sciences**
 - **Rare disease platforms (Chordoma Foundation, LAMsight, MMRF Researcher Gateway, MMRF CoMMunity Gateway...)**
 - **PatientsLikeMe**



Eboo Pharmaceuticals



DEUTERIA



Micropharmas



- Shoestring organizations (1-12 employees)
- Operated from homes, VCs, low rent facilities
- Often founded /run by big pharma veterans
- Everything is networked, open-sourced, outsourced
- VCs **love** them: 22 have raised over **\$800m** in 40 rounds of financing
- No failure
- 6 successful exits have created over \$2 billion for their investors



HYPERION THERAPEUTICS
A SPECIALTY PHARMACEUTICAL COMPANY



Possible scenario for the **(near) future**

- **Patients outfitted with biosensors that collect thousands of data points per day in non-invasive way**
 - **Mobility, effort, blood pressure, heart rate, dozens of blood and urine parameters, breathing rate, sleep, EKG, EEG, hi-res pics...**
- **Data sent wirelessly in real-time to “my-space-in-the-cloud” where it is processed by diagnostic software (e.g., IBM Watson)**
- **Patient alerted of developing problem(s) and potential corrections (lifestyle, diet, drugs, devices...)**
- **If problem, physician can “play the movie in reverse” to better understand etiology**

Impact on clinical research

- Data would provide **pre-symptomatic natural history** on thousands of diseases
- **Patient recruitment** greatly facilitated
- Enrolled patients can be **monitored at low-cost in real-time**
 - Impact of each dose can be carefully assessed on each patient
- Large amounts of **high quality data** available to scientists and regulators
- IRBs and regulators can **follow trials in real-time** at patient level
- **Analyses of sub-populations** greatly facilitated
- **Patient is in control**

It's observational data, but scientists and regulators have never had anything so good

The **transformation** of drug R&D

Cheap tools and data

- Proliferation of scientific databases covering every discipline, and accessible free of charge or at low cost
 - Genomic, biobanking, chemistry, pharmacology, toxicology...
 - Patient-level data
- Wide availability of software
- Effortless and costless collection of data
 - Apps, biosensors, movement-tracking, hi-res pics, plug-in devices...
- Unlimited cloud computing and cloud-based storage

Abundance of entrepreneurial talent

- MOOCs: massive open online course

The emergence of an innovation **supply chain**

- Data, tools, and platforms
- Cloud-based infrastructure
- Specialist companies (screening, chemical synthesis, toxicology, supply chain management...)
- Crowdfunding (Poliwogg, Microryza, Medstartr)
- e-commerce platforms (Assay Depot, Science Exchange)

It's a lot cheaper, easier, and faster to start a company

Open-source drug R&D **policy issues**

- Who owns and maintains the **infrastructure**?
- What **privacy** safeguards for patients?
- Who **owns the data**?
- Who **owns the IP** created from the data and bio samples?

Thank you!

Questions?

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