LIUC
Università Cattaneo

Innovazione e sviluppo del prodotto

Il settore farmaceutico (2)

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Key trends in healthcare

Increase of chronic and multiple diseases

Increased focus on prevention

Increasing expectations of citizens

Shift from public to private funding

Ageing populations
Budget constraints
HC worker shortages

Increasing e-solutions

Shift to community and homecare

Increased demand of Medical Technology and services

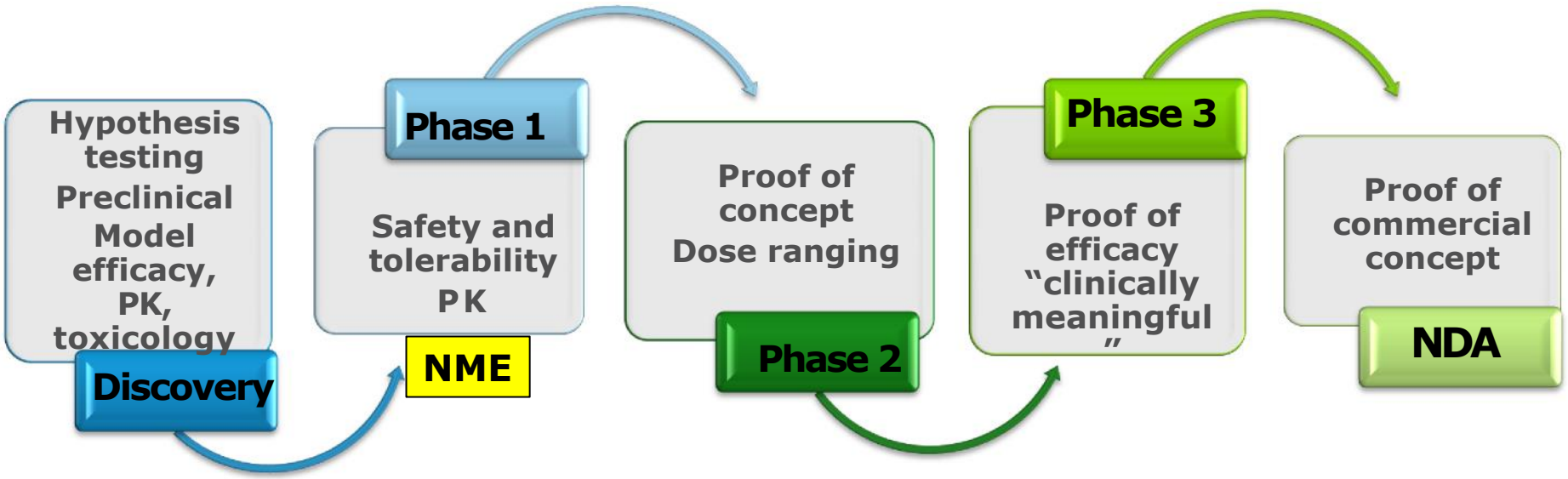
Increased role of purchasing & paying institutions

Declining power of HeathCare professional

Five trends are transforming our ecosystem

- Spiraling R&D costs coupled with decreased productivity
- Demand for safety and post-marketing surveillance
- Expectation of personalized medicine
- Reimbursement driven by medical and economic outcomes
- Proliferation and redistribution of healthcare outcomes information

Traditional Drug Development Process



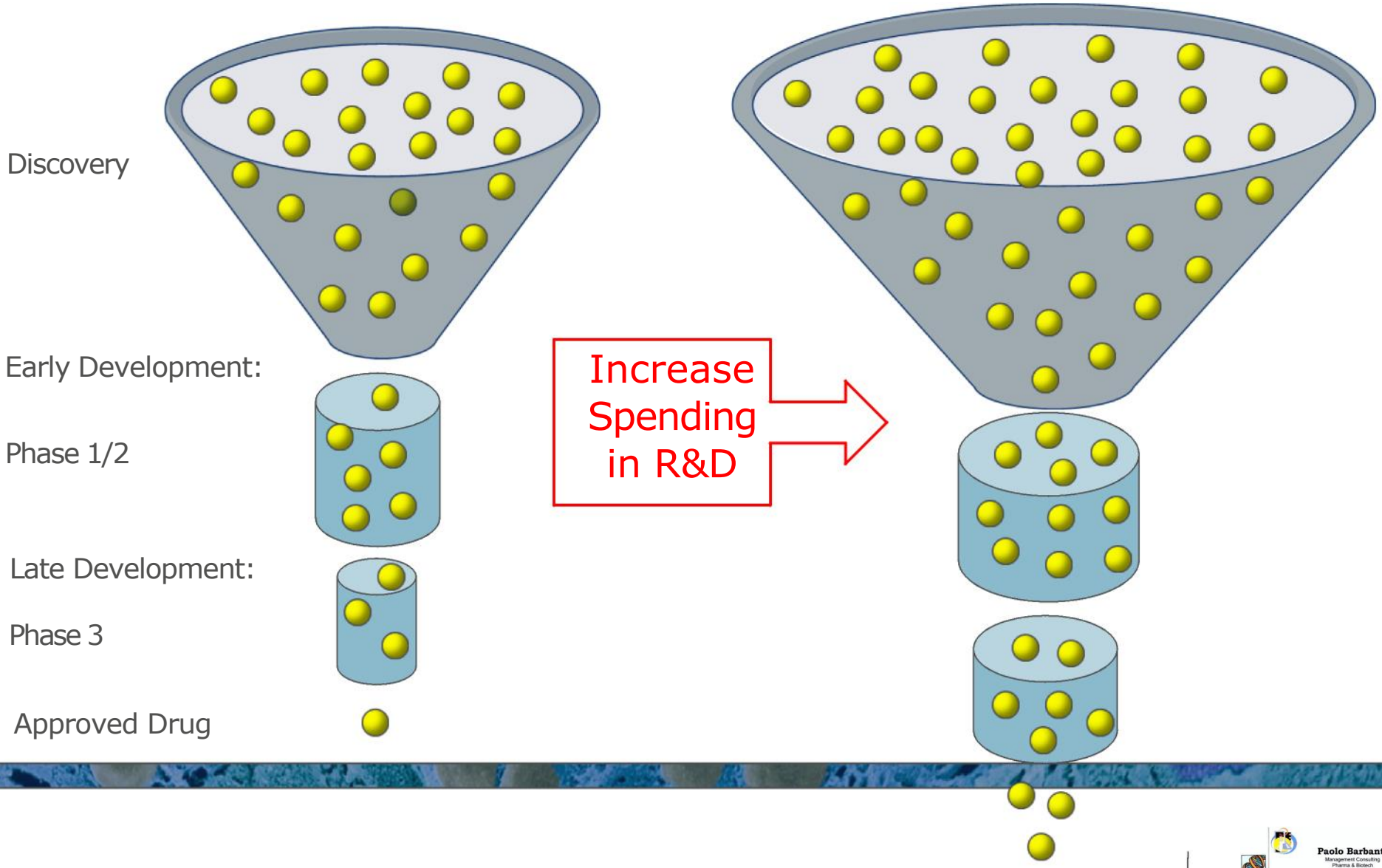
NME: New Molecular Entity

NDA: New Drug Application

Drug development

- Currently takes more than 11 years and requires an investment of over $2.5 Bln to bring a single innovative drug to market
- Clinical investigation, premarket application, and postmarket stages are heavily regulated in most developed countries
- Ongoing concern about ability of the drug development enterprise to translate innovative science and bring needed therapies to market
- Ongoing concern about the ability, and willingness, of societies to pay for novel therapies

The Pharmaceutical industry response



Industry Challenges

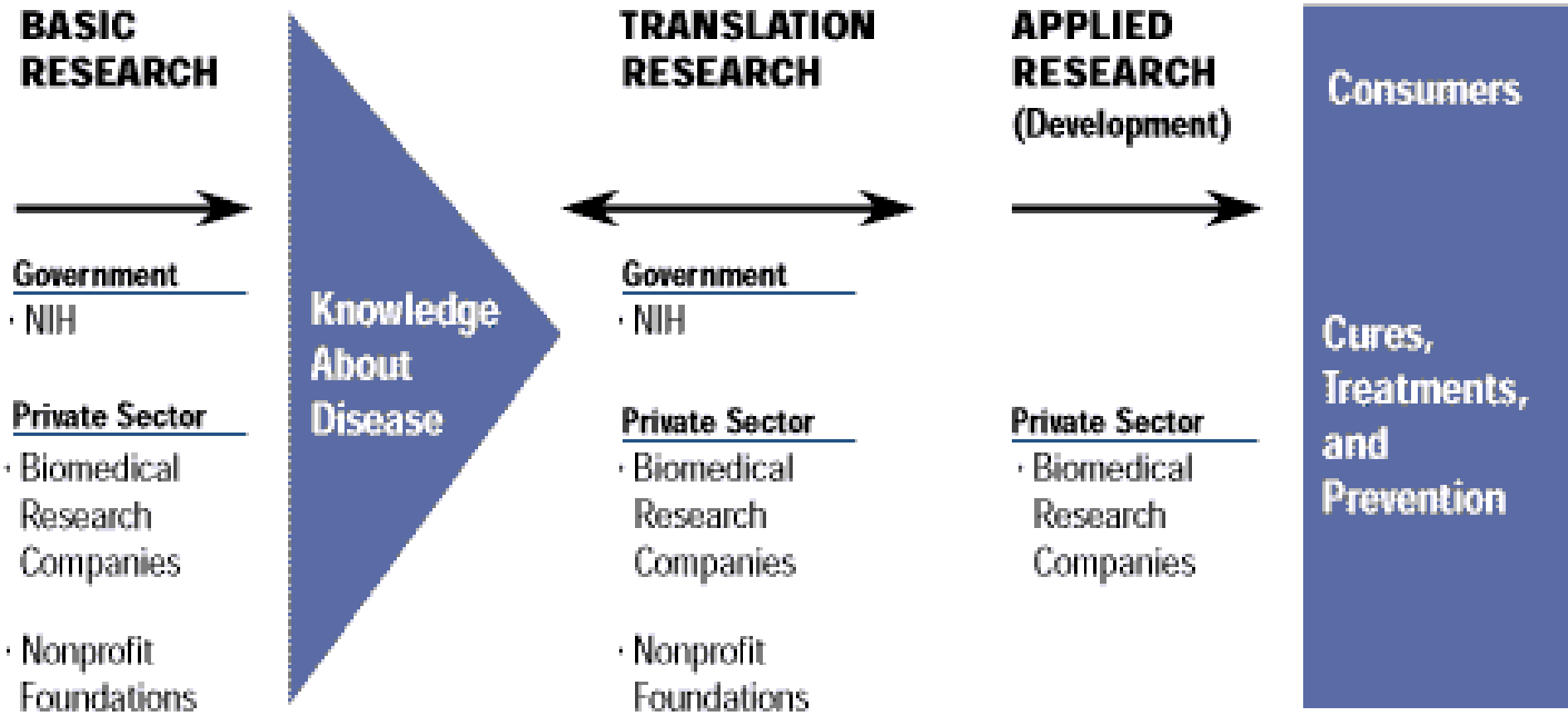
- Slow progression of pipeline
- Rising R&D costs.
- Unacceptable attrition rates in clinical drug
- Key patents have expired (the patent cliff)
- Downward pressure from payers on the price of medicines
- Challenges in the global economy development.
- Intense focus on new business models to decrease costs and increase efficiency in R & D
- Calls for greater collaboration amongst all stakeholders (industry, academia, regulators, payers)

These companies have not been delivering enough return on R&D investment (new medicines reaching the market) to satisfy the demands of the financial markets
Less venture capital available for 'discovery-end' biotech

FDA NME Approvals

- Basically stable output over long term (vs increased investment in basic research and R&D)
- Decline from late 1990s reflects primarily decrease in submission of “me-too” drugs: now difficult to get on formulary
- FDA seeing increased novelty in applications over recent 5 year period; more “game-changing” therapies
- Possibly reflects adjustment of industry strategies

The process of discovery

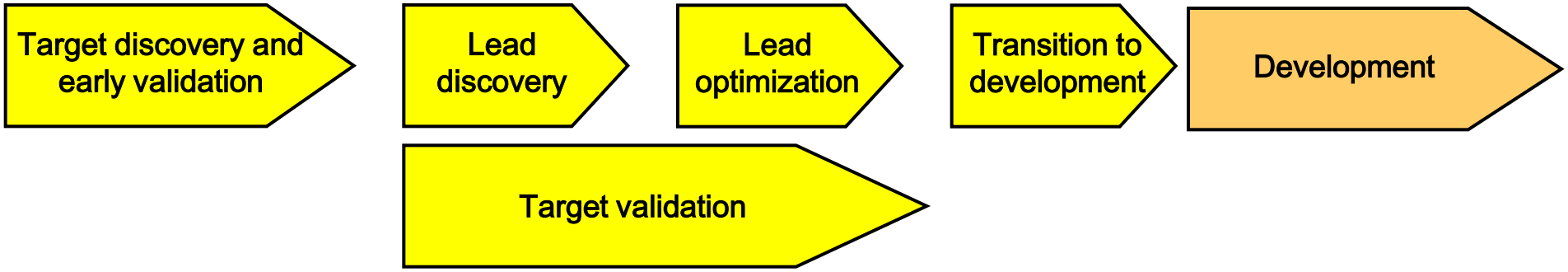


Source: FDA Council Congressional Briefing Series, "Molecules to Miracles," 1997.

Will new scientific discoveries revolutionize treatment of disease ?

- Advances in both science and technology are providing unprecedented opportunities for new approaches to disease prevention, diagnosis and treatment
- However, in some senses, the barriers to successful development have never been higher
- New paradigms for evaluation of diagnostic and therapeutic interventions must be developed
  - Faster
  - More efficient
  - But equally or more informative

Drug discovery: an evolving approach



0.5-1 year **0.5 -1 year** **1-2 years** **1 year**

3 – 5 years in discovery | **3 years in development**

Biology **Pharmacology**

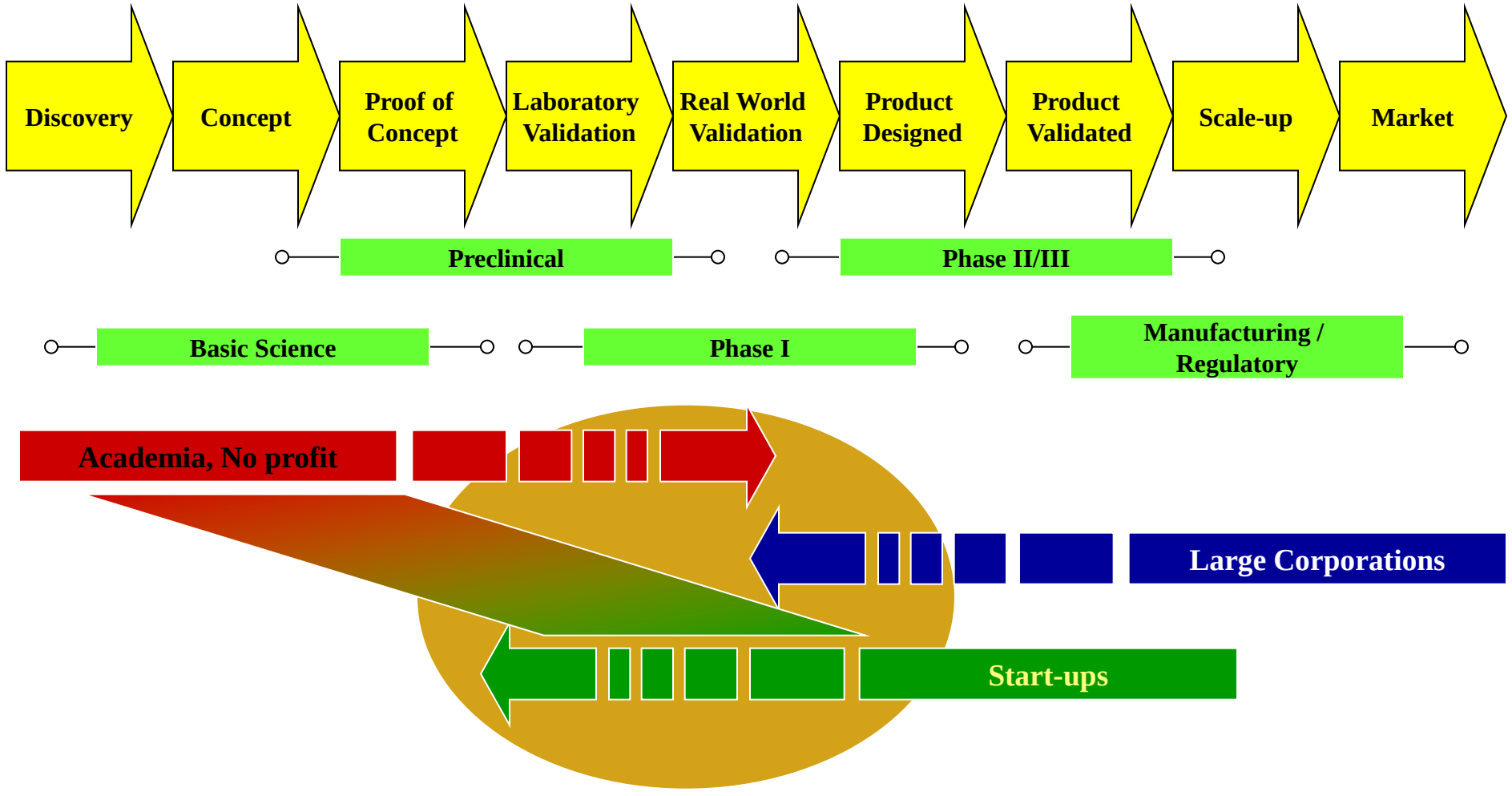
Chemistry

- Genomics
- Expression arrays
- Gene cloning
- Shotgun sequencing
- Population genomics

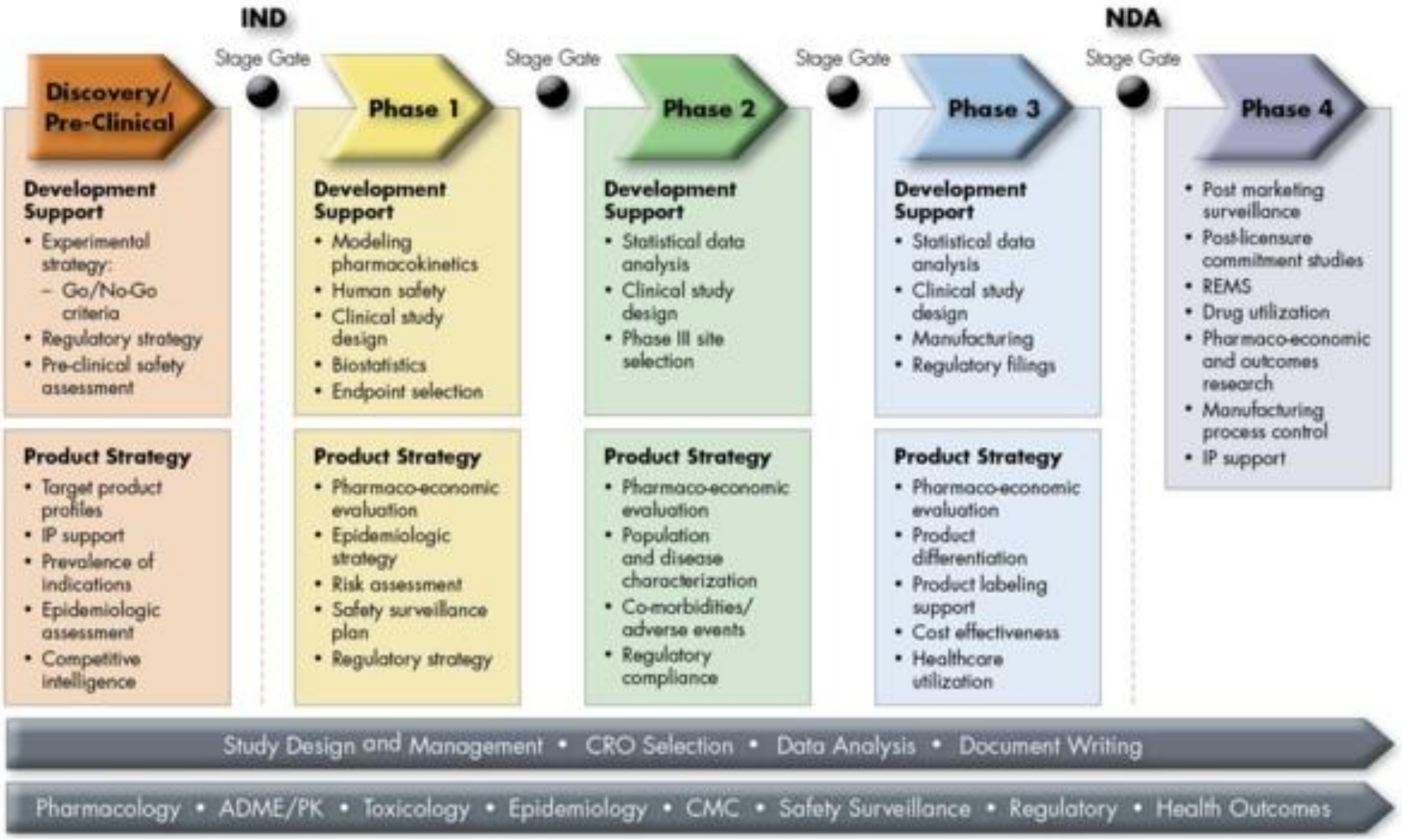
- HT protein sequencing
- HT Protein synthesis
- Tandem MS
- Whole genome chips
- Pathway predictive tools
- Single cell HTS
- Ab combi-chem
- Fluorescence technologies
- In silico HTS
- SNP analysis
- Genomics diagnostics arrays and biosensors

No one is capable of adequately covering all the phases of drug discovery

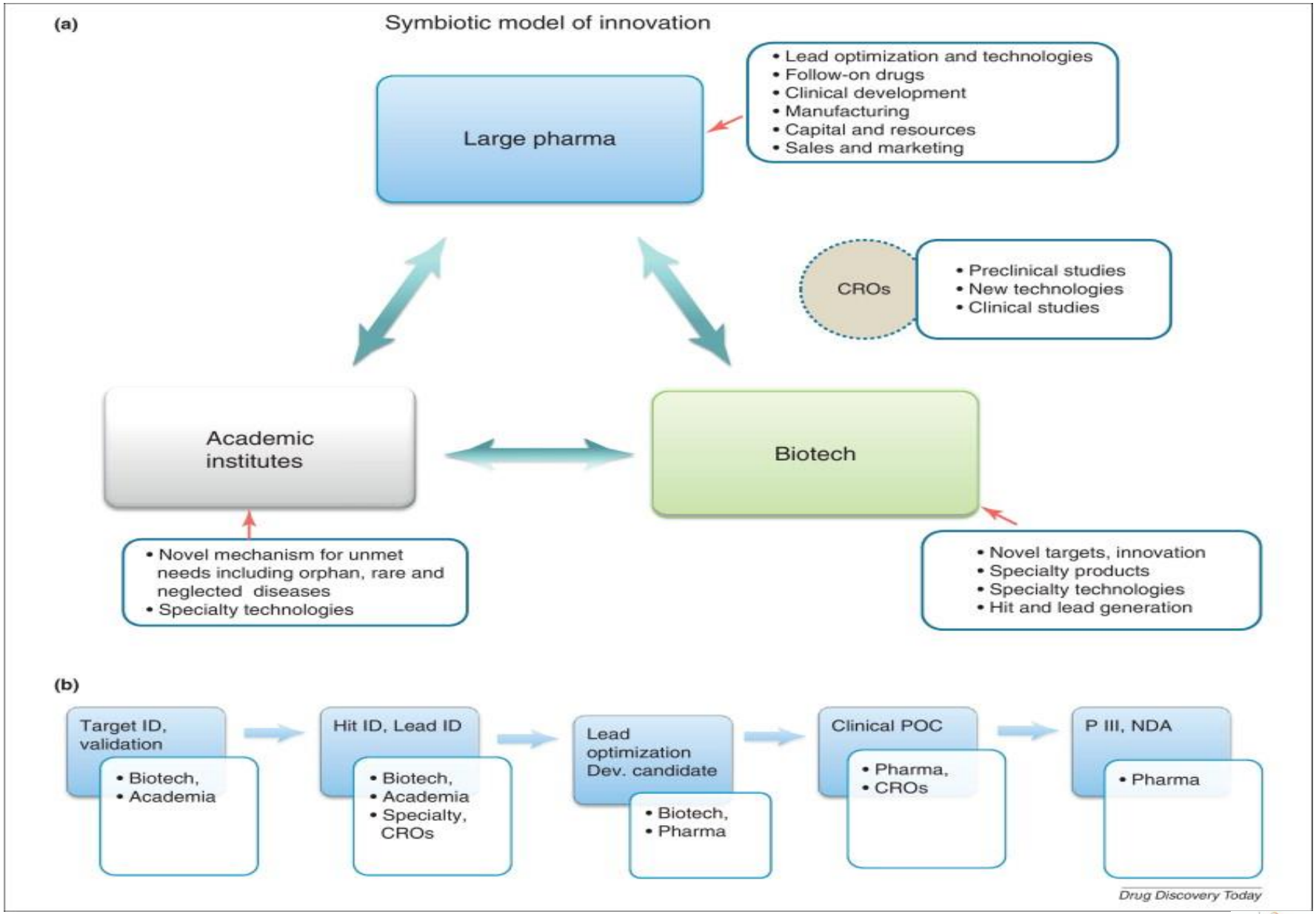
The product development value chain



Pharmaceutical Services



Drug discovery and critical partners

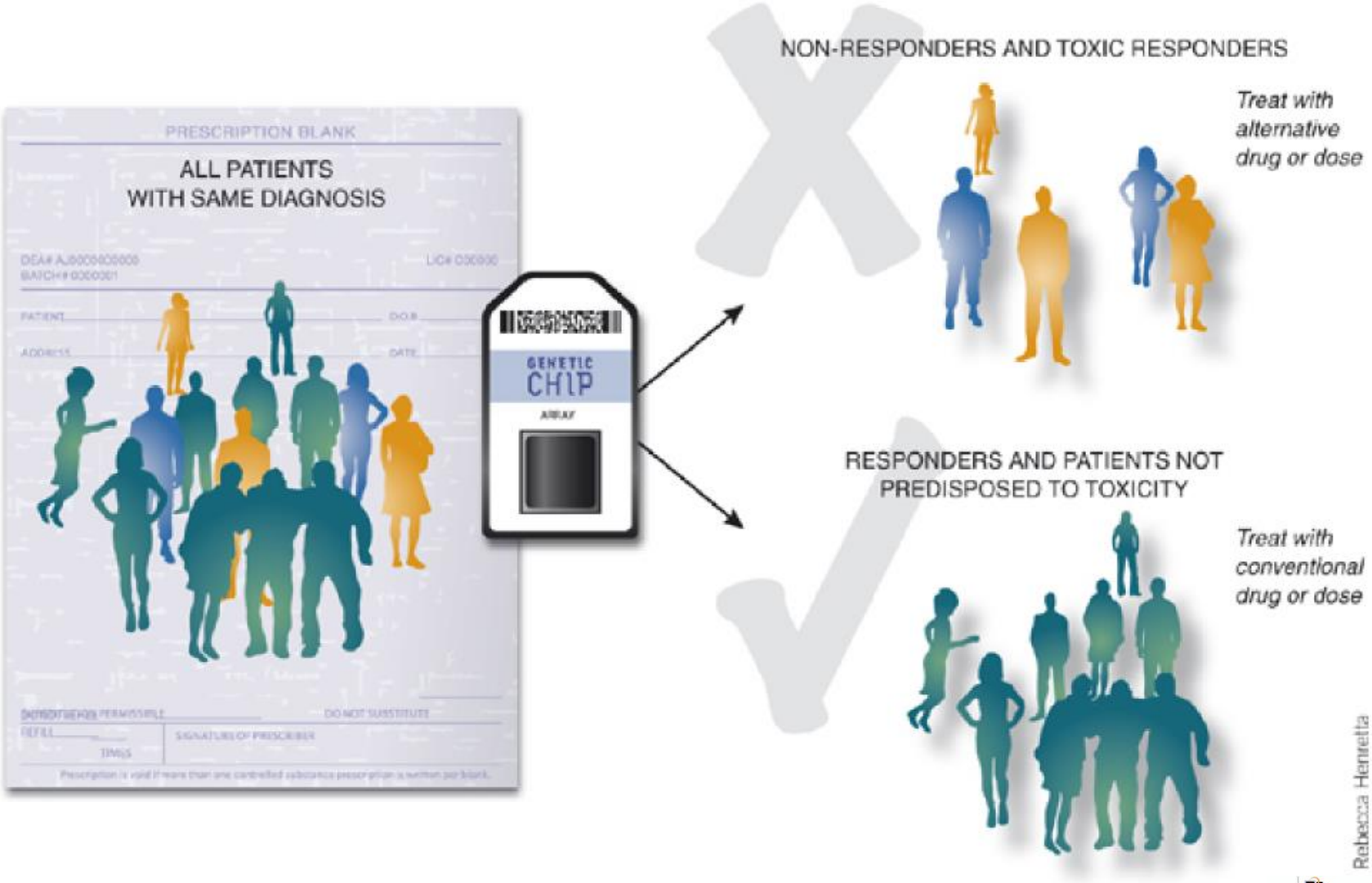


No "one size fits all" drug

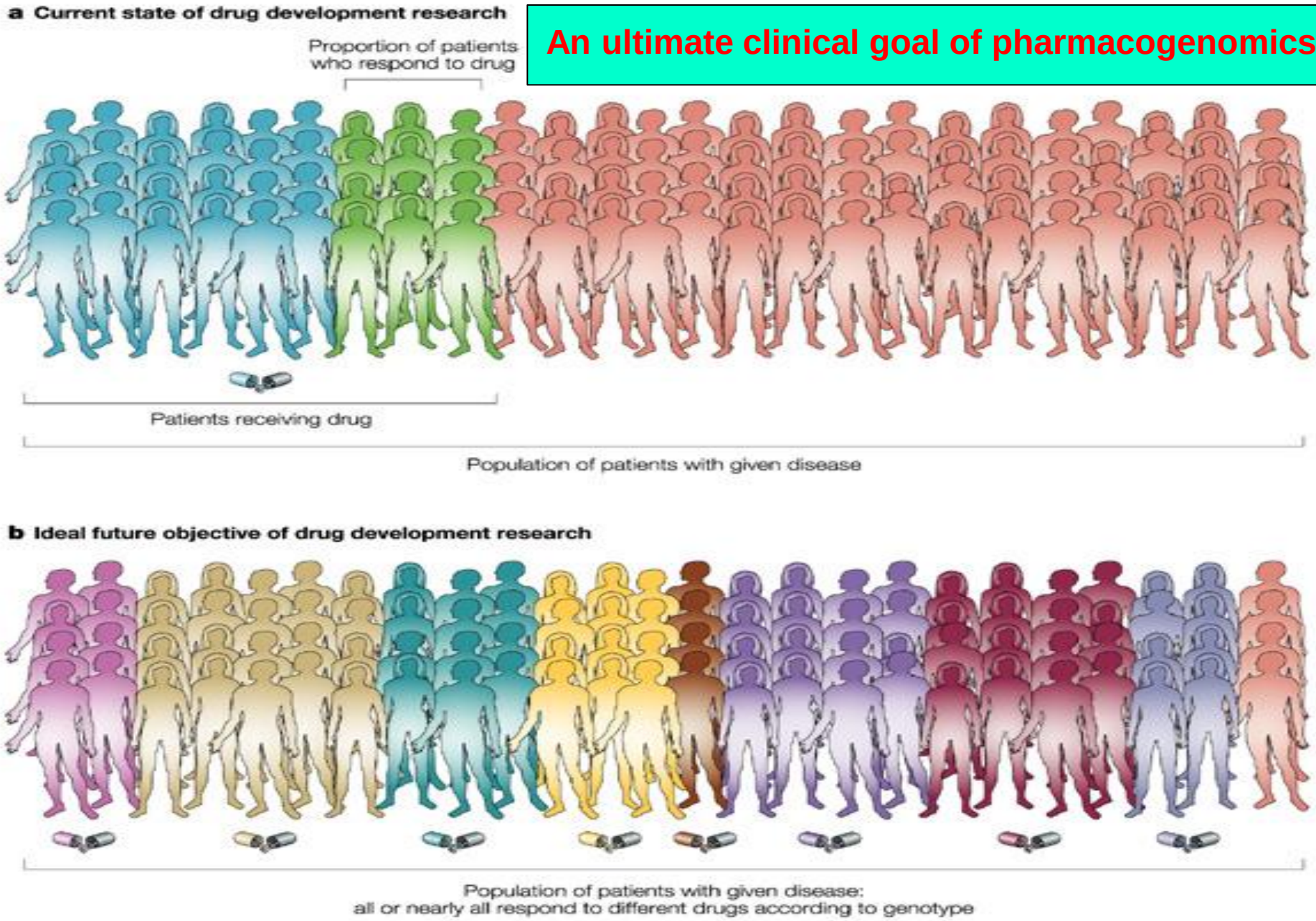
Most drugs work for 30% to 70% of patients

Multiple factors determine drug responses

Phamacogenetics is essential for individualized therapy



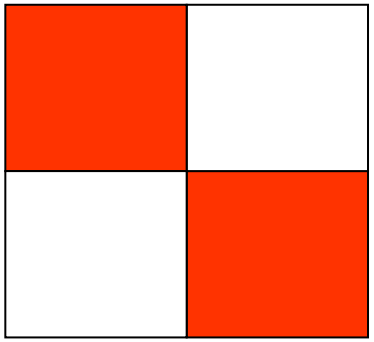
An ultimate clinical goal of pharmacogenomics

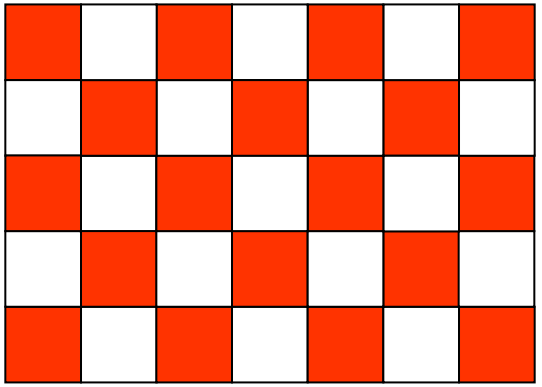


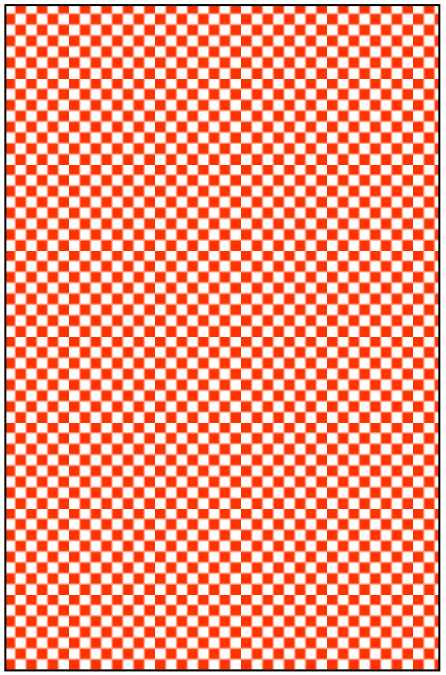
Nature Reviews | Drug Discovery

Segmenting into smaller markets ?

Blockbuster drug 100 – 500 m patients	Population medicine 10.000-100.000 patients	Personalised medicine 1- 50 patients
▪Limited pathophysiological depth of penetration for diagnostics and therapeutics ▪Low complexity and specifity of diagnostics ▪Limited differentation and effectiveness of therapeutics ▪High range of application for individual drugs	▪Increasing pathophysiological depth of penetration for diagnostics and therapeutics approaches ▪Expanding number of patients treatable by therapeutics ▪Decreasing range of application for individual drugs	■Novel therapeutic methods (eg, tissue engineering, stem cells) with applications limited to individuals







TODAY **TOMORROW** **FUTURE**

Pharmacogenomics shapes the healthcare business in 2000+

Past		Future
Disease definition by symptoms	→	Mechanism
Uniformity of disease	→	Heterogeneity
Uniformity of patients	→	Variability
Universal treatment	→	Individualized therapy
Sickness	→	Predictive/preventative care (wellness)

Molecular diagnostics: transforming the diagnosis (Dx) and the treatment of disease (Rx)

- **Replacement of current empirical "one-size-fits-all" approach by rational Rx selection**
  - **Diseases are not uniform**
  - **Patients are not uniform**
- **Diseases with identical symptoms may have different root causes (molecular pathologies)**
  - **Right Rx for right disease (subtype)**
- **Individual genetic uniqueness affects responses to Rx**
  - **Efficacy**
  - **Safety**

Personalized medicine
Same data, multiple subscribers

Pharmaceutical
Medical devices

- Cohort identification
- Drug performance
- Pharmaco-vigilance
- Outcomes analysis

Specialists

- Clinical research
- Best therapy for cost
- Trial candidate selection
- Drug performance
- Best practices

Hospitals

Hospitals / ER

Patients

Laboratories

Clinics

- Pharmaco-vigilance
- Drug safety
- Regulations

Government

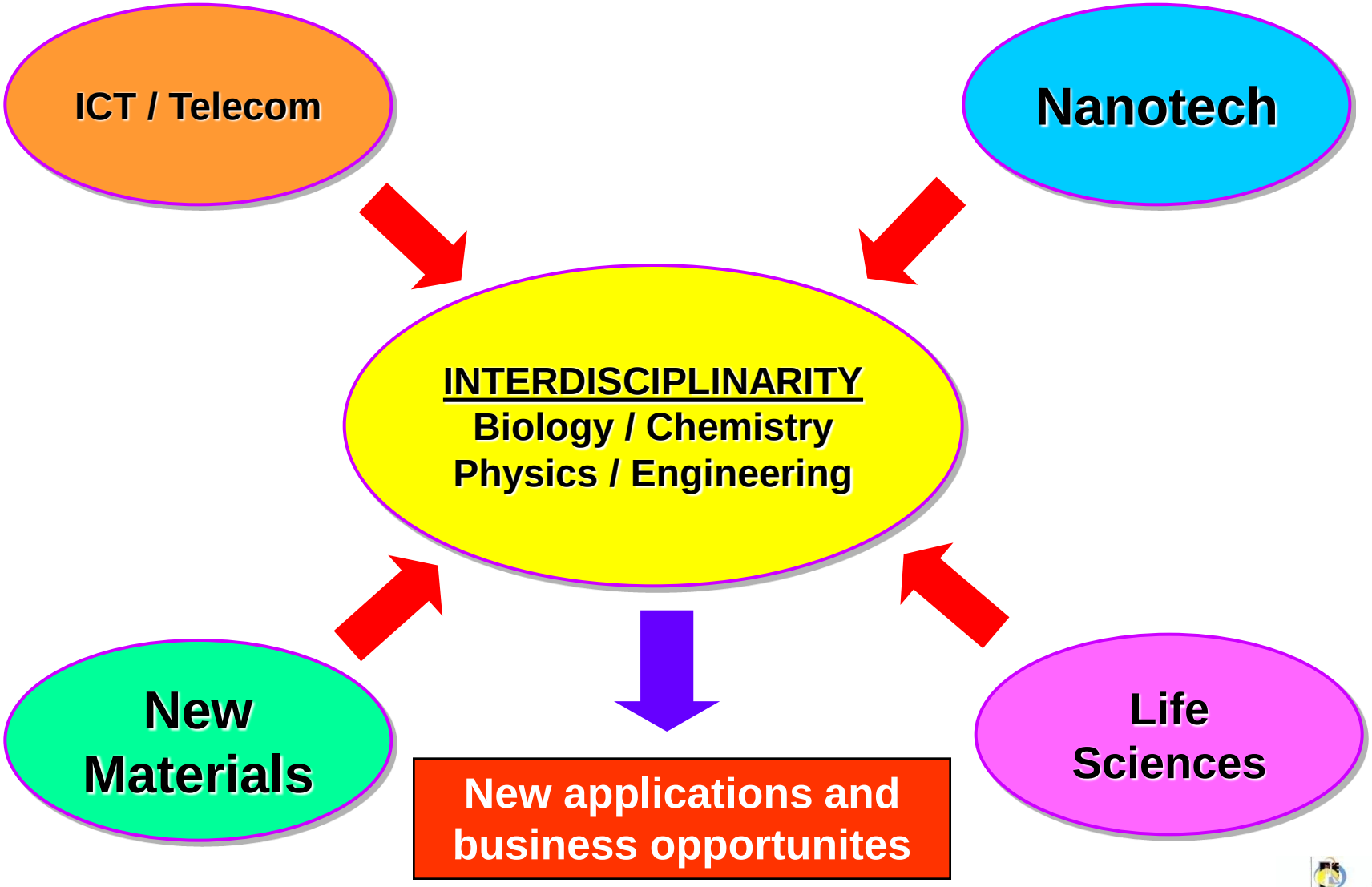
- Best therapy for cost
- Drug performance
- Best practices

Payers

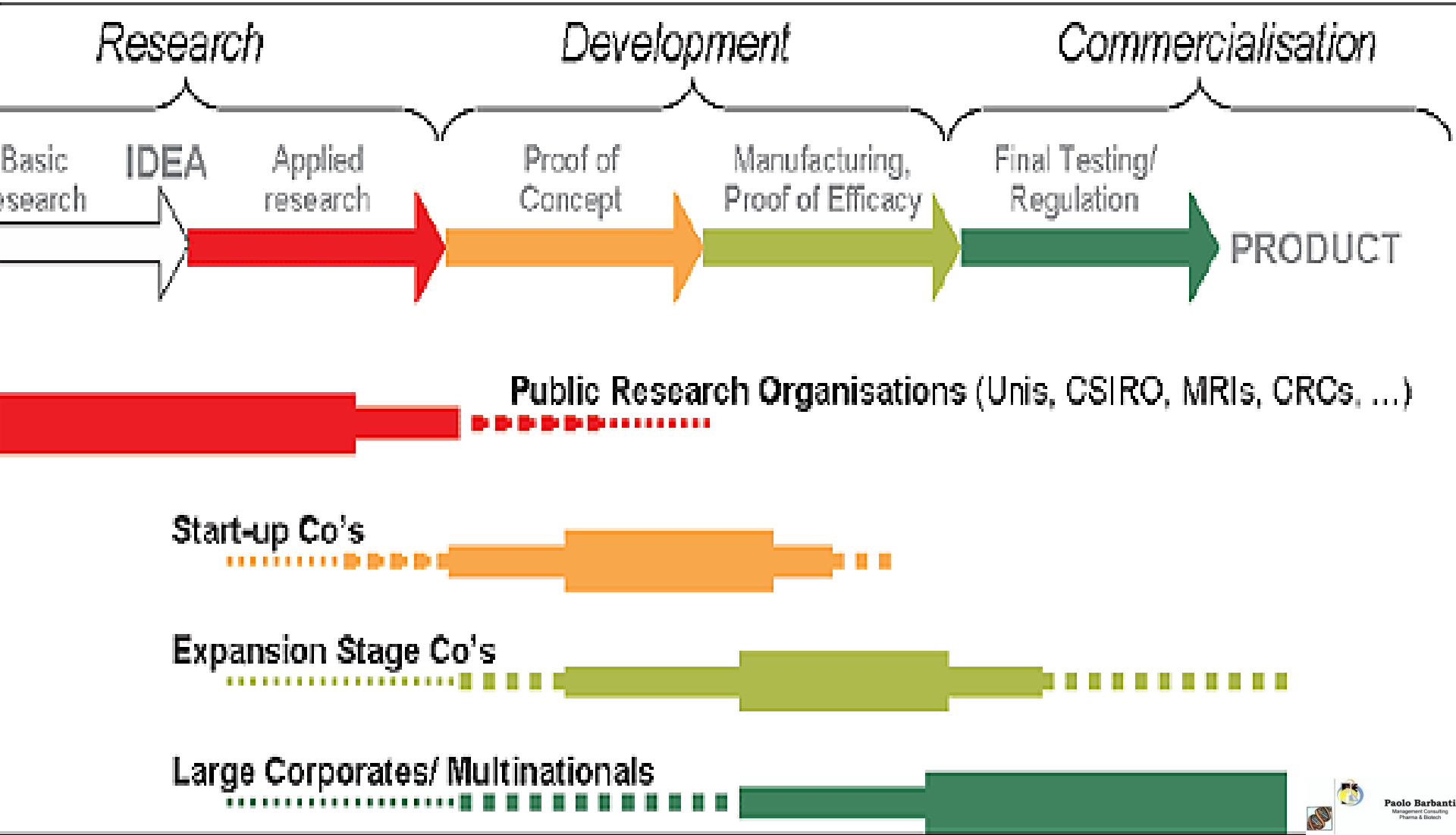
What's driving this change

- New technologies (biotech, systems biology…)
- An aging population (US, Europe)
- Consumers are consolidating
  - Managed-care
  - Little room for "me-too"drugs
  - Increased use of drugs vs. expensive hospital care
- Greater interest/access to "healthy foods*"(i.e., nutraceuticals),* supplements, and "total wellness"
- Virtual integration

Convergence in technology: scientific advances and new business opportunites



The product development value chain



New trends in Dx

- **Accellerated growth and expanding margins**

- **Convergence of diagnostics and therapeutics** *(personalized medicine)*

- **Value-based pricing**

Drivers of new diagnostics

- **New targets** *(genomics and proteomics)*
- **High cost of new drug development**
- **Medical utilization**
- **Adverse drug reactions (ADRs)**
- **Preventive / personalized medicine**

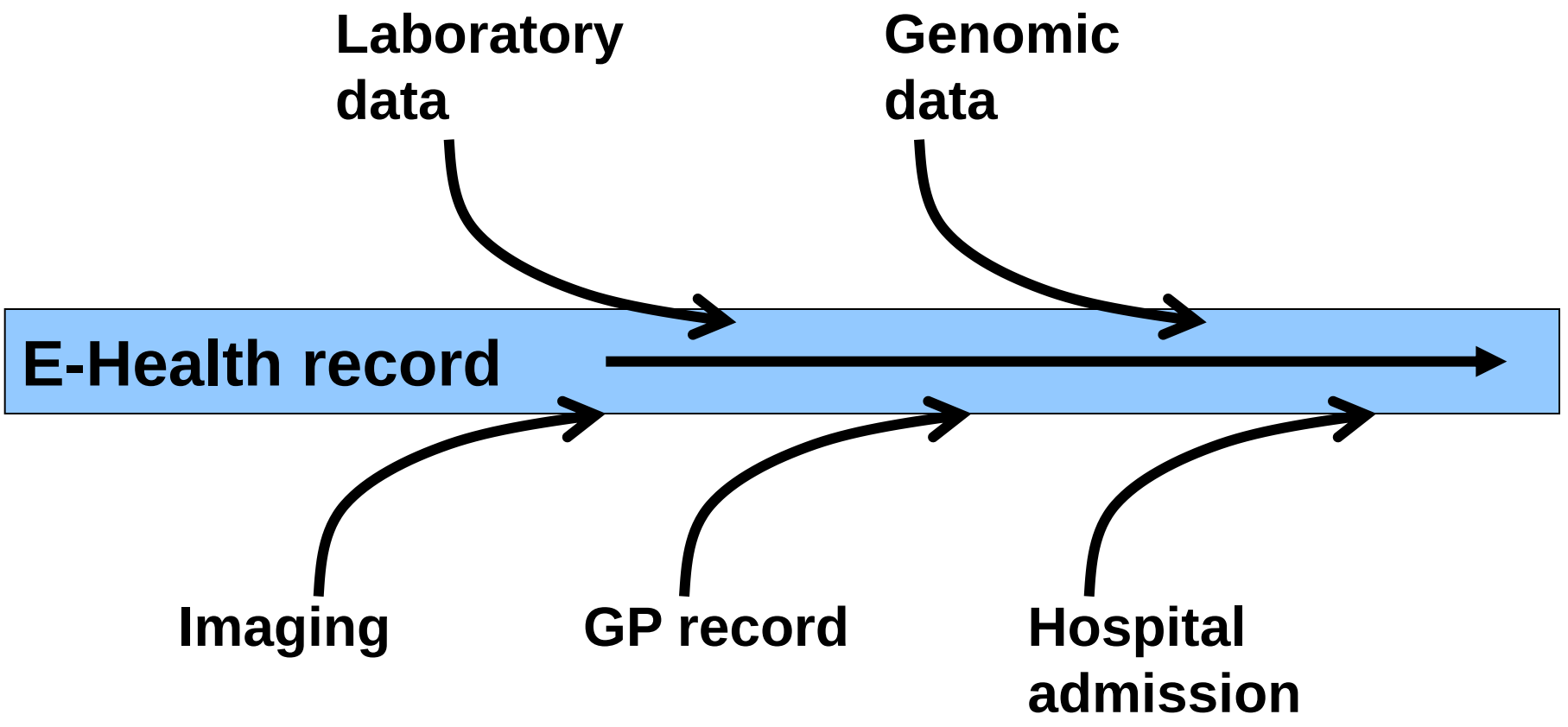
Types of medical data

- lab results
- administrative orders, appointments
- images
- signals, ecg
- microbiology results
- demographic
- familiar
- history of prescriptions
- genetics

Health informatics subdivisions

- Information level
  - (molecular, cellular, tissue, organ. patient, disease, population)
- Clinical specialties or diseases
  - (cancer informatics, cardiovascular, ...)
- User
  - (patient, clinical, pharma, nurse, bio)
- Agent
  - (HMO, hospital, government, ...)
- Technology
  - (Telemedicine, decision making, imaging, genomics)

Integration of patient data



ICT and Biotech

- Rapid **convergence** of biology and IT - a reality
- Areas of knowledge generation and collection:
  - Genomics and Proteomics
  - Clinical trials
  - Libraries
- Key functional areas:
  - Data acquisition
  - Storage
  - Analysis
  - Dissemination
- Key Issues: database integration, transparent accessibility, speed

DATA environment is rapidly changing

Healthcare organizations are facing a deluge of rich data that is enabling them to become more efficient, operate with greater insight and effectiveness, and deliver better service

Sources of the data deluge

Mobile | EMRs | Paper / Text Documents | Social Media | Images | Videos | Sensors / Devices

40-50%
Annual growth in digital data volume*

62%
Annual growth in unstructured data*

~9X
of unstructured data vs. structured data by 2020**

Advances in computing power and techniques

Smarter Algorithms

Faster Processing Speed

Improved Visualization

Retail Consumerism | **Value Based Care** | **Personalized Digital Health** | **Preventative Medicine** | **Drug Trials and Discovery**

Advances analytical and computing techniques coupled with the explosion of data in healthcare organizations can help uncover leading clinical practices, shrink research discovery time, streamline administration, and offer new personalized engagement paradigms at an industrial scale that align people's decisions and actions in ways that improve outcomes and add value

* HP Autonomy, *Transitioning to a new era of human information*, 2013

** Steve Hagan, *Big data, cloud computing, spatial databases*, 2012

Customer Centric | **Optimal Cost Structure** | **Adaptive Organization**

Healthcare Disruption is underway

24 months

Frequency at which **electronic healthcare data** doubles

150+

Exabytes of available healthcare data today

80%

Of data is unstructured

$7.2 trillion

In **global healthcare spending**; 10.6% of the global GDP

90%

Of the world's data has been created in the past 2 years.

75%+

Percentage of patients expected to use **digital health** services in the future

Source: IBM Watson Health

The Challenges of Big Data

Keeping up

There are 100,000+ clinical trials running in parallel.

A patient will generate >12 TB of personal health data in a lifetime (300 million books).

Medline: 424 million published articles in 5600 journals
1.8 million new articles published annually

80% Unstructured

A typical high-need patient has a 100+ page electronic health record.

Text where meaning is often derived from context

Images: X-rays, sonograms, electrocardiograms, magnetic resonance images, and mass spectrometry results

Noisy

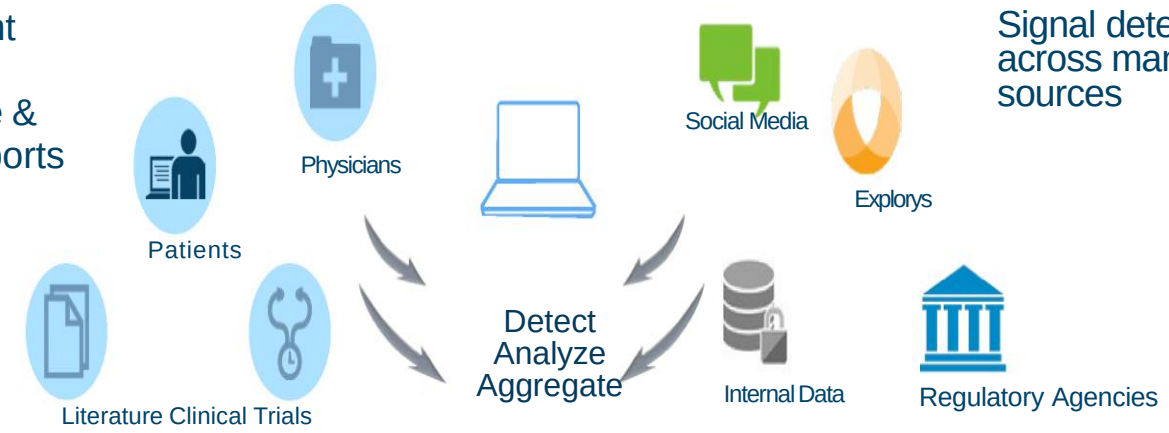
Problems of scale: finding the signal in the noise when its buried in millions of pages across multiple silos

Humans must collect, organize data and evaluate evidence

Introduces cognitive bias

Watson for patient safety

Adverse Event detection within literature & spontaneous reports



Signal detection across many data sources

Potential Healthcare

Benefits

v' Identify AEs within ICSRs and literature automatically and systematically using NLP
v'Scale AE detection with improved consistency, quality and accuracy

Potential Healthcare

Benefits

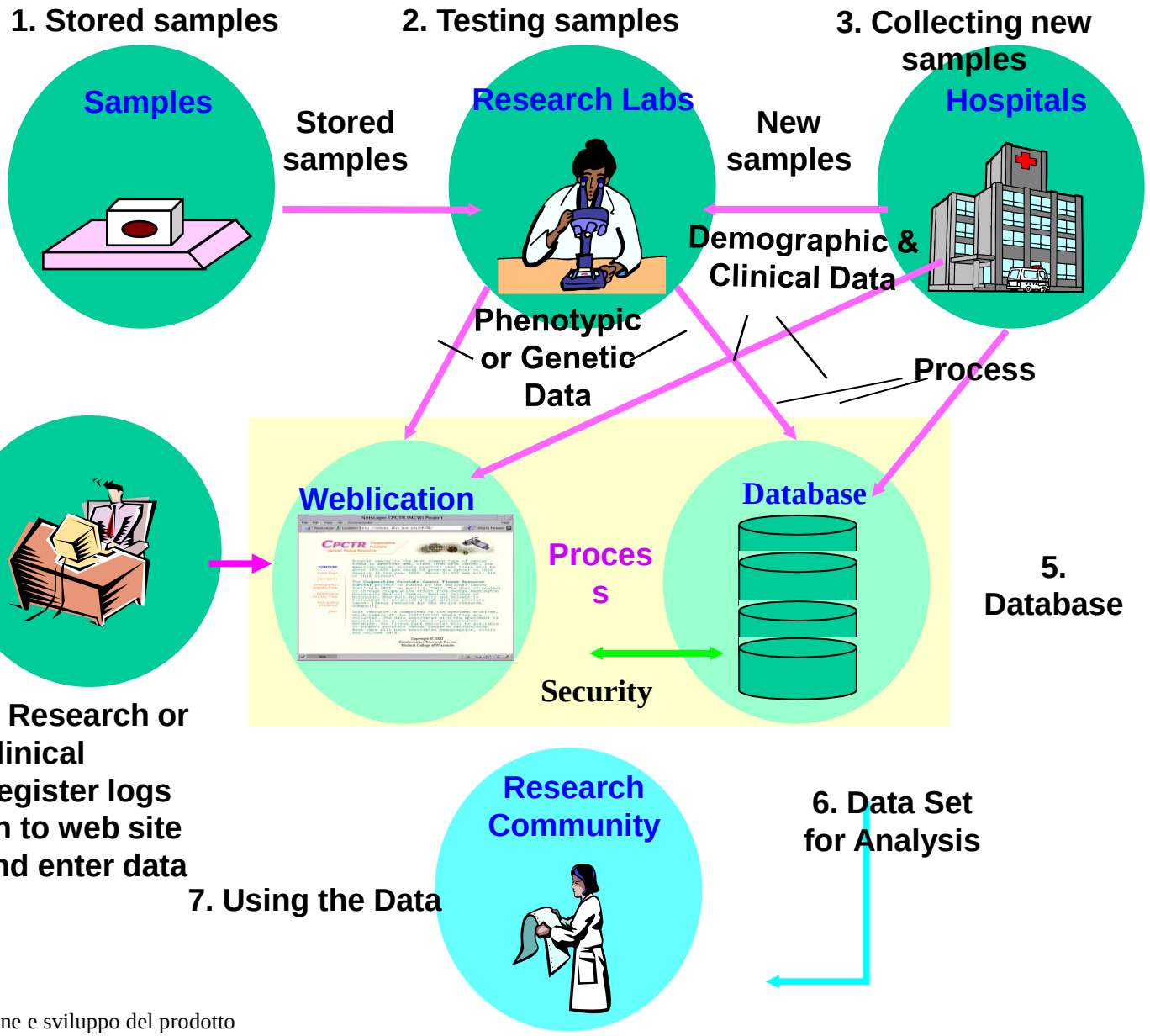
v' Detect potential AEs with greater accuracy and precision through automation
v' React sooner with more information through accelerated signal detection

Dashboard and Alerts for Safety Signals

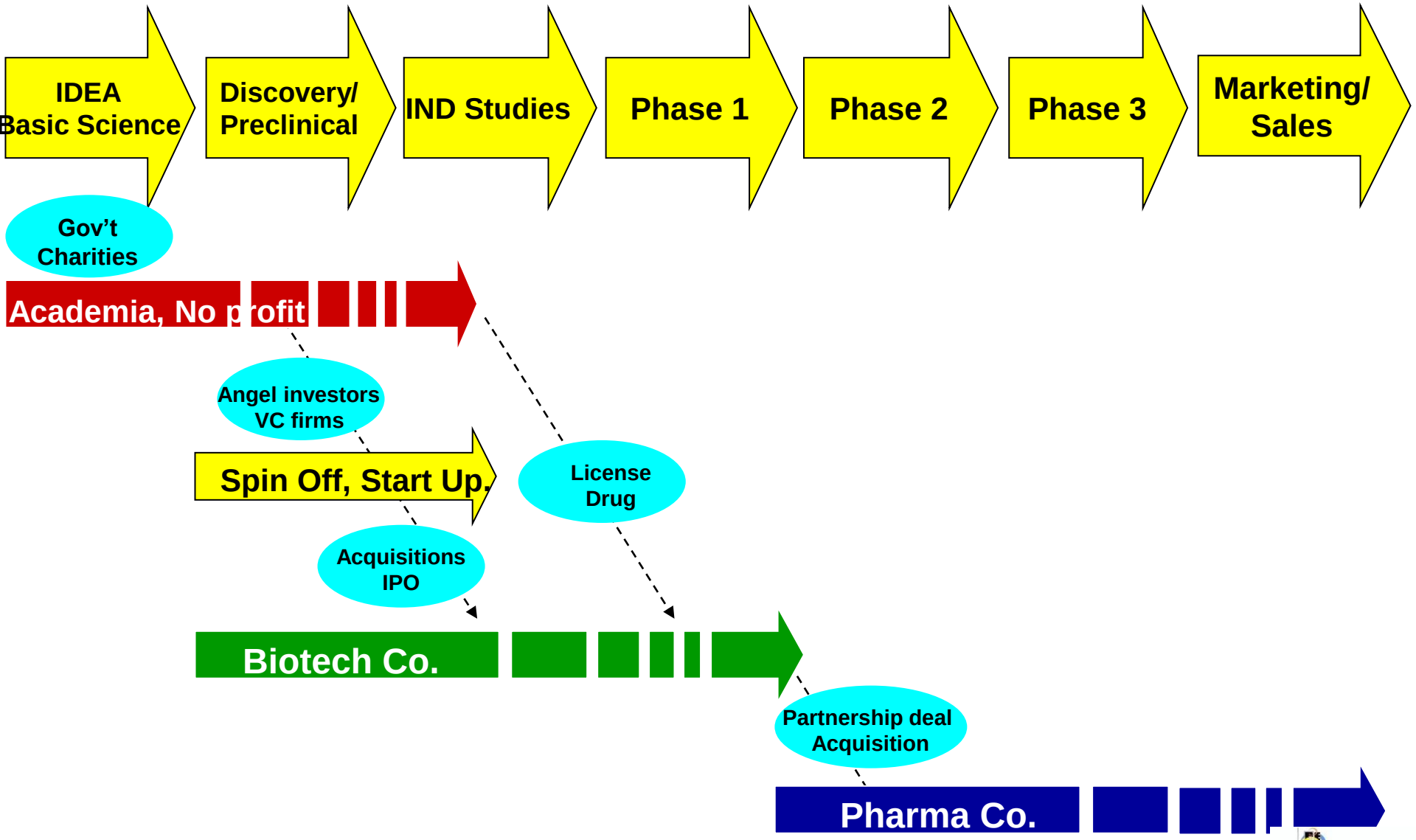
**Watson Health for Patient Safety is currently in development*

Paolo Barbanti
Management Consulting
Pharma & Biotech

Components



Drug discovery, development and industry adoption



Goals and Drivers

- **Academia**
  - Knowledge
  - Teaching/Training
  - Research
  - Research support
  - Open disclosure
  - Economic development
  - Serve public health needs
- **Industry**
  - Profit
  - Training
  - Product R&D
  - Growth
  - Confidentiality
  - ROI (stakeholders)
  - Serve public health needs

Industry-academia relationships: a venture that brings significant benefit

Short term

Consultancy and research by individual scientists
Industrial procurement of services (*Training, targeted contract research: problem solving; patents)*

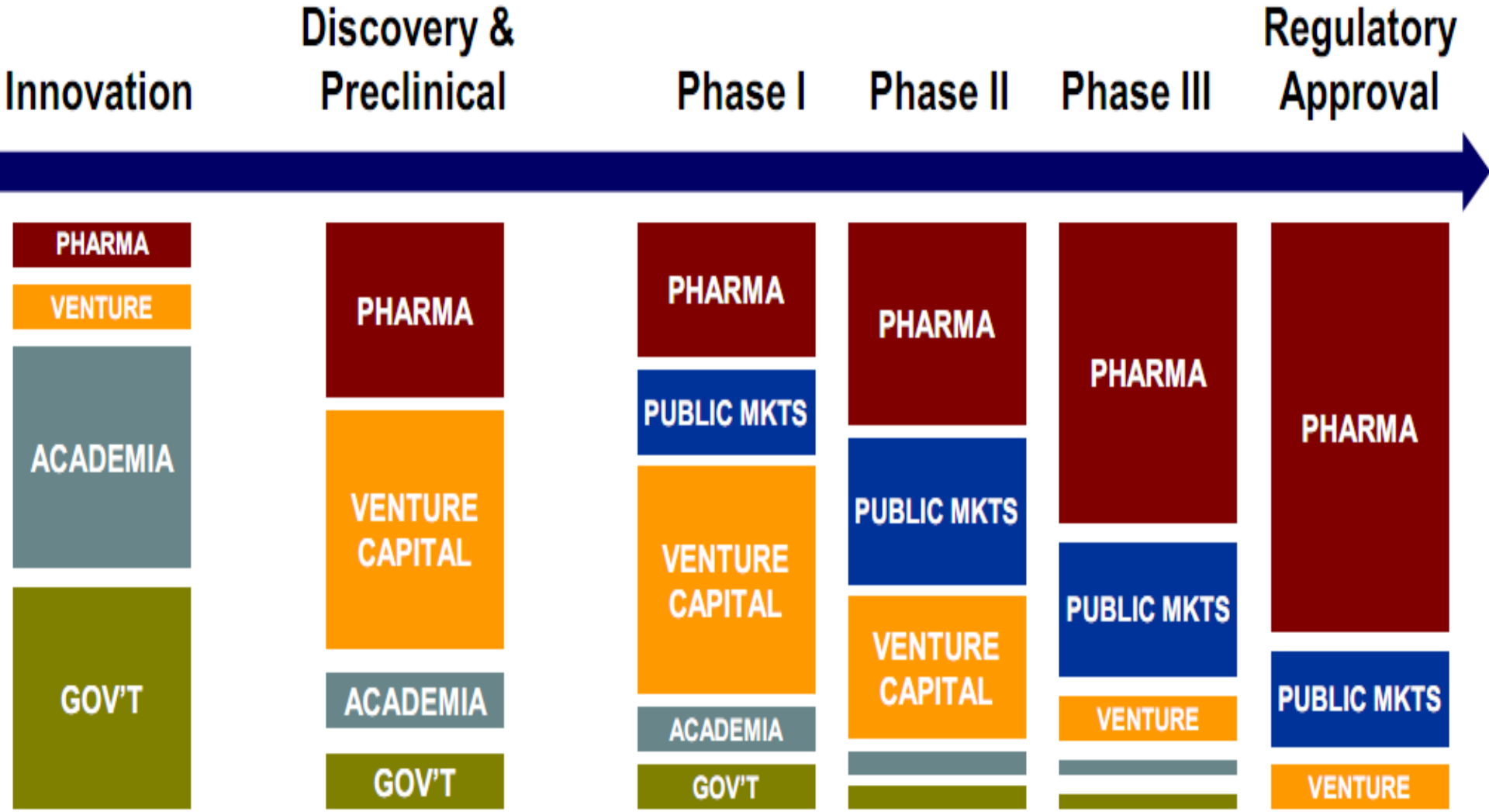
Medium term

Corporate contributions *(fellowships, targeted contract research: design and engineering, development, applied research; pre-competitive research)*
Cooperative research *(joint research programs; R&D consortia; Joint R&D labs)*

Long term

Privately funded research centres:(*multicorporate, single funder)*
Long-term research contracts *(basic, fundamental, precompetitive research)*
University-controlled companies to exploit research
Private companies that secure patents rights for resale

Collaboration required in pharmaceutical industry



The Industry-University interface is multifaceted

- Corporate sponsored research
- Corporate collaborations
- Material and knowledge transfer
- Consulting and other public service
- IP creation and licensing
- Gifts: cash, endowed chairs, equipment, sponsorship of graduate programs
- Graduate fellowships
- Industry consortia (memberships)
- Exchange of personnel, sharing of resources
- Investment in university employee-founded startups
- Networks of service providers and capital that support entrepreneurial activities

The changing landscape and partnering implications

Environmental Factors

- Internationalization
- Consolidation
- Cost containment
- Stakeholder pressure
- Evolving technology frontier

Implications

- Novel sources of Innovation
- Increased deal Throughput
- Diverse means of engagement
- Innovative alliance models

Opportunities of greatest interest

Considerations are based on a mix of attributes and strategic interests:

- Does this opportunity **complement** our existing portfolio?
- Does it offer entry into a **new area** of interest?
- Is it a **novel technology** to reduce attrition and increase R&D productivity?
- Does it address an area of **unmet customer need**?
- Would it be **First/Best in class**?
- What is the **opportunity size**?

What is Technology Transfer ?

The formal transfer to the commercial sector of new innovations, discoveries and inventions resulting from intellectual pursuits, such as scientific research, conducted at universities or other research institutes.

Technology Transfer Process

Three major steps

1. **Submission of an invention disclosure**
2. **Initiation of intellectual property protection**
3. **Formal licensing of the rights of an invention to industry for commercialization.**

The Technology Transfer process

Click the text boxes for more information about each step in the technology transfer process.

Research + Discovery

Disclosure is inappropriate

PRE-DISCLOSURE

DISCLOSURE

Too early

Inventors claim ownership

Ownership Review

Inventor Agreement

ASSESSMENT

Protection or market issues

Offer Back to Inventors

PROTECTION

MARKETING PLAN

MARKETING

No licensee

Post-Marketing Review

Offer Back to Inventors

File PCT

AGREEMENT NEGOTIATION

POST-LICENCE DILIGENCE

Revenue