Biotech Concerto #1

Brief Introduction to the Pharma/Biotech Business

December 2008

BSI HEALTHCAPITAL

Index

- Drug Research & Development
- Pharma and Biotech
- Biotech and Venture Capital

Drug Development

Drug Research & Development efforts are guided by two principles:

- are the underlying mechanism or cause of a disease understood?
- does this disease represent a significant unmet medical need in patients?

If the answer to both questions is "yes," then a research program is developed aimed at better understanding the disease and finding an effective therapy.

Source: Novartis, adapted

Drug Research & Development

Why are new drugs needed?

... due to unmet medical needs:

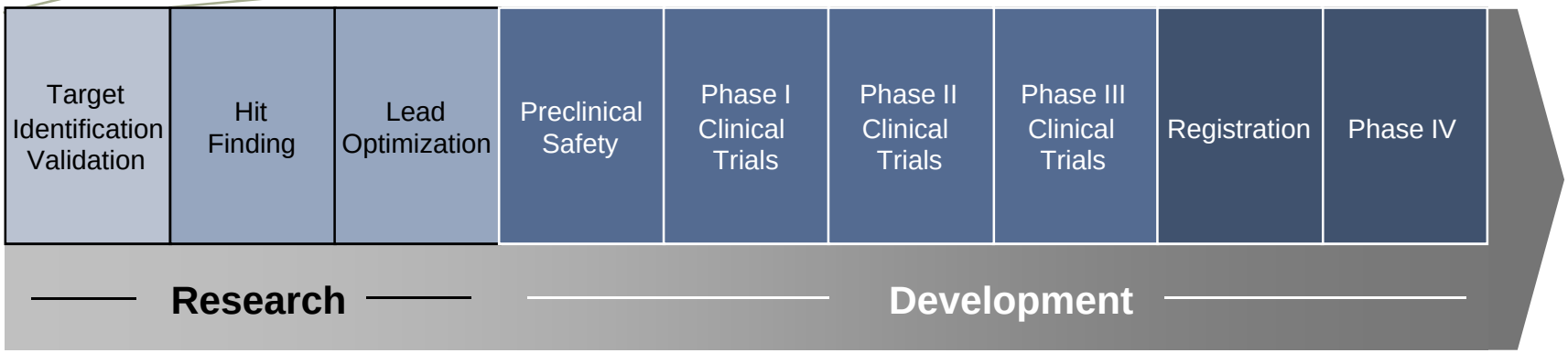
- not yet treated diseases
- new diseases
- low efficacy of existing drugs
- side effects of existing drugs
- cost of therapy
- downstream health costs
- etc.

Source: Ian Hughes, University of Leeds, adapted

Drug Research & Development

Target Identification and Validation

This step encompasses a wide range of scientific activities focused on identifying new targets and confirming their role in disease.

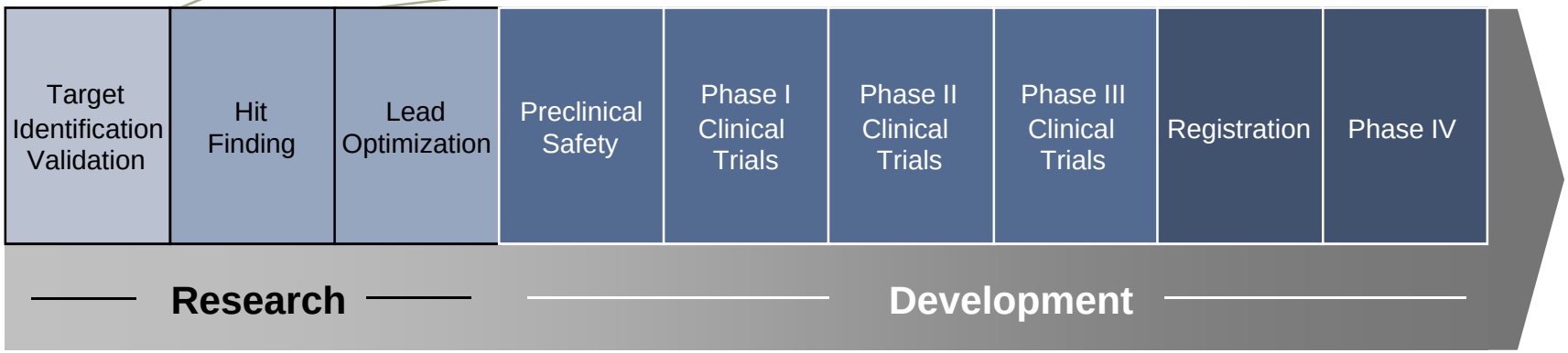


Source: Novartis

Drug Research & Development

Hit Finding

This entails development of robust assays to test small molecule compounds in High-Throughput-Screens. In case of Biologics this stage entails development of antibodies.

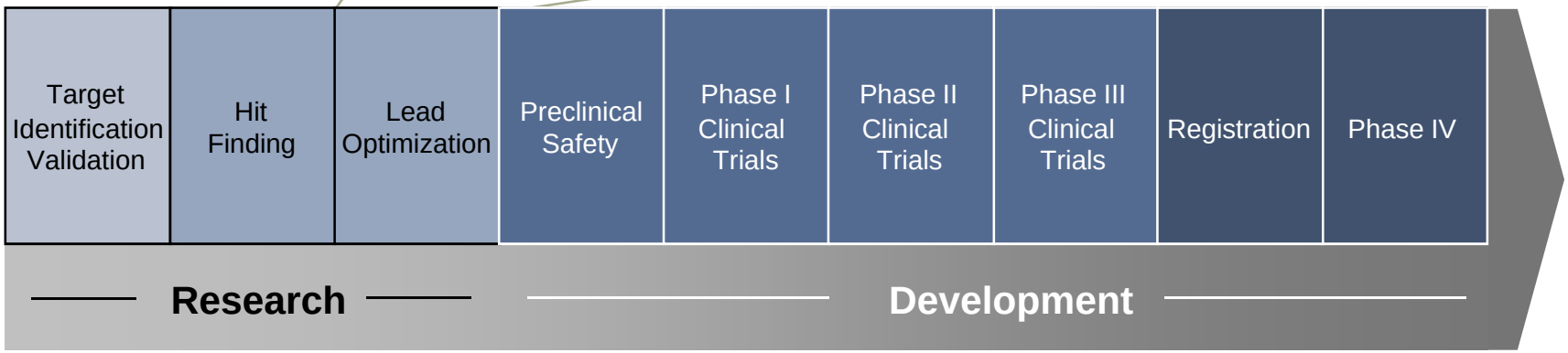


Source: Novartis

Drug Research & Development

Lead Optimization

In lead optimization “small molecules” are chemically altered to improved properties. In case of Biologics, antibodies are modified to increase their affinity for their target.

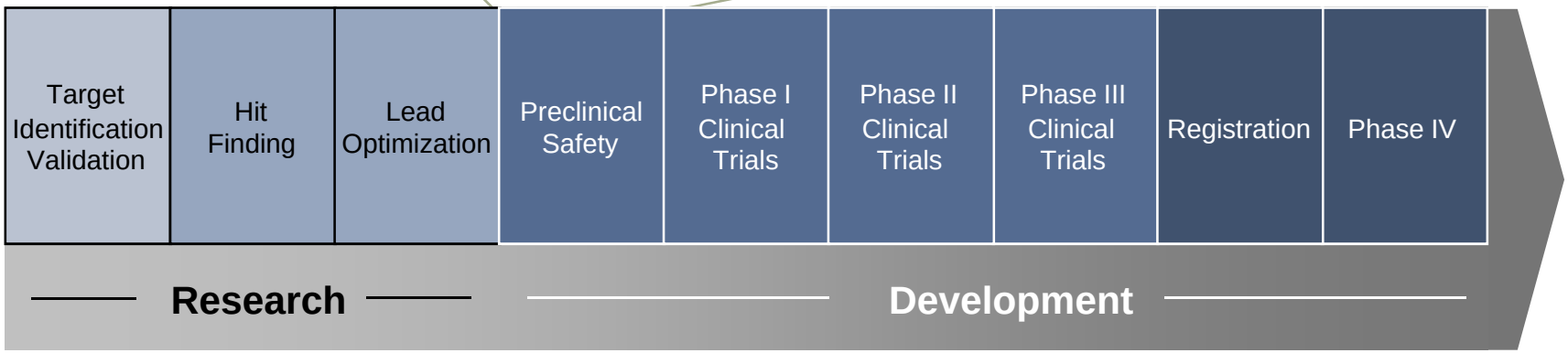


Source: Novartis

Drug Research & Development

Preclinical Safety

To establish an initial safety profile of the drug, extensive toxicological and safety pharmacological profiles are done using in-silico, in-vitro, and appropriate animal models.

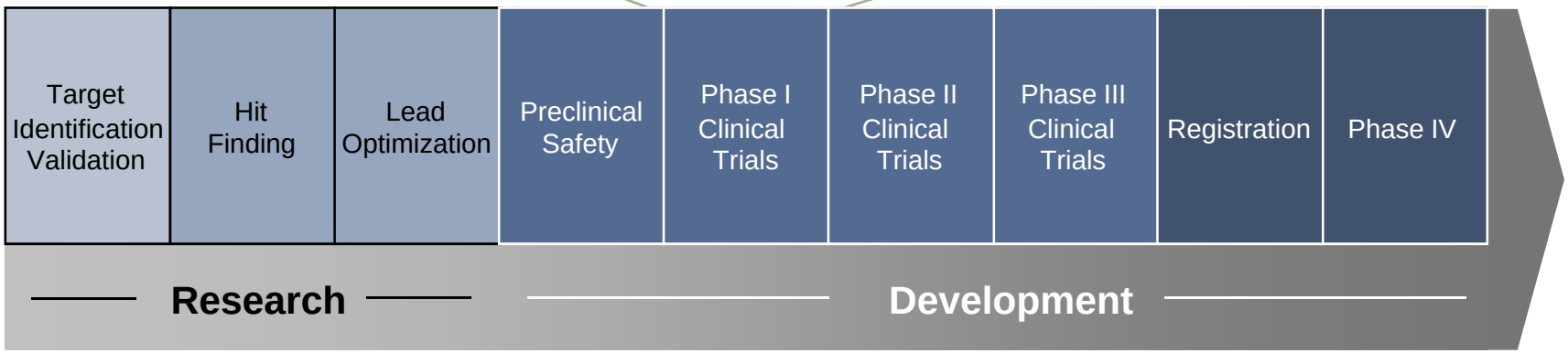


Source: Novartis

Drug Research & Development

Phase I Clinical Trials

In Phase I, the drug is tested in small groups of healthy volunteers (~20) to evaluate its safety, determine its safety dosage range and identify side effects.

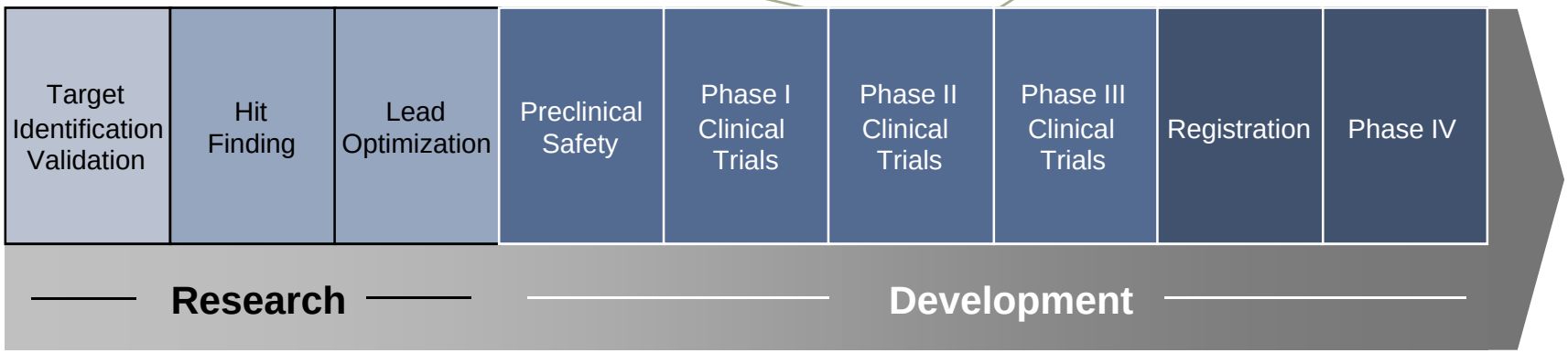


Source: Novartis

Drug Research & Development

Phase II Clinical Trials

In Phase II the drug is given to a larger group of people (~100-300 patients) to test its effectiveness, determine the effective dose range and to further evaluate its safety.

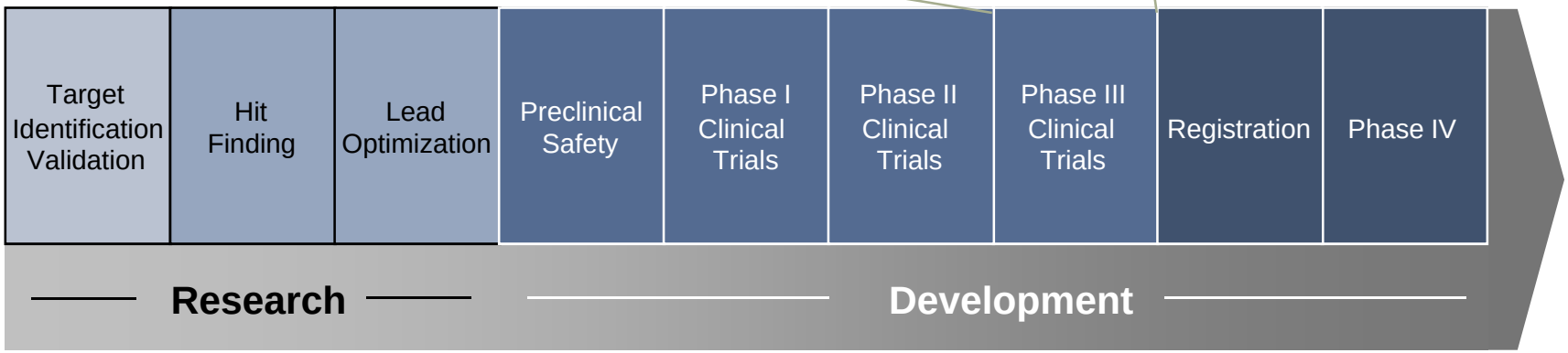


Source: Novartis

Drug Research & Development

Phase III Clinical Trials

In Phase III the drug is given to a large group of patients (~1000-3000) to confirm its effectiveness, monitor side effects, and compare it to existing treat-ments.

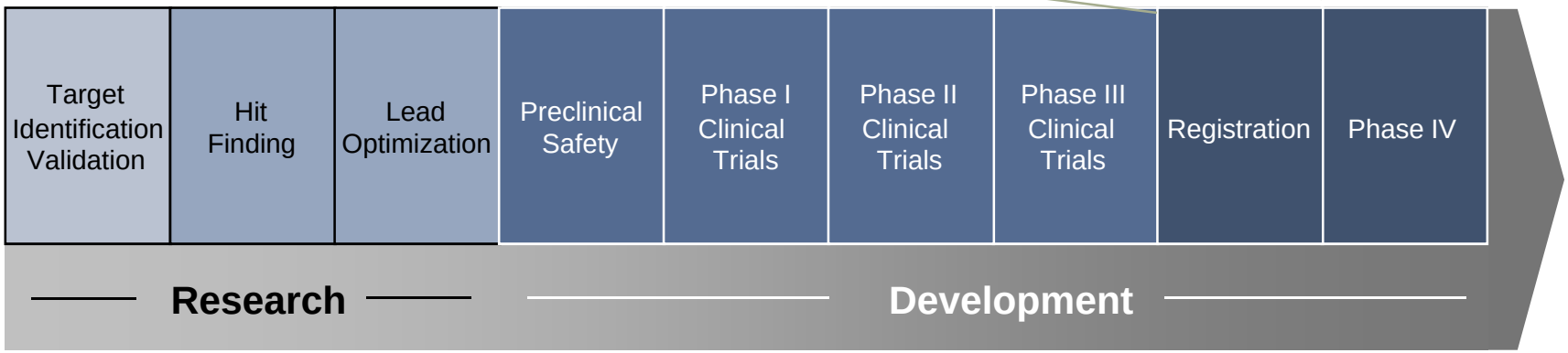


Source: Novartis

Drug Research & Development

Registration

For the registration of a new drug, the results of preclinical test and clinical studies, quality data, manufacturing process are reviewed by the regulatory authorities.

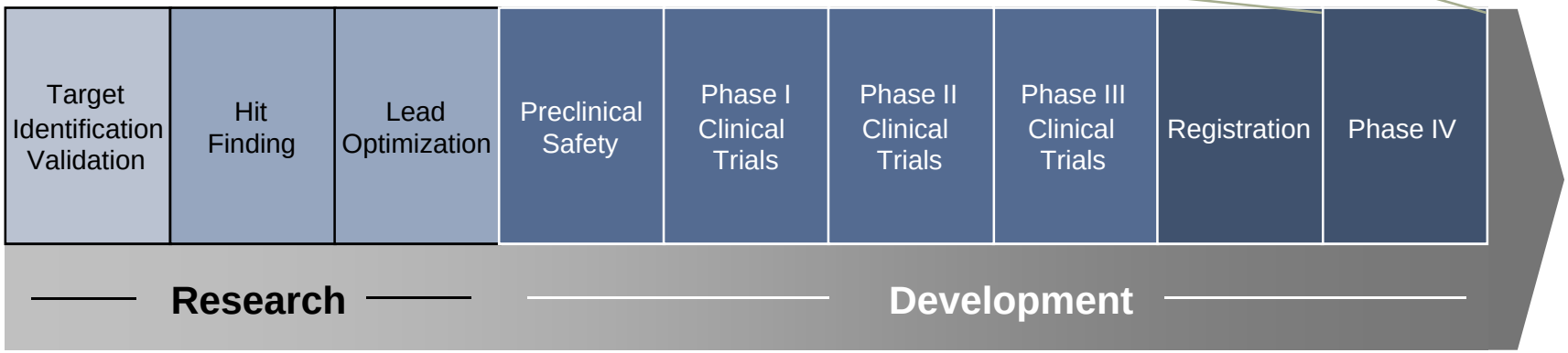


Source: Novartis

Drug Research & Development

Phase IV

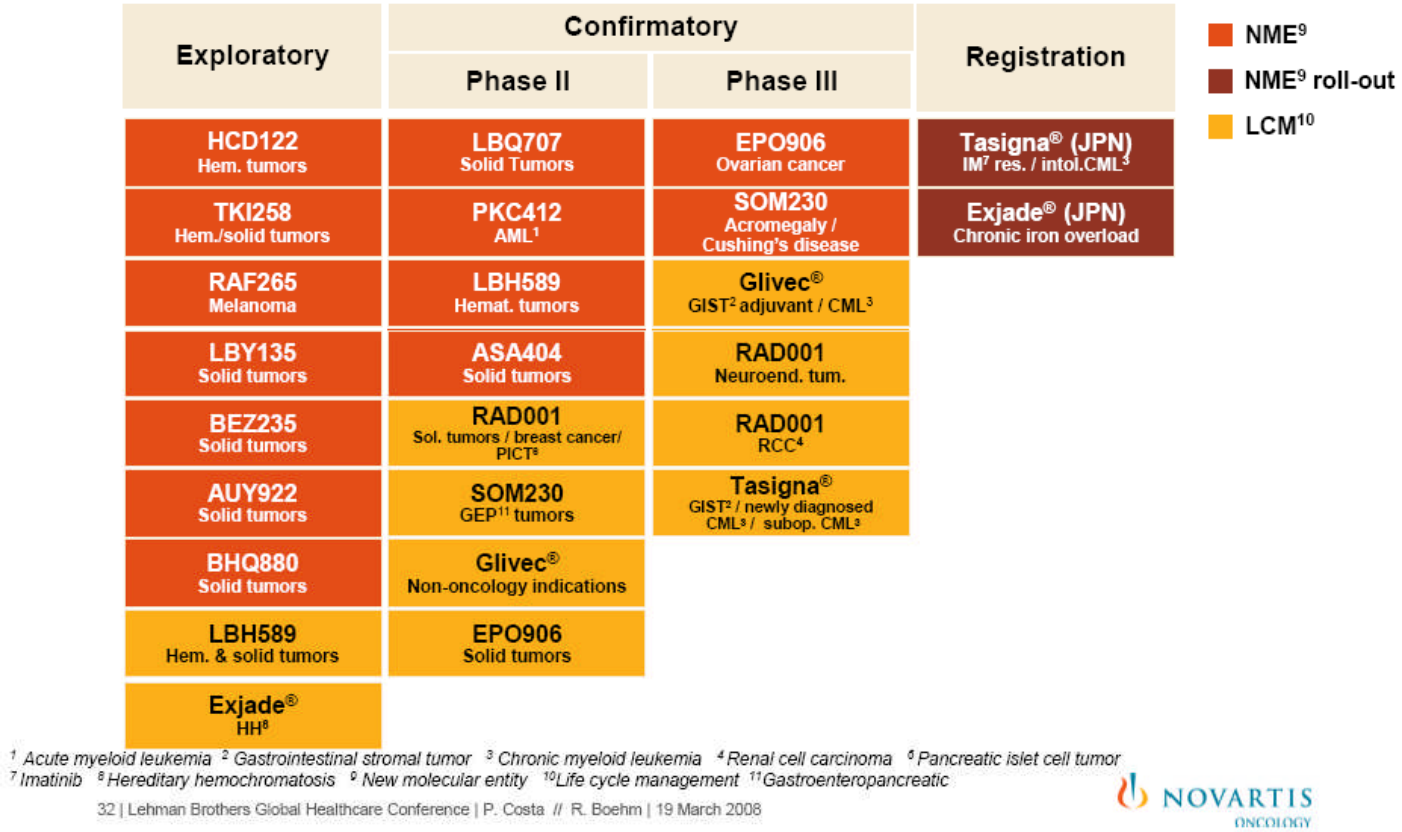
Once a drug is on the market, adverse effects need to be monitored/reported. Life-cycle management programs aims at new indications and/or improving formulations of the drug



Source: Novartis

Drug Research & Development

Example of a Drug Pipeline: Novartis Oncology, Q1/2008



Source: Novartis

Drug Research & Development

Industry-typical Key Data

	Discovery	Preclinical Studies	IND Filing	Clinical Phase 1	Clinical Phase 2	Clinical Phase 3	NDA Filing	FDA/ EMEA	Phase 4
Years	2 - 6	3.5		1	2	3		2.5	
Test Population	Laboratory	Laboratory and animal studies		20 to 80 healthy volunteers	100 to 300 patient volunteers	1'000 to 3'000 patient volunteers		Review and approval	Post-marketing testing
Purpose	Discovery of new chemical/ biological entities	Assess safety and biological activity		Determine safety and dosage	Evaluate effectiveness and side effects	Verity effectiveness and monitor adverse reactions			
Cost (USD mn)				0.1 - 1	10 - 100	10 - 500			
Success Rate	10'000	10 - 20		5 - 10	2 - 5	2		1	

FDA: US Food and Drug Administration
EMEA: European Medicine Agency
IND: Investigational New Drug Application at FDA/EMEA
NDA: New Drug Application at FDA/EMEA

Source: Pharmainfo.net, adapted

Pharma and Biotech

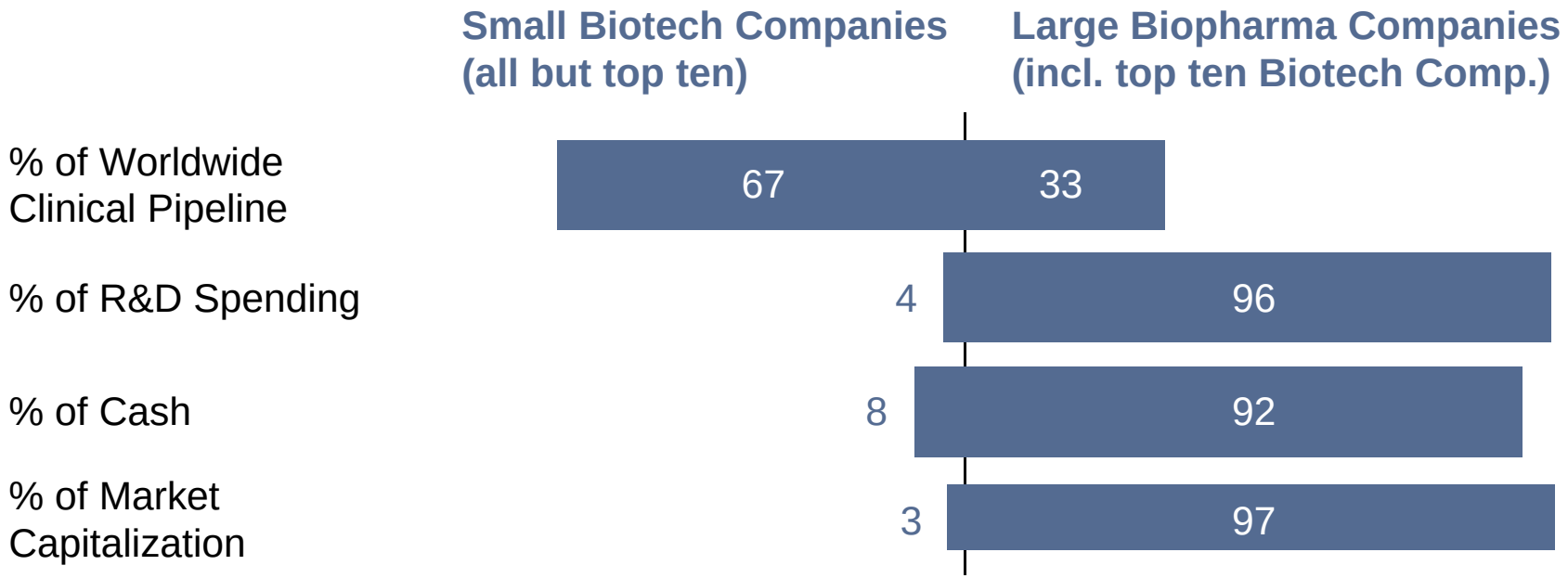
In earlier days, there was a specific distinction between old-style pharmaceutical companies and biotechnology companies. Biotechs genetically engineered proteins from the body, like human growth hormone, for use as therapeutics and produced them in mass quantities. Pharmaceutical companies were medicinal chemistry companies that explored the globe looking for compounds to screen against assays.

Nowadays that distinction is meaningless. When people use the phrase Big Pharma or pharmaceutical companies, they are referring specifically to large, global drug companies that research, develop and sell all kinds of drugs (and sometimes medical devices). Biotech now refers to development-stage companies of many ilks. Biotech encompasses genomics, gene therapy and combinatorial chemistry, as well as traditional protein development companies, and many other permutations.

Source: TheStreet, adapted

Pharma and Biotech

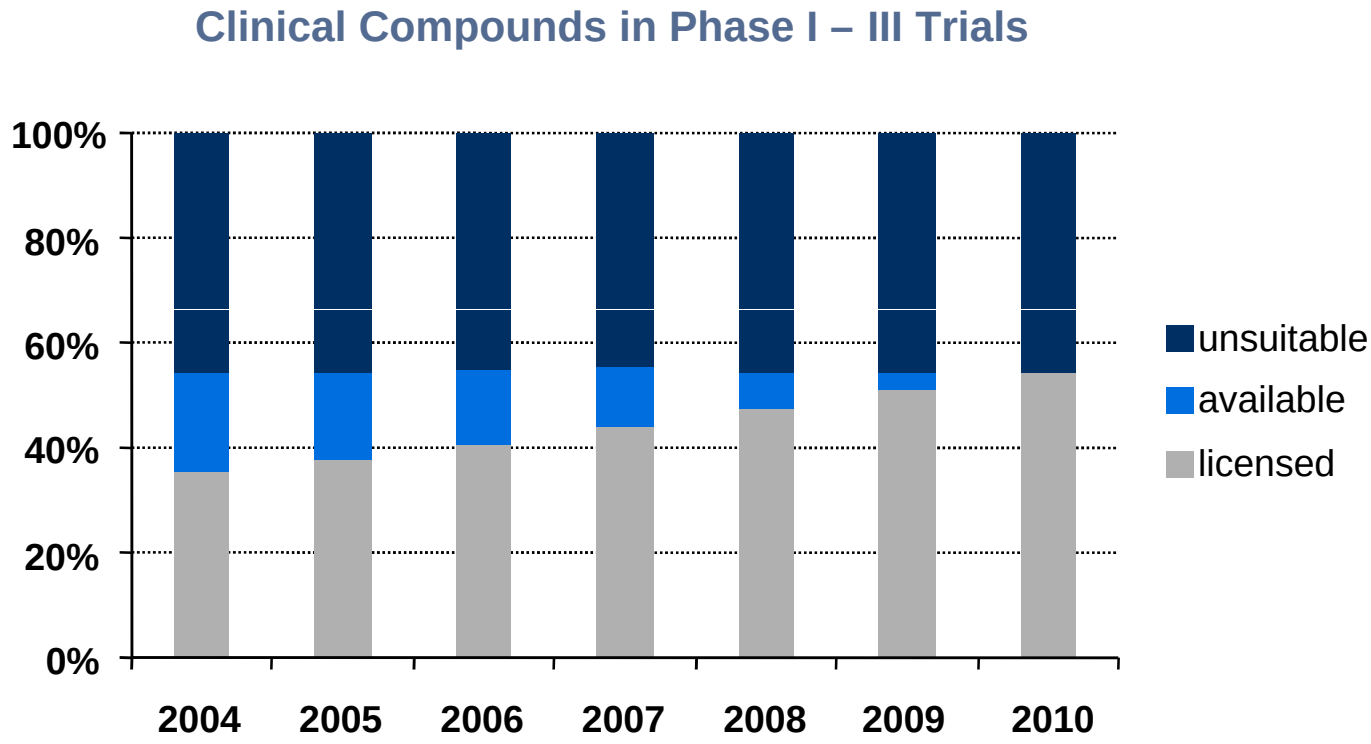
Biotech – The Innovation Powerhouse



Source: Lehman Brothers; Pharmaprojects; Value Lines; BCG

Pharma and Biotech

The Race for Licensing Deals



Source: Pharmaprojects; Recombinant Capital; FDA; BCG

Pharma and Biotech

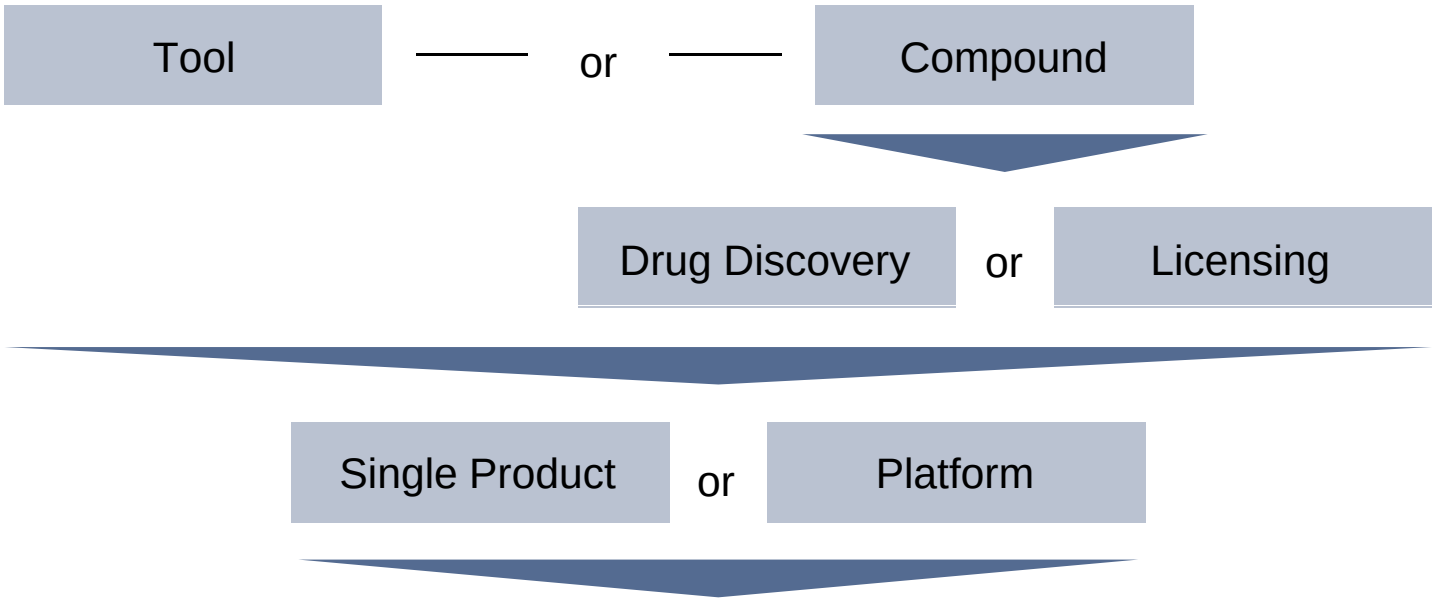
The Need to Play the "Dating Game"

Pharma Perspectives	Biotech Perspective
• Shrinking pipeline • Expiring patents • Decreasing revenues • R&D efficiency	• Ever existing financing needs

Source: Mayer Brown LLP

Pharma and Biotech

Biotech's Generic Business Model



Biotech and Venture Capital

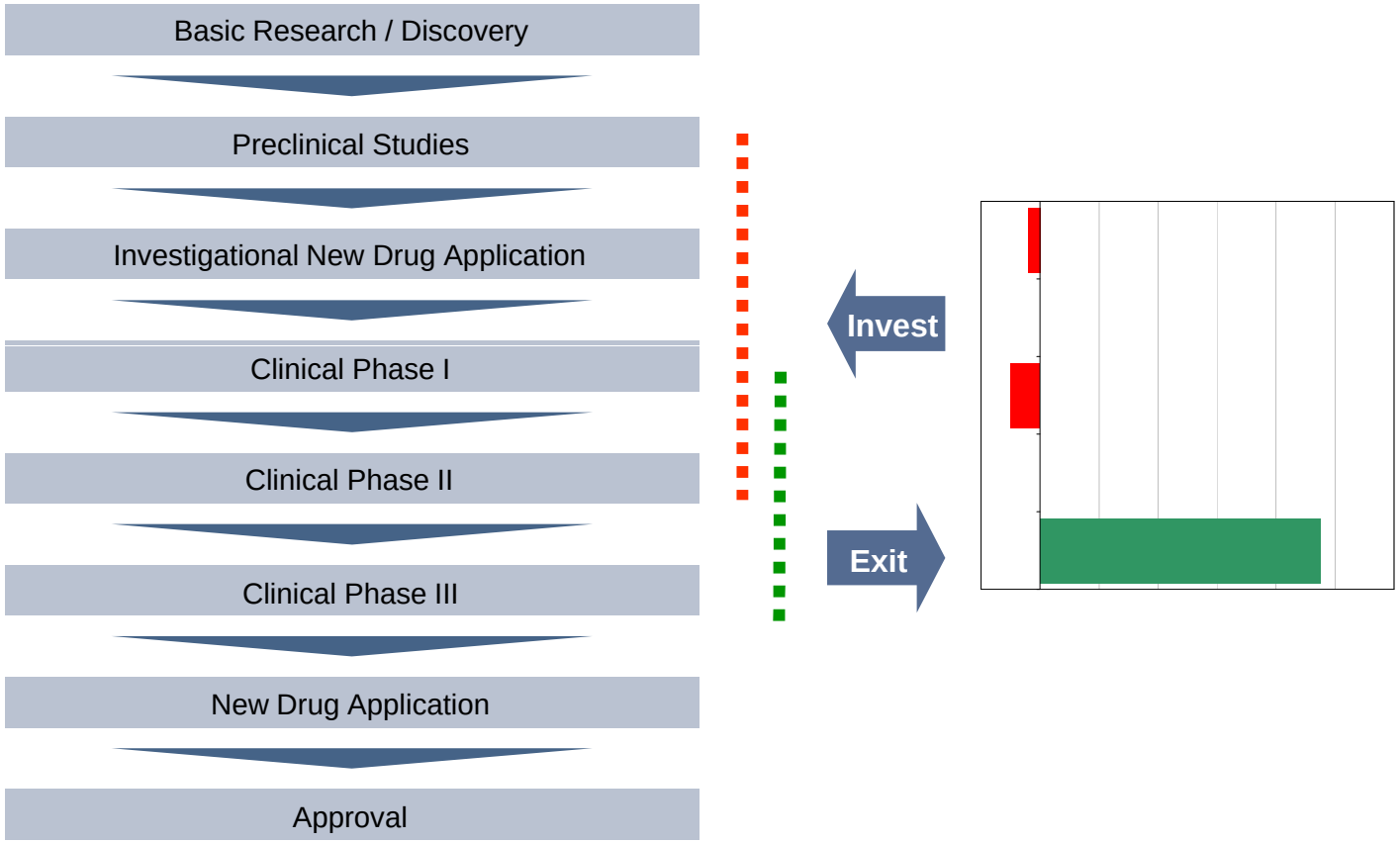
Having a great technology, a promising product pipeline and experienced management is the key to success within the biotech industry, but the proof is in the financing. Venture capital funding within the biotech industry accounts for a significant amount of the total funding, averaging roughly a quarter of all funding, including initial public offerings and additional follow-on funding.

The transition from angel money and early-stage federal grants to venture dollars is a daunting task for many startups and, in this hungry market, a competitive one.

Source: Bioentrepreneur

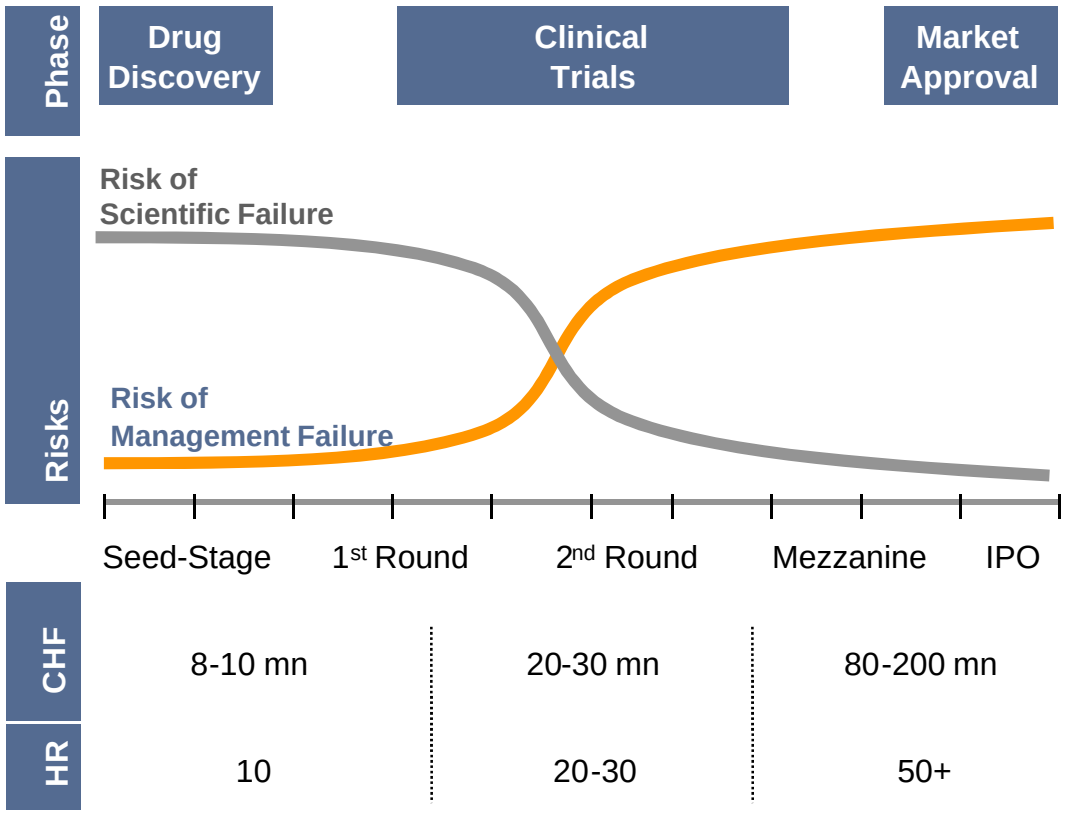
Biotech and Venture Capital

Venture Capital Strategy



Biotech and Venture Capital

Biotech Venture Risk Profile



Source: Aravis Ventures

Contact

BSI Healthcapital SA

Via F. Pelli 2
CH-6900 Lugano / Switzerland

Phone: +41-91-911-8415
Fax: +41-91-911-8414

info@healthcapital.ch
www.healthcapital.ch

BSI HEALTHCAPITAL