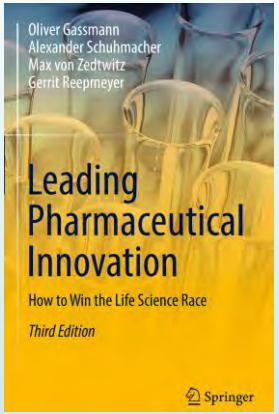
Future Trends and Directions

Presented by Gabrielle Kerkow

Application Specialist, Biotage
2nd Year MBT Student at SJSU



Three Levels of Future Change

Level 3: Leading people for innovation and change

Level 2: Redefining and rethinking the business model

Level 1: Increasing R&D efficiency and effectiveness

*Each level shown here are equally important, yet each company will need to prioritize actions and strategies differently and in accordance with their own intended path.

Level 1: Increasing R&D Efficiency and Effectiveness

Opening-up the Innovation Process

Companies rely external partners to improve efficiency of their R&D organizations → even more critical in the future.

Partnerships will be created that go far beyond the well-known technology sourcing strategies represented by early pharma-biotech alliances.

Why do companies increase R&D collaborations with external partners?

Intensive competition, access to markets, scarce own resources, lack of know-how, cost cutting or restructuring, growth aspirations, synergies and efficiencies, and risk reduction

More Focus on Commercialization Along the Value Chain

Any strategy aimed at improving the R&D pipeline will need to reiterate the focus on commercialization, even of early research results.

While the search for in-licensing candidates is widely accepted, the search for out-licensing candidates coming from own research and intellectual property is not sufficiently enforced.

Intellectual property should be actively created and bundled so that it can be marketed subsequently to potential licensees.

Level 1

New Discovery Technologies to Leverage the Potential of Personalized Medicine

NGS / WGS

(NGS) Next-Generation Sequencing is a high-throughput cost-efficient method to identify mutations in the whole genome of individuals to predict causes of disease

(WGS) Whole Genome Sequencing is a high-throughput profiling of patients that matches their genetic profile with the profile of investigational drugs.

Microchips

Microfluidic (lab-on-a-chip) = miniaturization technologies used for bioassays and cell biology in biomedical research.

This technique allows for high-volume biological testing in drug discovery, and mimic organs on a chip.

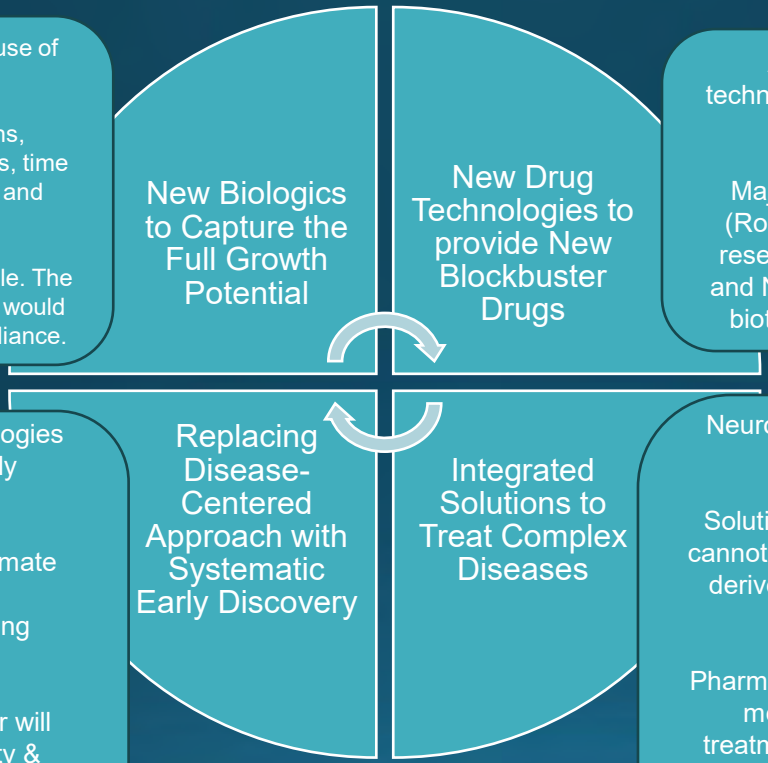
Expected to deliver exponential performance improvement over time, resulting in radical cost reduction.

Level 1

Biologics are trending in the industry because of the convenience and reduced costs.

Antibodies are administered by injections, associated with hospital administration costs, time investments for doctors & patients, travel and infrastructure.

Orally available antibodies could be available. The advantages of these kinds of new biologics would reduce costs and an increase patient compliance.



ADCs & IVT mRNA-based therapeutics are technologies that have the potential to launch the next wave of blockbusters.

Major research-based pharmaceutical companies (Roche, Pfizer and Sanofi), have invested in ADCs research. Other pharmaceutical companies (Eli Lilly and Novartis) have accessed ADC technologies from biotechnology companies through licensing deals.

With the implementation of the technologies already in use research will increasingly center around molecular drug design.

Use of these technologies lets us automate most of the discovery effort, having a comprehensive and consistent screening process.

Research bottlenecks and human error will be reduced. As a result, both the quality & quantity of resulting lead compounds are expected to increase.

Neurodegenerative diseases are a major burden for healthcare systems.

Solutions in the treatment of complex CNS diseases cannot solely come from drug discovery but need to be derived from a combination of pharmacological and non-pharmacological interventions.

Pharmaceutical companies will need to merge with the medical device business to expand their CNS treatment options with electric, robotic and software-based approaches.

Thus, pharmaceutical companies will need to change from pure drug makers to integrated healthcare solution providers.

Level 1

Reducing Serendipity in Discovery by Balancing Data Generation & Data Analysis Tools

- Huge amounts of data are being generated and collected **every day**.
- Following the success of these new information production capabilities, we are **still developing the techniques to manage and interpret all the accumulated data**.
- Medicine is looking for **new ways to find knowledge** in data sets we already have.

Focusing on Complexity and Diversity in Compound Libraries

- Besides applying more advanced screening methodologies, the quality of the substances to be screened must improve as well → **The structural complexity and diversity of the compound libraries must increase.**
- Goal to increase the probability to find substances capable to influence a given target in a specific desired way.
- The ability to manage complex knowledge—identifying relevance and dealing with information dynamics—will be key for R&D driven pharmaceutical companies in the future.

Level 2: Redefining the Business Model

Finding the “Sweet Spot” in the Pharmaceutical Value Chain

Decelerators in RnD: difficulties to transfer know-how and an unclear intellectual property situation

- The make-or-buy decision of a contracting pharmaceutical company (balance between in-house and external activities)
- Control within Collaboration.

Becoming a Knowledge “Leverager”

The pressure to innovate makes it necessary to realign R&D concepts to a more open and cost-efficient setting.

The Knowledge Leverager in open innovation is skilled at leveraging external innovation with the subsequent benefit of higher R&D output while reducing R&D costs.

Radical Knowledge Leverager Concept: virtual R&D teams & a high proportion of externally acquired pipeline projects.

Developing Downstream Capabilities within RnD

A stronger market-orientation in R&D is one of the primary tasks of all future R&D activities.

- This requires a stronger business orientation of research managers and key scientists. This is not easy since 80% of all scientists in the pharma sector will never see the commercial results of their efforts.
- Ultimately, a stronger market-orientation is expected to result in a shift from a product to a patient-driven strategy.

Level 2

Exploiting the Potential of Emerging Markets

Pharmaceutical companies with reduced R&D efficiencies cannot meet their growth objectives solely by product innovation, and they turn to:

- Generics
- OTC products
- Emerging Markets

Pharmaceutical companies need to expand their business models from purely research-based and focused on traditional markets to more diversified and balanced with respect to the specific needs of emerging markets.

Specialty Medicine as a Scope of Business

Traditionally, medicines for treating diseases with high prevalence and high earning potential have been driving pharmaceutical R&D pipeline decision making.

Torn between commercial needs and ethical interests, specialty medicine will have a future once development costs meet lifecycle returns.

Especially those specialty drugs with shorter time-to-market and leaner reimbursement and pricing have blockbuster potential; the trend toward creating blockbusters in this field will increase in the future.

Level 2

Strategies for How to Benefit from Scientific Breakthroughs

The integration of **genomics**, **proteomics**, **molecular design** and other technologies will lead to **improved target identification**, **enhanced lead optimization**, and improved clinical trial designs that speed up approval.

This will **signal a shift** from **broadly targeted drugs** to more **focused medicines** with much **higher therapeutic value** for the target population.

But access to genomics technologies is not cheap. An estimated **minimum US$100 million annual commitment**, top-tier pharmaceutical companies are likely to be the first to fully integrate these new technologies.

The more we understand the human genome, the more we will be able to use validated biological targets as starting points for new drug development.

The number of these targets is expected to grow twentyfold to over 10,000.

Level 2

Understanding and Exploring the Business of Consumer Electronics

New competitors are creating a market that requires the overlap of technology & health care. **Apple**, **Samsung** and **Google** work on wearable devices with **lifestyle management** and **accident**, **injury**, and **disease prevention functionalities**.

Their products do not need much clinical development **or FDA approval**. Instead, customer acceptance determines success of the innovation, and new business models based on individual information are generated.

Overall, pharma companies think in compounds and diseases and less in business models.

A business model is the integrated answer to four questions:

1. Who is your target customer?
2. What is your value proposition?
3. How is the value proposition implemented (supply chain architecture)?
4. And why is the business profitable (revenue model)?

Our textbook makes the following argument:

“Software “eats the world,” as Marc Andreessen pointed out in a Wall Street Journal article. Although the urbanization of the economy has not reached the pharma sector yet, a careful rethinking and redefining of the business model becomes more crucial. A business model creates and captures value for the company. The higher the pressure on its profit margins, the more a company needs to think in business models.”

Creating a Mindset of Change & Developing a Transformation Story

Competitive advantage is only temporary: Sustainable profitable growth is only possible if a company continues to challenge the sources of its success.

The annual McKinsey surveys show that **only 30% of strategic initiatives truly succeed** while 70% fail. Two thirds of those failures are caused by **resistance of employees** or **not-consistent management behavior**.

A convincing **transformation story** helps everybody affected to understand the need for change and to increase its acceptance **by nearly four times**, as research on change has shown. The content of a story can be a strong positive vision (“Win against HIV!”) or a threatening mission (“Innovate or die!”). Both kind of transformation stories can work if communicated consistently.

People can be resistant to change. Most companies show cultural inertia and complacency in the face of necessary transformations.

Questions Employees/ Managers may ask:

- Will this really work?
- How will this affect me?
- What will be my new role and my new tasks?
- With whom will I work together next?
- Will I be able to live up to the new expectations, and is my job still secure?

Level 3

Overcoming Functional Silos

People are at the center of organizations. Emphasizing employee development and training is critical. Long lead times and specialized jobs require companies to overcome the distance between early-stage research and the final product by adapting their incentive and job assignment systems.

Product Conception → Company Organization → Sale of Product

Upstream RnD, Discovery, Product Development	Clinical Trials, Drug Formulation, Downstream RnD	Scale-Up, Manufacturing	Marketing, Business Development, Sales
Marketing experts should be included as early in the R&D process as possible. The most successful pharmaceutical companies differentiate themselves by actively confronting the tension between a pure functional organization versus an organization along product groups.	Feedback loops (from the clinical trials back to basic research and the screening stages) should be proactively pursued. Modern drug discovery requires the integration of knowledge from a broad array of disciplines. The formation of multidisciplinary teams, including biologists, physiologists, biochemists, as well as specialists in the traditional disciplines of synthetic chemistry and pharmacology, and more esoteric specialists like molecular kineticists, can help to overcome bottlenecks in creativity and develop unexpected downstream opportunities.	Not every scientist wants to be involved in marketing, but every researcher should have had the exposure to the rest of the business, and the opportunity to pursue a modern, more flexible career. All employees in R&D should ask themselves what their contribution could be across all levels of the R&D process. This approach differs from the traditional, linear structured process and places more emphasis on group and team-oriented R&D.

Level 3

Balancing Scale and Creativity

Creativity in pharmaceutical R&D is more likely to occur in small teams rather than large ones.

Beware! Mega-mergers and the resulting even larger companies endanger R&D's ability to be creative.

Henkel has established the 3 X 6 teams:

Six people work for 6 months together in an autonomous team; six specific product concepts are expected.

The idea of small teams and high time pressure can be transferred to pharma companies. Embedded small functional units within the larger unit will help to retain creativity within the firm.

Fail Often to Succeed Sooner

It is more economical and productive to terminate less prospective projects and concentrate resources on objectively more promising ones.

There is a cut-off point after which project management gives discipline absolute priority over incremental improvements. Before this cut-off, the R&D organization should be designed for maximal creativity and effectiveness of its discovery effort.

Google X deliberately focuses to solve the most difficult questions at the beginning of a project with the goal to learn fast, get external feedback early, and then do the next loop. The more a team fails, the more it learns and has the chance to adapt and succeed. Overall, the combined thrust of all project activities determines a company's competence areas and thus its therapeutic fields.

Level 3

Remember That Drugs Solve Global Problems

Is global R&D a consequence of business decisions, or is global business a consequence of R&D decisions?

Pharmaceutical products can be applied anywhere in the world. It's important to align R&D strategy with corporate strategy. Companies need to clarify what the global decision criteria are and which criteria would influence the initial mission, ramp-up and evaluation of new R&D projects.

The pharmaceutical industry relies on predictable production of new medicines and therapies.

- FDA approvals or NCE released are quantitative ways of measuring R&D productivity. If these are low, there will be criticism on the pharmaceutical management.
- Company-wide knowledge management systems and communication technologies play a critical role in the overall internationalization process.

Strategies for Success:

- Incorporation of market-oriented aspects
- Improved human resource and project leadership
- Better R&D pipeline management including a balanced approach to outsourcing and collaborations
- Having a strong internationalization strategy

Executive Summary

The three levels of future trends are (1) increasing RnD efficiency & effectiveness, (2) redefining the business model and (3) leading people for innovation and change.

While we can go into a lot of detail for each of these 3 categories the general theme goes as follows:

Level 1: The R&D efficiency and effectiveness in the pharmaceutical industry increases with the company's abilities to absorb, create, transform and interpret relevant knowledge. The effect in terms of productivity will be like the introduction of platform development in the automotive industry. Pharma R&D mainly becomes a knowledge management challenge in which the speed to identify and interpret relevant patterns of knowledge generated inside and outside the company distinguishes the winners from the losers in the innovation race. **Key take-aways: High-Throughput, Personalized Medicine (NGS, WGS), Microchips, Automation, ADCs, (AAVs)**

Level 2: While there are many threats & risks that endanger the prospects of individual companies, overall the future for the biotech industry is positive. New drug discovery technologies are in the works and expected to revolutionize the way pharmaceutical companies manage innovation. In addition, new markets emerge. **Key take-aways: wearable tech, collaborations with Tech companies, outsourcing, IP**

Level 3: Management and leadership on all levels play a critical role for the success of a company. In times when innovation and change become more important, focus on people is crucial. The companies that will win the innovation race and therefore the race to sustainable competitiveness in a global scale are the ones that win the war on technology and talent. **Key take-ways: transformation story, fluid company organization, creativity & failture, international RnD mindset**