Science and Society

Pharmaceutical industries: do they prefer treatment to cure?

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Despite the emergence of novel life-science-based approaches to drug discovery and development and the unprecedented growth of new companies and research organizations competing in the healthcare sector, the industry continues to be dominated by the marketing of small-molecule 'blockbuster' therapies developed by large multinationals. The hope that 'genomics' would shift the focus from the treatment of symptoms associated with disease to the prevention or cure of many debilitating illnesses has yet to translate into reality. The question is whether traditional pharmaceutical companies simply prefer treatment to cure and, if so, where must society look to ensure that the best science is translated into the most innovative, effective and beneficial clinical products.

I argue that although traditional big pharma companies do currently appear to prefer treatment to cure, in the long term, change will be inevitable. Radically new and complex options for healthcare are emerging from the growing number of small, innovative biotech and genomics companies, and truly novel basic research is being conducted within the public sector. Coupled with various social, commercial and technological challenges now facing traditional pharmaceutical innovators[1], big pharma companies will probably be forced to reconsider the wisdom of the 'blockbuster pill' approach. However, change is likely to be slow, as the major companies dominating the industry exhaust their existing models and processes for drug R&D.

Key words: cure, innovation, life science, pharmaceutical, therapy.

A complex and evolving pharmaceutical sector

The pharmaceutical sector has undergone profound structural change over the past 20 years. It is now commonly referred to as a complex 'system' or 'network' in which innovative activities are widely distributed and depend upon diverse actors and institutions; including small, medium and large pharmaceutical and biotechnology firms, genomics companies, public sector research organizations, universities, financial institutions, regulatory authorities, governments, health care systems, consumers and citizens[2]. This transformation, from a relatively homogenous industry dominated by large incumbent firms developing small molecule treatments for diverse therapeutic markets, to a complex 'distributed innovation system', began when large, fully-integrated drug-discovery companies were confronted by 'disruptive' life science technologies. They were forced to develop 'innovative capacity' within fields where they had no previous expertise, and adapt to a changing industry structure.

Although advances in biology undoubtedly opened up new avenues for drug discovery and development, and helped create and shape new kinds of industry relations and business models, it appears that we may have been too optimistic in our expectation that radically new, safer and more effective therapies or cures would soon be made available to patients.

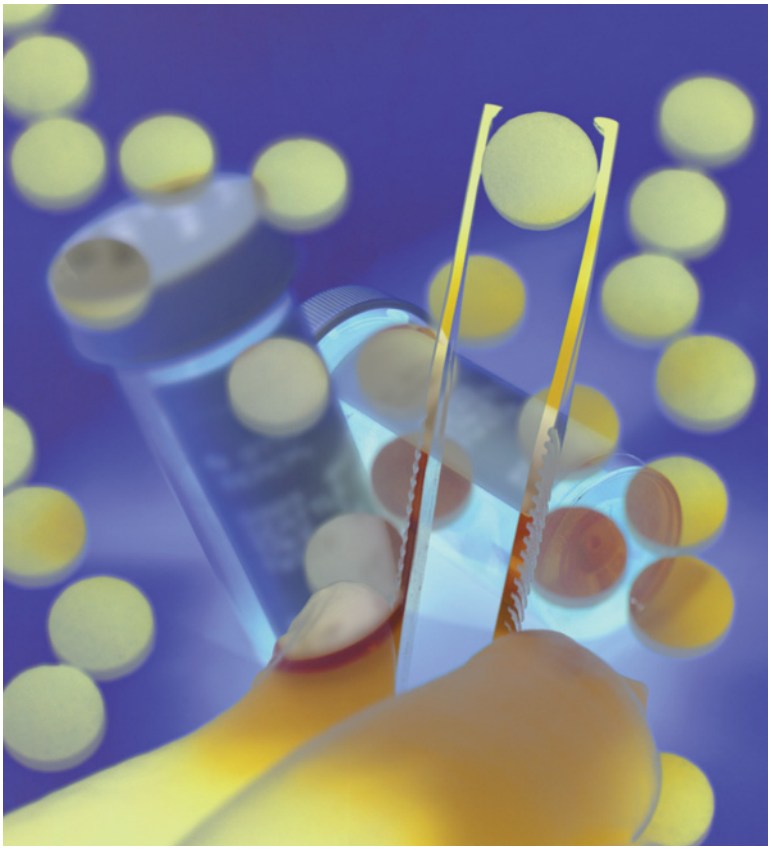
'Treatment' versus 'cure': what is happening in big pharma companies?

While most big pharma companies have recognized the importance of life science based approaches to drug R&D, as evidenced by the growing trend for them to acquire or become partners of smaller biotech and genomics companies, there is little evidence yet that the

largest and most profitable companies are actively pursuing an alternative to the 'treatment paradigm'.

A cursory glance at the R&D pipelines of the top ten pharmaceutical companies and their most recent product launches, reveals the continuing dominance of traditional therapeutic products. While innovations in discovery techniques and instrumentation, such as combinatorial chemistry, high-throughput screening and biotech approaches to target identification and validation, have certainly had an impact on early-stage R&D, the vast majority of products marketed by big pharma companies, and currently being developed in-house, are small-molecule treatments in complex, but high-value, therapeutic areas, such as cardiovascular, oncology, depression and the central nervous system. Some authors have argued that the very idea of a 'biotech revolution' in big pharma companies is a myth and that any major changes in products and processes will be slow and incremental[3]. The large number of 'me-too' therapies currently in pharmaceutical development also suggests that simply altering existing chemical-based drug treatments continues to be the most efficient and lucrative strategy for a big pharma company to employ.

Although it is easy to blame large pharmaceutical companies for the lack of radically new therapies or cures on the market, one cannot really expect rapid change in a for-profit industry that has built its technological capabilities and fortunes on a highly specialized and expensive research trajectory. At a time when big pharma companies are struggling to deal with the challenges of innovation deficit, rising costs of R&D, regulatory hurdles, cost-containment pressures and increasing competition, it is not surprising that they focus on extracting as much value as possible from models that have proved so successful for them in the past.



It is still an open question whether big pharma has the technical ability or commercial inclination to really abandon the 'blockbuster treatment' model and push for novel cures. To remain competitive, such companies already have to rationalize internal processes and product foci. Identifying potential markets for new drug treatments is a core activity for the modern big pharma firm. To develop a cure for diabetes, heart disease or cancer would represent a fundamental change to its traditional business model and could potentially render existing high-value therapies redundant. To invest money and resources into a paradigm of prevention and cure, big pharma companies would have to perceive either realistic commercial benefits, or potentially significant losses accruing from a failure to adapt and change.

So where are 'cures' likely to be researched and developed?

If there is little evidence of any major pharmaceutical company actively seeking cures, from where are the radically new and innovative approaches going to emerge, and how likely is it that they will translate into deliverable healthcare options? There is great expectation that the life sciences will eventually lead to cures for many common diseases, and publicly funded research has perhaps been at the forefront of new developments.

In 2003, it was reported in the magazine *Chemistry and Industry* that scientists had for the first time been able to convert the liver cells of mice into pancreatic cells using a single injection, and that a treatment for people with diabetes might be available in the next 10 years[4]. More recently, it has been reported by the BBC that scientists at The London

NHS Trust believe they are close to developing a miracle cure for heart disease, using stem cells from the patient's own bone marrow. A four-year research programme involving 600 patients is currently being established[5].

Reports like these are becoming increasingly common and generating hope for many patients and healthcare providers that cures are now on the horizon. However, because the pharmaceutical industry remains so powerful, and large companies are often still required to develop late-stage products, there is concern that these scientific breakthroughs will not fulfil their promise.

Nevertheless, the increasing diversity of firms in the industry may help renew our early optimism. It is no longer just the public sector that is pursuing new approaches to healthcare. Many biotech companies (e.g. Amgen, Biogen and Genzyme), as well as a few medium-sized pharmaceutical companies (e.g. Novo-Nordisk and Ferring Pharmaceuticals), appear open to research in this area and quite willing to collaborate with scientists in the public sector. Many of these companies built their success on the development of novel treatments for niche markets, so they were never constrained by the blockbuster model favoured by big pharma companies. By focusing on areas of unmet medical need, and developing therapies based on the latest life science technologies, these companies have demonstrated much greater willingness to adopt very different business models.

Novo-Nordisk, a medium-sized Danish company, is a good example of this. The company specializes in diabetes products, where it has a global market share of 46%. From 2002, it began to shift resources from small-molecule development to the research of therapeutic proteins produced by biotechnology. It has also begun to explore the potential of new technologies for curing diabetes. It recently established the Hagedorn Research Institute to perform peer-reviewed research on stem cells, and has secured access to Transition Therapeutics' technology for regenerating insulin-producing cells in a $48 million deal. There are commercial companies now willing to invest in potential cures, even though their existing business models might be disrupted. Companies like Novo-Nordisk, which have sought to build market leadership in narrow therapeutic areas such as diabetes, may be seeking to ensure that they benefit from any cure that is developed. Maybe they are now beginning to realize that the 'treatment paradigm' cannot last forever and that they must now adapt.

Looking to the future

It is desirable for society that the latest lifescience technologies and approaches to healthcare are used to improve the therapeutic options for those suffering many common debilitating illnesses. The evolution of the lifesciences has provided hope that prevention and cure of today's major killer diseases, as happened in the past with infectious disease, might come to replace treatment as the primary goal of health policy. However, the speed at which such radically new therapies reach the market will depend on the balance of initiative between multinational companies that continue to be driven by the marketing of small-molecule, 'blockbuster' treatments, and companies that choose to develop strategies aimed at prevention and cure rather than long-term treatment.

There is now growing recognition, even within big pharma companies, that the traditional model is unsustainable. Companies have already had to adapt to new technologies and come to recognize the importance of innovation within public sector research organizations and small- and medium-sized biotech companies. These organizations are at the forefront of exploring new technological and business paradigms for healthcare, and are likely to affect the future strategies of big pharma companies. Policymakers, healthcare providers and the public must now begin to recognize the diversity of firms within the pharmaceutical industry, and their very different capabilities and business objectives, when considering how to ensure potential cures reach the market.

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