Affordable Drugs: a Developer's Perspective

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Conflicts of Interest

President and CEO, ESSA Pharma, Inc

Board Director: Threshold, Cerulean, Tocagen

Origins of My Perspective

Trained as a hematologist/oncologist; career in academic medicine as clinical investigator (Boston, Houston)

National Cancer Institute: years directing new drug development, clinical trials activities

Responsible for global oncology therapeutics development: Novartis, Amgen, Biogen-Idec

President & CEO, Nodality, Inc; ESSA Pharma Inc (current)

Venture Partner, New Enterprise Associates (NEA)

Previous member and chairman of FDA advisory committees (Biologics Advisory Committee, Science Board)

Previous member, National Cancer Policy Board and Forum (National Academy of Medicine)

Goals of an Ideal Pricing System

Pricing a complicated topic, sitting at the end of the hugely complex process of drug development

An ideal pricing system should stimulate the creation of new, improved therapeutics: the business opportunity should attract innovators, investors

Should limit opportunities for “gaming”

Should align current stakeholders: -patients & their advocates, drug developers, providers and payers

The Perversity of the Current System

The pharma business model is drugs as products: market opportunity drives pricing, greatest return is related to most product sold at highest price, little direct relationship to actual value returned at the personal and societal levels

Unique among product businesses?

Longer, more complex development rewarded with shorter time after approval to experience return on investment

Ample opportunities for “gaming”

Drug performance beyond that necessary for approval is not a major driver: drug makers are not incentivized to most accurately profile the patients most likely to benefit, despite enabling technology which could enable them to do so

Current Reality of Drug Development

Much drug creation “discovery” has migrated from large pharma companies to smaller companies

These smaller companies are more flexible, faster moving, closer to the science, participants are motivated by outcome rather than process, all advantages for successful development

…by contrast the larger companies have the resources, longer timelines, expertise, global reach necessary for global registration and necessary linkage with eventual commercialization

Goal of blockbuster drug developmen has shifted to niche drugs in defined speciality markets: rare diseases as the extreme example

Current economic drivers still encourage broad an indication on label as possible, rather than precision

Challenge to developing assets whose clock is running relative to value return even during the development process

Steps in Drug Development & Availability

Uncovering disease biology – identifying new “targets” (scientists, their institutions)

Creating the drug “drug discovery” and establishing the clinical activity of the drug “clinical development” in order to assemble a body of evidence sufficient for registration (Biopharma R&D)

Commercialization, entry into drug formularies (marketing and sales)

Distribution, reimbursement (patients, providers, payers)

Stakeholder Agendas

Scientists, Institutions

- Scientists want to see practical applications of their work
- Both would like to gain financial return

Biopharma R&D

- Face every increasing costs, bureaucratic burdens, challenges in conducting clinical development
- Challenges in financing of early stage companies “valley of death”
- Major development risks still exist, reflecting incomplete biological understanding of diseases
- No viable business model for speciality diagnostics companies

Marketing and Sales

- goal to maximize revenue

Patients, Providers, Payers

- All want better treatments which are available and affordable
- payers want predictable, controllable costs

How to Change This Situation: Opportunities for Improving Efficiency

Significant, continuing advances in biological understanding of diseases offer abundant opportunities for improved therapeutics development

Major improvements in drug creation technologies: e.g. antibodies, gene therapies, gene editing, etc.

Associated technology developments enable accurate biological characterization of patients

Potential of combining these elements: more efficient drug development, faster, less costly, less risky “personalized medicine, precision medicine”

Failures to Maximize These Opportunities: Hurdles to Improving Efficiency

Drug approvals are based on meeting some minimal levels of evidence for some threshold of activity

Drug reimbursement is not related to improved drug performance; incentives post initial approval relate to expanding use of drug to new indications, not to improving the performance of the drug

Many of the efficiency improvements would require the development of better diagnostics (better linking individual patients with drugs, more accurate dosing in patients, etc):

Yet…….there is currently no viable business model related to the development of accurate predictive diagnostics, hence limited investment in these companies

Aligning Stakeholders to Achieve Societal Goals: Ideas Related to Pricing Policies

Consider drugs more as a service than a product and thereby aligning all stakeholders:

- Perhaps enable earlier availability of drugs once safety and a certain level of efficacy has been established, but at some base price
- Reimbursement to increase as the drug's value is improved: perhaps lower levels at initial registration, improved levels of evidence for performance, with certain situations justifying "pay for performance" at the individual patient level
- Performance-related reimbursement at the individual patient level would encourage development of diagnostics
- Diagnostic reimbursement could either be separate, in which case reimbursement also should reflect levels of evidence developed, or incorporated into the reimbursement for the drug

Summary and Thoughts Going Forward into the Panel Discussion

Drug development represents an expensive, regulated, long cycle time activity with great risks and potential rewards

Current stakeholders are not aligned in this process; current reimbursement realities do not motivate improvements in drug performance after initial registration, or reward drug developers for doing so

The current model of drugs as products is behind much of this situation

The solution to this will need to be holistic, and extend beyond pricing; for example it could includes incentives to reward diagnostic incorporation earlier to further more efficient drug development and clinical use of drugs