Demonstrating the Value of Innovation

Meeting the Needs of FDA, Payers, Providers, Consumers

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There is an important feedback loop from market authorization, access, and adoption to incentivize further investment in research and development.
The Reverse of Moore’s Law: An Exponential Decrease in New Drug Launches per $Billion Spent on R&D
Value as Defined by the FDA

- FDA interprets product value as efficacy when used in *ideal research settings* and safety relative to unmet need.
- It recognizes that social value will depend on clinical performance and cost under *real world conditions* but wants to allow products passing its threshold to prove themselves to payers, physicians, and patients.
- Evidence required to demonstrate (this concept of) value traditionally consisted of randomized trials and clinical endpoints, with a lower bar for follow-on products (generics, biosimilars, substantially equivalent devices).
- In each case, FDA weighs the value of additional data against the burden of time delay, cost, rapid product cycles.
Lowering the Bar to Market Authorization

- In recent years FDA has made a dramatic shift towards reducing evidentiary demands in order to accelerate approval and reduce regulatory costs
- Promising drugs (esp. oncology, orphan) receive special consideration/advice, priority review, approval for special designs (e.g., one-arm trials, surrogate endpoints)
- This makes it easier to get on the market. It is being combined with requirements making it more difficult to stay on the market: post-launch follow-on studies (if used surrogate endpoints), studies using real world evidence (RWE) over the product life cycle, safe use requirements (REMS), and site of care (COE) restrictions
Which Roles for Real World Evidence?

- The 21st Century Cures Act enabled/exhorted the FDA to increase its use of RWE, but challenges include:
  - There is no gold standard of RWE data quality across EMR, insurance claims, patient surveys, sensors on wearables or in the home, etc.
  - These data sources typically are not integrated with one another
  - Machine learning methods are sifting thru them to find patterns, but users feel uncomfortable about inferences when they cannot understand the algorithms and feel confident about causality
- Most immediately, RWE can be used for:
  - Control arm for trials where randomization not possible due to small patient populations, ethical limits on assigning patients as controls, etc.
  - Insurance claims post-adooption can document complications, changes in utilization and costs, etc. across diverse patient sub-groups
  - Patient experience data provides endpoints supporting claims of safety/efficacy where hard clinical endpoints are not available
Example: Orphan Drugs Benefit from Research Grants, Tax Credits, Smaller Trials with Surrogate Endpoints, Extended Regulatory Exclusivity

*Source: EvaluatePharma* February 2017
Payer Coverage and Pricing

“You don’t know how lucky you are! A quarter of an inch either way, and it would have been outside the area of reimbursable coverage!”
Now payers are defining value in terms of comparative clinical and cost effectiveness

They are tightening coverage (formulary inclusion, prior authorization, step therapy), provider payment incentives, and consumer cost sharing as means to obtain reductions in price and utilization

These tools are effective.

**Insurers and employers traditionally were under pressure to approve all technologies that improve outcomes, no matter how small, regardless of cost, no matter how large. Evidence demands were minimal (beyond FDA authorization)**
More Intense Prior Authorization and Cost Sharing Are Slowing Drug Adoption Relative to Projections

![Graph showing percent of potential post-launch adoption actually achieved, with changing intensity of payer management.](image)

‡ Source: QuintilesIMS, Payer and Managed Care Insights, Novartis
The Emerging Logic of Value-Based Pricing and Patient Access Criteria

Comparative clinical assessment:
Does the new drug offer better safety and/or effectiveness than other options?

Yes

Does the drug’s price represent a reasonable value, based on comparative clinical and cost performance?

Yes

VALUE-BASED PRICING

Value-based pricing is accompanied by value-based patient access:

Payers include drug in formulary. Prior authorization and step therapy are limited to clinical (not economic) criteria. Purchasers and producers promote appropriate adoption and adherence. Multi-year contracts

No

REFERENCE PRICING:
Purchaser limits payment for new drug to the price charged by the cheapest, equivalent option

No

MARKET PRICING:
Purchasers exclude drug from formulary or include subject to strict prior authorization, step therapy, cost sharing requirements
Example: Manufacturer Reduces Price to ICER ‘Value-based’ Benchmark in Exchange for Lighter Prior Authorization and Cost Sharing

Regeneron and Sanofi Plan to Cut Cholesterol Drug Price in Exchange for Wider Coverage

They seek to offer rebates and discounts for Praluent and want insurers to ease restrictions on some patients.

A cost-effectiveness analysis by an independent group incorporated new clinical trial data showing that Praluent reduced the risk of death. PHOTO: SANOFI AND REGENERON PHARMACEUTICALS/ASSOCIATED PRESS

By Joseph Walker
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Provider Prescription and Purchasing

“Geez Louise—I left the price tag on.”
In the ‘medical arms race,’ hospitals competed for physician affiliations and admissions via adoption of state-of-the-art technology, regardless of cost.

In the new context of bundled payment and shared savings, hospitals are developing new strategies with respect to technology assessment, purchasing, and use.

Hospitals, and physicians aligned with hospitals, interpret value as quality, measured by short-term outcomes, and cost, measured by impact on the hospital budget and physician gain-sharing.

They do not focus on cost and benefit to society.
Alignment, Assessment, and Purchasing

- **Physician alignment & incentives**
  - Hospital employment of physicians; joint ventures and co-management for ambulatory infusion, imaging and surgery centers; physician payment linked to organizational performance with gain-sharing bonus for reducing hospital costs; limits on ties with manufacturers (‘conflicts of interest’)

- **Technology assessment: ‘value committees’**
  - Major new clinical equipment and supplies need to be approved by committee dominated by physicians, based on evidence of performance and cost to the hospital budget. Committees also serve a cultural function: physicians begin to think of value as including cost (to the hospital)
Capital Equipment and Supplies

- High-value capital equipment (e.g., radiotherapies)
  - Major capital purchases have always been subject to financial review to a greater extent than consumable ‘physician preference items’, but the bar is rising as payers consider ‘Center of Excellence’ contracting to obtain better prices (and perhaps better outcomes) by contracting with more limited numbers of facilities. COE contracting also potentially helps payers counter the consolidation of local markets. This makes hospitals more aware of clinical equipment as either a path towards COE or an unreimbursed cost burden if COE status is not achieved.

- High-value supplies (e.g., implantable devices)
  - Increasing reliance on formal RFP and external benchmarking; reduce number of suppliers to simplify supply chain and maximize discounts; physician alignment supports creditable threat to switch suppliers.
Example: Bundled Episode Payment for Device-Intensive Procedures

- Medicare CCJR initiative was mandatory in 67 markets, combining Part A, B payments. Alternative models combine post-discharge readmissions and subacute services in bundle. Was to expand to cardiac, spine procedures. Trump administration switched from mandatory to voluntary and halted expansion to cardiac.
- Strategy: create incentives for physicians to make clinical choices aligned with hospital concept of value
- Peer-reviewed evaluations report:
  - Reduction in discharges to post-acute
  - Small decline in costs
  - No decline in quality, patient mix

Source: AM Ryan. JAMA 2018; 320(9):877-879
Consumer Engagement & Adherence

“The gentleman at the other register would like to cover your co-pay.”
Patients often have lacked the information and incentives to demand high-value health care.

They now increasingly have the incentive, but still need information and support to make value-based choices.
## Decision Support

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<th>Company</th>
<th>Target Population</th>
<th>Product Offering</th>
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| Online Transparency Tool       | • Patients with significant cost sharing requirements facing choice among providers with significant price and quality differences                                                                                   | • Information on price, quality, location of alternatives  
• Titrated to individual’s network and benefit design  
• Employers offered analytics                                                                                                                                 |
| AIM Specialty Health           | • Patients prescribed high cost radiology or infused drug                                                                                                                                                             | • AIM staff phone patients once receive referral info through prior authorization. Suggest lower priced sites of care                                                |
| Sutter Health                  | • Patients with multiple ED visits, inpatient admissions, unplanned readmissions, diagnosis of CHF, COPD, three or more chronic conditions                                                                                    | • Complex case management program                                                                                                                                 |
| Aetna Health                   | • Patients benefitting from screening & preventive services, chronic disease management, maternity (‘beginning right’)                                                                                        | • Employer contributes to HSA, reduces deductible, offers gift cards if patient completes health risk assessment and indicated preventive and/or management programs |
| Healthy Actions Program        | • Patients facing complex treatment alternatives                                                                                                                                                                   | • Nurses counsel members on treatment alternatives, low-cost site of care, medication choices and adherence, lifestyle change                                    |
| OPTUM Health                   |                                                                                                                                                                                                                      |                                                                                                                                                               |
Digital Technologies to Support Consumer Engagement: Effectiveness to be Demonstrated
Bottom Line: Innovators Need to Prove Value to All Stakeholders, According to Each Definition

**FDA**
- Regulators are making it easier to get on the market, but also easier to get pulled off the market: demanding life cycle RWE

**Insurers and employers**
- Payers are using HTA evidence (informally) when assessing value and seeking price-volume contracts that protect budgets

**Physicians and hospitals**
- Physicians and hospitals increasingly are budget-holding purchasers, focused on cost (to them) as well as performance

**Consumers and patients**
- Patients increasingly are engaged and price-sensitive consumers; care experience and out-of-pocket cost are key
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