Market- & drug development-trends potentially affecting prices

Jonathan J. Darrow, SJD, LLM, JD, MBA

Faculty, Harvard Medical School
Program on Regulation, Therapeutics, & Law (PORTAL)
Brigham & Women’s Hospital

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1. Market trends affecting price
2. Drug development trends
Market trend #1: Growing generic share

Generics as percent of prescriptions in US

Brand-manufacturer profits come from a shrinking share of prescriptions.


Market trend #2: More orphan drugs

On average, profits come from a shrinking number of patients.
Revenues = Quantity x Price
Caveats (examples)

- Shrinking share, but total number of prescriptions has increased
- Some drugs have multiple orphan (or non-orphan) designations
  - E.g., Humira, Enbrel, Remicade, Crestor…
Market Trend #3: More biologics

Percent of new drugs that are biologics*

*excludes biologics approved under an NDA, and vaccines or other CBER products

Biologics can be more expensive to manufacture and administer.
Hospitals receive 2.5x what they paid to acquire these medicines. (After Price Negotiations)


From: https://www.phrma.org/graphic/hospitals-mark-up-medicine-prices-nearly-500
1. Market trends affecting price

2. Drug development trends
Drug development trend #2:
More fast-track (1988) approvals*
(can be based on Phase II trial)

Drug development trend #1: More accelerated (1992) approvals*
(i.e., based on surrogate endpoints)

The Strength of Association Between Surrogate End Points and Survival in Oncology
A Systematic Review of Trial-Level Meta-analyses

Vinay Prasad, MD, MPH; Chul Kim, MD, MPH; Mauricio Burotto, MD; Andrae Vandross, MD

Surrogate Outcomes in Clinical Trials

A Cautionary Tale

Staffan Svensson, MD, PhD
David B. Menkes, MD, PhD
Joel Lexchin, MSc, MD

JAMA INTERN MED/VOL 173 (NO. 8), APR 22, 2013
Drug development trend #3: 
More priority (1992) reviews* 
(6 month FDA review, rather than 10 months)**


** Prior to 1992, we considered “A” and “B” to be “priority” and “C” to be “standard”
The risk we bear: The effects of review speed and industry user fees on new drug safety

Mary K. Olson*

Department of Economics, Tulane University, 306 Tilton Hall, 6823 St. Charles Avenue, New Orleans, LA 70118-5698, United States

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The Complications of Controlling Agency Time Discretion: FDA Review Deadlines and Postmarket Drug Safety


Daniel Carpenter   Harvard University
Jacqueline Chattopadhyay   Harvard University
Susan Moffitt   Brown University
Clayton Nall   Stanford University
Drug development trend #4: More “Breakthrough Therapies”*
(approval in 4.8 rather than 8.0 years**)


Precision Medicines Have Faster Approvals Based On Fewer And Smaller Trials Than Other Medicines

- 52% based on Phase 1 or 2
- 45% based on single trial
- 42% did not have either a placebo or active control

*Oncology BT drugs
Implications for future spending

• Less data at time of approval
• Shift of data collection from Phase 3 to 4
• Less time for FDA to review each drug
• Result:
  – Greater uncertainty about risk/benefit
  – Payors must make decisions amid uncertainty
Increased pressure for payor coverage

• Uncertain benefit…
  – …but superlative labels

• “Breakthrough” designation can increase pressure for payor coverage*
  – E.g., pimavanserin (Nuplazid) (~$2800 / 30 days**)
  – E.g., ivacaftor/lumacaftor (Orkambi) (~$21,000 / 112 tablets)
  – E.g., uridine triacetate (Xuriden) (~$45,000 / 30 packets)
  – E.g., pirfenidone (Esbriet) ($27,000 / 270 tablets)


** Prices from https://www.goodrx.com/
Required Medicare coverage*

1. Anti-convulsants
2. Anti-depressants
3. Anti-neoplastic [cancer medicines]
4. Anti-psychotics
5. Anti-retrovirals
6. Immunosuppressants for the treatment of transplant rejection

Subjects Who Received Tisagenlecleucel (Kymriah)
voretigene neparvovec (Luxturna)
Conclusion

- Less evidence required for FDA approval
- “Guaranteed” payment, keyed to FDA approval
- No price limits
- Poor understanding of FDA efficacy standard

Prediction: prices will continue to increase.
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