FDANEWS

WIDETRIAL
Expanded Access Clinical Trials

THE NEW MARKETPLACE FOR EXPLORATORY TREATMENT

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West Coast Symposium on Expanded Access

The unique reimbursement landscape in the U.S.

Fragmented payer system = Fragmented reimbursement policy

21 CFR 312.8 limits charging at "Cost Recovery"

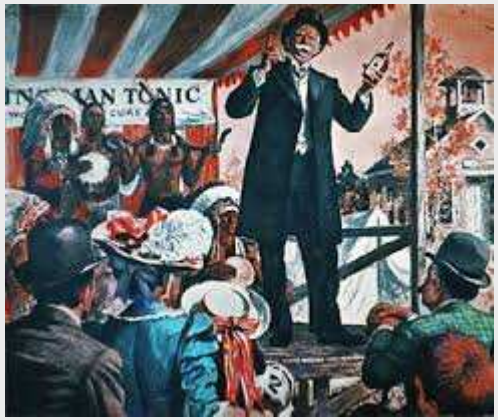
	Permitted	Covered by Health Plans
1. Cost Recovery for Sponsor:	**Yes**	**Rarely**
2. Full Breakeven for Sponsor:	**No**	**No**
3. Full charging by Medical and Service Providers:	**Yes**	**Sometimes**

New Approaches under Current Conditions

2017 Expanded Access Summit, Panel Session 3:
Cost Recovery Models. Rabourn Balch, Pitts, Farber

- Sliding pay scale with Patient Assistance Fund and financial means test
- Charitable partnerships to fund selected number of patients (allow others to buy in?)
- Third-Party Sponsor buys from manufacturer, resells at cost. (not for profit)

Marketplace for Remedy



1840- 1937

1962- Present

Marketplace for Remedy

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Marketplace for Options

- Lower evidentiary standard
- No affirmative claim of pharmacological benefit or patient outcome
- Cultural belief in individual decision making for people with unmet need

Normative Analysis of Possible Regulatory Changes

1. **Right To Try (HR 5247). No restrictions**
   (-) Back to 1937
   (-) Misaligns development priorities of legitimate medicines
   (-) Who pays? And at what price?
   Net: Minimal improvement in feasibility

2. **Current environment**
   (-) Reveals manufacturer's cost structure
   (-) Hard costs only; not full resource expenditure
   (-) Potentially bad optics for manufacturer
   Net: No change in feasibility, limited improvement in access

Normative Analysis of Possible Regulatory Changes

3. **Cost Plus Standardized Margin**
   (+) May increase cash flow feasibility
   (-) Reveals cost structure
   (-) Who pays? And at what price?
   Net: Moderately increased feasibility, greater with reimbursement

4. **Fraction of Projected Market Price**
   (+) Greater alignment with commercial risk / value prospect
   (+) Does not reveal cost structure
   (-) Requires payer
   Net: Moderately increased feasibility, greater with reimbursement

Normative Analysis of Possible Regulatory Changes

4. **New York Taxicab (Tiered pricing)**
   (+/-) Not related to cost structure
   (+) No projection of market pricing
   (+) Can include associated treatment costs
   (+) Adoptable by CMS, health systems, and employer plans
   Net: Significantly Increased feasibility for some

Example Tier	Example Set Price Per Patient /Yr
Inorganic Molecules	2500
Semi-Synthetic Sourced	4500
Plant-Derived Recombinant Protein	6000
Humanized Antibodies	/
Oligonucleotide	/
HCT/P	/

From: **Access to Experimental Drugs for Terminally Ill Patients**

JAMA. 2008;300(23):2793-2795. doi:10.1001/jama.2008.828

Table. Patient and Societal Concerns Regarding Access to Experimental Agents

Domain	Concern	Strategy
	Individual Patients	
Clinical	Patients harmed by investigative drug	(1) Select appropriate evidentiary cutoff (at least post–phase 1, which FDA has flagged for extreme deference to patient desires); (2) allow for case-by-case FDA review only if broad definition of "terminally ill" is adopted
Equity	Manufacturers and/or health care professionals charge exploitative prices to vulnerable patients	(1) Manufacturers penalized for charging more than the average sales price after launch; (2) health care professionals prohibited from marking up administration-related procedures or earning more than a predetermined spread on the drugs
Equity	Systematic bias: only rich, insured, or well-connected will be able to access investigational drug	(1) Reimbursement for those who assist patients through the process; (2) permit manufacturers to earn a profit on experimental drugs so that they have an incentive to streamline the process; (3) profits collected from the sale of investigational compounds that are never approved are used to fund FDA-approved treatments for those who cannot afford it; and (4) if *terminally ill* is narrowly defined, restrict FDA review to issues involving clinical trial enrollment
Equity	Arbitrary system: FDA review for adverse effect on clinical trial enrollment degenerates into de facto review on the merits with random intervention	Transparency
	Society	
Science: generation of new knowledge	For specific studies: trial enrollment impaired if patients can acquire drug outside of clinical trials	Access granted only if clinical trial enrollment is unimpaired
	Full body of knowledge about new agent: insufficient body of evidence generated	Require all patients who receive investigational drugs to enroll in a registry so that their data are helpful in some fashion
Regulation of new agents: FDA submission	Companies will not submit for FDA approval if they can sell drug anyway; incentives to start fly-by-night companies	Manufacturers do not retain profits unless FDA approval is obtained

Abbreviation: FDA, US Food and Drug Administration.

Solutions for the Buy Side

1. **Collaborative Patient Assistance Funding**
    1. Means test / Sliding Scale out of pocket

2. **Legislation to recognize "medical appropriate exploratory treatment"**
    1. Fund CMS coverage with PDUFA fee sponsored pilot (e.g. vax)
    2. Threshold test for eligible products

3. **Evolving Private Sector Health Systems**
    1. Specialty Plans / Concierge Medicine
    2. Self-insured employer plans

4. **Federally mandated "escrow"**
    1. Payable upon market approval in indication
    2. Can tie regulation with CMS coverage

THANK YOU!

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