

# Specialty Pharmaceuticals: Market Overview



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# Presentation Overview

- Defining Specialty Pharmacy
- Current and Future Trends in Specialty Pharmacy
- Coverage and Management of Specialty Pharmaceuticals
- Future Challenges

# Defining Specialty Pharmacy

# What Are Specialty Pharmaceuticals?

- A category of drugs resulting from advances in drug development research, technology, and design
- Target and treat specific chronic or genetic conditions typically with an unmet need for therapeutic intervention

Specialty Pharmaceuticals Include	Specialty Pharmaceuticals Require...
■ Biopharmaceuticals (bioengineered proteins)	■ Detailed patient education for safe and effective use
■ Blood-derived products	■ Patient-specific dosing
■ Complex molecules	■ Monitoring for serious side effects
■ Select oral, injectable, and infused medications	■ Administration via injection, infusion or orally



# Current Biotech Diseases/Therapies and Related Drug Examples

Disease/Therapy	Drug Name	Disease/Therapy	Drug Name
Asthma	<i>Xolair</i>	Multiple Sclerosis	<i>Avonex, Betaseron Copaxone, Rebif, Tysabri</i>
Anemias (Hematopoietics)	<i>Procrit, Epogen, Aranesp, Neupogen</i>	Pulmonary HTN	<i>Remodulen Flolan Tracleer</i>
Crohn's Disease	<i>Remicade, Humira</i>	Psoriasis	<i>Amevive Raptiva</i>
Gaucher's Disease	<i>Cerezyme</i>	Oncology	<i>Rituxan Gleevec Herceptin Avastin Tarceva</i>
Hemophilia	<i>Recombinate, Benefix, other Factor products</i>	Rheumatoid Arthritis	<i>Enbrel Humira Remicade</i>
Hepatitis C	<i>Pegasys Peg-Intron</i>	Growth Hormone	<i>Nutropin Genotropin</i>
Infertility	<i>Gonal-F. Follistim</i>	RSV	<i>Synagis</i>

# High Cost Specialty Therapies: Monthly and Annual Costs

<b>Disease State</b>	<b>Average Monthly Drug Cost Per Patient</b>	<b>Avg. Annual Drug Cost per Patient</b>
Hemophilia	\$20,000++	\$240,000++
Pulmonary Hypertension	\$3,600	\$43,200
Multiple Sclerosis	\$1,600	\$19,200
Hepatitis C	\$1,650	\$19,800
Rh. Arthritis	\$1,400	\$16,800
Psoriasis	\$1,400	\$16,800

# Specialty Pharmacy Trends

# Key Drivers of Specialty Trend

## High cost per patient

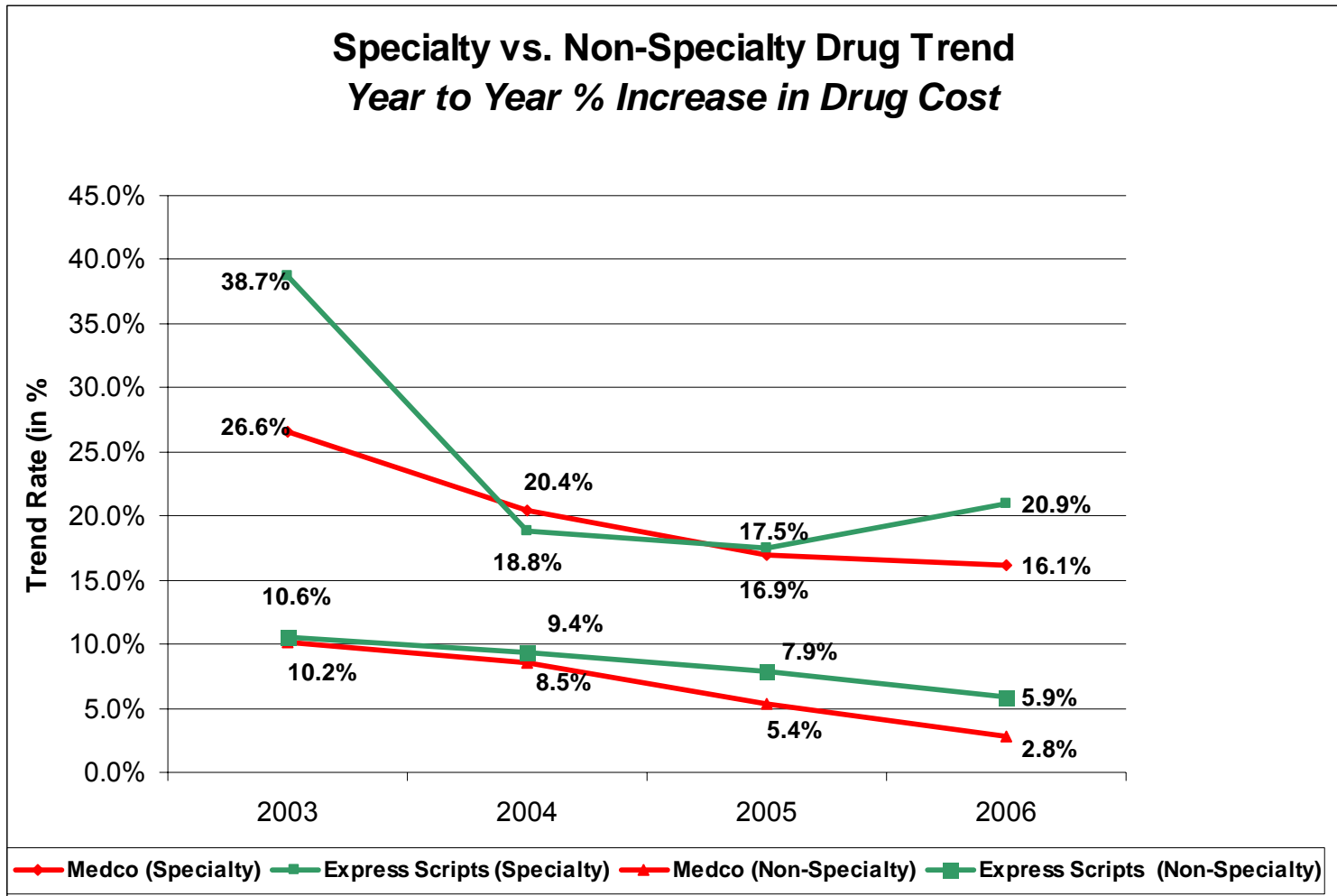
- Accounts for 15% of pharmaceutical spending in the US
- Annual growth at 15-20%
- Annual drug cost ranges from \$15,000 - \$250,000+ per patient
- Manufacturer price increases for existing drugs
- No generics available as products mature

## Increasing utilization

- Flourishing pipeline
- Multiple indications for existing drugs
- Earlier use of biologics in treatment
- Move from rare diseases to more common chronic diseases
- Episodic vs. chronic treatment

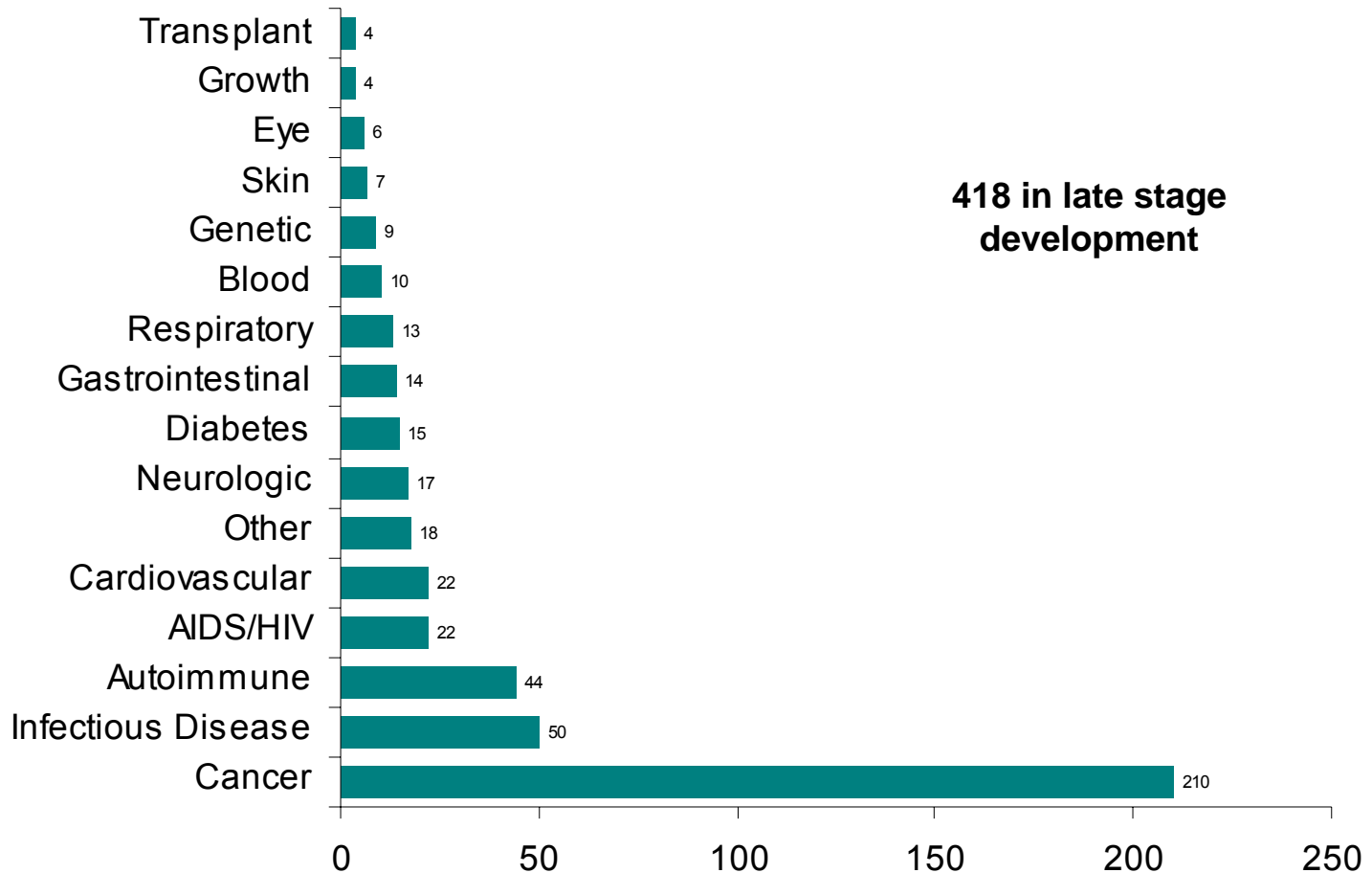


# Specialty Pharmacy Trend Rate Outpaces Traditional Drugs



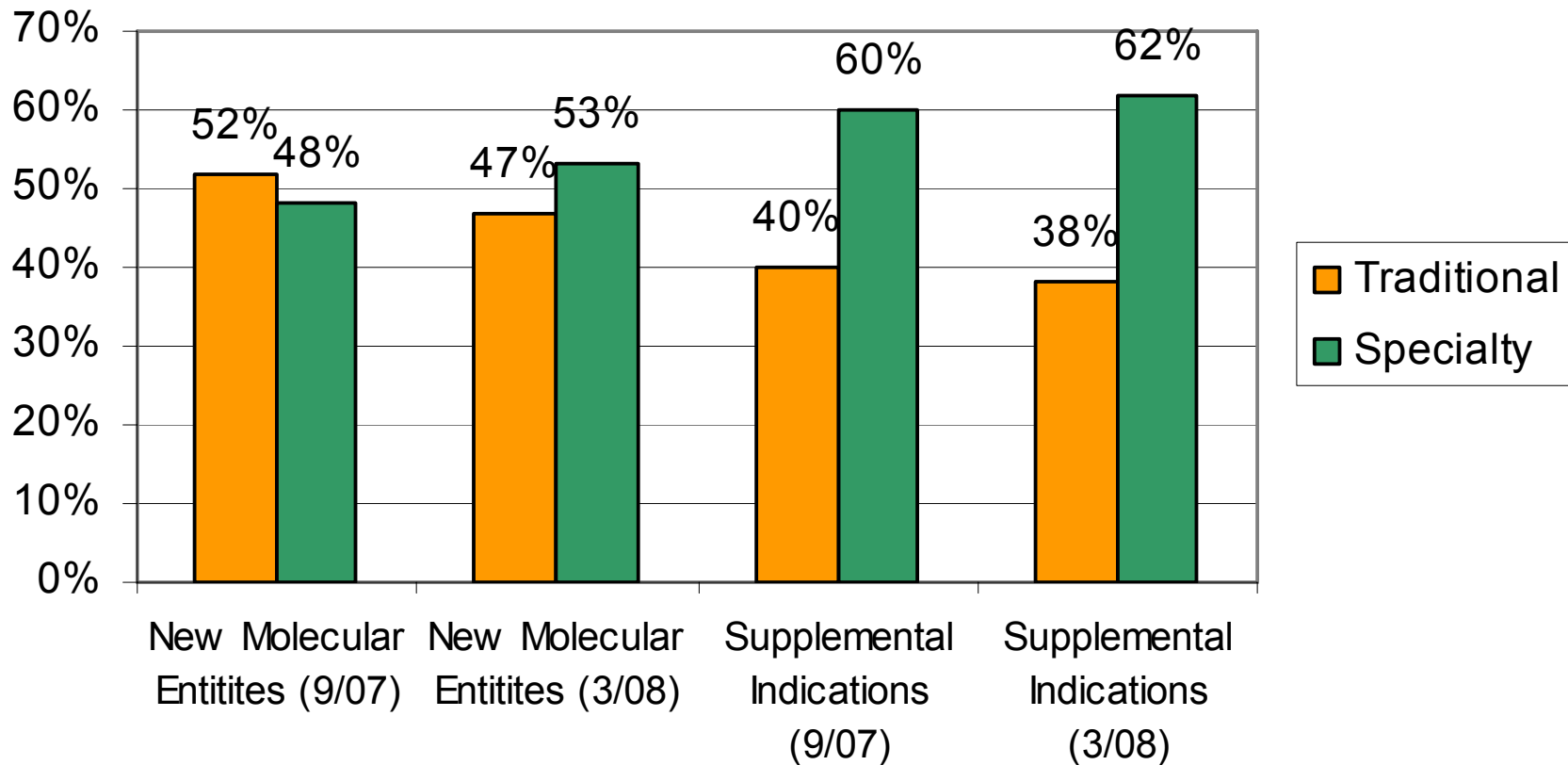
# Pipeline View by Therapy Class

## Biotech Drugs in Development

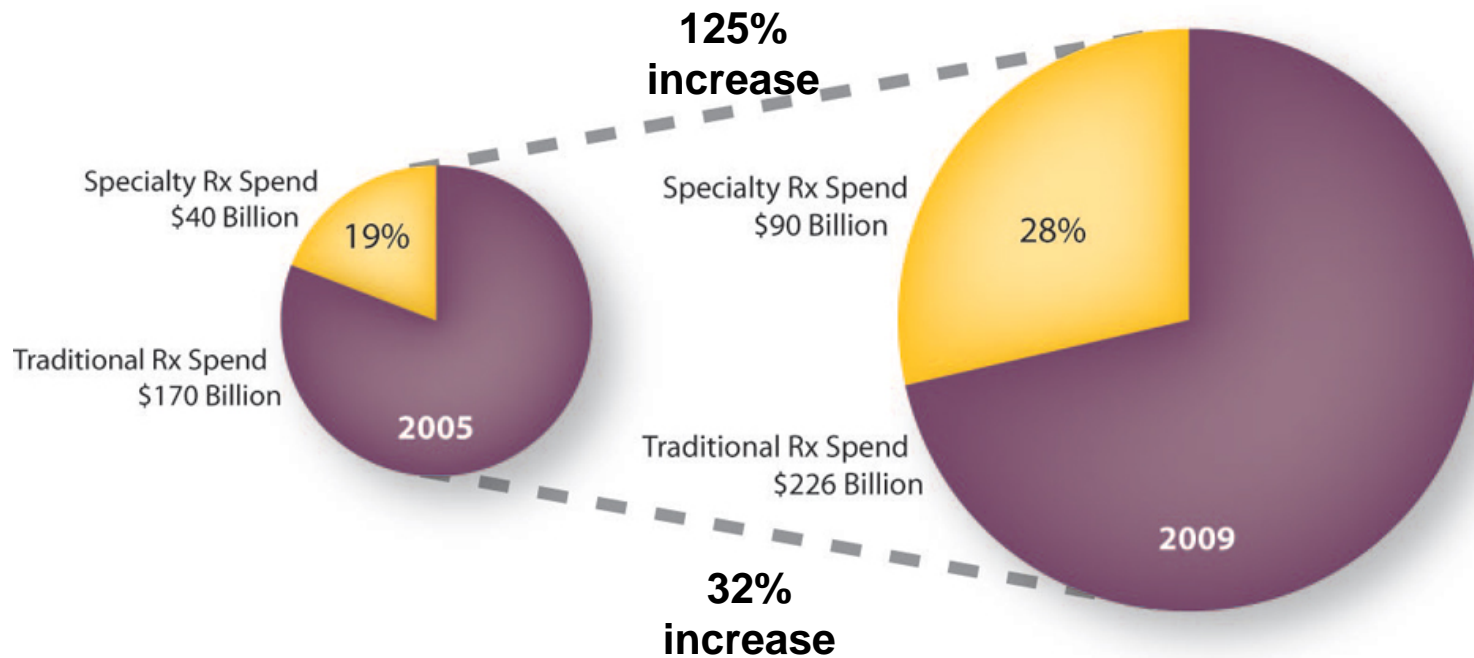


# Biotechnology Pipeline

## *New Molecular Entities and Supplemental Indications in Phase III Development*



# Projected Spend for Specialty Medications: 2005 vs. 2009



**2009-2011 projected trend = 20% annually**

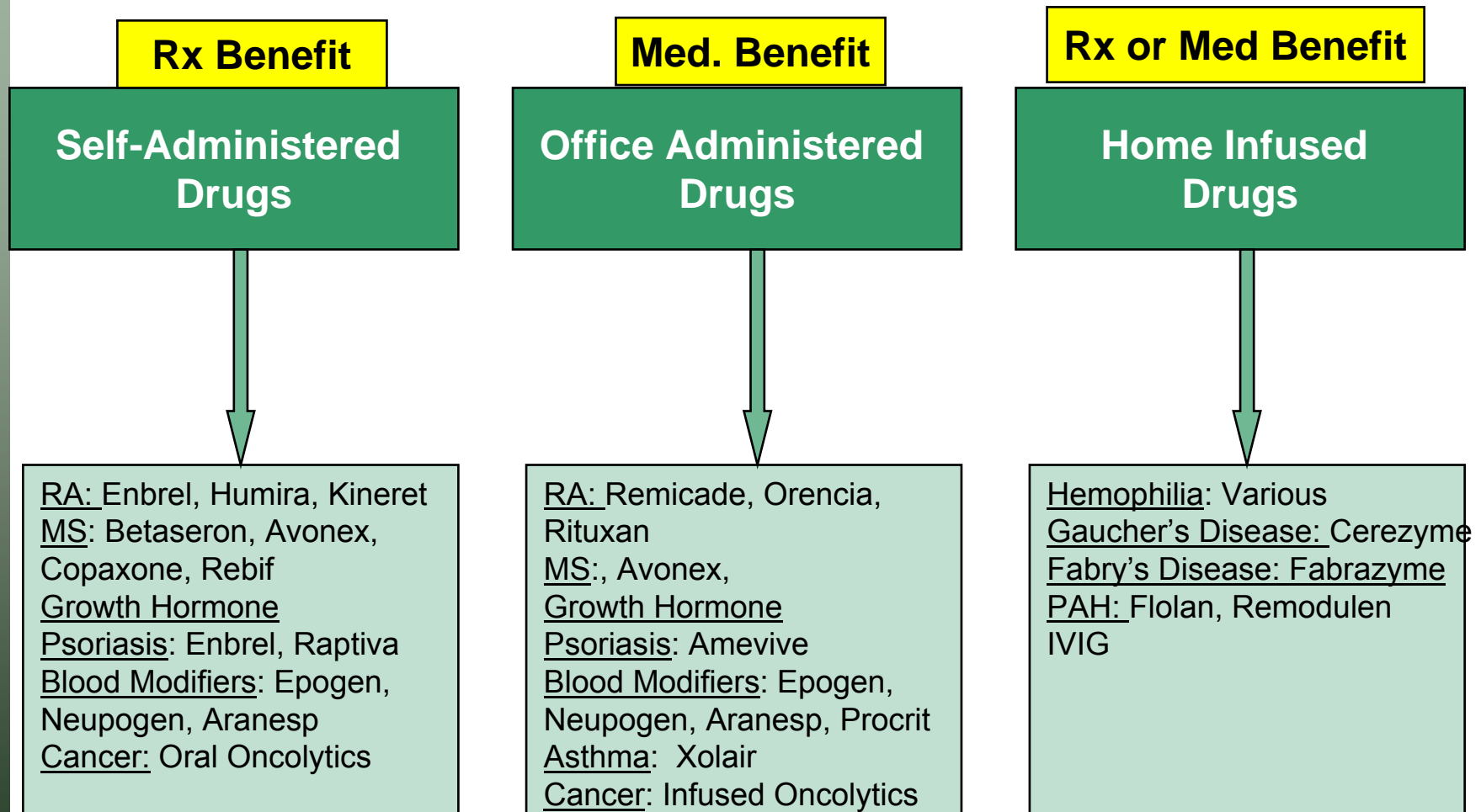
*Express Scripts 2007 Drug Trend Report*

# Coverage and Management of Specialty Pharmaceuticals

# Impact of Specialty Injectables

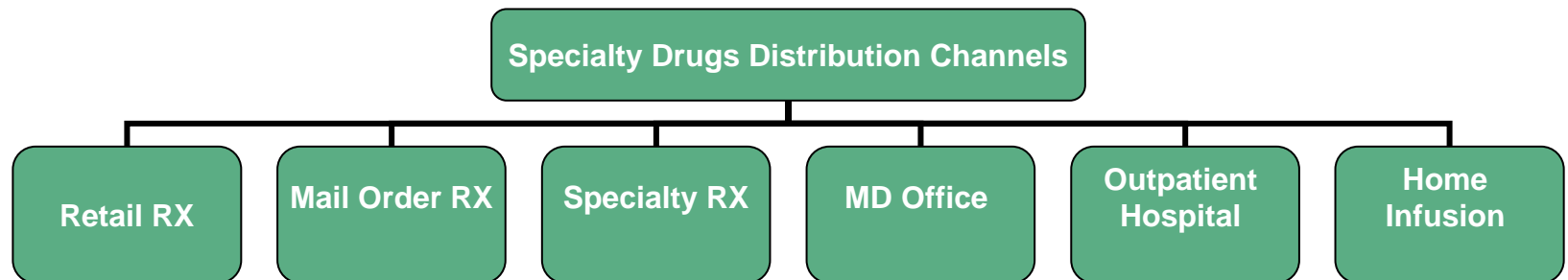
- High cost biotechnology drugs are changing the managed care “paradigm”
  - Traditional drug management strategies are not enough
  - “Siloed” drug management mentality not applicable
- Specialty drugs have been the fastest growing segment of drug spend
- SpRx represents significant challenges for private and public payers
  - Cost Management
    - Network
    - Patient Cost Share
  - Clinical Management
  - Utilization Management
  - Benefit Design
    - Is it Pharmacy or is it Medical?

# Three Types of Specialty Drugs



**Some Crossover Occurs Between Sites of Administration**

# There Are Many Distribution Channels for Specialty Drugs



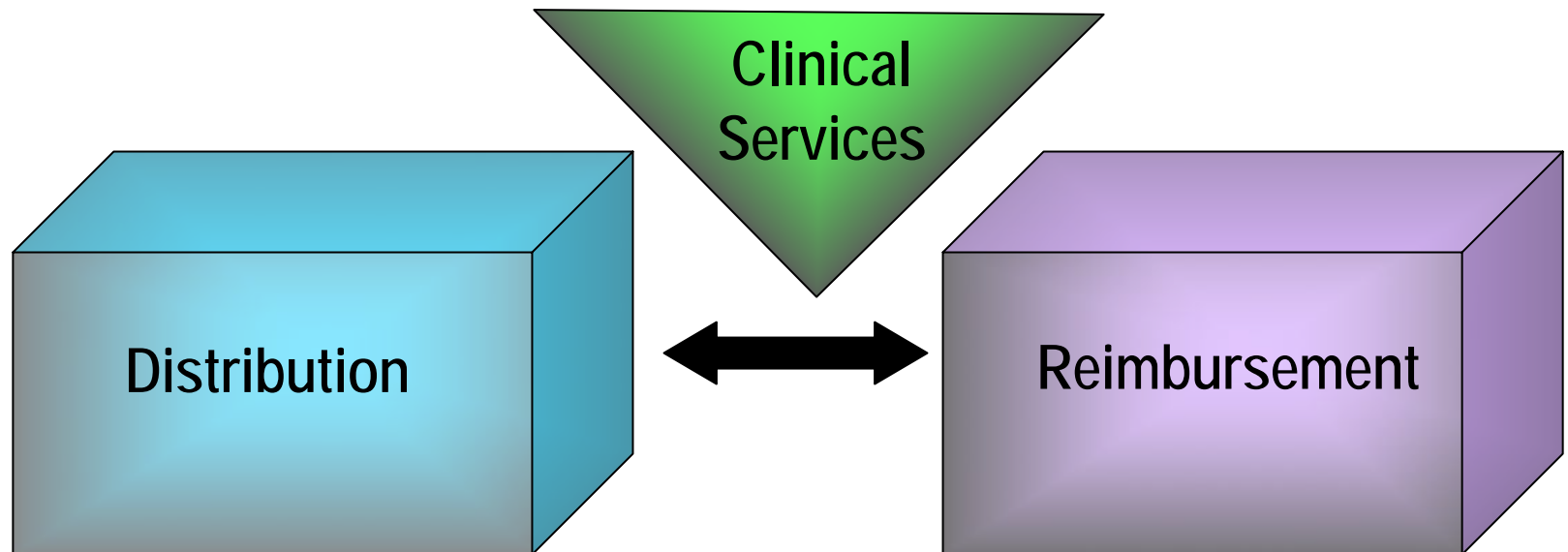
Each channel may receive a different reimbursement and be subject to different management practices



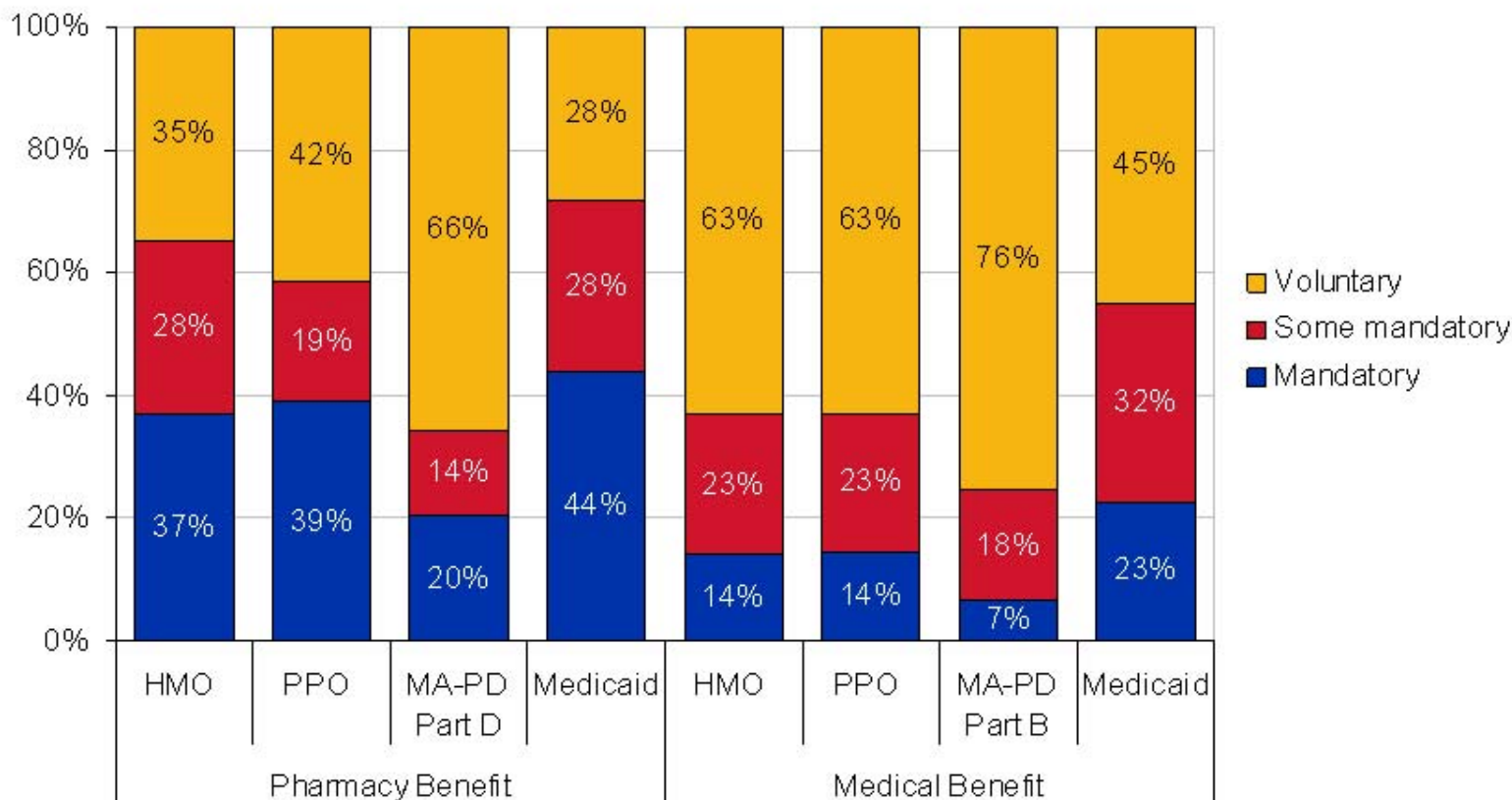
# Specialty Pharmacy Providers (SPP)

*Owned by PBMs, health plan, drug chains, wholesalers, home infusion companies*

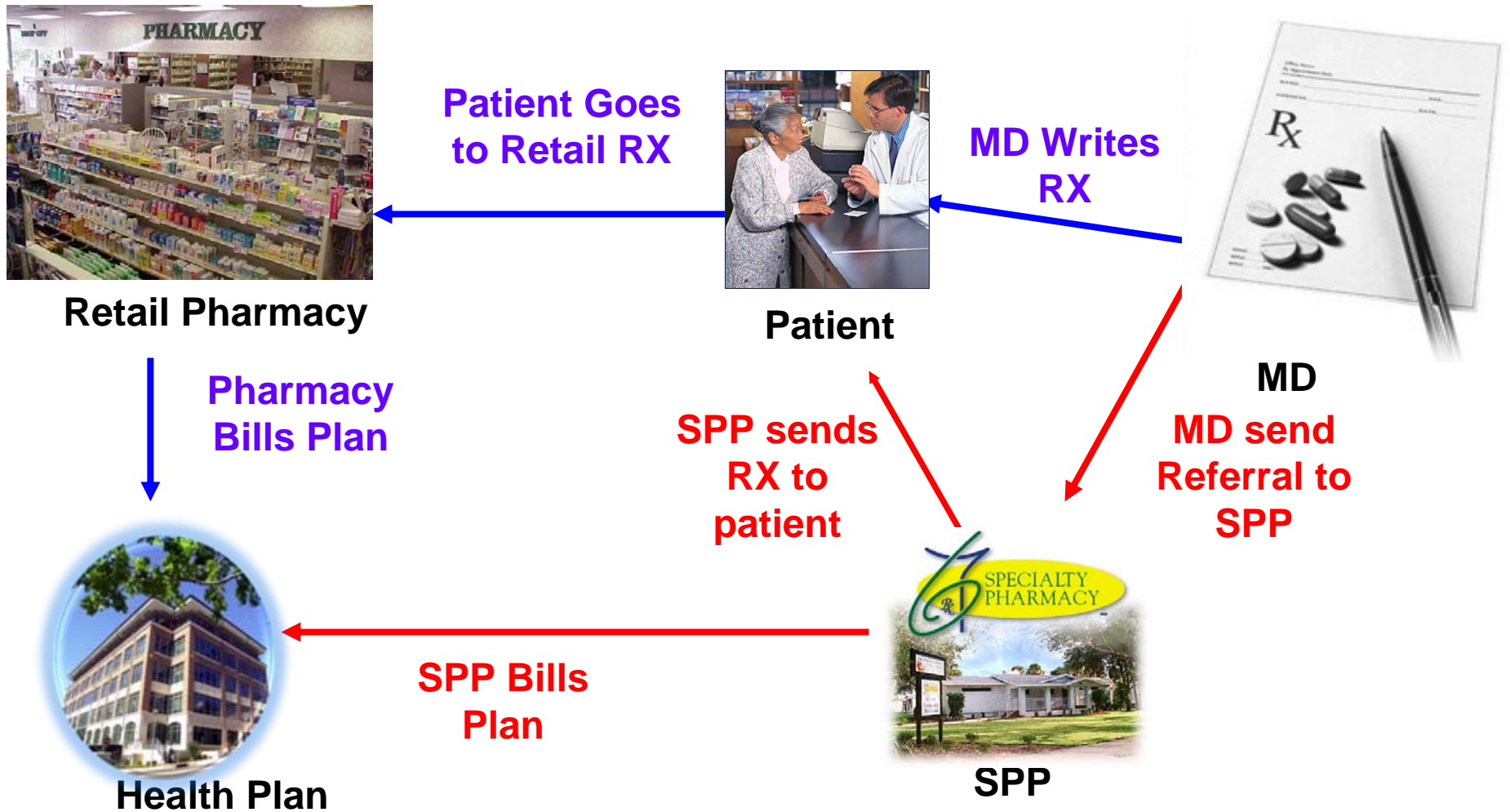
*Pharma trend to limit distribution of SpRX to select SPPs and restrict access*



# Voluntary vs Mandatory Use of SPP

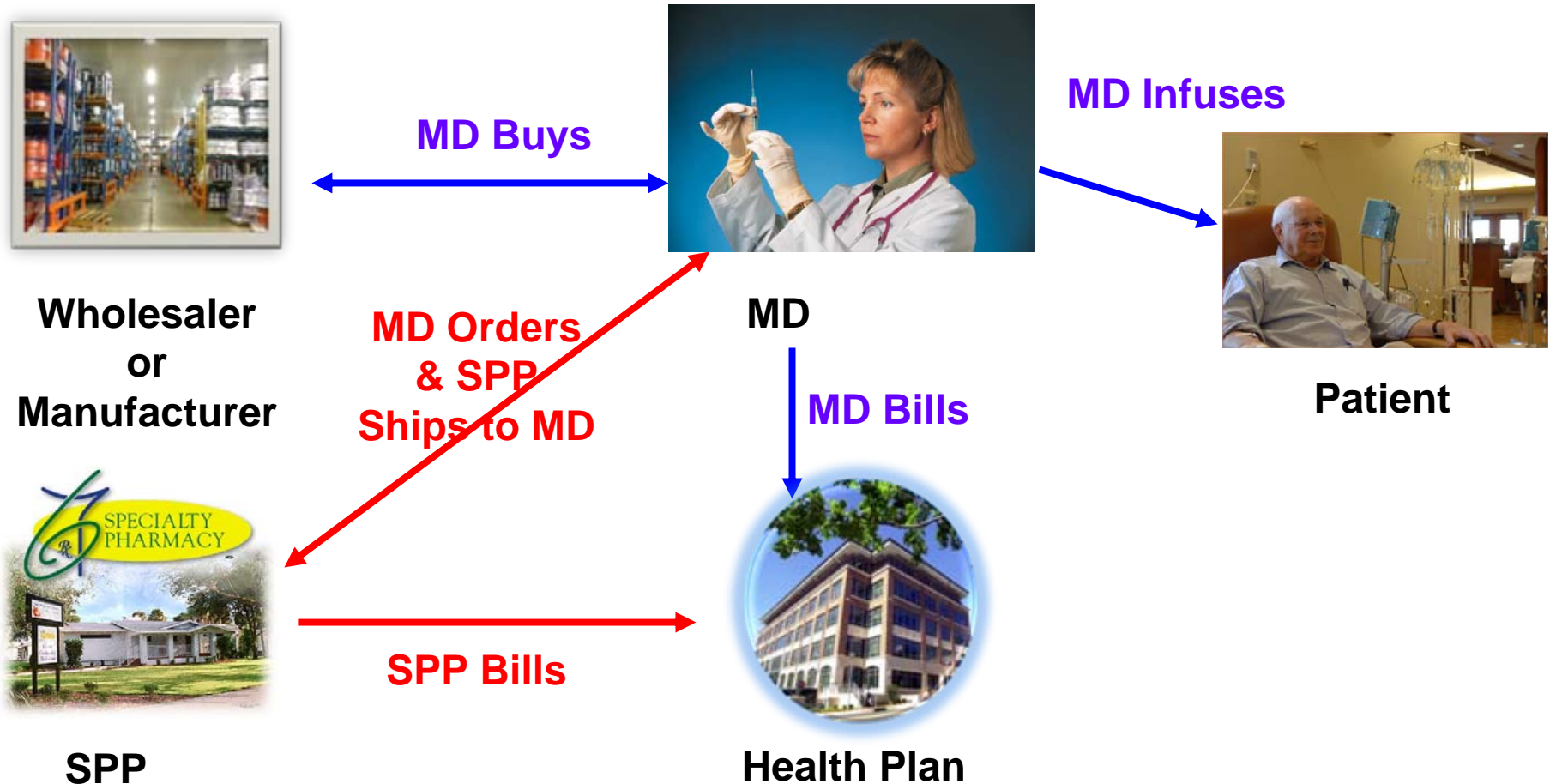


# Process Flow for Self-Administered Drugs



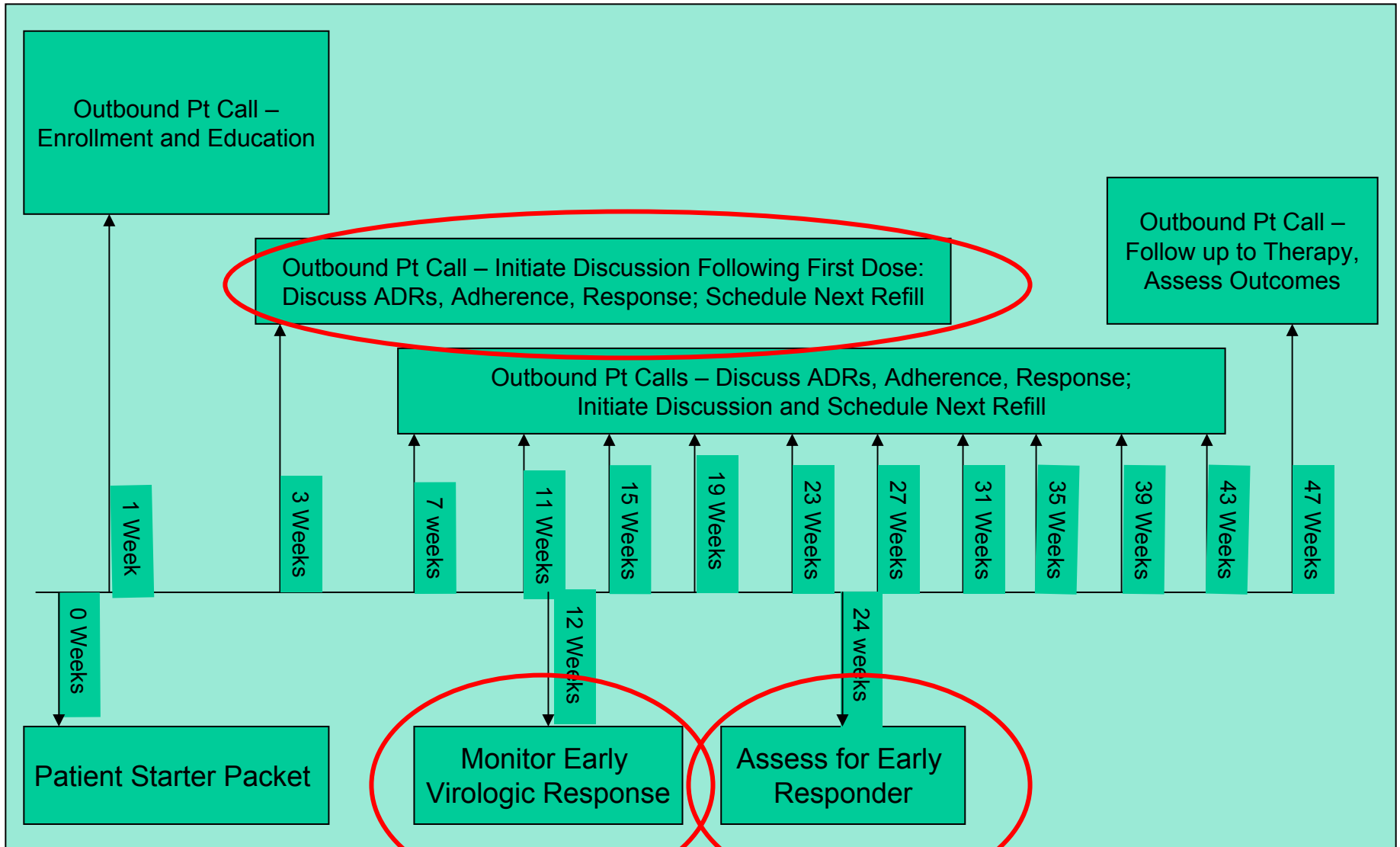
Over 50% of health plans allow patients to obtain self administered injectables through retail and SPP

# Process Flow for Office Administered Drugs



Over 60% of health plans that contract with SPPs allow physicians to continue to “buy and bill”

# Specialty Pharmacy Therapy Management of Hepatitis C



# Clinical and Utilization Management

# Payers Want to Ensure Appropriate Utilization

## Right Drug



*Is there another medication that may be more appropriate?*

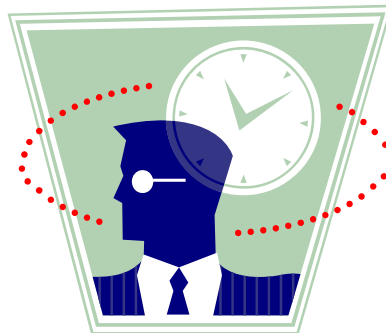
*Or may be less expensive yet equally effective?*

## Right Time

*Is this the correct dose?*

*Is this the right time in the regimen?*

*Does the pt. have enough meds? Too many?*



## Right Patient



*Should therapy be discontinued?*

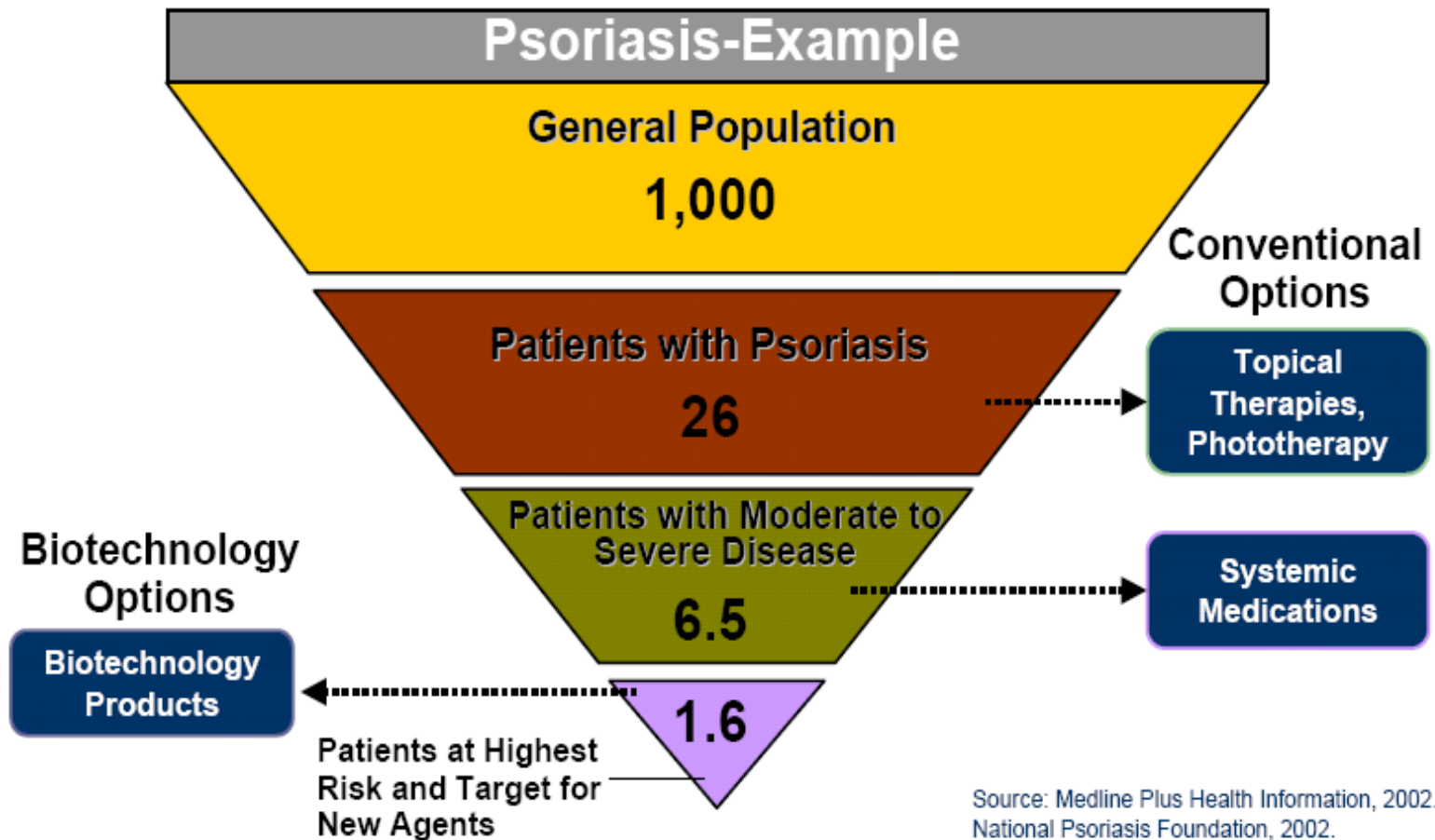
*Have labs been performed at the right time to measure results?*

# Payers Employ a Variety of Utilization Management Techniques

- Development of clinical guidelines and criteria
- Prior authorization to ensure appropriate use
- Step therapy guidelines
- Promote formulary with preferred products
- Reduce waste and over-use

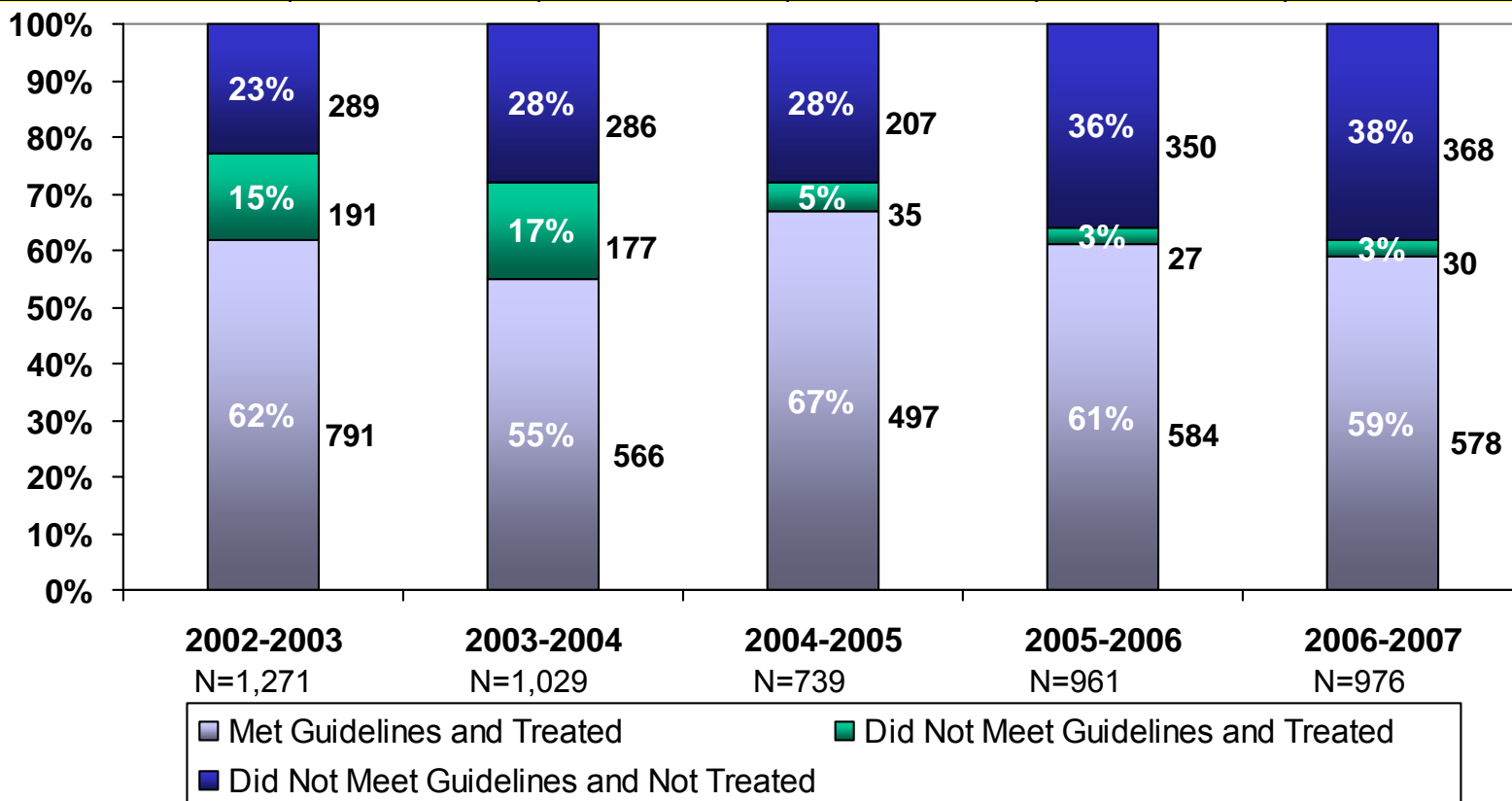


# Managing Specialty Therapies: Identifying the Patient



# Specialty Guideline Management Condition Specific Case Study – RSV

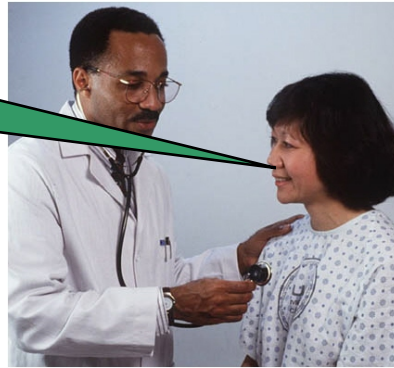
Program Financial Results	2002-2003	2003-2004	2004-2005	2005-2006	2006-2007
Did not meet AAP Guidelines	23%	28%	28%	36%	38%
Number of Doses Avoided	1,300	1,287	941	1,278	1,391
Cost Avoidance	\$ 1.9M	\$ 1.9M	\$ 1.5M	\$2.1M	\$2.5M



# Benefit Design Issues

# Misaligned Financial Incentives Among Stakeholders

I want the lowest copay



**Patient**

I want to make a profit on the drug



**Doctor**

I want the highest reimbursement



**Specialty Pharmacy**

# Payers are Looking at New Benefit Strategies to Manage Specialty Drugs

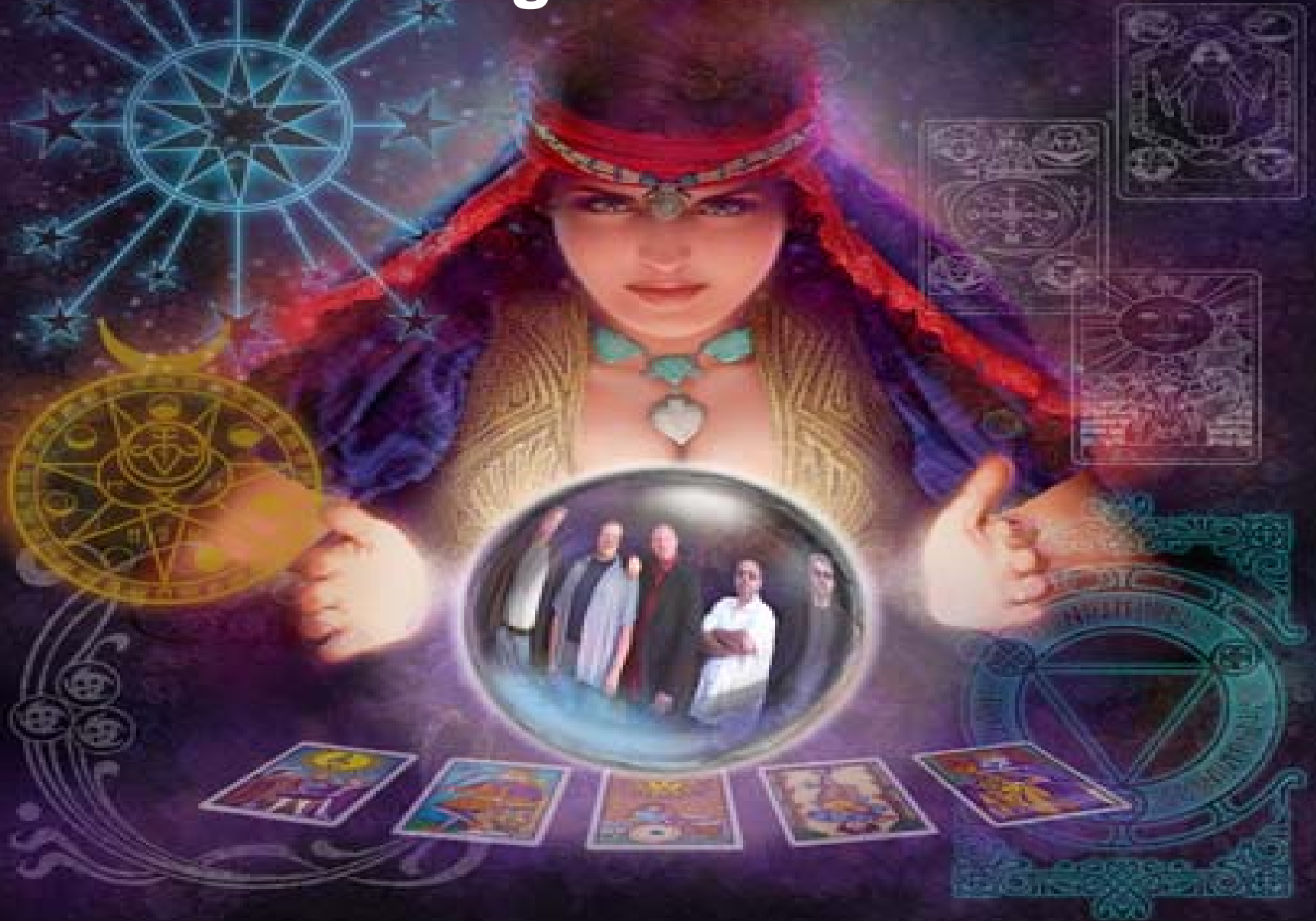
## Changing Benefit Design

- Trend to move from Medical Benefit to RX Benefit
- New Specialty Benefit
- Results in equalizing cost sharing, deductibles, clinical review

## Increased Pt. Cost Share

- Creation of 4<sup>th</sup> copay tier
  - % coinsurance/higher flat copay
- Out-of-pocket maximums per RX
- Deductibles
- Annual or lifetime maximum benefit

# Looking to the Future



# Payer Shifting Focus on Specialty

## Previous Expectations

- Biologics were indicated for treating rare diseases only
- Payers implemented few management tactics
- Physicians were given “carte blanche” related to prescribing
- Some therapeutic categories viewed as “off-limits”
  - Oncology
  - HIV/AIDs
  - Genetic disorders



## New Expectations

- Biologics will shift from treating rare disease to more common diseases
- No drug categories are “off-limits” if there is sufficient utilization and concern about their value and appropriate use
- New biologic treatments must result in favorable outcomes – both clinical and economic
- Shift burden of proof of overall value to pharma
- Develop a standardized approach to manage total specialty spend regardless of site or method of administration
- Physicians will be more accountable for their therapy selections and reimbursement will be equalized across channels

# Focus on Outcomes

- Uncertainty Related to Outcomes
  - Safety, efficacy and cost concerns
    - 75 new or revised black box warnings in 2007
  - Outcomes data not available when a new drug comes to market
  - Efficacy varies across patient populations and indications
  - Payers demanding pharmacoeconomic value from new therapies
  - Many new therapies do not provide quantifiable outcomes
  - FDA accelerated approvals
    - Product approval for life threatening diseases available on the market on the basis of preliminary evidence prior to formal demonstration of patient benefit.
  - Pharma may be required to document outcomes via a Pay for Performance (P4P) model.
  - P4P's intent is to improve evidence base for safety, comparative effectiveness and value
    - Move focus from EBM to outcome based medicine
    - Comparative effectiveness of competing agents
    - Improve value for money paid as well as quality of care

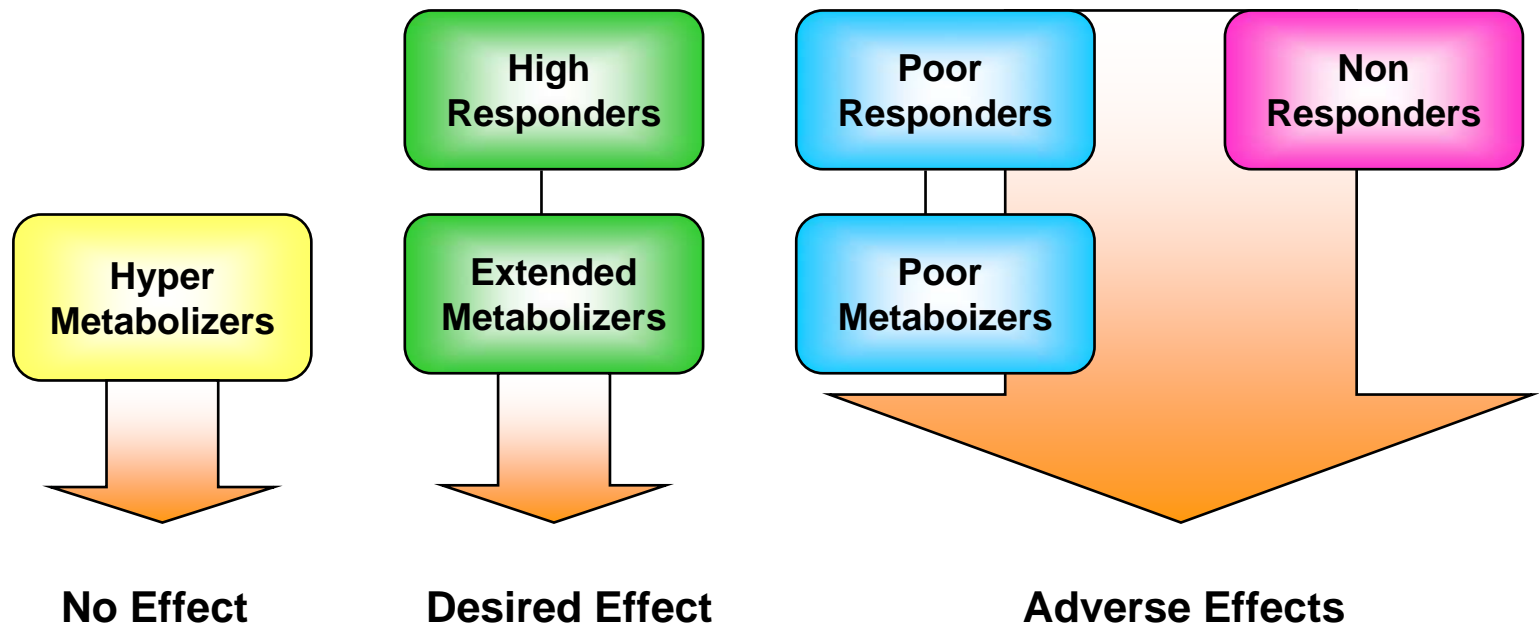


# Global P4P Models: Will This Work in the US?

Type of Model	Description	Example
<b>Price/Volume</b>	Payment is linked to predetermined utilization based on prevalence or sub-groups to limit budget impact and inappropriate use	55 agreements in Australia including anti-TNF and Spiriva.  Other agreements in France and Italy.
<b>Performance or Risk-based</b>	Different reimbursed price depending on patient outcomes	<b><i>NICE/UK</i></b> <u>Multiple sclerosis DMDs</u> Manufacturers at risk for cost if defined outcomes are not achieved  <u>Velcade (bortezomibe)</u> Manufacturer refunds cost if target outcome not achieved

# Selecting Optimal Therapy

*40-60% of patients do not benefit from the drugs they are prescribed*



# Examples

<i>Disease Treatment</i>			
<b>Disease</b>	<b>Drug</b>	<b>Mechanism</b>	<b>Diagnostic test</b>
Breast Cancer	Herceptin®	Inhibit HER2 receptor	IHC and FISH
CML & Malignant gastrointestinal stromal tumors (GIST)	Gleevec®	Inhibits Bcr-Abl protein	BCR-ABL (CML), C-KIT (GIST) Genetic test to monitor emergence of Gleevec resistance
Various solid tumors	Various	Identify resistance to chemotherapy and clinical failure or potential success	Oncotech EDR® Assay
HIV/AIDS	Various		Phenotypic and genotypic resistance testing
<i>Reduce Adverse Drug Reactions</i>			
<b>Marker</b>	<b>Drug</b>	<b>Mechanism</b>	<b>Test</b>
Cytochrome P450 metabolism	30 different forms of CP450 coded by a different gene	Variations in genes lead to ↑ or ↓ in metabolism of drugs	Amplichip® detects variations in 2
	Camptosar	Predict patient's safety-related response	UGT1A1 assay
<i>Disease Prevention</i>			
<b>Disease</b>	<b>Genetic Variant</b>	<b>Indication</b>	<b>Results</b>
Breast and Ovarian Cancer	BRCA1 and BRCA2	Indicates hereditary propensity for cancer	Guide preventive measures

# Public Perception of Specialty Pharmaceuticals






# Specialty Drug Costs are Viewed as a “Side Effect”

“Payers Aim to Rein in Specialty Drug Spending”

*Wall Street Journal 3/20/08*

## Side Effects<sup>2</sup>

The average cost of a monthly prescription is going up for many drug classes.

Drug Class	Cost (2007)	Change from 2006
Cancer	\$1816.38	 15.8%
Multiple sclerosis	\$1647.00	 12.1%
Blood-cell deficiency	\$1724.51	 9.0%
Inflammatory conditions	\$1547.97	 4.6%
Growth-hormone deficiency	\$2569.10	 1.8%

Source: Express Scripts (prices are paid by the pharmacy-benefits manager)

- Current specialty drug spend is \$60 billion
- Expected to reach \$99 billion by 2010
- Annual trend rate is 15–20%

# Specialty Pharmacies, Pharma and Health Plans Portrayed as “Pushing Prices Higher”

*New York Times, 4/14/08*

**“Co-payments Soar for Drugs with High Prices”**

*Blames employers and plan sponsors for the high cost of biotech treatments.*

*New York Times, 4/19/08*

**“Paid to Control Drug Costs, Yet Pushing Some Prices Higher”**

*Blames high cost of biotech drugs on those who dispense and manage biotech products.*

*Focus on drugs with limited or exclusive distribution.*

# Issues to Consider...

- How private and public payers can continue to offer affordable benefits to all enrollees with increased use of biologics
- How to equitably adjust cost share for persons who need to take expensive biologics
- How to remove administrative decisions about benefit design and reimbursement when selecting the appropriate drug for the patient
- The lack of a viable biosimilar pathway
- How to monitor therapy adherence and outcomes that show effectiveness of therapy
- How to overcome data management challenges within the legacy medical claims system

# Discussion



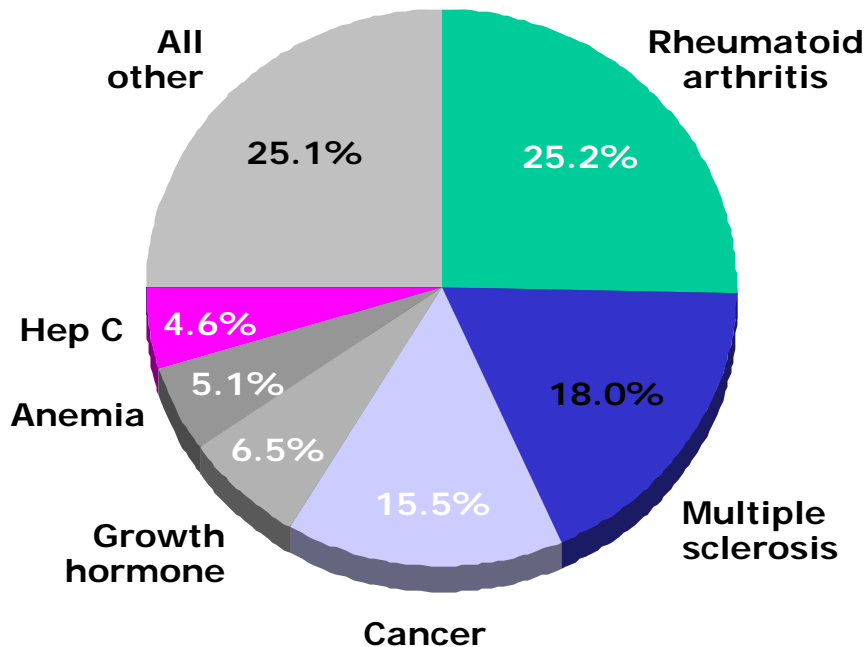


# Top specialty drug spend

## Pharmacy benefit and medical benefit – 2006

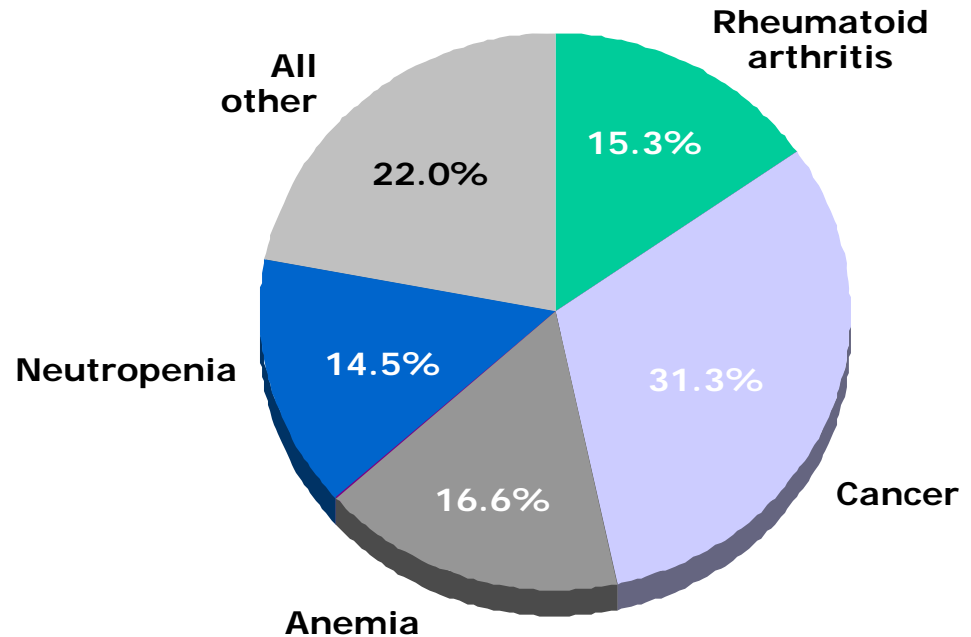
### Pharmacy

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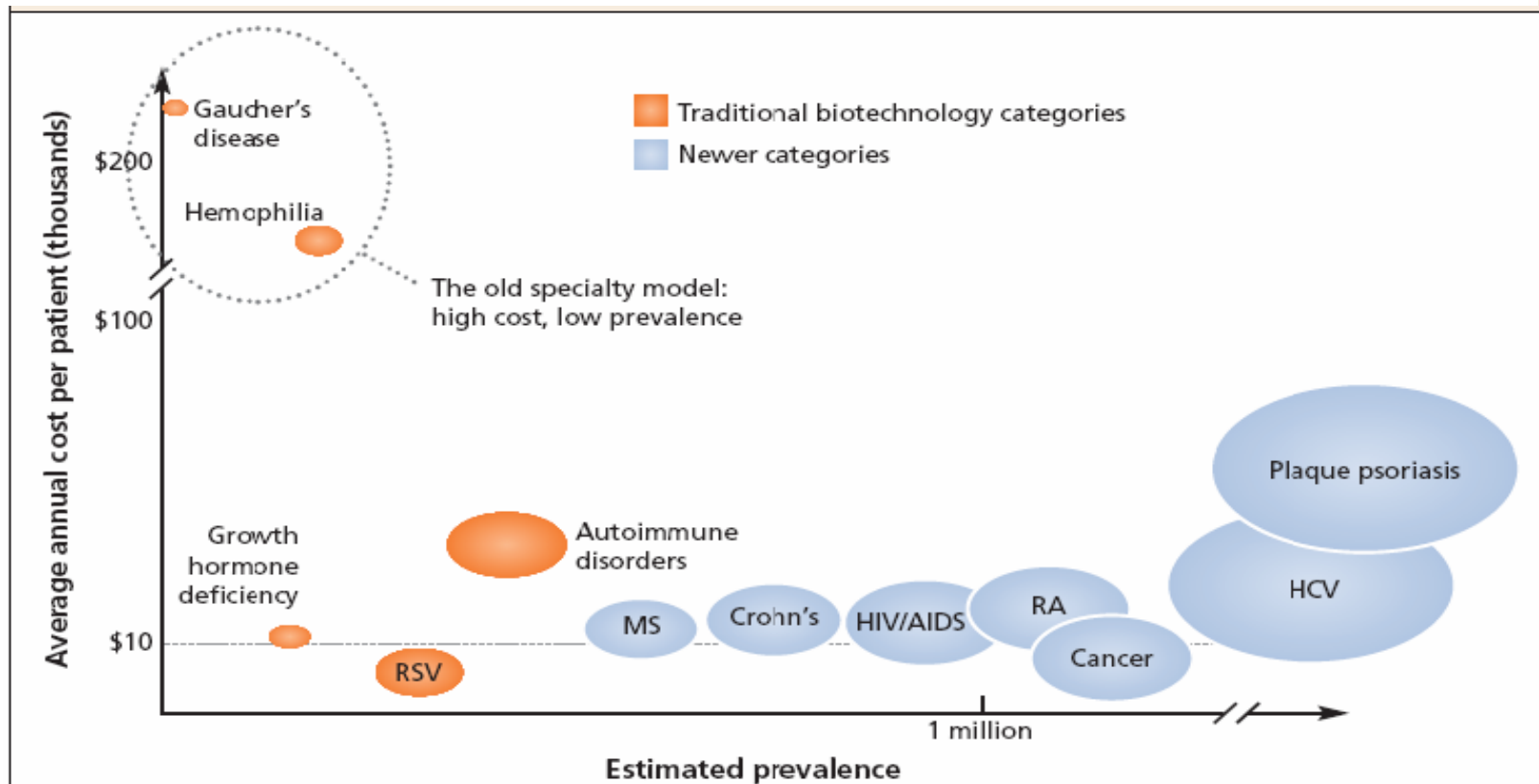


### Medical

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# Changing Cost and Prevalence in Biotechnology : Shift Toward Larger Target Populations



SOURCES: Raymond James & Associates Equity Research; AdvancePCS; Atlantic Information Services, *Specialty Pharmacy: Stakeholders, Strategies, and Markets*; William Blair & Company Equity Research; Jacobson 1997.

HCV=hepatitis C virus, MS=multiple sclerosis, RA=rheumatoid arthritis, RSV=respiratory syncytial virus.

# The Promise of Personalized Medicine

## Better diagnoses and earlier interventions.

- Detect disease at earlier stage
- Reduce adverse drug reactions
- Shift emphasis from REACTION to PROACTION

## More efficient drug development.

- Design targeted therapies based on molecular pathways
- Smaller defined patient population to reduce initial cost and duration of clinical trials
- Reduce time, cost and failure rate of clinical trials

## More cost-effective therapies.

- Cost savings realized through proactive and preventive interventions
- More patients seeking care once identified
- Less patients requiring “shotgun” or ineffective therapy
- Select optimal therapy and reduce trial and error prescribing