# Outcome-based Risk-sharing Agreements

Washington Prescription Drug Price and Purchasing Summit Series – Part 1

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# Acknowledgments


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# Disclosures

- Josh Carlson is a partner in Veritech, Inc., a healthcare consultancy.
- Original sponsors of UW PBRSA Research Project: Novartis, Johnson & Johnson, GSK, GE Healthcare, Roche, Pfizer, Eli Lilly, Sanofi-Aventis, Abbott Laboratories, Amgen

# Agenda

- Background
- Review of performance-based arrangements
- Informative case examples
  - Januvia/Janumet for diabetes in the US
  - Risedronate for Osteoporosis in US

# Performance-Based Risk-Sharing Arrangements: A Variety of Names

- Managed entry agreements (MEA)
- Outcomes-based schemes
- Risk-sharing agreements
- Coverage with evidence development (CED)
- Access with evidence development
- Patient access schemes (PAS)
- Conditional licensing
- And others?

The New York Times

Business



# Pricing Pills by the Results


Illustration by The New York Times

By ANDREW POLLACK
Published: July 14, 2007

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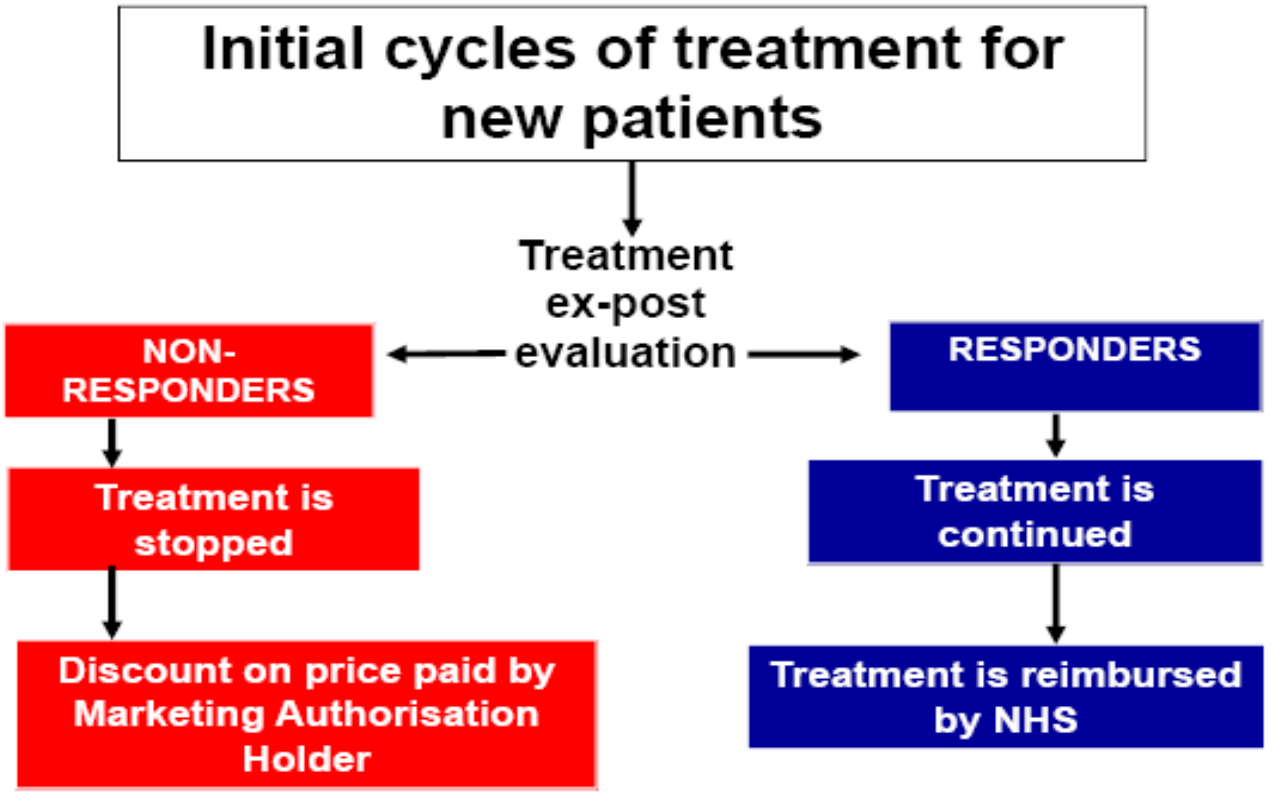
REPRINTS

SHARE

Drug companies like to say that their most expensive products are fully worth their breathtaking prices. Now one company is putting its money where its mouth is — by offering a money-back guarantee.

Johnson & Johnson has proposed that Britain's national health

# AIFA Risk Sharing procedure




AIFA

AGENZIA ITALIANA DEL FARMACO

Source: Paolo Siviero, AIFA

# Background

- **Increasing medical expenditures:**
  - Rising cost and use of pharmaceuticals
  - Prescribing beyond evidence and approved indications
  - Increasing use of complex diagnostics?
  - Other factors (aging population, fewer resources, etc)

- **The Pervasiveness of Uncertainty:** Medical products are approved, launched, and reimbursed under conditions of uncertainty related to:
  - Efficacy (heterogeneity)
  - Effectiveness in real world
  - Risks (safety)
  - Models, including links between surrogate markers and long-term outcomes
  - Cost-effectiveness
  - Budget impact

# Payer Response to Increasing Cost Pressures

- Public and private sector payers are facing these challenges with various cost-control instruments and management strategies:
  - Increasing patient co-payments
  - Pre-use authorization (targeting appropriate patients and appropriate use)
  - Quantity and dose limitations
  - Specialty pharmacy vendors
  - Benefit restrictions (e.g. generic-only benefits)
  - Denial of coverage

# National Association of Medicaid Directors (NAMD)

- "Policymakers have failed to address the cost and reimbursement issues associated with faster or increased pathways for the development of high-cost therapies and treatments."
- "While the immediate focus and challenges present with hepatitis C treatments, we know this is a harbinger of the promises and challenges that will emerge in the years ahead."
- "The challenge…is the intersection of a high-cost therapy and a potentially large population eligible for the therapy."
- "It is not practical to expect Medicaid programs to finance the significant upfront costs of Sovaldi…on the promise of seeing savings 10, 20, or 30 years later."

# NAMD policy suggestions

- Direct price controls for public payers
- Federal purchasing (negotiated discount) and distribution to public programs (e.g. vaccines)
- Mandated rebates for high volumes (i.e. Price volume)
- Modify "best price" to include selling price in other countries (reference pricing)
- Allow Medicaid programs to utilize cost-effectiveness research to identify whether or not a particular drug will be included in the program's formulary by granting Medicaid the flexibility to exclude products that are found to not be cost-effective
- Allow innovative payment arrangements. For example, allow states to enter into outcomes-based contracts with manufacturers, where payment is made per successful course of treatment rather than per pill.

# PBRSA—Five Key Characteristics

1. **There is a program of data collection** agreed between the manufacturer (or provider, in some instances) and the payer..
2. **This data collection is typically initiated during the time period following the regulatory approval** (which may be full, conditional, or adaptive), and linked to post-launch coverage decisions..
3. **The price, reimbursement, and/or revenue for the product are linked to the outcome of this program of data collection** either explicitly by a pre-agreed rule or implicitly through an option to renegotiate coverage, price, and revenue at a later date
4. **The data collection is intended to address uncertainty about …. For example:**
   - efficacy or effectiveness in the tested population as compared to current standard of care;
   - the efficacy or effectiveness in a broader, more heterogeneous population than used in registration trials or in pre-licensing testing;
5. **These arrangements provide a different distribution of risk between the payer and the manufacturer than the historical manufacturer-payer relationship.**

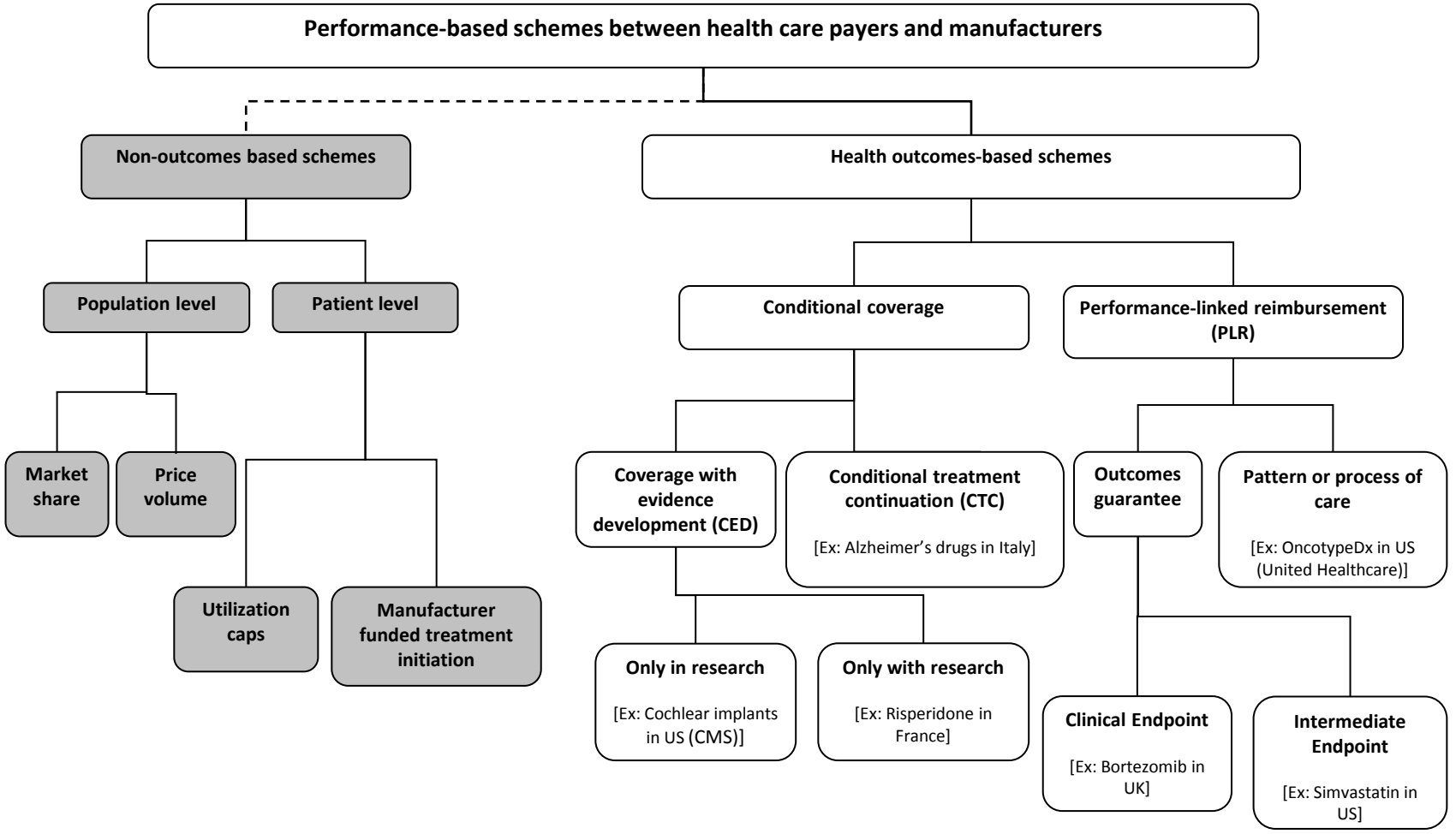
*Source: ISPOR PBRSA Task Force Report*

# Review of Performance-Based Arrangements: Methods

- Sources:
  - PubMed
  - Google
  - Government payer and reimbursement agency websites
  - Reports and communications from colleagues and healthcare experts.

- PBRSA Definition:
  - Arrangement between a payer and a pharmaceutical, device, or diagnostic manufacturer where the price level and/or nature of reimbursement is related to the actual future performance of the product in either the research or 'real world' environment.

- University of Washington Performance Based Risk Sharing Database®


Performance-based schemes between health care payers and manufacturers
Non-outcomes based schemes
Health outcomes-based schemes
Population level
Patient level
Conditional coverage
Performance-linked reimbursement (PLR)
Market share
Price volume
Utilization caps
Manufacturer funded treatment initiation
Coverage with evidence development (CED)
Conditional treatment continuation (CTC)
[Ex: Alzheimer's drugs in Italy]
Outcomes guarantee
Pattern or process of care
[Ex: OncotypeDx in US (United Healthcare)]
Only in research
[Ex: Cochlear implants in US (CMS)]
Only with research
[Ex: Risperidone in France]
Clinical Endpoint
[Ex: Bortezomib in UK]
Intermediate Endpoint
[Ex: Simvastatin in US]

# Taxonomy 2 X 2

| | Patient Level | Population Level |
|---|---|---|
| **Health Outcomes** | • Performance-Based Reimbursement: Patient Level<br>• Conditional Treatment Continuation | • Coverage with Evidence Development: Only in Research<br>• Coverage with Evidence Development: Only with Research<br>• Performance-Based Reimbursement: Population Level |
| **Financial/Utilization Outcomes** | • Manufacturer-funded treatment initiation<br>• Individual Budget caps<br>• Utilization Caps | • Price Volume<br>• Market Share<br>• Traditional Rebates<br>• Price parity |

Note: Distinction between Patient and Population Level relates to the way in which the data generated is applied or impacts the decision to cover or reimburse the product

# Coverage with Evidence Development: What problems are being addressed?

- Coverage with evidence development: coverage is conditioned on collection of additional population level evidence, from pre-specified study, to support continued, expanded, or withdrawal of coverage

- Problem: Insufficient evidence at product launch or time of coverage decision.
- Solution: Creates a middle ground b/n coverage and no coverage for promising products without sufficient evidence to support full coverage.
- Payer Benefit:
  - Provides access while generating additional evidence to support future coverage decision
- Manufacturer Benefit:
  - Access
  - Reduced cost of data collection

# Conditional Treatment Continuation: What problems are being addressed?

- Conditional treatment continuation : continuation of coverage for individual patients is conditioned upon meeting short-term treatment goals.

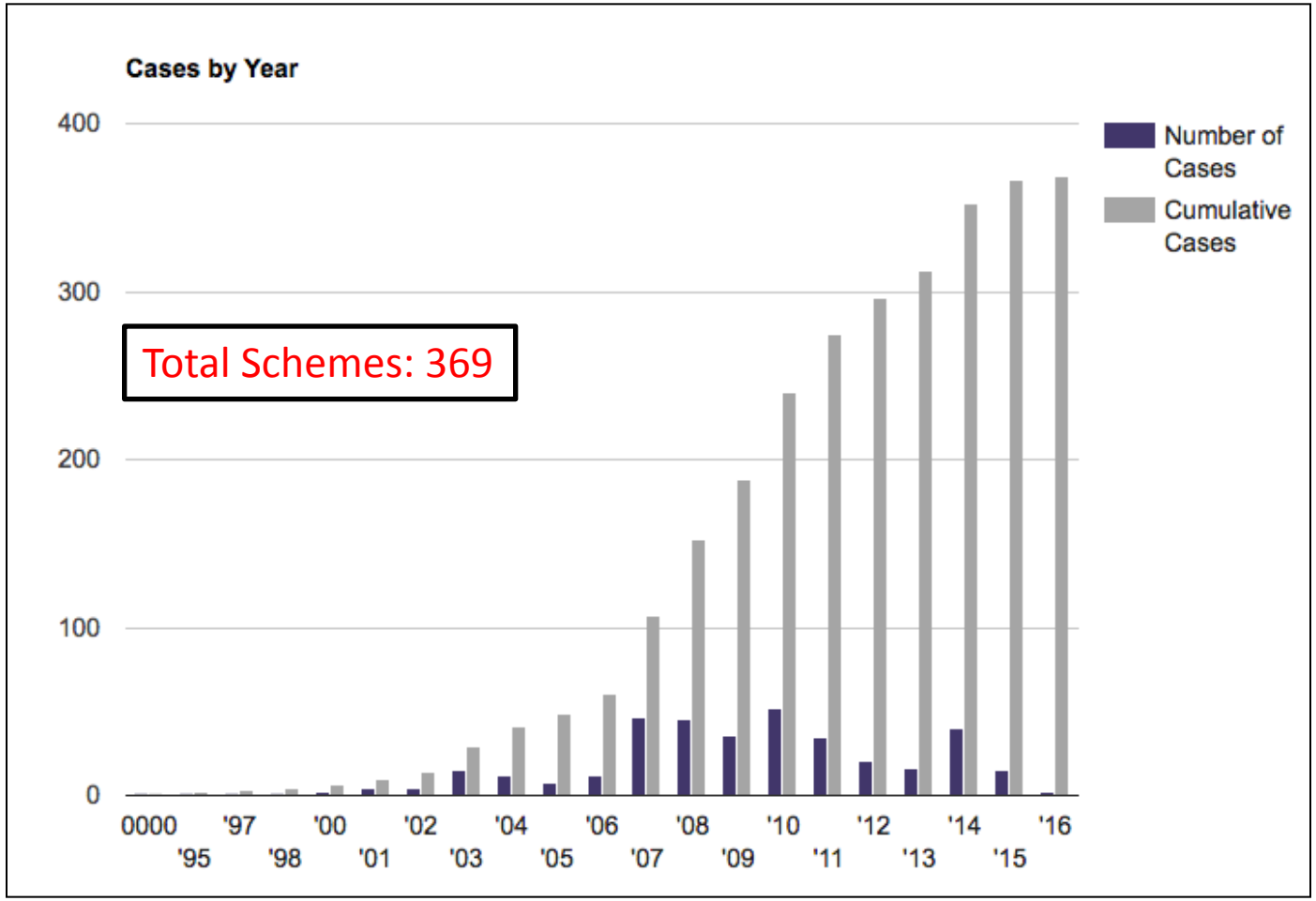
- Problem: Medical products are used in inappropriate patient populations
- Solution: Conditioning coverage on short-term treatment goals helps ensure that only patients benefiting from treatment remain on treatment.
- Benefit to Payer:
  - Minimizing their long-term cost exposure
  - Improving a product's cost-effectiveness
  - Replaces need for limits on patient access (e.g. prior authorization)
  - Assuage payers' concerns over patients receiving continued treatment despite a lack or loss of benefit.
  - Advantages increased when manufacturers cover cost of treatment initiation (E.g. Alzheimer's drugs in Italy).
- Benefit to Manufacturer
  - Access

# Performance-Linked Reimbursement: What problems are being addressed?

- Problem 1:  Payers may desire more evidence to support manufacturer's claims
  - Expensive: Direct costs & lost revenues due to delays in market access.
- Solution: Manufacturers provide payers a guarantee for certain outcomes linked by formula to the reimbursement level in place of additional product research.
- Problem 2: Pricing transparency can limit price discrimination for individual markets
- Solution: Alternative mechanism to provide discounts without changing list prices.
  - Example: List price for 3.5 mg vial of Velcade is £760 in U.K. , after rebate for non responders the effective price paid is closer to £540 per vial—yet the list price remains the same.
- Benefit to Payer:
  - Provide access to patients at a discounted net price
  - Decreased financial exposure for underperforming products
- Benefit to Manufacturer
  - Access at or near launch
  - Can be used to provide a discount

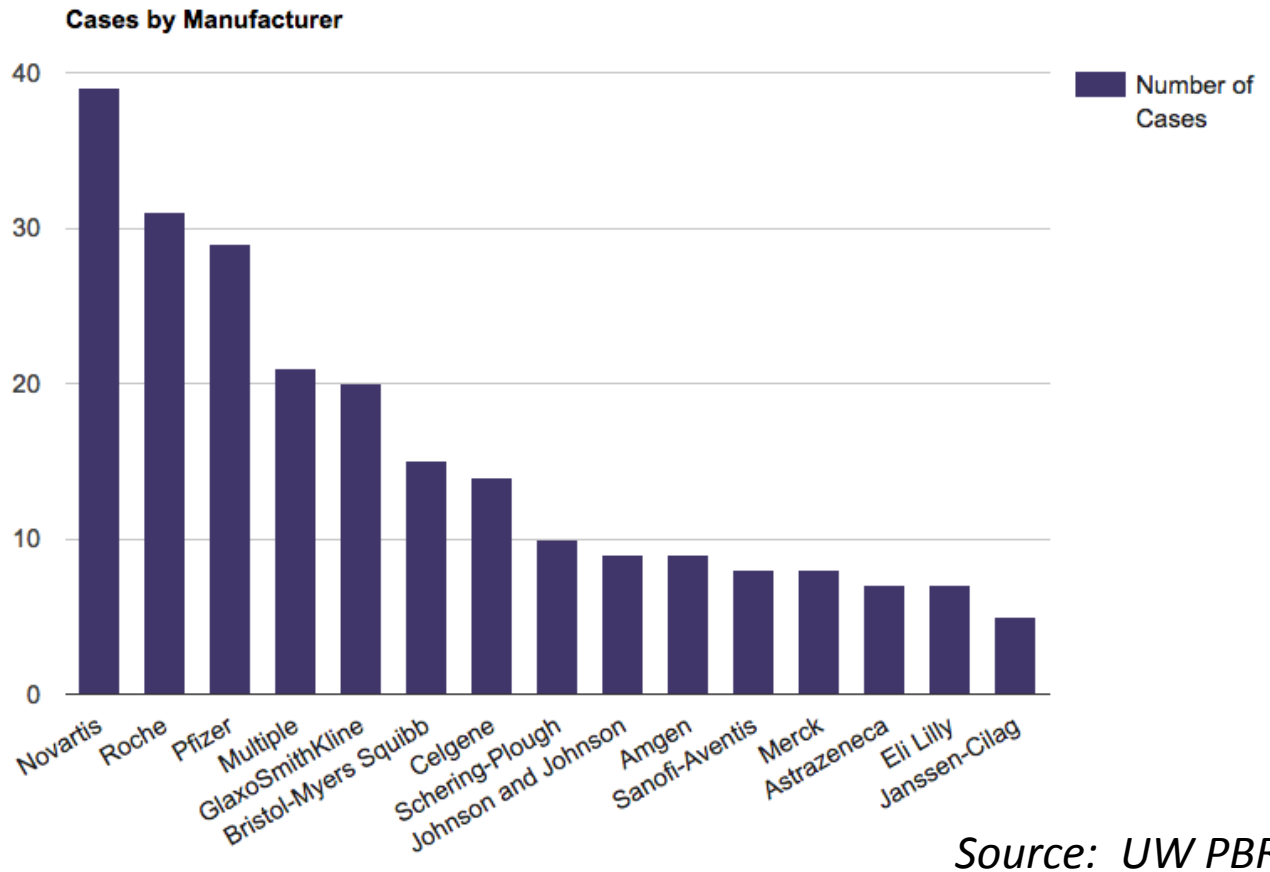
# Summary: What problems are being addressed?

1. Uncertainty
   - Resolve residual uncertainty:
     - Coverage with Evidence Development
   - Mitigate the negative consequences of uncertainty:
     - Payers – bad buy if product under delivers relative to expectation
       - Sub optimal patient heath, financial losses, inefficient resource allocation.
     - Developers/Manufacturers—no or limited market access.
       - Performance-linked reimbursement, conditional treatment continuation.
2. Inefficient pricing:
   - Allows for differential reimbursement without changing a list price



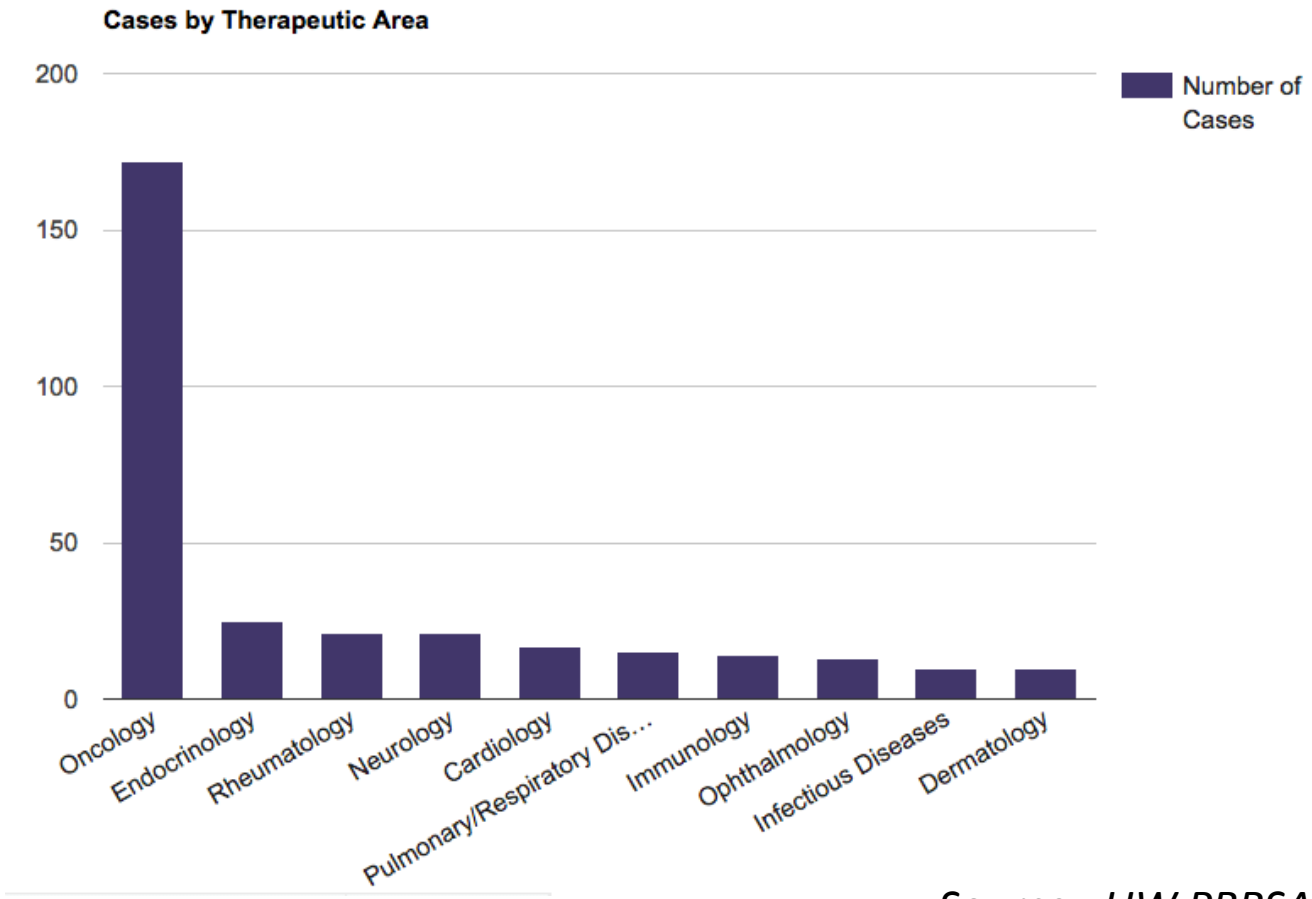

*Source: UW PBRSA Database*

# Cases by Manufacturer




*Source: UW PBRSA Database*

# Cases by Therapeutic Area




*Source: UW PBRSA Database*

# EXAMPLES

# Junuvia and Janumet (Merck) for Diabetes and CIGNA

- Scheme has three core components:
  1. CIGNA will assess the blood sugar levels (A1c lab values) for pts on any oral antidiabetic medications.
     - If the A1c values, in aggregate, improve by the end of the agreement period, the discounts will increase by a pre-agreed amount.
  2. CIGNA will use claims data to determine if patients are taking Januvia and Janumet as prescribed
     - Merck will further increase the discounts
  3. Better placement on CIGNA's formulary + lower copayment versus that for other branded drugs.

# Junuvia and Janumet (Merck) for Diabetes and CIGNA

- Different from other schemes → deeper discount when patients improve their A1c lab values.
- Benefit all the key parties—payers, manufacturers, and patients.
  - Diabetes patients who are more adherent tend to have better outcomes.
  - Pts with better adherence and outcomes utilize fewer resources→ cost savings
  - Manufacturers can improve sales volumes with better patient adherence
    - Offset the lost revenues related to the per unit discount offered by Merck.

- As a recent New York Times article stated,

"Merck is betting not only that its drugs prove superior but that CIGNA's incentives to reap the benefits of the deeper discounts will prompt the insurer to try to keep patients on those drugs."

# Risedronate (Proctor & Gamble, Sanofi-Aventis) for Osteoporosis and Health Alliance

- Two companies agree to reimburse the insurer for the costs of treating non-spinal fractures suffered by patients who consistently take their medications.
- First published example of a manufacturer agreeing to cover the cost of disease-related sequelae as opposed to discounting or refunding the cost of their product.
- Hip and wrist fractures cost approximately $30,000 and $6,000, respectively.
- The benefit to the manufacturers:
  - Keeps patients from switching to generic version
  - Maintains a lower copayment level than their competitor, ibandronate.

# Risedronate (Proctor & Gamble, Sanofi-Aventis) for Osteoporosis and Health Alliance

- Clinical trials of risedronate failed to show a statistically significant reduction in non-spinal fractures, whereas some competitors have demonstrated this benefit in their trials.
- Benefit to payer:
  - Outcome guarantee on uncertain clinical endpoint
- Makers of risedronate are betting:
  - Product will reduce non-spinal fractures in actual practice and/or,
  - The cost of treating them will be offset by maintaining or even expanding their market share in a highly competitive market in which it may not be the market leader.

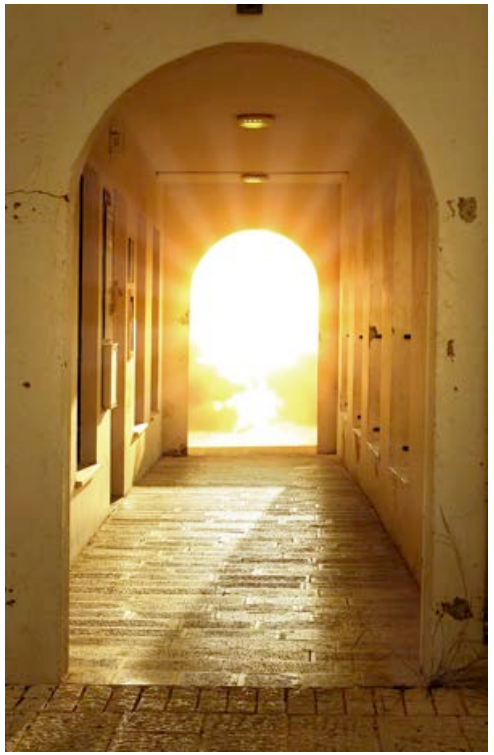
# Palmetto GBA and Noridian and ConfirmMDx

- Coverage with data development, i.e. CED: only with research
  - Creation of a physician registry
  - Data collection: PASCUAL trial
- Palmetto GBA expects 50% of Medicare cases to be in PASCUAL trial.
- PASCUAL trial is expected to take 2 years.
- Outcome measure at interim analysis:
  - If test substantially lowers re-biospy rate w/out adverse events, physician participation in ConfirmMDx registry program will be expanded—increasing number of patients tested and covered.
  - If trial demonstrates poor patient accrual or fails to demonstrate a substantially decreased re-biopsy rate, limited coverage will continue until either 1200 patients are tested or 3 years from start date
- After trial:
  - Favorable findings: Full coverage and removal of registry requirement
  - Unfavorable findings: non-coverage

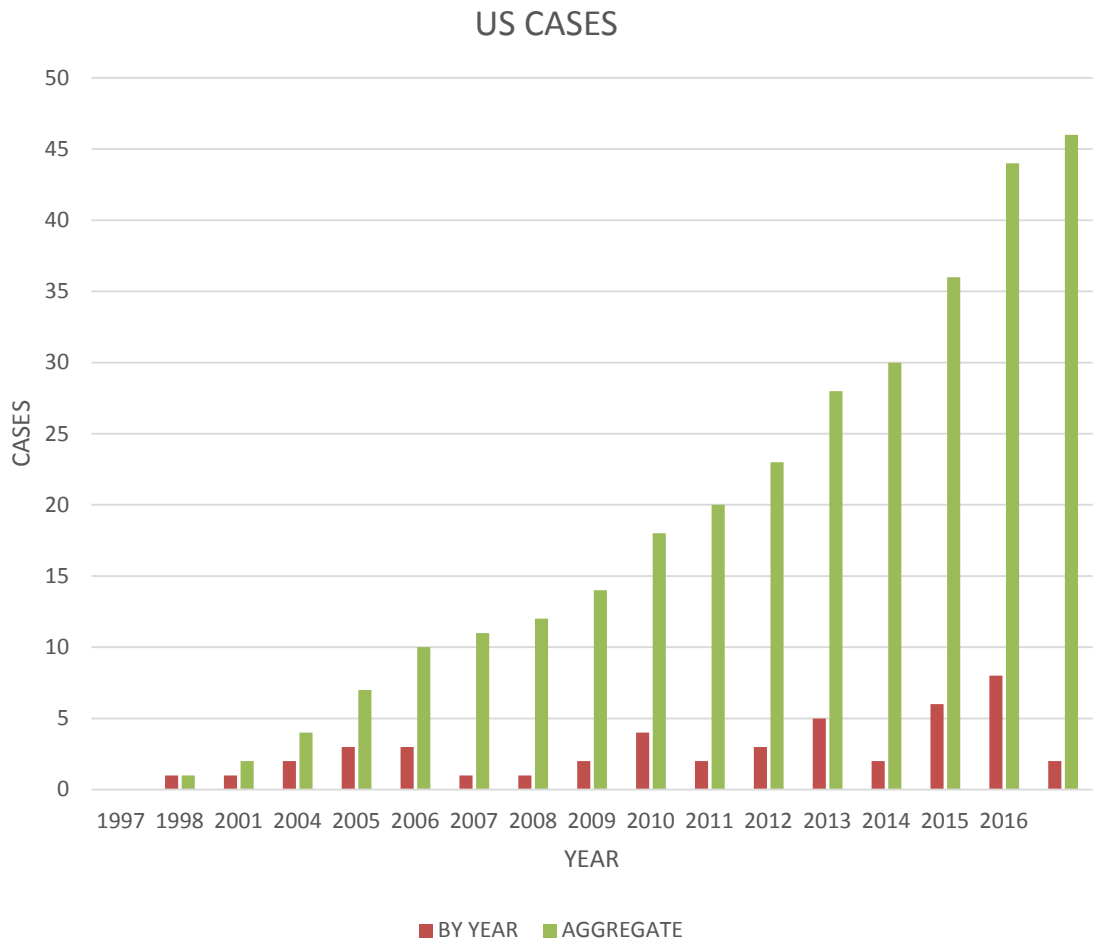
https://www.genomeweb.com/clinical-genomics/medicare-payment-scheme-mdxhealths-prostate-cancer-test-provides-model-condition

# Results of Implemented Schemes

- Difficult: Very little published, and even less on the results
- Payer: Cost savings and uncertainty reduction
- Manufacturer: Access



# U.S. Results




- CMS and CED:
  - Data used to inform two policy decisions
  - Other studies failed to be designed, funded, or implemented due to costs, measurement issues, and legal challenges.
- Cigna and Januvia/Janumet:
  - Blood glucose levels improved by more than 5 percent
  - Adherence was 87 percent for patients taking Januvia or Janumet
- Health Alliance and Actonel
  - Reimbursement rate 79% at 9 months
  - Lower than contract maximum
  - Incidence of non-spinal fractures consistent with clinical trial data.

# RECENT U.S. ACTIVITY

- **Proposed Rule: Part B Drug Payment Model**
  - **CMS proposes to test new Medicare Part B prescription drug models to improve quality of care and deliver better value for Medicare beneficiaries**
  - **Risk-sharing agreements based on outcomes.** This proposed test would allow CMS to enter into voluntary agreements with drug manufacturers to link patient outcomes with price adjustments.
  - https://www.federalregister.gov/articles/2016/03/11/2016-05459/medicare-program-part-b-drug-payment-model

# RECENT US ACTIVITY

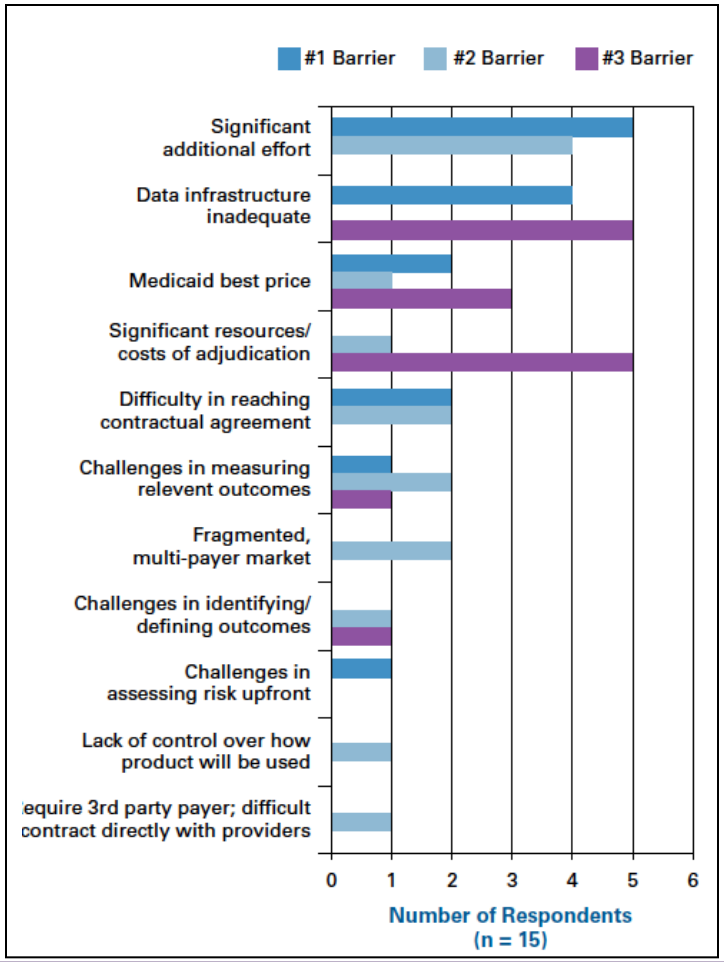
- Drugs:
  - Entresto and Cigna (2016)
    - Cigna's payments to Novartis will be based on a reduction in the proportion of customers who are admitted to the hospital for heart failure.
  - AstraZeneca PLC and Express Scripts (2016):
    - AZ with reimburse costs of the lung-cancer drug Iressa if a patient stops treatment before the third prescription fill.
- Diagnostic Tests
  - Palmetto GBA Coverage with Data Development
    - ConfirmMDx epigenetic assay for prostate cancer
    - Decipher (GenomeDx)
    - Prolaris (Myriad)
    - Oncotype Prostate (Genomic Health)
- Devices:
  - TYRX Antibacterial Envelopes (2015):
    - Medtronic will cover the cost of treating the patient's infection if a hospital's infection rate for procedures performed with Tyrx are higher than the infection rate for similar procedures without it.
  - Biopatch (2015)
    - A risk-sharing program is planned for Biopatch linked to catheter-related infections

# Private Sector Risk-Sharing Agreements in the United States: Trends, Barriers, and Prospects

Louis P. Garrison, Jr, PhD; Josh J. Carlson, PhD; Preeti S. Bajaj, PhD; Adrian Towse, MA, MPhil; Peter J. Neumann, ScD; Sean D. Sullivan, PhD; Kimberly Westrich, MA; and Robert W. Dubois, MD, PhD

**Figure 4. Potential Barriers to RSA Use in the United States**

1. Significant additional effort required to establish/execute RSAs (eg, compared to traditional rebates/discounts)
2. Challenges in identifying/defining meaningful outcomes
3. Challenges in measuring relevant real-world outcomes
4. Data infrastructure inadequate for measuring/monitoring relevant outcomes
5. Difficulty in reaching contractual agreement (eg, on the selection of outcomes, patients, data collection methods)
6. Implications for federal (Medicaid) best price
7. Payer concerns about adverse patient selection
8. Fragmented multi-payer insurance market with and significant patient switching among plans
9. Challenges in assessing risk upfront due to uncertainties in real-world performance
10. Lack of control over how product will be used
11. Significant resources and/or costs associated with ongoing adjudication



# Understanding and Developing Performance-Based Arrangements

- Understand the intervention
  - Nature of uncertainty
  - Available short-term efficacy and safety measures
  - What innovative schemes might address the uncertainty
    - Leverage existing data collection efforts
- Understand the market factors
  - External market factors that may impact the approach
    - Unmet need
    - Competitive landscape
    - Country/payer type
- When might additional investment into evidence generation studies be needed?
- Explore impact of schemes using cost-effectiveness and revenue models

# Conclusions

- Performance-based agreements in line with healthcare trends
- They are intrinsically appealing
  - Align incentives toward realized value
- Substantial barriers to implementation that will limit both the short-term and long-term impact
- They will not apply to all medical products, but rather to a select group where the payer and manufacturer can find common ground
- Performance-based schemes are a viable option for the coverage and reimbursement of new medical products in many health systems.

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# Thanks!
# Questions?

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