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Keynote Address

Increasing the Relevance and Quality of Health Care Value Assessment

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Overview

❖ Practical Value Assessment

- Value Matters
- Practical Significance Matters: Magnitude + Measurement

❖ Issues and Opportunities for Value Assessment Impact

❖ Better Support for Coverage Decisions Based on Prior Evidence

❖ Better Support for Individual Treatment Choices

- Improved clinical guidelines and shared decision-making
- Guidance and support for real-world evidence initiatives

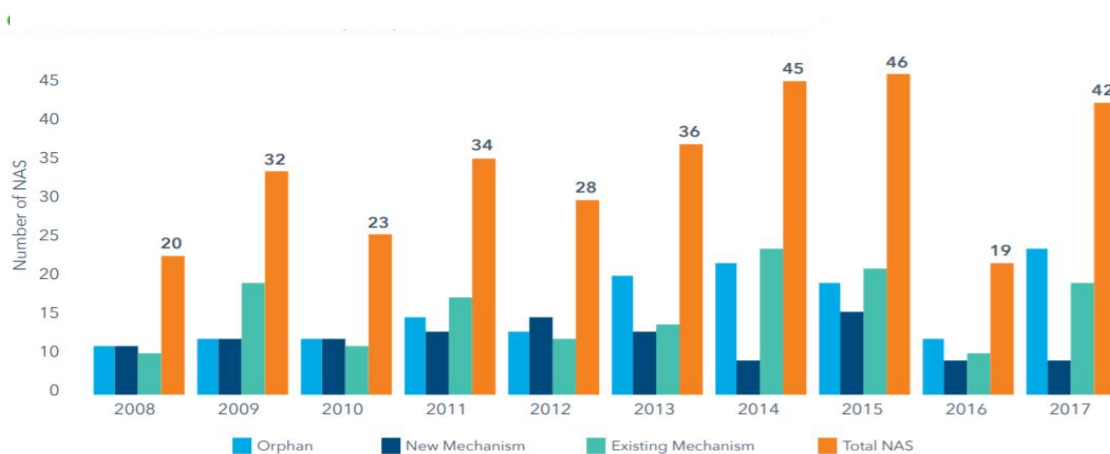
❖ Implications for Results-Based Payment

Health care fundamentals favor growing attention to value assessment

❖ Valuable breakthroughs in medical technology

- *HIV* → chronic disease instead of high-mortality diagnosis
- *Cancer* → 20% reduction in death rates over 25
- *Cardiovascular disease* → Over 60% reduction in last 50 years
- *Hepatitis C* → Potential for elimination
- Significant biomedical opportunities in many other disease areas

Continuing progress in biomedical innovation



Source: IQVIA LifeCycle New Product Focus, IQVIA Institute, Mar 2018

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IQVIA Institute for Human Data Science, "Medicine Use and Spending in the U.S. A Review of 2017 and Outlook to 2022" Accessed October 16, 2018. <https://www.iqvia.com/-/media/iqvia/pdfs/institute-reports/medicine-use-and-spending-in-the-us-a-review-of-2017-and-outlook-to-2022.pdf>

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❖ But questions about realization of value in practice

- Leveling/increase in mortality rates for CV disease
- High variation in apparent cost-effectiveness of cancer treatments
- Potential variation in response across patients – genomics/proteomics, care model, adherence, preferences

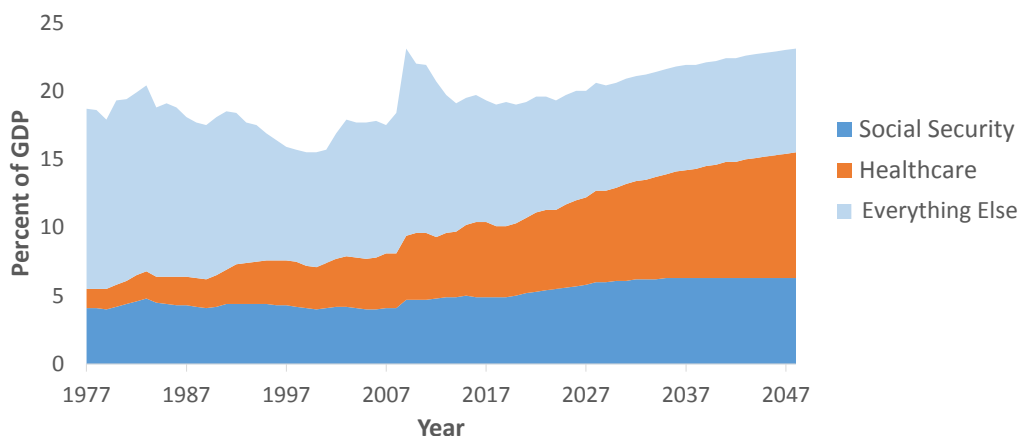
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Historical and projected federal spending

Health Care and Federal Spending



Relevance of value assessment

❖ Coverage and pricing decisions

- Additional resource for payers and manufacturers for use in pricing/coverage negotiations, especially for drugs that do not appear to have close substitutes
- Ex-US, eg NICE: explicit basis for access and coverage decisions
- US relevance, eg ICER and Drug Abacus: potential consideration in prior authorization, tier placement, coverage

❖ Individual patient care decision-making and basis for clinical guidelines

- ASCO, NCCN, ACC/AHA Frameworks: designed to support for appropriate clinical decision pathways and effective shared decision-making between patients and caregivers
- FasterCures Patient Perspective Framework:
 - Understanding value from a patient perspective along five domains: patient preferences, patient-centered outcomes, patient and family costs, quality and applicability of evidence, usability and transparency
 - Can be deployed as a shared decision-making tool or to incorporate patient-centered domains into existing value frameworks

❖ Foundations for value-based payment models

- Implicit or explicit value framework
- Limited applications so far beyond key quality or outcome measures, utilization, and costs

ISPOR Special Task Force: Potential elements of next generation value assessment

- Unmet need
- Rare diseases
- New mechanism/potential for clinical extensions
- Equity: does a new technology provide improved benefits for populations that historically have worse outcomes?
- Labor Productivity
- Better Adherence
- Better Targeting
- Contagion and fear of contagion
- Financial protection value
- Value of hope/some potential for cure
- Option value: value of opportunity to benefit from future treatment

Obstacles to uptake and impact of value frameworks

❖ **Conceptual Validity and Acceptance**

- Intended Purpose/Audience
- Elements of Value
- Methodology to Determine Value

❖ **Data Availability**

❖ **Reliable Measures and Evidence Based on Data**

❖ **Feasibility + Expected Magnitude of Impact**

Precedence for explicit “contextual considerations” of a broader range of value dimensions: ICER examples

- Intervention addresses unmet need (conditions of high severity affecting quality of life and/or high lifetime burden of care)
 - *Example: ICER analysis of vortigene neparvovec (inherited retinal blindness) notes the long-term benefits are significantly higher for children with blindness versus adults.¹*
- Intervention is the first to offer any improvement for patients with this condition
 - *Example: Drugs treating orphan diseases such as Duchenne muscular dystrophy.²*
- Significant improvement in reduction of side effects
 - *Example: Next-generation hepatitis C virus (HCV) direct-acting antiviral (DAA) drug regimens reduced treatment time and severe side effects.³*
- Significant uncertainty about the magnitude or durability of long-term benefits
 - *Examples: Newly-emerging gene therapies approved on expedited pathways.⁴*

ISPOR Special Task Force: Potential elements of next generation value assessment

- Unmet need
- Rare diseases
- New mechanism/potential for clinical extensions
- Equity: **Unmet/poorly-met need for population subgroups with historically worse outcomes**
- Labor Productivity: **Depression treatment for employed individuals**
- Better Adherence: **Long-acting antipsychotics**
- Better Targeting: **Some genetic tests for cancer susceptibility (can be co-labeled/marketed)**
- Contagion and fear of contagion: **Ebola vaccine; antimicrobials for resistant organisms**
- Financial protection value: **High-cost conditions (with potential for risk selection)**
- Value of hope/some potential for cure: **early gene therapies for inherited retinal blindness; current treatment for Duchenne Muscular Dystrophy**
- Option value: value of opportunity to benefit from future treatment

Enhanced value assessments to inform coverage and treatment decisions: Showing that additional dimensions of value have real-world impact

- Real-world data analysis for better evidence is increasingly feasible
- Can be supported by coverage decisions in cases where significant uncertainty exists about potential benefits and risks, but general agreement that better evidence could improve care
- Example: CED for TAVR
 - CMS provided relatively broad initial TAVR coverage in conjunction with requirement to submit patient data to a registry for outcomes analysis, in conjunction with demonstration of sufficient infrastructure to perform TAVR procedures.¹
 - Meta-analyses have found that TAVR procedures have improved outcomes relative to SAVR² and have failed to detect significant reductions in access to TAVR due to procedure volume requirements.³
- Example: EMA/IHI adaptive coverage pilots

Challenges to development of real-world evidence

- Data consistency/reliability and interoperability
- Determining causal relationships vs associations
 - Pragmatic trials
 - Methods issues for observational studies
- Methods transparency
- Study governance

Recent legislation directs FDA to explore further uses of RWE within the regulatory framework

Prescription Drug User Fee Act VI

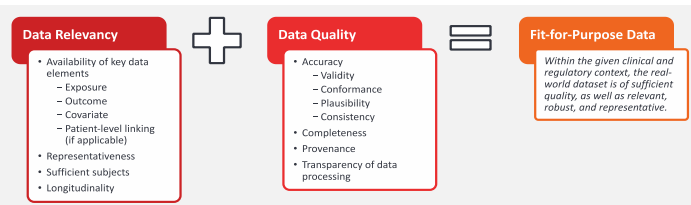
- Requires FDA to enhance use of RWE for use in regulatory decision-making
- FDA must:
 - Hold a public workshop with key stakeholders (e.g., patients, industry, academia) by the end of 2018
 - Initiate (or fund) activities (e.g., pilot studies or methodology development projects) aimed at addressing key concerns and considerations in the use of RWE by the end of 2019
 - Issue draft guidance by the end of 2021

21st Century Cures Act

- Requires FDA to establish a program to evaluate the potential use of RWE to:
 - Help support the approval of **new indications for an approved drug**
 - Help support or satisfy **post approval study requirements**
- FDA must issue:
 - A draft framework for this program by the end of 2018
 - Draft guidance by the end of 2021

Improving Availability of Real-World Data and Evidence to Support Application of Value Frameworks

- Improving RWD relevancy and quality



As value assessments have growing potential to rely on Real-World Data, key challenges include ensuring sufficient data quality while also reducing the cost and potential provider burden of developing fit-for-purpose datasets – and applying valid methods for assessing value from such data

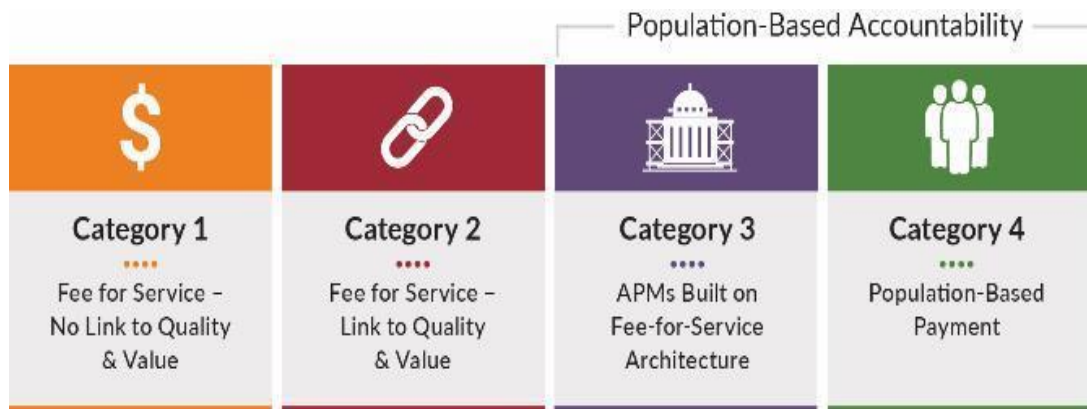
- Building and leveraging existing infrastructure for RWD Sharing



Future Directions for Value Assessment: Importance of Care System Context

- Effective treatments for unmet health needs often increase costs, at least in their early years with patent protection and limited competition – and consideration of additional dimensions of benefits may increase costs further.
- But the care systems in which technologies are used may significantly increase their benefits and reduce their costs, through such features as:
 - Innovations to better target use of medical technologies to patients who will benefit
 - Wireless/ remote personal health tools and supports, telemedicine
 - Lower-cost methods of treatment or sites of care
 - Better care coordination for team-based care
- Applying value frameworks to *systems* of care rather than technologies in isolation could help improve access and outcomes, and advance the transition to “value-based” care systems

Payment reform to sustain new care models– framework from US Health Care Payment Learning and Action Network



Types of Accountable Care Payment models

Episode Based

Payment linked to quality and cost for a specified episode of care

Examples:
 Elective procedure episodes
 Hospital admission episodes
 Diagnosis-based episodes (e.g., pregnancy, back pain)
 Chronic disease episodes (e.g., CHF, cancer)

Whole Patient

Payment linked to quality and cost for a specified population

Examples:
 Accountable care organizations
 Medical home with pop. health accountability
 Comprehensive care for high-risk patients
 Specialty-based care teams with accountability
 Capitated care with pop. health accountability

Framework for Value-Based Payment for Drugs and Devices

	Evidence-Based Price Tied to expected value	Outcomes-Based Contracts Tied to patient/population outcomes	
Category 1	Category 2	Category 3 <i>Limited shift from FFS</i>	Category 4 <i>Substantial shift from FFS</i>
FEE-FOR-SERVICE	LINKED TO PRIOR EVIDENCE	3A: LIMITED OUTCOME-BASED REBATE	4A: PARTIAL POPULATION-BASED
<ul style="list-style-type: none"> Vast majority of drug volume Low co-pay generics Formulary tiers and utilization review to target use of more costly drugs 	<ul style="list-style-type: none"> Indication-specific pricing based on available evidence for new products Entresto – Novartis/Cigna and Aetna Effient – Eli Lilly/Humana 	<ul style="list-style-type: none"> Symbicort - Astrazeneca/UPMC Repatha - Amgen/Harvard Pilgrim Repatha - Amgen/CVS Praluent - Sanofi-Regeneron/Cigna 	<ul style="list-style-type: none"> May include partial PMPM tied to performance for a population None
		3B: MEANINGFUL OUTCOME MEASURES	4B: Fully Population-Based
		<ul style="list-style-type: none"> May include measures such as clinical and cost outcomes 	<ul style="list-style-type: none"> May include a subscription model: PMPM linked to outcomes across a population

Conditions for impact from outcomes-based payments

- Promising, potentially large but uncertain benefit and cost impacts in practice
- Uncertain longer-term impacts
- Potential for treatments and their applications to improve with experience and support from payments focused on value
- Potential for reliable performance measurement and linking product to outcomes
- Alignment with value-based payment reforms for health care providers and value-based insurance design reforms for patients

Future of value assessment in era of value-based pricing

- Reduced need for prior authorization, high copays with accountable provider systems – link to value-based insurance design
- Role of value assessment in subscription models
- Shift to consideration of impact of product in care systems
 - Shift from emphasis on incremental cost efficacy to true incremental cost effectiveness, including capacity of product developers/health care providers to integrate product
 - Evidence relevant to health care systems impact

Some general principles to enhance trust/support for implementing and sustaining value frameworks

- Patient Centricity – But Consider Societal Perspective
- Clarity of Purpose
- Stakeholder Engagement – Including Patients
- Transparent Methods
- Captures Important Outcomes – Including Long-Term Outcomes and Impactful Outcomes Not Traditionally Measured
- Ability to Adapt: Variations in Preferences, Evidence
- Evolutionary Path – Start with Most Important Dimensions and Technologies
- Synergistic with Other Policies to Improve Care and Evidence
- Dynamic- Consider Likely Impact of Market Policies/ Competition in Future
- States Likely to Consider Budget Impact- and Respond to Value Frameworks that Limit Need for Early (and Uncertain) “Investments” with Potential Payoff Later

Increasing the Impact of Value Frameworks

- Further Development, Broader Engagement, and Broader Support of Frameworks
- Support for Better Evidence Development
- Pilot Applications Involving Value Frameworks To Assess Impacts
 - Quality of Patient Decision-making
 - Clinical Guidelines
 - Refined Pricing/Coverage Determinations
 - Value-Based Drug/Device Payment
 - Alternative Payment Models Incorporating Drugs or Devices
 - Accounting for outcome improvements