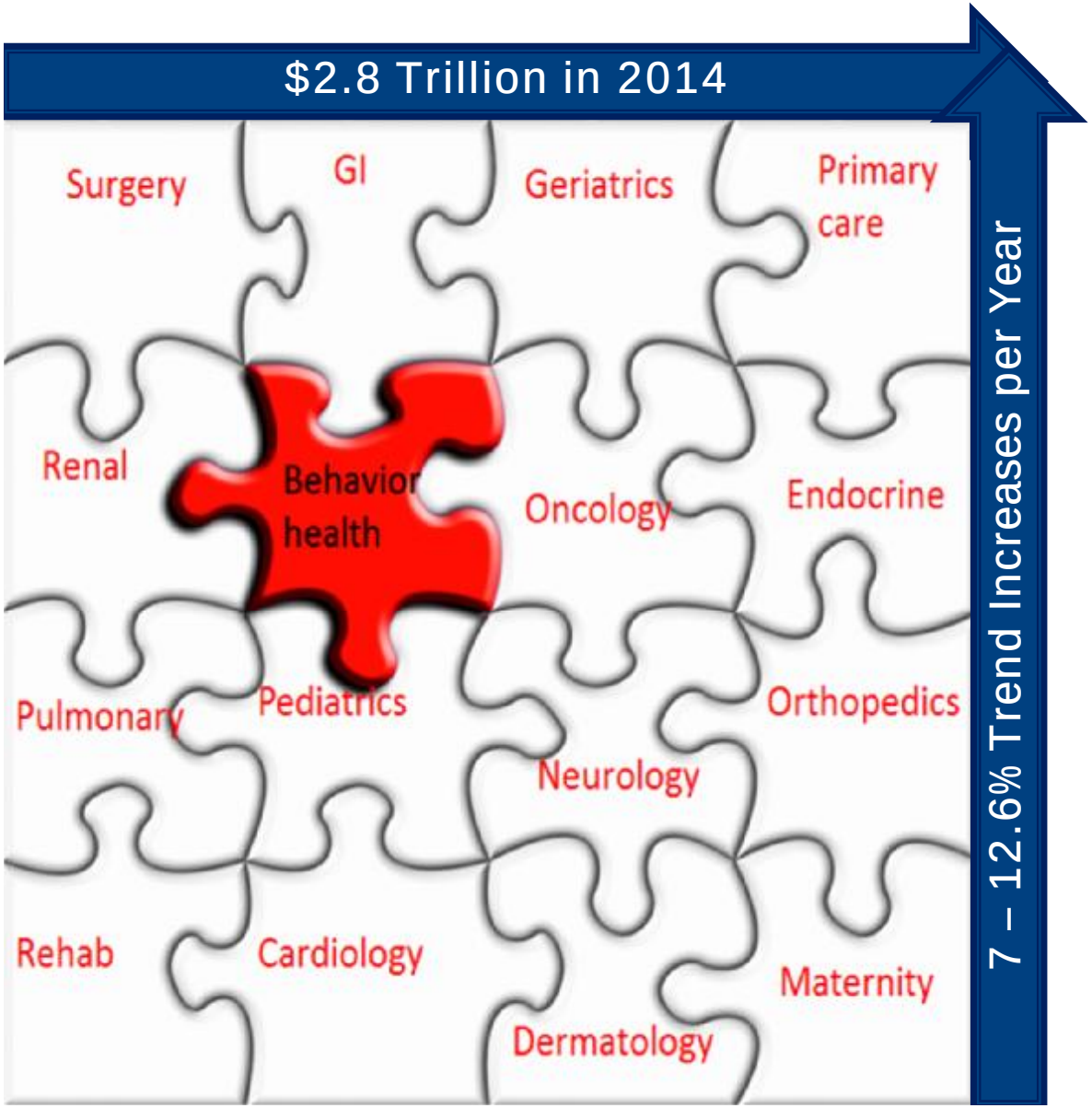
Multimodal Therapies for Brain Disorders
A Payer's Perspective

Rhonda J. Robinson Beale. M.D.
SVP and CMO
Blue Cross of Idaho

Major Dilemma: Unsustainable Health Care Cost

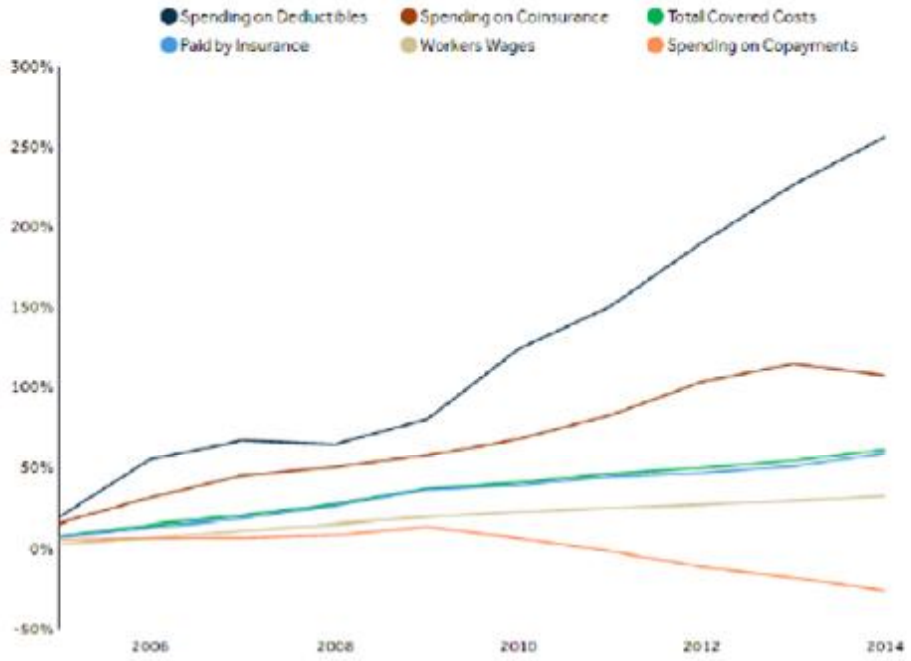


- Health care cost increases are not tolerable.
- Premium increases are not tolerated by the public.
- Drivers of cost linked to:
  1. Practice Variation
  2. Uninformed Preference
  3. Supply/ Demand
  4. New Technology

Long-term trends consistently show an increase in member costs and a simultaneous reduction in benefits

Average deductible spending rises while average copayment spending falls, 2004-2014

Cumulative increases in health costs, amounts paid by insurance, amounts paid for cost sharing and workers wages, 2004-2014



Source: Kaiser Family Foundation analysis of Truven Health Analytics MarketScan Commercial Claims and Encounters Database, 2004-2014; Bureau of Labor Statistics, Seasonally Adjusted Data from the Current Employment Statistics Survey, 2004-2014 (April to April).

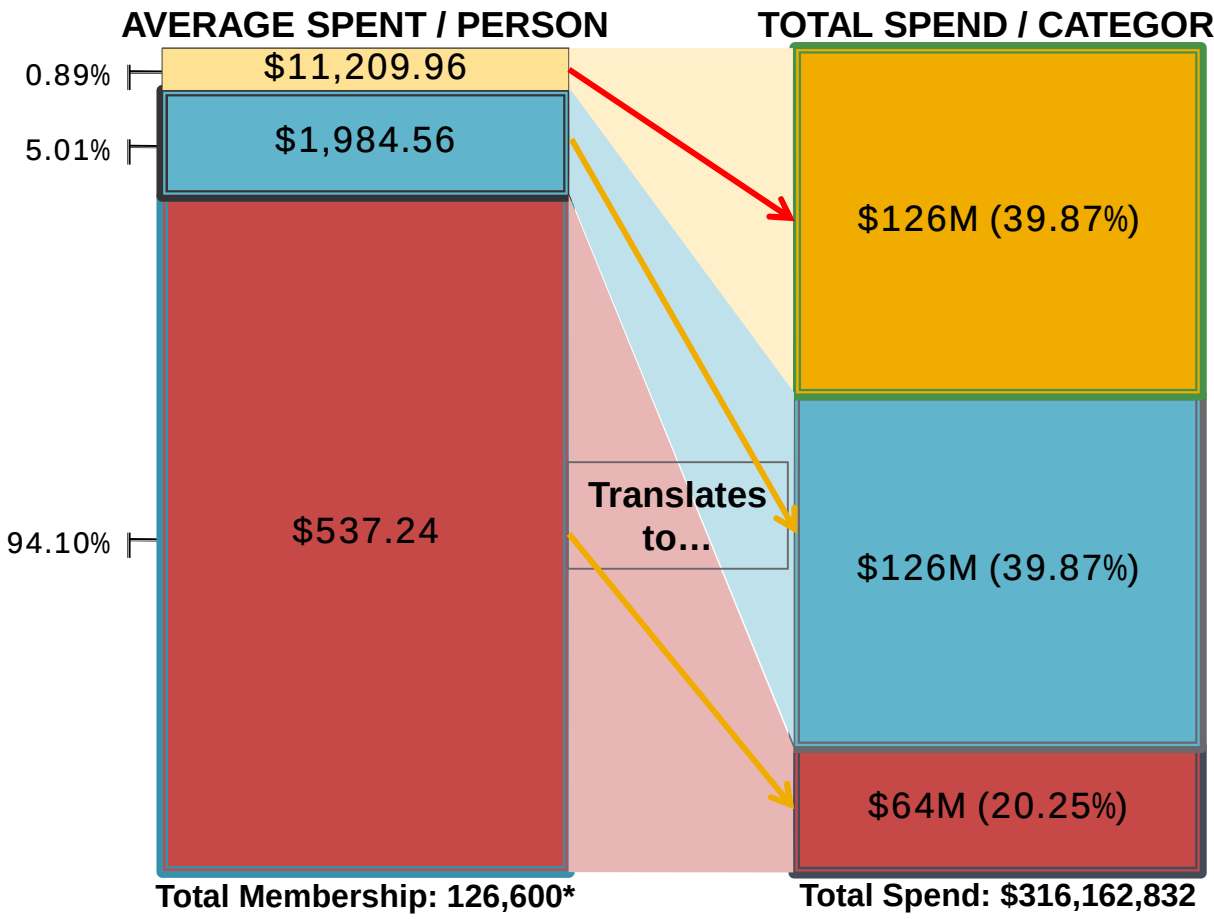
Rising Health Insurance Costs Reduce Take-home Pay

From **2000-2013**, overall health insurance costs for workers increased **66%**. This increase left less money for take-home pay — which increased only **3%**.

Source: Addressing Rising Health Care Costs: A Resource Guide for Consumer Advocates, ConsumersUnion

High Cost Threshold & Drivers Have Changed

- The old 20/80 rule, 20 % of the population drives 80% of the cost, has changed.
- Now within the Exchange population only 6% of the population drives around 80% on the cost.



* *All* members, active and termed

Cost Is Driven by Highly Complex Care and Technology

Gene Therapies -

- *Currently there are more than 320 ongoing gene therapy clinical trials that are regulated by the Food and Drug Administration (FDA). The FDA's mission is to advance the public health by helping to speed innovations that make medicines safer and more effective. The diverse gene therapy products in clinical development for a wide range of disorders offer an opportunity for the FDA to fulfill that mission.*
- *Estimation of cost per patient >1,000,000*

Combination Drug Therapies –

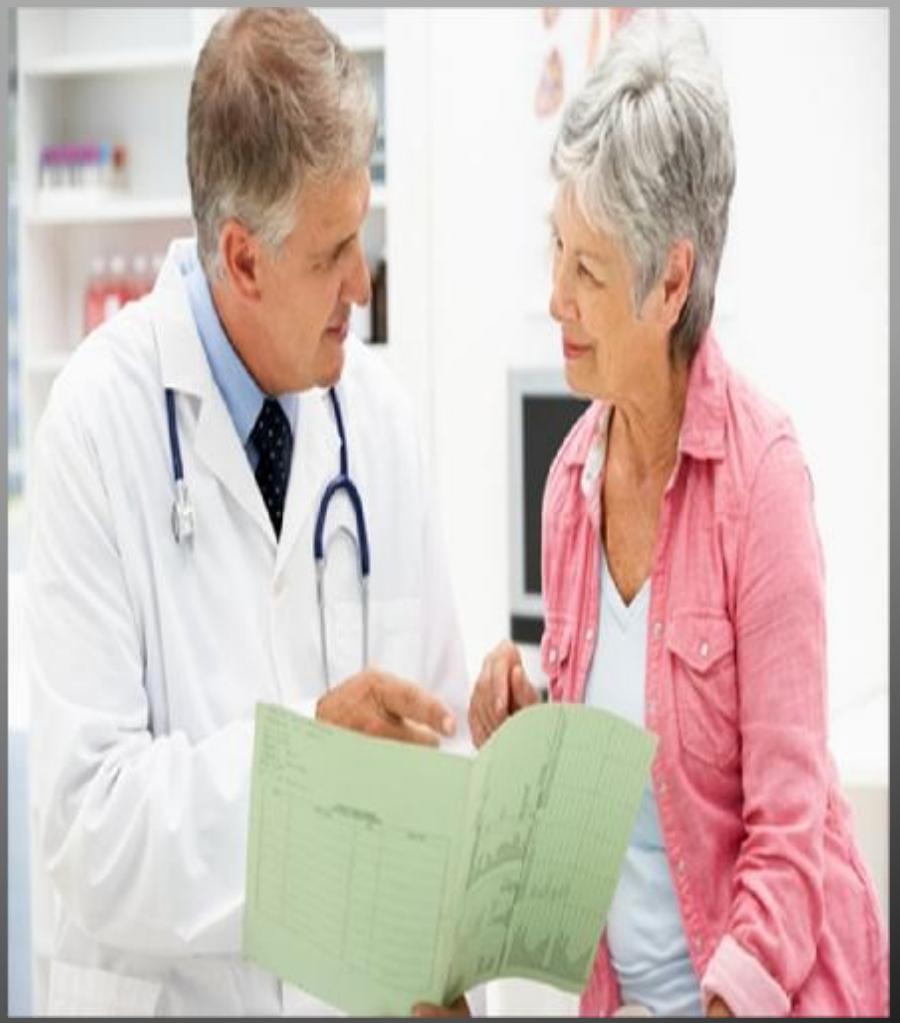
- *"Last year, the U.S. Food and Drug Administration approved the first immunotherapy combination, Bristol-Myers' Opdivo and Yervoy, to treat melanoma. It costs more than $250,000 a patient for the first full year of treatment".*

- *"AbbVie Inc. and its partner companies are testing a combination of the drugs Imbruvica and Venclexta to treat a form of leukemia. For their currently approved uses, Imbruvica costs at least $116,000 a year and Venclexta costs $109,500 for the first year of treatment".*

Ethical Issues Arise When Cost of is Too High

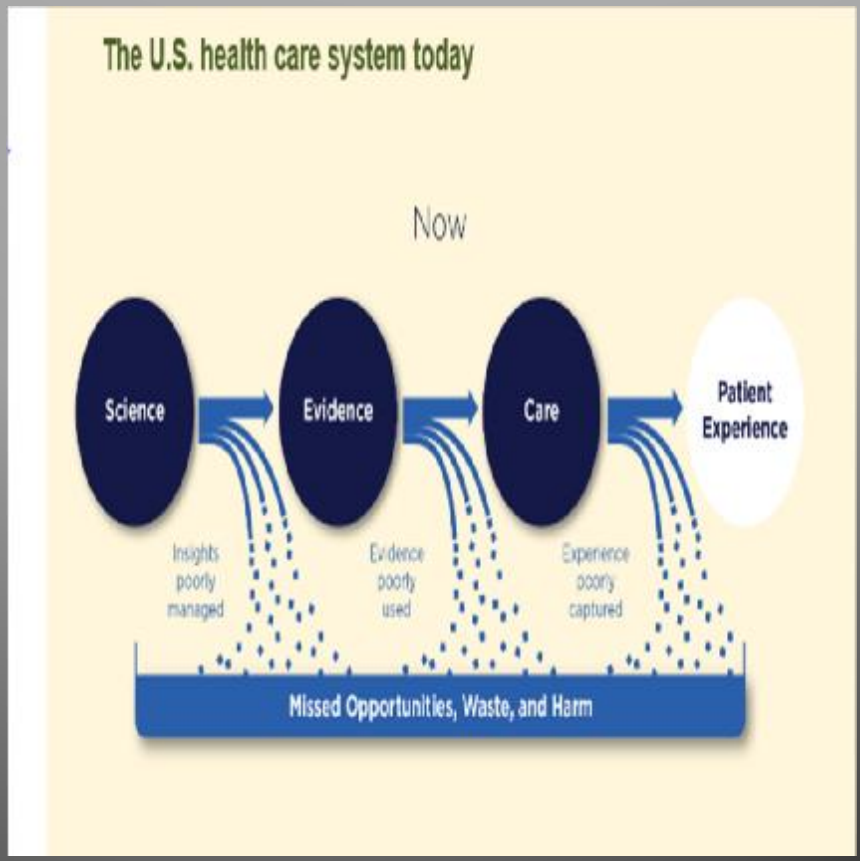
High cost of medical care is forcing difficulty decisions to the fore front for providers and patients.

- Decision regarding the utility of extension of life with the use of a extremely high cost treatments become an ethical dilemma for payers, patients and providers.
- In the brain health space the issue is different.
- Risk of mortality may be smaller but the risk of life time disability is tremendous



Current Treatment System is Set Up to Standardly Waste Resources

"Excess and waste estimates suggest that perhaps one-third of all health care spending is excessive and unnecessary, draining resources for expanded coverage, preventive care, and other important societal priorities"



IOM Best Care at a Lower Cost 2013

"Medical Necessity " Defined

- "Medically Necessary" or "Medical Necessity" means health care services that a Physician, exercising prudent clinical judgment, would provide to a patient for the purpose of evaluating, diagnosing or treating an illness, injury, disease or its symptoms, and that are:
  - (a) in accordance with generally accepted standards of medical practice;
  - (b) clinically appropriate, in terms of type, frequency, extent, site and duration, and considered effective for the patient's illness, injury or disease;
  - (c) not primarily for the convenience of the patient or Physician, or other Physician, and
  - (d) not more costly than an alternative service or sequence of services at least as likely to produce equivalent therapeutic or diagnostic results as to the diagnosis or treatment of that patient's illness, injury or disease.

- For these purposes, "generally accepted standards of medical practice" means standards that are based on credible scientific evidence published in peer-reviewed medical literature generally recognized by the relevant medical community, Physician Specialty Society recommendations, the views of Physicians practicing in relevant clinical areas and any other relevant factors.

Medically Necessary as a Bases for Insurance Coverage

- a term that describes the criteria used to determine which services will be covered by an insurance product and therefore added to the cost pool.
- Based on:
  - demonstration of comparative effectiveness,
  - generally accepted medical practice backed by evidence,
  - reproducibility with fidelity, and
  - important in achieving preservation of life and a reasonable level of functionality.
  - not more costly than an alternative service or sequence of services at least as likely to produce equivalent therapeutic or diagnostic results as to the diagnosis or treatment of that patient's illness, injury or disease.

Current Evidence Sources for Determining Medical Necessity

- Scope of evidence used for establishing Med Nec –
  - New technology reviews –
    - reviews, evaluates and ranks research to determine if the effectiveness of pharmaceuticals/technology is proven/established
  - Explicit practice guidelines from credible subspecialty organizations using IOM-like methodology
  - Credentialing/privileging criteria, from research protocols or device training manuals, for assuring competency and fidelity of treatment delivery by the clinician and/or device.

Typical Gaps in the Evidence

- Detailed demographic details of the responsive population(s).
  - Populations not researched
  - Gradation of response by other demographic groups
  - Application creep/off label use/practice experimentation.
- Routine clinical trials including testing against standard treatments as a part of pre-FDA review.

  Comparative cost benefit analysis not done
- Long term and post clinical trial information sporadically available.

Typical Gaps in the Evidence

- Dosages and ideal duration of treatment to achieve initial response and remission and the corresponding quantitative measurement.
- Routine clinical trials including testing against standard treatments as a part of pre-FDA review.
- Comparative cost benefit analysis.
- Long term and post clinical trial information sporadically available.

Recommendations

- Research evidence should clearly identify the specific responsive population(s).
  - based on more specific neurological characteristics not on just DSM diagnostic classification.
- Establish clearly populations not tested or unresponsive.
- Clear delineation of drug effect, duration of effect, long term outcomes.
- For combined therapies, clear indications for use, step therapy protocol, timeline trajectory for response and remission and
- Routinely establish an after market patient registry process for high cost drugs or rush to market drugs.
- Evidence review from FDA should be practice guideline-ready. Practice guidelines should be practice -ready

Opportunities for Health Care Organizational Leadership

"Health care in America has experienced an explosion in knowledge, innovation, and its capacity to manage previously fatal conditions. Yet our heath care has also experienced poorly managed increases in complexity, unsustainable growth in costs, and shortfalls in quality and outcomes. The result is unacceptable occurrence of missed opportunities, waste, and harm to patients. Americans need a health care system that becomes more effective and efficient with each care experience."

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rrobinsonbeale@bcidaho.com

Thank You!

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