

Connecticut Bioscience Growth Council  
New England Bio  
We Work for Health CT

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# RARE DISEASE POLICY – RESEARCH & DEVELOPMENT CONSIDERATIONS

Hartford, Connecticut

September 8, 2017



# How are rare diseases treated?

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The same way as other diseases –

meaning . . .

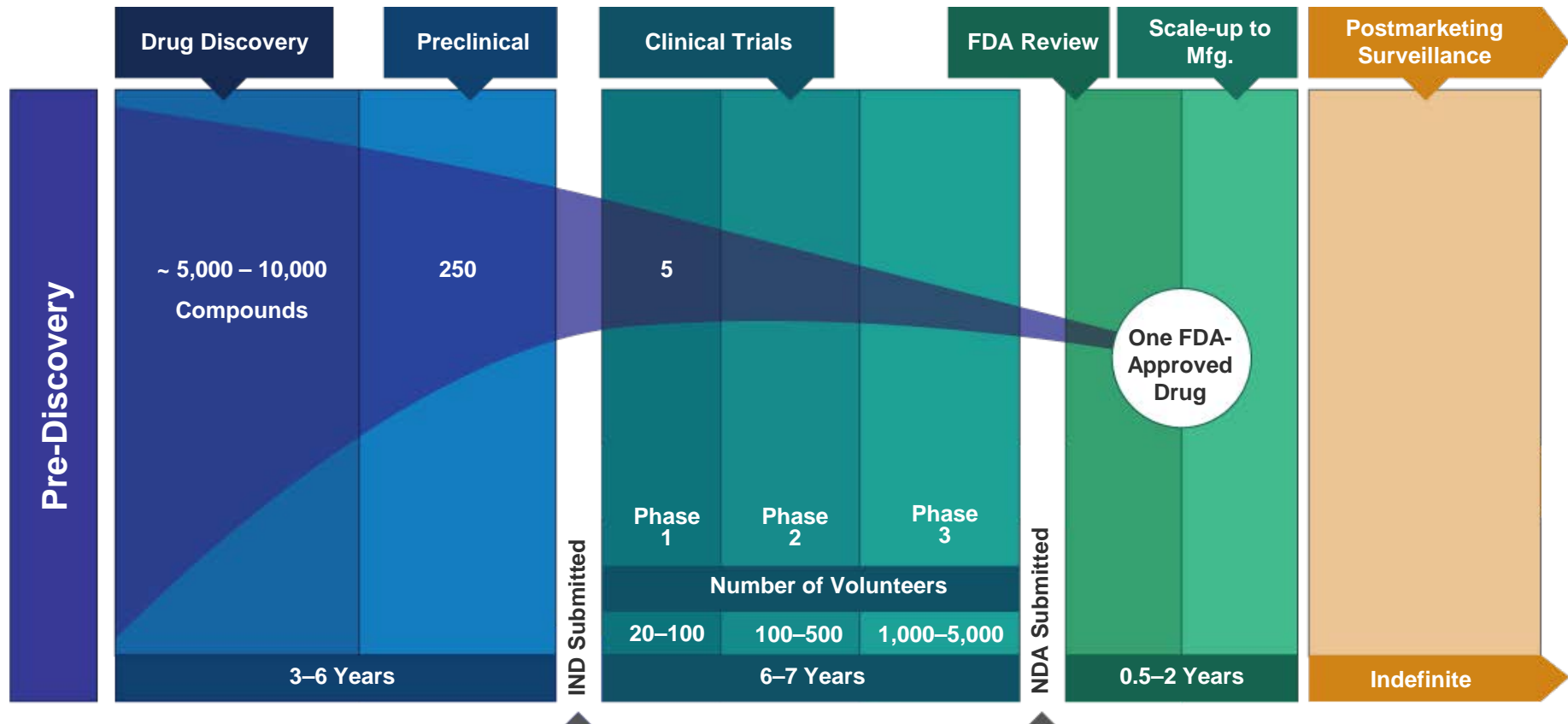


the costs and R&D burdens are the same,

*only more so*

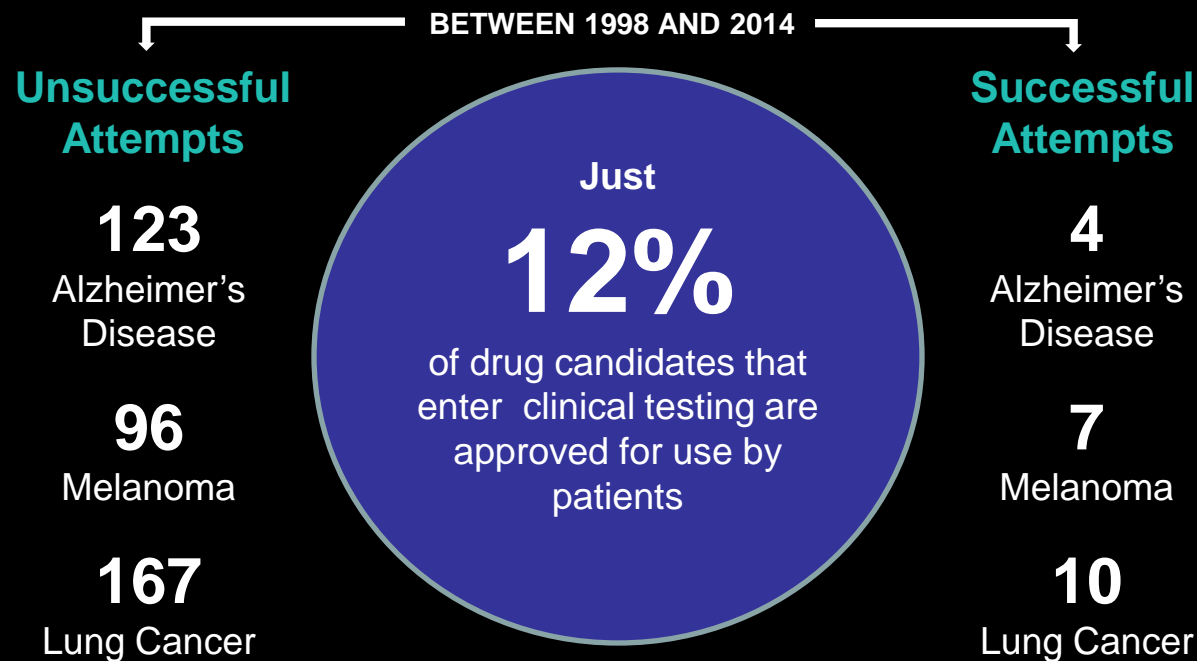
# Medicine Discovery – A Risky and Expensive Proposition

High Risk Process: 12–15 years; \$2.6 Billion



# Public Policy Should Recognize and Reward Risk Taking

On average, it takes more than  
**10 years and \$2.6B** to research and develop a new medicine.



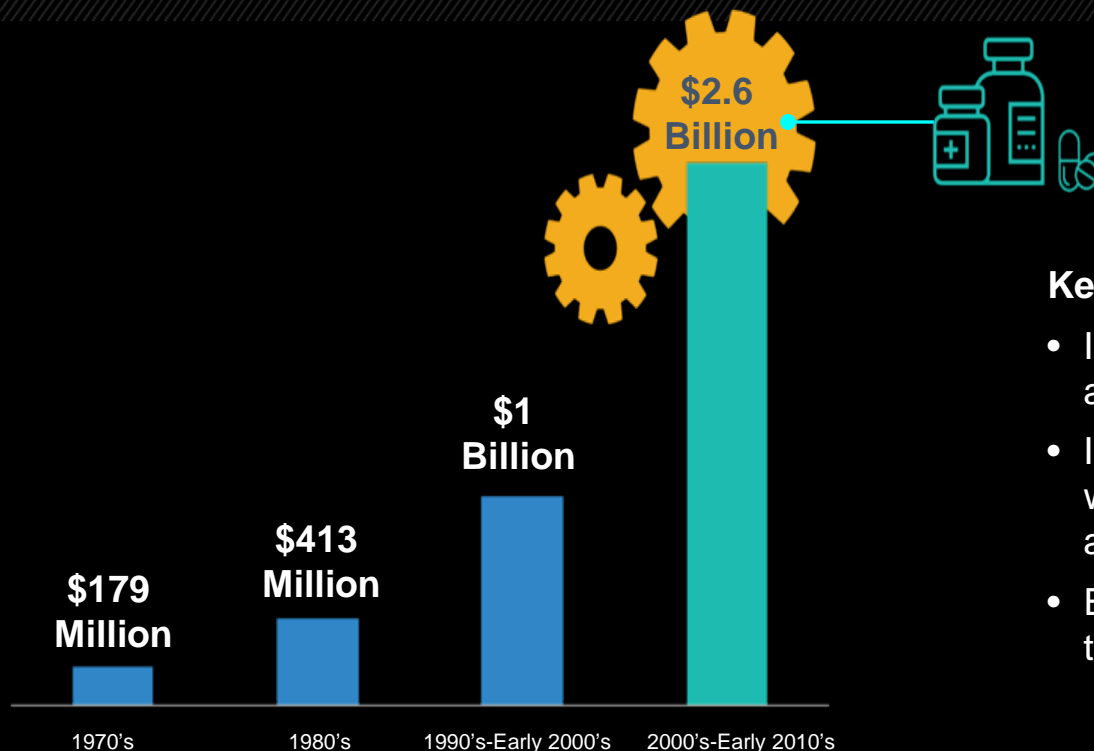
Source: Tufts Center for the Study of Drug Development (CSDD).

Source: Pharmaceutical Research and Manufacturers of America (PhRMA), "Researching Alzheimer's Medicines: Setbacks and Stepping Stones," 2015.

Source: Pharmaceutical Research and Manufacturers of America (PhRMA), "Researching Cancer Medicines: Setbacks and Stepping Stones," 2014.

# Cost to Develop a New Medicine More Than Doubled Over Past Decade

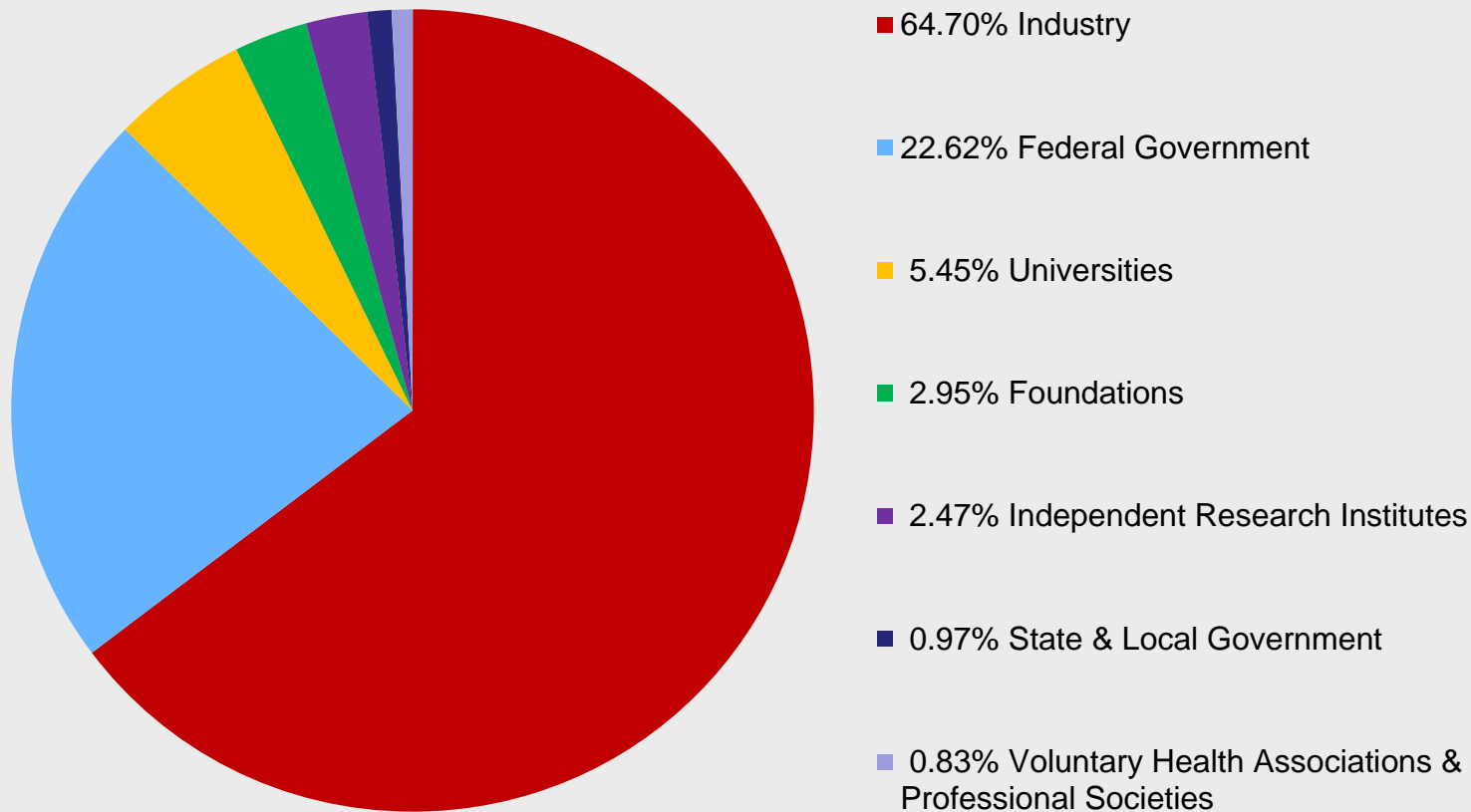
## Average Cost to Develop an Approved Medicine – Including Setbacks



### Key Drivers Include:

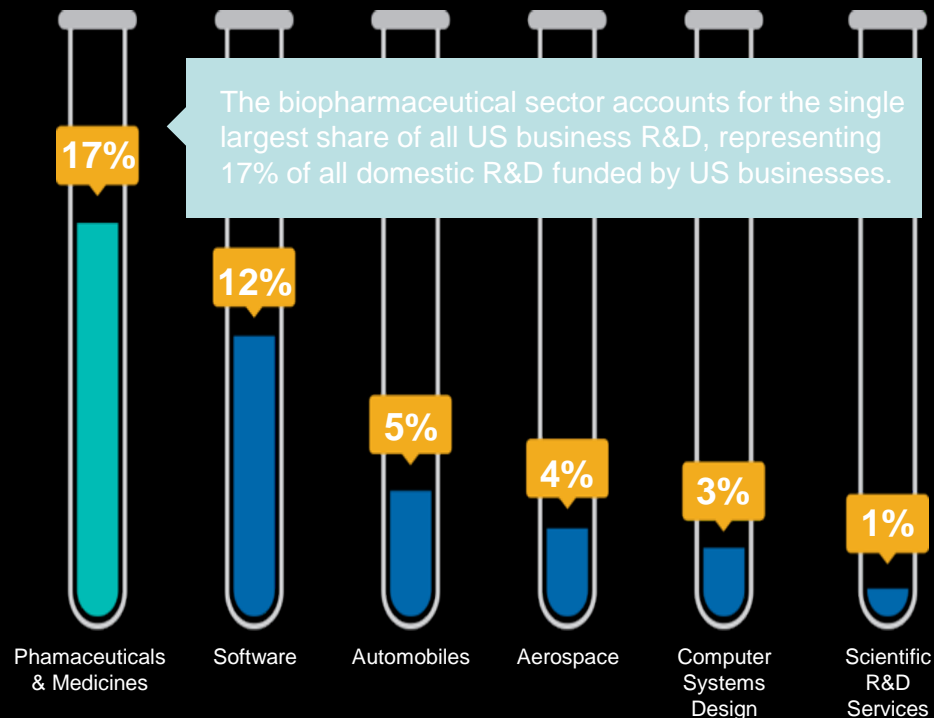
- Increased trial complexity and regulatory burdens
- Increased focus on areas where science is difficult and failure risks high
- Expanded research burden to meet payer demands

# U.S. Medical and Health R&D Expenditure, 2015



# The Biopharmaceutical Sector is the Single Largest Funder of Business R&D in The United States

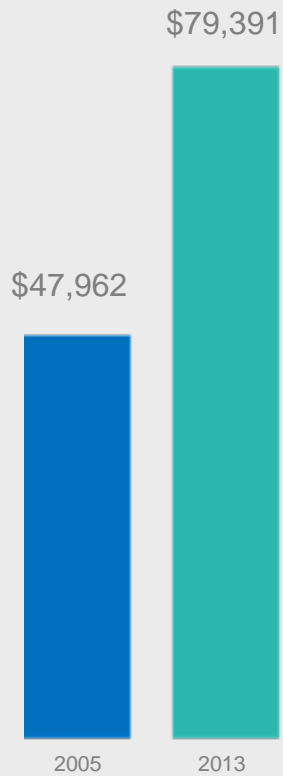
## Share of Total US Business R&D by Industry, 2014



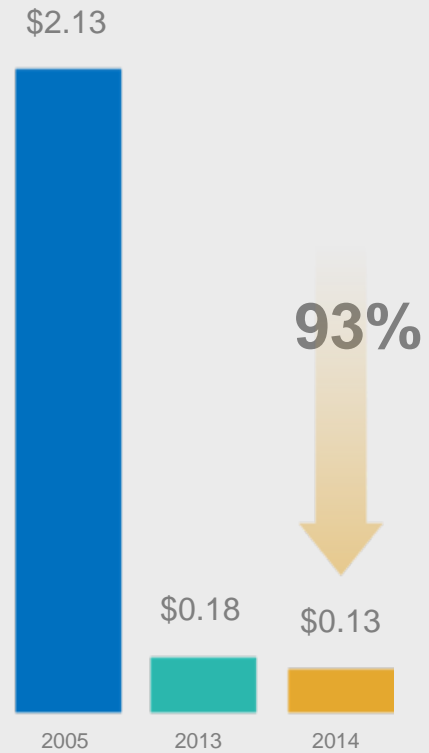
NOTE: The remaining 57% share of business R&D spending is conducted by other industries, including subsectors of the machinery sector, the electrical equipment sector, and the professional, scientific, and technical services sector.  
Source: PhRMA analysis of National Science Foundation data.

# Medicines Lower Healthcare Costs

## Percutaneous Coronary Angioplasty (PTCA)

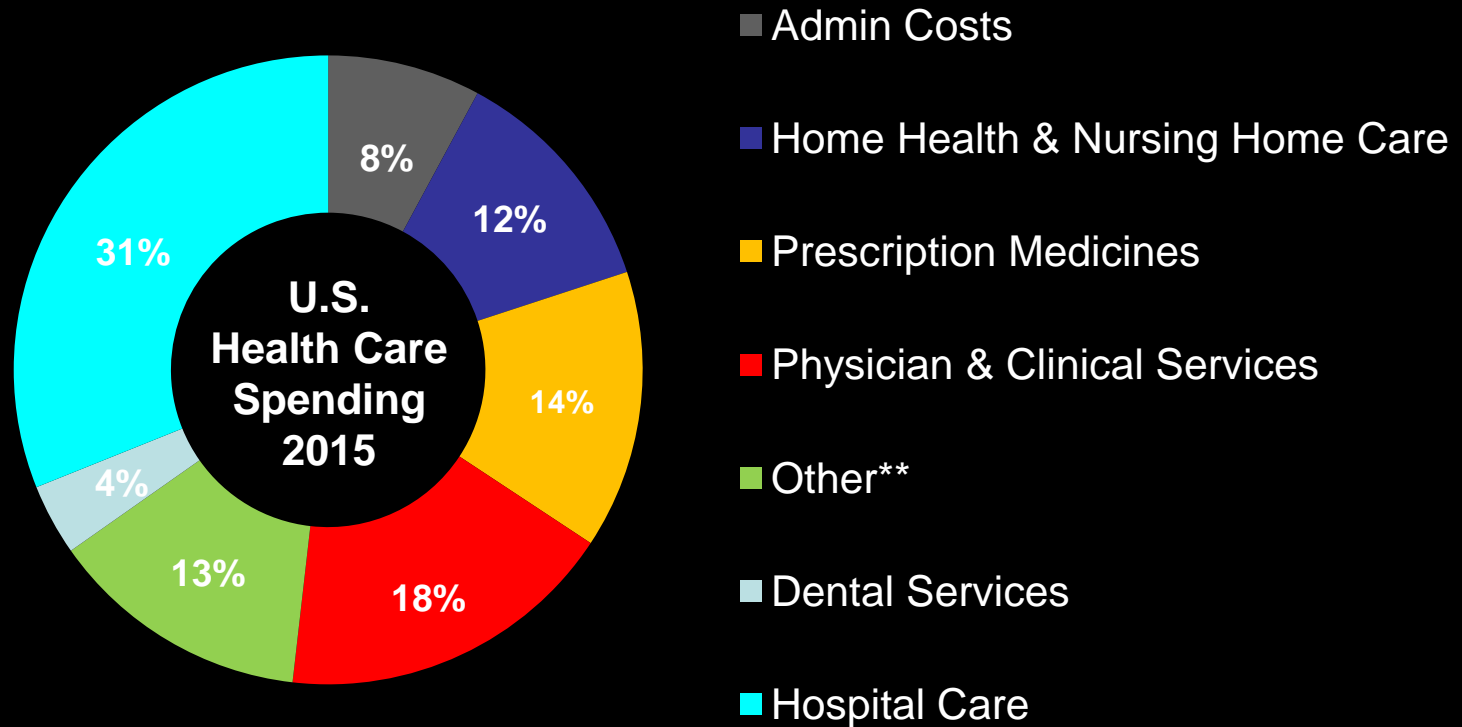


## Atorvastatin 10mg





# Spending on Retail and Physician-administered Medicines Continues to Represent just 14% of Spending

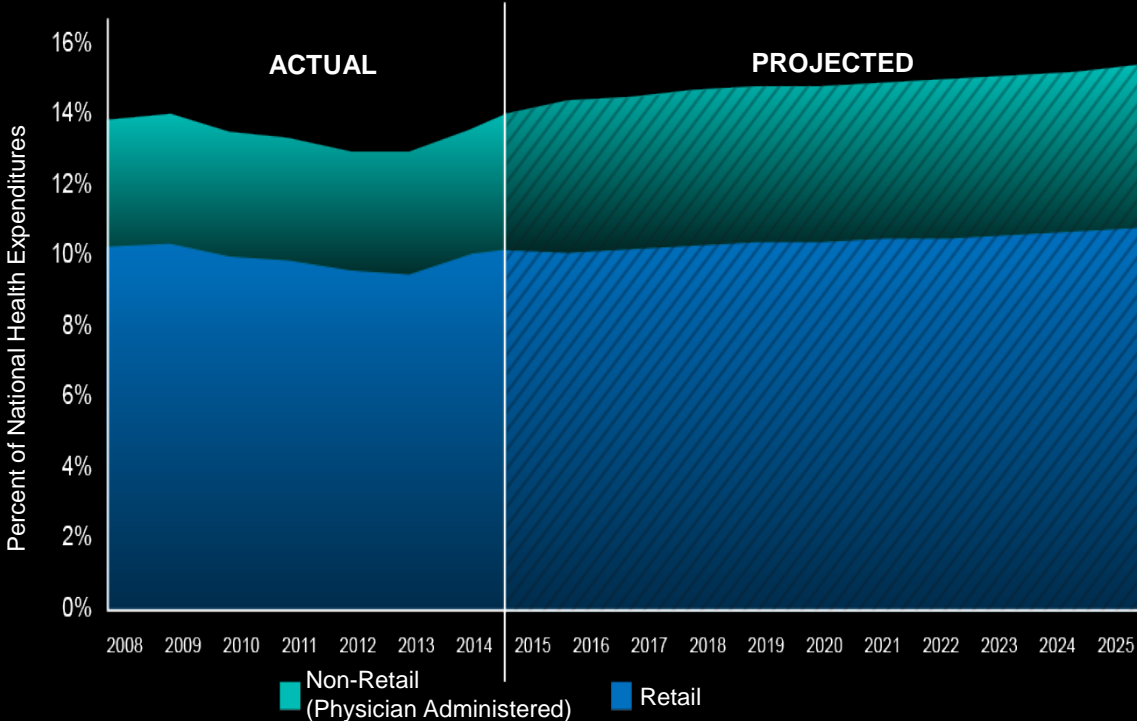


Source: PHRMA analysis of CMS National Health Expenditures data, Altarum Institute study and Berkley Research Group study.

\*\*Supply chain entities- stakeholders involved in bringing medicines from manufacturer to patient, including wholesalers, pharmacies, PBMs and healthcare provider locations.

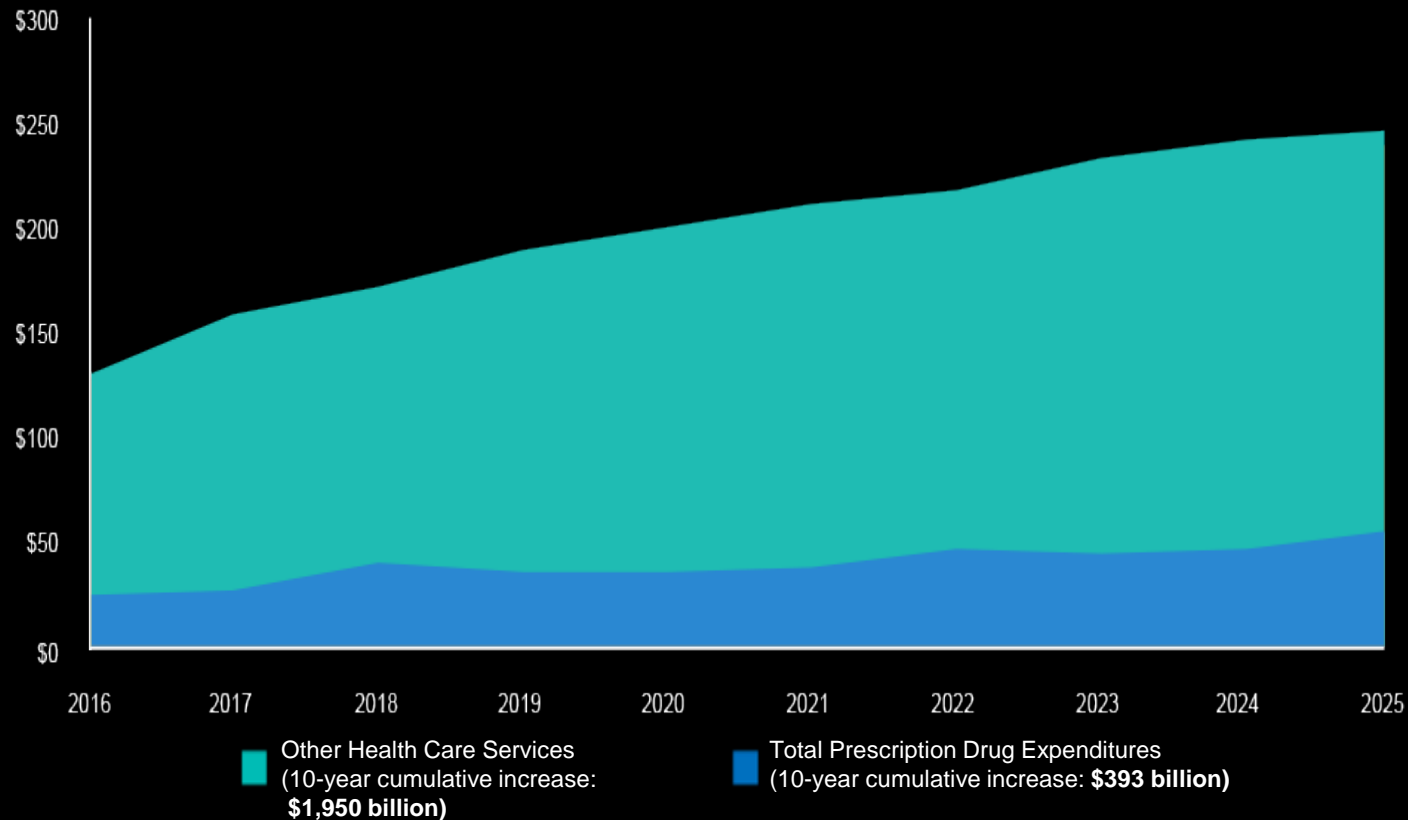
# Medicines Are a Stable Share of Health Care Spending

## Health Care Expenditures Attributable to Retail and Non-Retail Prescription Drugs, 2008-2025



Source: Altarum Institute, "A Ten Year Projection of the Prescription Drug Share of National Health Expenditures Including Non-Retail," May 2017.

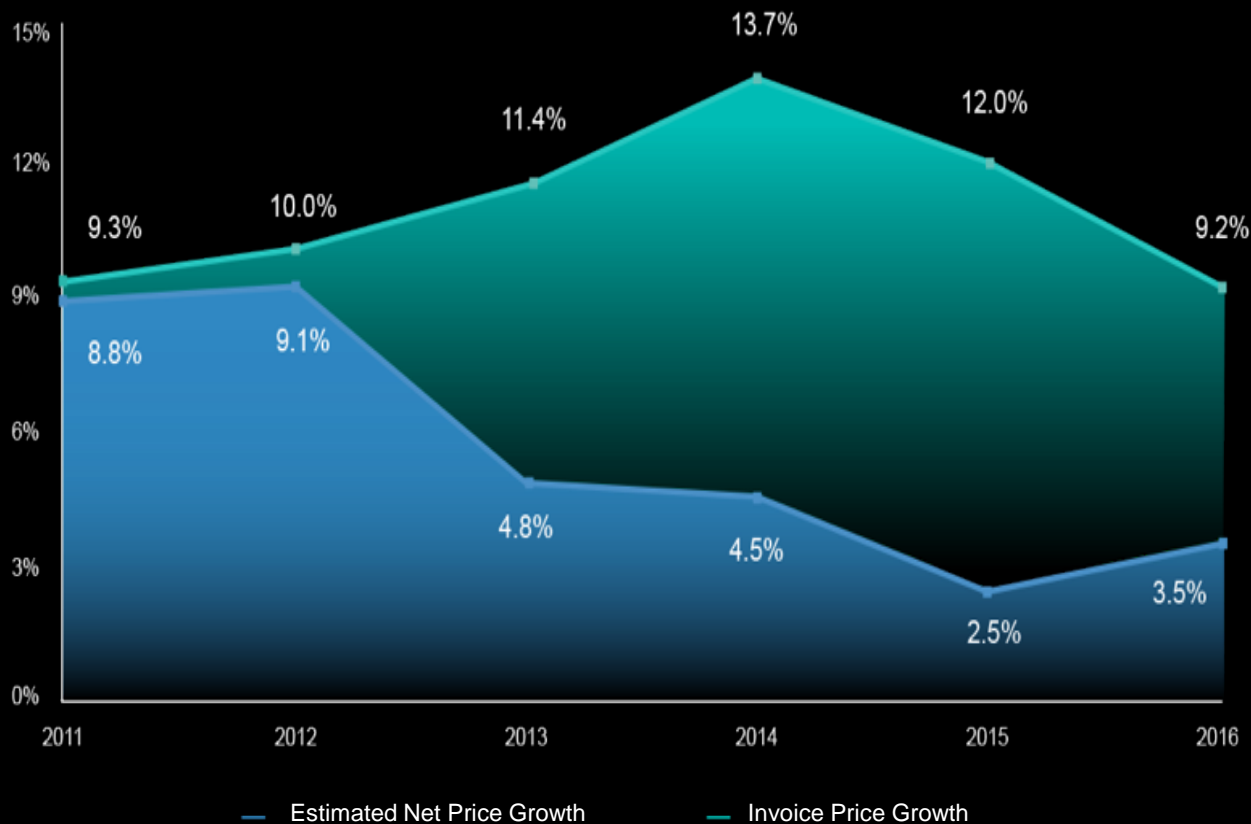
# Growth in Other Health Care Services Will Be 5 Times Total Medicine Spending Growth Through Next Decade



Source: CMS National Health Expenditures Report, July 2016.

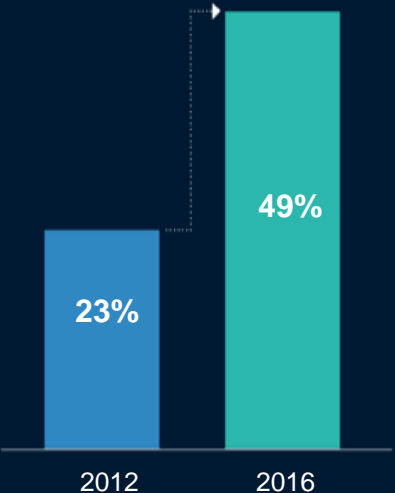
Source: PhRMA analysis of Altarum Institute, "A Ten Year Projection of the Prescription Drug Share of National Health Expenditures Including Non-Retail," August 2015.

# After Discounts and Rebates, Brand Medicine Prices Grew Just 3.5% in 2016

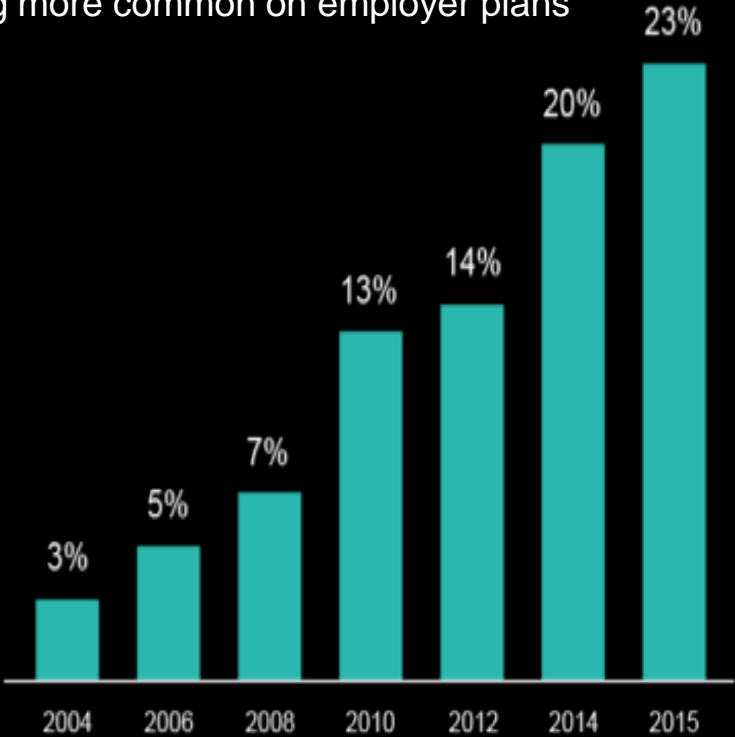


# Patients in the United States are Facing Rising Out-of-Pocket Costs and Other Barriers to Care

Percent of plans with deductibles on prescription drugs



The use of four or more cost-sharing tiers is becoming more common on employer plans



Source: PWC, Health and Well-Being Touchstone Survey, June 2016.  
Source: Kaiser Family Foundation/Health Research & Educational Trust, Employer Health Benefits: 2015 Annual Survey.

# Importance of . . .

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- R&D tax credits
- Special clinical trial protocols
- Orphan Drug Act
  - 7 year patent exclusivity after approval
- Accelerated Approval
- “Fast Tract” / “Breakthrough” designation
- Priority review

# What can we do?

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- Support policies that promote research and development
- Protect intellectual property
- Support legislation to create rare disease councils

# Consider . . .

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- Model legislation regarding payer coverage of rare diseases
- Assessing if Connecticut has a rare disease research and development cluster
- More effective communication regarding the value of rare disease R&D to broader patient populations