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

Paul R. Pescatello, J.D./Ph.D.

RARE DISEASE POLICY – RESEARCH & DEVELOPMENT CONSIDERATIONS

Hartford, Connecticut
September 8, 2017
How are rare diseases treated?

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

Drug Discovery
- 3–6 Years
- ~ 5,000 – 10,000 Compounds

Preclinical
- 250

Clinical Trials
- 5
- Phase 1: 20–100 Volunteers
- Phase 2: 100–500 Volunteers
- Phase 3: 1,000–5,000 Volunteers

FDA Review
- 6–7 Years

Scale-up to Mfg.
- 0.5–2 Years

Postmarketing Surveillance
- Indefinite

One FDA-Approved Drug

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

Just **12%** of drug candidates that enter clinical testing are approved for use by patients.

Unsuccessful Attempts

- Alzheimer's Disease: 123
- Melanoma: 96
- Lung Cancer: 167

Successful Attempts

- Alzheimer's Disease: 4
- Melanoma: 7
- Lung Cancer: 10

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

- 64.70% Industry
- 22.62% Federal Government
- 5.45% Universities
- 2.95% Foundations
- 2.47% Independent Research Institutes
- 0.97% State & Local Government
- 0.83% Voluntary Health Associations & Professional Societies
The biopharmaceutical sector accounts for the single largest share of all US business R&D, representing 17% of all domestic R&D funded by US businesses.

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

Source: Average hospital charges for Atorvastatin 10mg data adapted from HCUP Hospital Charge database 2005 and 2013. IMS National Sales Perspective (NSP) Invoice Price in 2005 (branded Lipitor), 2013 (generic) and 2014 (generic).

<table>
<thead>
<tr>
<th>Year</th>
<th>PTCA Charges</th>
<th>Atorvastatin Price</th>
</tr>
</thead>
<tbody>
<tr>
<td>2005</td>
<td>$47,962</td>
<td>$2.13</td>
</tr>
<tr>
<td>2013</td>
<td>$79,391</td>
<td>$0.18</td>
</tr>
</tbody>
</table>

93% cost reduction from 2005 to 2013.
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, PBM 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

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

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

Source: IMS Institute for Healthcare Informatics, National Sales Perspectives, March 2016.
Patients in the United States are Facing Rising Out-of-Pocket Costs and Other Barriers to Care

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

Percent of plans with deductibles on prescription drugs:

- 23% in 2012
- 49% in 2016

Importance of . . .

• 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?

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

• 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