BIO / BioMedTracker
Clinical Trial Success Rates Study

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BIO CEO & Investor Conference
February 15th, 2011
MOST RECENT STUDIES

TUFTS 2010*

• 1,738 drugs (Only Lead indications)

• Must have entered into a Phase I 1993-2004

• Developed by the 50 largest Pharmas

BioMedTracker 2011

• 4,275 drugs

• 7,300 indications (All indications)

• Any Phase during Oct 2003- Dec 2010

• All companies, Biotechs + Pharma including Private

* DiMasi, et al. Nature Clinical Pharmacology and Toxicology, May 2010
OVERALL SUCCESS RATES

Tufts ’03
Lead Indications: 21%

Tufts ’10 *
Lead Indications: 19%

BMT All Indications: 9%

BMT Lead Indications: 14.5%

BMT Secondary Indications: 3.2%

* DiMasi, et al. Nature Clinical Pharmacology and Toxicology, May 2010
CALCULATING OVERALL SUCCESS RATES
(simple compounded probabilities)

<table>
<thead>
<tr>
<th>Phase Transition</th>
<th>Phase Success</th>
<th>Phase LOA</th>
</tr>
</thead>
<tbody>
<tr>
<td>P1 to P2</td>
<td>63%</td>
<td>9%</td>
</tr>
<tr>
<td>P2 to P3</td>
<td>33%</td>
<td>15%</td>
</tr>
<tr>
<td>P3 to NDA/BLA</td>
<td>55%</td>
<td>44%</td>
</tr>
<tr>
<td>NDA/BLA to Approval</td>
<td>80%</td>
<td>80%</td>
</tr>
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</table>

\[.63 \times .33 \times .55 \times .80 = .09\]
LOWER SUCCESS WITH SECONDARY INDICATIONS AT EACH PHASE

- Lead Indications
- Secondary Indications

<table>
<thead>
<tr>
<th>Phase</th>
<th>Lead Indications</th>
<th>Secondary Indications</th>
</tr>
</thead>
<tbody>
<tr>
<td>Phase I</td>
<td>67%</td>
<td>54%</td>
</tr>
<tr>
<td>Phase II</td>
<td>41%</td>
<td>23%</td>
</tr>
<tr>
<td>Phase III</td>
<td>65%</td>
<td>37%</td>
</tr>
<tr>
<td>NDA/BLA</td>
<td>83%</td>
<td>72%</td>
</tr>
</tbody>
</table>
OVERALL SUCCESS RATES BY MOLECULE TYPE

- **Lead Indications**
  - NME: 14%
  - Biologic: 26%
  - Non-NME: 41%

- **Secondary Indications**
  - NME: 3%
  - Biologic: 7%
  - Non-NME: 10%
OVERALL SUCCESS BY DISEASE

- **Infect. Dis.**
  - Lead Indications: 15%
  - Secondary Indications: 3%

- **Endocrine**
  - Lead Indications: 14%
  - Secondary Indications: 2%

- **Autoimmune**
  - Lead Indications: 12%
  - Secondary Indications: 7%

- **Respiratory**
  - Lead Indications: 13%
  - Secondary Indications: 2%

- **Neurology**
  - Lead Indications: 12%
  - Secondary Indications: 4%

- **CV**
  - Lead Indications: 7%
  - Secondary Indications: 3%

- **Oncology**
  - Lead Indications: 11%
  - Secondary Indications: 2%
OVERALL SUCCESS AT PHASE II AND III

Infect. Dis. 41% 55%
Endocrine 36% 60%
Autoimmune 30% 63%
Respiratory 24% 61%
Neurology 32% 55%
CV 28% 46%
Oncology 29% 34%

Phase II  Phase III
OVERALL SUCCESS RATES - ONCOLOGY

- Head and Neck: 19%
- RCC: 15%
- Ovarian Cancer: 8%
- Breast Cancer: 7%
- Multiple Myeloma: 7%
- HCC: 6%
- CLL: 6%
- Prostate Cancer: 4%
- Colorectal Cancer: 3%
- MDS: 3%
- NSCLC: 2%
“IF AT FIRST YOU DO NOT SUCCEED... TRY, TRY AGAIN”

- Lead Indications
- Secondary Indications
- NME

40% 50% 60% 70% 80% 90% 100%

First Second Third Fourth Fifth

51% 64% 75% 78% 87%
Trial and Error: Breaking Down Clinical Trial Success Rates

13th ANNUAL BIO CEO INVESTOR CONFERENCE
Tuesday, February 15th
12:00 - 12:55 pm, Jade Room

Moderator:
• David Strupp, Managing Director, Rodman & Renshaw, LLC

Panelists:
• Ted Buckley, PhD, Chief Economist, Bloomberg
• Lawrence T. Friedhoff, MD, PhD, FACP, CEO, Pharmaceutical Special Projects Group, LLC
• Michael Hay, Senior Biotechnology Analyst, Sagient Research Systems
• David Thomas, Director, Industry Research & Analysis, BIO