LST Expert Meeting, May 13-14 2013
Clinical trial case studies
What has worked well and what lessons have been learned?
Reducing complexity and cost

1. design
2. set up
3. implement
4. analyze
Reducing complexity and cost

1. design
2. set up
3. implement
4. analyze
Align design with practice

Keep it simple...

<table>
<thead>
<tr>
<th></th>
<th>Practice</th>
<th>Protocol</th>
</tr>
</thead>
<tbody>
<tr>
<td>PCI patient population</td>
<td>SA/ UA/ NSTEMI/ STEMI</td>
<td>✔️</td>
</tr>
<tr>
<td>Clopidogrel loading</td>
<td>300 or 600 mg</td>
<td>✔️</td>
</tr>
<tr>
<td>Clopidogrel timing</td>
<td>After angiography</td>
<td>✔️</td>
</tr>
<tr>
<td></td>
<td>Before <em>or</em> after PCI start</td>
<td>✔️</td>
</tr>
<tr>
<td>Minutes lapsed to treat</td>
<td>0</td>
<td>12</td>
</tr>
</tbody>
</table>
Site selection matters

Pick winning sites...

<table>
<thead>
<tr>
<th></th>
<th>NEJM-1</th>
<th>NEJM-2</th>
<th>NEJM-3</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patients</td>
<td>13,000</td>
<td>18,000</td>
<td>11,000</td>
</tr>
<tr>
<td>Sites</td>
<td>707</td>
<td>650</td>
<td>150</td>
</tr>
<tr>
<td>Rate (pt/site/mo)</td>
<td>1</td>
<td>1</td>
<td>4</td>
</tr>
<tr>
<td>Enrollment period (mos)</td>
<td>26</td>
<td>29</td>
<td>20</td>
</tr>
</tbody>
</table>
God is in the details…

tape the blinded study
move the 12-lead
attach the post PCI meds to the patient ECG machine into blood sample orders to record…

tape the cath lab…

The outgoing gurney…
2. set up

Restrain data exuberance

4. analyze

Data variable counts $\alpha$ data acquisition cost

Data cost per patient

$0$

$500$

$1,000$

$1,500$

Data variables per patient

$200$

$250$

$300$

$350$

$400$

ACUITY (N=13,800)

PCI-PLAT (N=14,500)

PHOENIX (N=11,000)

REPLACE-2 (N=6,000)

$R^2 = 0.86$

$\text{Data cost} \propto \text{data acquisition cost}$
2. set up

■ Restrain data exuberance

4. analyze

Data variable counts modestly $\alpha$ queries

![Graph showing data queries per patient vs. data variables per patient for ACUITY, REPLACE-2, PHOENIX, and PCI-PLAT datasets. R$^2 = 0.33$.]
Restrain data exuberance

Trial complexity may drive costs of data + queries

- **ACUITY (N~13,800)**: $3,000
- **PCI-PLAT (N~14,500)**: $2,000
- **REPLACE-2 (N~6,000)**: $1,000
- **PHOENIX (N~11,000)**: $0

Data + query cost per patient

Data variables per patient

$R^2 = 0.77$
$6,500
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Meredith Todd
THE MEDICINES COMPANY

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