What is a cure worth?

Mark W. Skinner, JD
Gene therapy for hemophilia – future scenarios for cost, capacity, and impact on therapy
The 9th WFH Global Forum on Research and Treatment Products for Bleeding Disorders
Montreal, Canada
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## DISCLOSURES FOR:
### MARK SKINNER

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<tr>
<th>Conflict</th>
<th>Disclosure - if conflict of interest exists</th>
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<tr>
<td>Research Support</td>
<td>Baxalta, Bayer, Biogen, Novo Nordisk, SOBI</td>
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<td>Director, Officer, Employee</td>
<td>WFH USA, BloodSource,ATHN</td>
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How much are we willing to pay?

• $1,000,000
• $100,000
• $10,000
• Parity for 1, 2, 3 years treatment cost
• ???
World’s Most Expensive Medicine: Is it Worth the Price?

Is it worth $1 million to treat a potentially fatal genetic disease that causes painful swelling of the pancreas and can require repeated trips to the emergency room? Germany says it’s not sure.
The safest drug that no one can afford or that arrives too late is of no benefit to a patient.
Bringing a new discovery from lab to market: a long, expensive and risky road
## Finite target patient population

- **All Patients with Hemophilia B** – 28,500 Worldwide
- Minus those with moderate / mild disease
- Minus those with anti AAV neutralizing antibody
- Minus patients with active HCV
- Minus those with Inhibitors
- Minus those with other major disease (?)
Cell Phones & Landlines

For people in resource-poor countries, where access to factor is limited and transportation to a medical center can be a formidable challenge, the ability to undergo a single high-tech intervention that renders patients much less likely to experience life-threatening bleeding events can spell the difference between life and death.
Coming Challenges of Affordability

Typically health economic analyses fail to take a whole-of-life view omitting the cost of avoidable comorbidities, life long management of complications and economic impact over a lifetime for individual patients and their families due to loss of social, educational and career opportunities.
Answering Payer Demands

• Evidence-based evaluations of rare diseases should not be confined to traditional analysis
• Utilize new concepts including:
  - Societal willingness to pay
  - Cost savings for bleeds prevented
  - Reductions in inhibitor incidence
  - Cost to manage co-morbidities other than joint disease
  - Quality of Life improvement
Special Case of Gene Therapy Pricing

A large one-time payment for gene therapy may be the simplest approach, it is fraught with substantial practical and policy risks.

A pay-for-performance model based on the thoughtful development of efficacy metrics that can be transferred between succeeding insurers seems to present a reasonable and practical solution.
Risk-Sharing Background

- Risk sharing deals were born out of necessity as a reaction to a payer’s “no”, rather than as part of a proactive, collaborative strategy.
- Thus far, they have allowed pharma to retain confidential pricing, protecting them against the downward pressure of external reference pricing.
- Payers are increasingly interested in funding outcomes, not drugs.
- Very few, if any drugs, can prove their real world value prior to launch, reimbursement and pricing are increasingly likely to be evidence-linked.
- Risk-sharing may be the only option for high-priced / orphan drugs where the outcome is not guaranteed.
UK NICE Example – VELCADE

- VELCADE – Treatment of Multiple Myeloma, Mantle Cell Lymphoma
- Example of “No-cure, No-pay” scenario
  - Combines access with value for money
- UK NHS pays upfront but able to claim refund for non-responders
  - J&J to reimburse for drug costs for patients who fail to show a full or partial response after 4 cycles
  - Defined response according to levels of serum M protein
- Forces response rate data collection
- Lessons learned
  - Complex to administer
  - Inadequacy of data reporting systems

Adapted from METEOS PharmaDiplomacy Dialogue, Oxford UK
France Example - XOLAIR

• XOLAIR – Treatment of persistent, allergic asthma
• Example of “Real World Evidence” generation
  - Agreed to provide drug at current price
  - Novartis promised to develop evidence of the drug’s effectiveness
    • Pre-agreed price would fall if the evidence didn’t hold-up; if it did the price would be maintained
    • Collaboration on study / protocol design
  - Proactive upfront engagement between payer and mfg.
• Lessons learned
  - Significant cost all borne by mfg.
  - Risk-share agreement allowed mfg. to maintain a premium price while generating peer-reviewed RWE

Adapted from METEOS PharmaDiplomacy Dialogue, Oxford UK
Italy Example - ESBRIET

• ESBRIET – Treatment of Idiopathic Pulmonary Fibrosis
• Example of reimbursement based on limited evidence of efficacy
  - Orphan drug
  - InterMune agreed to reimburse drug costs for patients whose forced vital capacity (FVC) (measure of lung function) declined by 10% within first 6 months of treatment
  - Reimbursement trigger based on one relatively easy to collect data point
  - Forced close payer – mfg. collaboration
• Lessons learned
  - Potentially complex to administer / collect reimbursement

Adapted from METEOS PharmaDiplomacy Dialogue, Oxford UK
UK NICE Example - IRESSA

• IRESSA – Used to treat breast, lung and other cancers

• Example of a Patient Access Scheme
  - Provides budgetary certainty - AstraZeneca offers the first two months free, after which NHS pays a fixed fee per patient
  - Drug access for eligible patients
  - Drug only available directly from manufacturer
  - Encourages closer provider-pharma interaction, systematic data collection

• Lessons learned –
  - Administratively complex
  - Supply logistics complex
  - Evidence generated is limited

Adapted from METEOS PharmaDiplomacy Dialogue, Oxford UK
New Pricing Paradigms

- Risk sharing agreements
- Value-based pricing systems
- “Pay for Performance”
- Managed entry
- Conditional reimbursement
- Coverage with Evidence Development / Generation
  - Real world evidence