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ESTIMATING THE COST OF PHARMACEUTICALS MANAGING COST AND EXPECTATIONS

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Outline

- Background
- Life Cycle Phases
- Estimating Cost
- Risk and Uncertainty
- Managing Costs and Expectations
- Summary

Background

- Pharmaceutical product pricing has been catching the headlines in recent history
- Many factors go into the price, cost of goods sold is just one
  - Drugs are typically a chemical process and found in tablet form
  - Biologics are manufactured with live/killed organisms/viruses and a more complicated than tablets
  - Lots of negotiations with insurance
  - Investor or stockholder returns
- Estimating the cost to manufacture is relatively straight forward
- The approach is the same for either a commercial or federal product
- Risk and uncertainty are big drivers for cost
- Managing expectations is key to managing costs

Life Cycle of Pharmaceuticals

- Research and Development
  - Discovery/Science and Technology
  - Development
  - Advance Development
- Production/Manufacture
- Operation and Sustainment/Market
- Phase 4 – Post Marketing Surveillance
  - Follow up
  - Tracking any adverse reactions
  - Filling required FDA reports
  - Potential for additional use/indications

Estimating Cost

- Throughout the entire life cycle process the steps involved are well known and easily definable.
- Estimating approach
  - Typically estimate from detailed build up
  - Few if any CERs
    - Companies will not share
  - Analogies are useful
  - Plug and play models

Estimating Cost

- RDT&E
  - Early on it is more art than science
    - Cannot plan the outcomes of science, but you can plan the steps of the research
  - Mid-phase RDT&E includes:
    - Non-clinical studies
    - Developing desired clinical outcomes
    - Looking for scalability
  - Late RDT&E involving clinical trials are well defined, and defined by both the FDA and manufacturer
  - License application fees
    - Who will hold the license
    - Direct cost or pass through

Estimating Cost

- Production
  - Both drug and biologics are defined as they come out of RDT&E – License approval
  - Manufacturing is very well defined for estimating
    - Materials
    - Labor
    - Processes
  - Insurance and indemnification
- Phase 4 – Post Marketing Surveillance
  - Study the long-term risks and benefits of using the drug and to discover any rare side effects
  - Annual and facility licenses
  - Long term production
    - Market demand
    - Warm base
  - Required Reporting

Assessing Risk

- RDT&E
  - Big difference between federal and commercial is cutting less promising solutions faster
    - Commercial entities meet weekly to review product pipeline and cull those that appear to be developing slowly
    - Federal decision process will allow products to move along longer and farther through the pipeline before a decision to stop is made
  - Risk is going from bench top to full scale manufacturing
    - Size matters in biologic products
  - Clinical trials
    - Safety is paramount
    - Appropriate sizing of cohorts
    - Engineering runs at full production volumes
    - Ensuring that product meets specified outcomes

Assessing Risk

- Production
  - Maintaining:
    - SOPs
    - Training
    - Cleaning to required standards
  - Making sure product is up to standards
  - Facility is maintaining its standards
  - Establishing appropriate volume
  - Determine how to address warm base if needed
    - Federal products
    - Orphan drugs

Uncertainty

- FDA
  - Changing type of product
    - Device
    - Biologic
    - Drug
  - Can seem elusive in agreeing to processes for conducting trials non-clinical and clinical
    - Does not dictate outcomes
    - Does not stipulate size clinical cohorts to prove outcomes
    - Will hold discussions to help bound, but is up to manufacturer to propose and then gain FDA approval

Uncertainty

- Materials
  - Source may withdraw from the market
  - Availability
  - New materials may not perform as planned
  - FDA scrutiny
- Non-clinical studies
- Clinical trial size
- Product may not perform at larger scale during clinical trials
- Shelf life

Managing Expectations

- Commercial
  - Fastest to market
  - Logistic chain
  - Market Size
    - Orphan Drug products
  - Pricing
    - Recover R&D
    - Medicare/Medicaid pricing
    - Return to Stock Holders
    - Negotiating with insurance companies
    - Negotiating with doctors
  - Finding additional indications

Managing Expectations

- Federal
  - Understanding R&D process and decisions to keep pursuing
    - Need detailed research plans
    - Calming fears of failure and variations from plan
    - Researchers are typically not managers or product planners
  - Understanding procurement quantity
    - Processes are licensed as part of the regulatory approval process
  - Shelf life and stockpiling
  - Surge
    - Possible for drugs
    - Less likely for biologics
      - Difficult and expensive to increase current manufacturing volumes

Summary

- Commercial or federal life cycle is the same
  - Life cycle phases have different periods of performance
- Estimating is relatively straightforward
  - Build up or analog
  - Solid basis for estimate can go a long way to successfully managing expectations
- Risk
  - Researchers understand where the issues typically are found
- Uncertainty
  - Many factors come into play
    - Materials
    - Regulatory
    - Outcomes
- Expectations
  - Solid estimate can significantly manage what is expected
    - Details come out in order to complete the estimate
    - Help with assessing market's impact

Questions

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