CTI CLINICAL TRIAL & CONSULTING
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May Newsletter

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Pricing, Economic Reimbursement, Market Share (PERMS): A Holistic Approach to Rare Diseases

Contributed by:
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There are approximately 30 million Americans who are inflicted with one of the 7,000 rare diseases and over 450 medicines in development for rare disease according to the NIH. A rare disease is defined in the European Union as a disease with a prevalence of less than 5 patients per 10,000 people and in the United States as having a maximum of 200,000 patients, which makes it challenging to achieve orphan drug approvals and reimbursement. Market exclusivity, tax breaks, and historically high prices have been permitted to encourage pharmaceutical and biotech companies to develop products to serve small markets and patients with no or limited therapeutic options. However, the success of these incentives has also brought about a concern regarding the budget impact of orphan drugs on the health care system. **Annual costs for treatment of rare diseases can be as much as $500,000 per year for a single patient.**

Collectively, the orphan drug market is large with current estimates of $86 billion. These high costs have led several European countries to demand significant price concessions from orphan drug manufacturers in the past year. Reimbursement at a viable level for the pharmaceutical company is no longer assured with the regulatory approval of the orphan drug. Therefore, drug companies are burdened with the risk of developing a drug for a rare disease that may have a high societal value, yet inadequate coverage.

According to one of CTI's prominent academic affiliates, John A. Rizzo, PhD, Professor of Economics and Preventive Medicine at Stony Brook University, and a leading health economist who has published more than 140 peer-reviewed articles in health economics and outcomes research:

"In an increasingly cost-conscious environment; stakeholders, who may be less familiar with economic methods and models, nevertheless realize all too well that understanding reimbursement and related economic issues at an earlier stage in the orphan drug development process is critical to the eventual market success of the drug.

***Simulation modeling is often the tool of choice for providing a holistic assessment of orphan drugs.** This approach allows for the simulation of numerous different scenarios. For example, an over-arching model can be developed that accounts for pricing, economics, reimbursement, and market size. A well-developed model will be adaptable to the changing requirements throughout the drug development process.*

Through these interactive decision-analytic models, stakeholders can assess pricing strategies as well as cost-effectiveness. The models are typically populated with cost and clinical information taken from the published literature and hospital/payer/EMR or registry database analyses. Extensive sensitivity analyses are performed to highlight the substantial uncertainty in disease prevalence and costs associated with rare diseases. An interactive interface can be developed to communicate to stakeholders how changes in model input values affect outcomes of interest."

As the orphan drug landscape becomes increasingly more competitive, using simulation models can be useful and informative for understanding potential reimbursement levels, attracting investors, and making an informed decision to proceed to the next phase of drug development.

CTI offers clients a complete portfolio of outcomes research services to evaluate the effect of health care interventions on patient-related, clinical, and economic outcomes. Understanding the end results of

CTI Cares Spotlight

Leukemia and Lymphoma Society

LEUKEMIA & LYMPHOMA SOCIETY®
fighting blood cancers

Nominated by:
Ed Riestenberg - Senior Manager, Clinical Systems
Mary Wersel - Project Financial Analyst
Jane Riestenberg - Administrative Assistant

The Leukemia & Lymphoma Society (LLS) is the world's largest voluntary (nonprofit) health organization dedicated to funding blood cancer research and providing education and patient services. Since 1949, LLS has been dedicated to curing leukemia, lymphoma, and myeloma.

Click here to learn more and donate!

particular health care practices may provide evidence about benefits, risks, and results of treatments for the clinicians or patients. For healthcare managers and purchasers, it can identify effective strategies to improve quality and value of care.

For more information:

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CTI Upcoming Meeting Spotlight

International Society Pharmacoeconomics and Outcomes Research (ISPOR) 19th Annual Meeting

CTI will have posters presented at the following sessions:

- **POSTER SESSION II, Monday, June 2, 2014 - 3:45PM - 7:45PM**
  - *Poster ID #PCV57*
    Moore M, Mallow PJ, Rizzo JA. **The Variable Cost of an Operating Room Minute for Valvular Procedures**
  - *Poster ID #PCV14*
    Hunter TD, Quiroz ME, Gunnarsson C, Mollenkopf SA. **Characterization of Ischemic Stroke Patients and Second Stroke Rates by Risk Scores**
  - *Poster ID #PCN60*
    Ghosh SK, Roy S, Ryan MP, Gunnarsson C, Yoo AC.**Incremental Burden of Anastomotic Leaks in Colorectal Surgeries**
  - *Poster ID #PCN71*
    Swanson S, Miller D, McKenna R, Meyers B, Marshall MB, **Ghosh SK,** Fegelman E, Roy S, Ryan M, Gunnarsson C, Howington JA. **Economic Burden of Prolonged Air Leak after Lung Resection: Open versus Video Assisted Thoracoscopic Surgery (VATS)**
- **POSTER SESSION III, Tuesday, June 3, 2014 - 8:30AM - 2:15PM**
  - *Poster ID #PHS167*
    Mallow PJ, Rowlandson I, Tasic D. **Outpatient Visits to the Cath Lab for Coronary Angiography Resulting in Minimal Action in the Short Term**
- **POSTER SESSION IV, Tuesday, June 3, 2014 - 3:45PM - 7:45PM**
  - *Poster ID #PRM139*
    Mallow PJ, Rizzo JA, Irish W, Okere CE.**A Comprehensive Economic and Pricing Modeling Framework for Understanding Orphan Drug Development**

Stop by and visit us at Booth #92!

To schedule a meeting with us while we're here, please click here.

DIA 2014 50th Annual Meeting

CTI's Peter J. Mallow, PhD, Assistant Director, Health Economics, will chair the session **"Pricing, Economic, Reimbursement, Market Share (PERMS) Strategy: An Interactive Holistic Approach to Rare Disease" on Monday, June 16th at 11:00am - 12:30pm PST**. The session will present a strategy to assess orphan drug development that accounts for pricing, economics, reimbursement, and market size. During the session, Dr. William Irish, CTI's Vice President of Health Outcomes and Biostatistics, will present on the topic "Rare Disease Aspects of Pricing, Economics, Reimbursement, Market Share (PERMS) Strategy". CTI Consultant, Dr. John Rizzo, Professor Economics and Preventative Medicine at Stony Brook University, will also present at this session on the topic "Economic Aspects of Pricing, Health Economics, Reimbursement, Market Share (PERMS) Strategy". Click here for more information.

CTI's Dr. Kathryn Wekselman, Director, Regulatory and Scientific Affairs, will lead a session titled **"Successful Drug Development: Best Practices for Clinical Trial Design, Agency Interactions and Regulatory**

Document Writing" on Sunday, June 15, 8:30am -12:00pm PST. Click here for more information or to register for this session.

Stop by and visit us at Booth #2316 throughout the week!

To schedule a meeting with us while we're here, please click here.

Upcoming Meetings We'll be Attending

Israel Innovation Conference (MIXiii)
Tel Aviv, Israel - May 20 - 22

International Society Pharmacoeconomics and Outcomes Research 19th Annual Meeting
Montreal, QC - May 31 - June 4

Drug Information Association Annual Meeting 2014
San Diego, CA - June 15 -19

ICE/ENDO (Endocrine Society)
Chicago, IL - June 21-24

2014 BIO International Conference
San Diego, CA - June 23 -26

2014 World Transplant Congress
San Francisco - July 26 - 31

To schedule a meeting with us at one of these, please click here

New Additions & Promotions at CTI

Jessica Sheridan joins as Senior Manager, Business Development & Client Management

Jaqueline Aguiar joins as Operations Manager, Latin America

Yuhui Qiu joins as Biostatistician I

Robin Brown joins as Clinical Data Associate II

Teresa Murrell-Bohn joins as Study Manager

Betty Wharton joins as Patient Recruiter

Ed Probst joins as Information Technology Support Specialist

George Ranson promoted to Contract Manager

Join our Team!! We're looking for individuals to fill these positions:

Clinical Research Associate (US, Spain, UK, Germany, France, Belgium, Poland, Australia, Brazil)

Director, Health Outcomes Research (Cincinnati, OH)

Quality Assurance Auditor (Cincinnati, OH)

Regulatory Specialist (Cincinnati, OH)

Study Manager (Cincinnati, OH)

Patient Recruiter (Cincinnati, OH)

Click here for more information and to apply!