RTI HEALTH SOLUTIONS®

Real-World Data for Health Economics and Outcomes Research Studies: an Overview

RTI Health Solutions
- Research Triangle Park, NC, USA
- Ann Arbor, MI, USA
- Barcelona, Spain
- Ljungskile, Sweden
- Manchester, UK
- Waltham, MA, USA

www.rtihs.org
e-mail: rtihealthsolutions@rti.org

March 16, 2015

Agenda

- Introductions
- Overview of Health Economics Services
- Real-World Data Needs and Designs
- Potential Data Sources
- Case Studies
- Questions

Introductions

- Head, Health Economics, at RTI Health Solutions
  - >15 years' professional experience
    - Primary focus on retrospective, observational data analyses
    - Lead team of 10 health economists, all accomplished SAS programmers with extensive experience as statistical modelers/econometricians
  - Formal academic training in economics and pharmacy administration
    - PhD, Pharmacy Administration, The Ohio State University, Columbus, OH
    - MS, Economics, The Florida State University, Tallahassee, FL
    - BS, Economics, The Florida State University, Tallahassee, FL
    - BS, Biological Sciences, The Florida State University, Tallahassee, FL

RTI-HS: The Organization

- RTI International was established in 1958

- RTI-HS was formed in 2000 as a business unit of RTI International
- ~250 employees worldwide located in:
  - Research Triangle Park, NC, US
  - Manchester, UK
  - Barcelona, Spain
  - Ljungskile, Sweden
  - Ann Arbor, MI, US
  - Waltham, MA, US

Full Spectrum of Health Economics Services

What

Early Assessments

- Valuation
- Economic strategy
- Protocol development (clinical trials)
- Early compound assessment (clinical and economic)
- Value-based pricing (threshold analyses)

Prep for Market

- Burden/cost of illness (literature review or analyses of data)
- Treatment patterns
- Risk of events (clinical or resource use)
- Cost-effectiveness/budget impact analyses
- Within-trial economic analyses
- Meta-analyses
- HTA submissions

Postmarketing Support

- Comparative effectiveness
- Adherence/compliance/persistence studies
- Drug-specific treatment patterns (discontinuation, switching, augmentation)
- Benefit-risk analyses
- Market dynamics (analysis of data)
- User-friendly field models
- Indirect burden (e.g., productivity)

How

Methods

- Literature review
- Analysis of claims, EMR, and survey data
- Chart abstractions
- Advisory boards
- Decision-analytic modeling
- Meta-analyses
- Workshops/training sessions
- Publications/presentations

Real-World Data Needs and Designs

Pharmaceutical, biotech, and medical device clients often seek to design and implement studies addressing a wide-range of HEOR-related research questions:

- Clinical and economic burden of illness
- Health care resource utilization
- Treatment patterns
- Medication adherence and persistence
- Comparative effectiveness and costs of alternative treatments
- Indirect cost burden (e.g., lost workplace productivity)

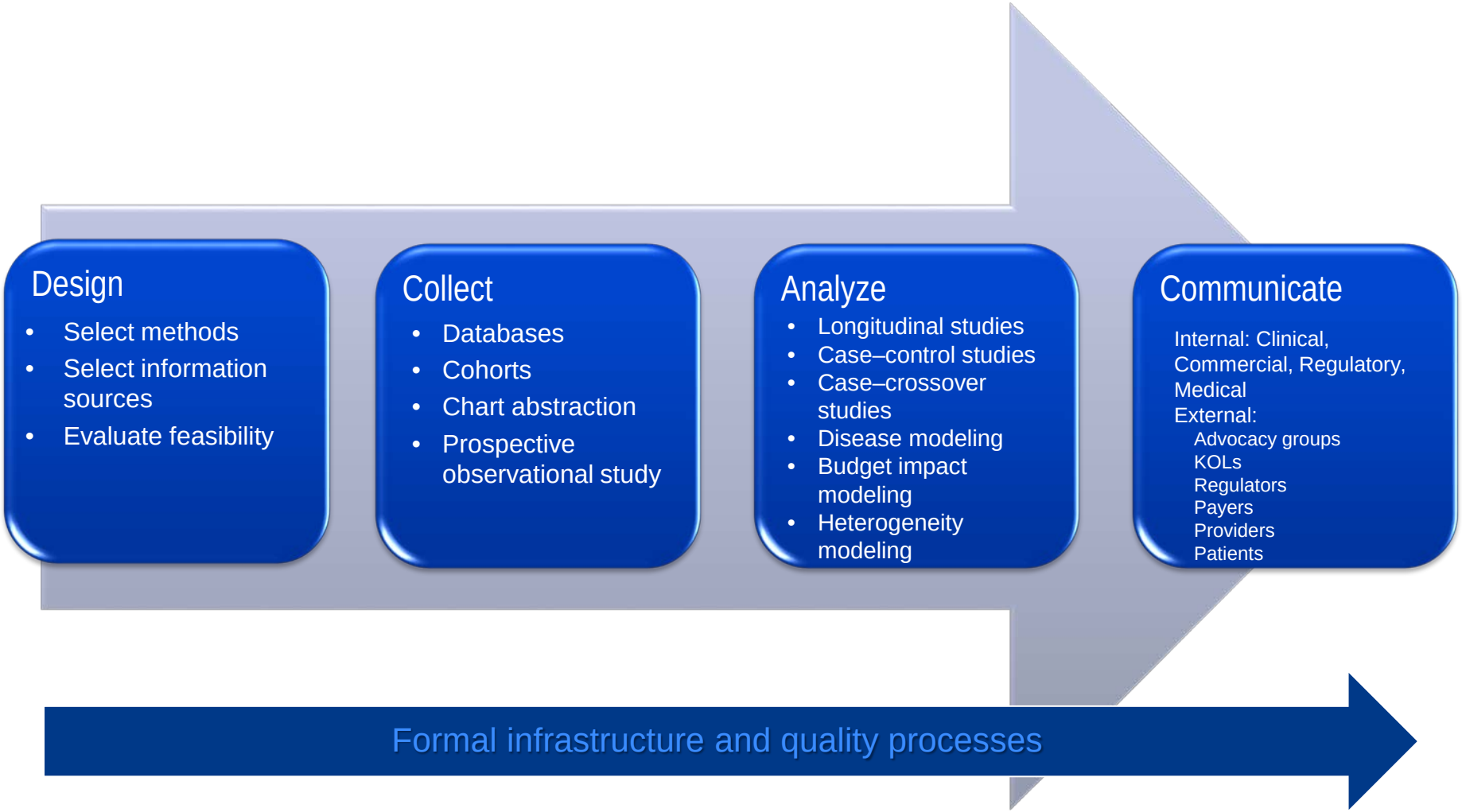
Two primary research designs:

1. Retrospective analyses of existing administrative claims databases
2. Retrospective, non-interventional chart/medical record reviews
   - Heavy focus in oncology
   - Highly customized study protocols and data collection tools
   - Multinational

Somewhat less frequently, conduct analyses of:

1. EMR data
2. Publicly available health survey data

Elements of Real-World Data Strategy



Real-World Evidence: Database Studies

Project Approach

Develop research questions

Design study

Select and acquire data

Program and analyze data

Prepare reports and slide decks

Develop publications

Advantages

Access to large patient samples – more diverse and representative

Useful for more prevalent diseases

More accurate resource use and cost estimates (claims-sourced)

Relatively low cost of data acquisition

Shorter turnaround time

Potential Data Sources

- Behavioral Risk Factor Surveillance System (BRFSS)
- CPRD (UK)
- Drug Abuse Warning Network
- Friuli-Venezia-Giulia databases (Italy)
- GE Healthcare (EMR)
- MedMining (Linked claims and EMR from Geisinger)
- Hospital Episode Statistics (HES) (UK)
- Health and Retirement Survey (US)
- Health Search Database (Italy)
- Health Survey of England (HSE)
- Henry Ford Health System
- Healthcare Cost and Utilization Project (HCUP) databases
- i3 Innovus Integrated Health Care Information Systems (IHCIS)
- i3 Innovus Lab Rx
- Japan Medical Data Center (JMDC)
- MarketScan Health and Productivity Management (HPM)
- MarketScan Commercial Claims and Encounters (CCAE)
- IMS MediPlus (UK, Germany, France)
- Medical Expenditure Panel Survey (MEPS)
- Medicare and Medicaid claims data
- National Comorbidity Survey (NCS)
- National Health and Nutrition Examination Survey (NHANES)
- National Health Interview Survey (NHIS)
- National Hospital Discharge Survey (NHDS)
- National Medical Expenditure Survey (NMES)
- National Nursing Home Survey (NNHS)
- National Ambulatory Medical Care Survey (NAMCS)
- National Household Survey (NHS) (UK)
- National Hospital Ambulatory Medical Care Survey (NHAMCS)
- National Inpatient Surveys (NIS) (UK)
- National Medical Expenditure Survey (NMES)
- National Survey On Drug Use and Health (NSDUH)
- Organ Procurement and Transplantation Network
- PharMetrics/LifeLink
- Pharmo (Netherlands)
- Premier
- Saskatchewan Health (Canada)
- Scandinavian cancer registries
- Studies of Left Ventricular Dysfunction (SOLVD)
- Surveillance, Epidemiology and End Results (SEER) cancer registries
- SEER-Medicare Linked Database
- The Health Improvement Network (THIN) (UK)
- US Renal Data System
- Veterans Affairs claims data

Noticeable Increased Interest in Chart Review Approaches to Retrospective Data Analyses

- Chart abstractions can provide a rich source of data that often cannot be assessed through other data sources
- Chart review studies can be/often are conducted within US and ex-US markets to:
  - Describe prescribing patterns
  - Evaluate disease progression, survival, and treatment response
  - Evaluate clinical characteristics of disease
  - Assess resource utilization
  - Develop country-specific economic endpoints to populate cost-effectiveness or budget impact models
- Chart data can be linked to other patient data (e.g., patient surveys)

Real-World Evidence: Retrospective Chart Abstractions

Project Approach

Develop research questions

Design study and develop protocol

Develop web-based customized data collection forms

Obtain IRB/ country-level ethics approvals

Recruit physicians; manage fieldwork

Program and analyze data

Prepare reports and slide decks

Develop publications

Advantages

Useful for hard to reach patient populations

Smaller samples but highly targeted

Ability to collect detailed clinical data

Ability to collect uniform data across multiple countries

Case Study 1

Chart Review in Chronic Myeloid Leukemia

GOAL

To assess chronic myeloid leukemia (CML)-related treatment patterns and prognostic indicators of treatment response among patients in North America, Europe, and the Asia-Pacific region

APPROACH

- Team was able to capture data from nearly 1,700 patients in the US, Canada, Australia, Japan, South Korea, Germany, and the UK through chart abstractions
- Patients selected were >18 years of age and in chronic phase at the time of diagnosis and were Philadelphia chromosome and/or BCR-ABL positive
- Team was responsible for the data collection form, study protocol and analysis plan, results tables, and final study report, as well as three publications and numerous poster presentations

RESULTS

- Several factors, in addition to Sokal score, were found to be prognostic for clinical response in 1st-line therapy, including younger age, white race, and no comorbidity
- In addition, in 2nd-line therapy, the likelihood of response was generally greater in patients treated with dasatinib than in those who received nilotinib

VALUE

This study used an innovative approach to conducting retrospective, observational chart reviews, allowing the study sponsor to expand the initial scope of the project and broaden the geographic reach of the study

Case Study 2

Administrative Claims Analysis to Evaluate Hydroxyurea Medication Adherence among Medicaid Enrollees with Sickle Cell Disease

GOAL

To assess adherence to hydroxyurea treatment among patients with sickle cell disease and investigate associations between adherence and clinical and economic outcomes

APPROACH

- Retrospective, observational database study of claims for Medicaid enrollees in North Carolina who have sickle cell disease
- Inclusion criteria included age < 65 years, continuous Medicaid enrollment ≥ 12 months before and following hydroxyurea initiation, and ≥ 2 hydroxyurea prescriptions

RESULTS

- Three hundred twelve patients, mean age 21 (± 12.2) years, met inclusion criteria and 35% were adherent, defined as a medication possession ration (MPR) ≥ 0.80, with mean MPR of ~0.60.
- In the 12 months following hydroxyurea initiation, adherence was associated with:
  - reductions in health care costs, including all-cause and SCD-related inpatient and ancillary care, vaso-occlusive event-related, and total costs (all $p < 0.0001$)
  - reduced risk of SCD-related hospitalization, all-cause and SCD-related emergency department visit and vaso-occlusive event (all $p < 0.05$)

VALUE

This was the first published study to document adherence to hydroxyurea in a real-world population of Medicaid enrollees, and emphasizes the need to monitor and improve medication adherence, as doing so will likely improve outcomes, from both patient and societal perspectives

Thank You!

Questions and Discussion

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