



# The Role of Patient Registries in Evidence Development: Similarities and Differences Between Europe and North America

Eric K Gemmen MA; Chris L Pashos PhD; Christopher M Blanchette PhD, MS, MA; Yvonne Lis PhD; Gabriel Sandblom MD, PhD; Dimitris Polygenis PharmD; Sally Thompson PhD, MSc; Alex Exuzides PhD; Carl J Gibbons BSc (Psych)

\*Representing the ISPOR Patient Registry Classification, Strategy & Design Working Group

# Acknowledgements

- Diana Frame, MPH
- Shital Kamble, PhD
- Michael Carter
- Elizabeth Molsen, RN

# Purpose

This workshop will compare and contrast the role of patient registries in evidence development in Europe with that emerging in North America. Examples of patient registries and their uses will be provided.

# Registry Definition

- **Prospective** observational study of subjects, with certain shared characteristics, that collects ongoing and supporting data over time on well-defined outcomes of interest for analysis and reporting

# Context: Uses of Patient Registries

- Observe the course of a disease
- Illuminate practice patterns and variations in them
- Assess clinical outcomes: effectiveness and safety
- Explore humanistic outcomes, including health-related quality of life & other patient-reported outcomes
- Assess economic outcomes
- Examine associations between care and outcomes
- Inform clinical and policy decision-making

*Patient Registries provide real-world data that complement clinical trial evidence*

# Context: Key Stakeholders

- Clinicians
- Physician and disease organizations
- Health technology agencies
- Health insurers / payers
- Regulatory authorities
- Patients

# Work Plan

- Review literature on the use of patient registries in decision-making
- Review websites of primary stakeholders/decision-makers in Europe and North America
- Review current guidance for the use of patient registries  
Gliklich RE, Dreyer NA, eds. Registries for Evaluating Patient Outcomes: A User's Guide. AHRQ Publication No. 07-EHC001-1. April 2007.
- Perform qualitative assessment of the evidence
- Characterize the role of patient registries in the evidence base in Europe and North America



# Use of Registries in HTA

## UK Experience

(Yvonne Lis)

# HTA process in the UK

- HTA has been a long-term UK policy priority
- Main drivers in UK include
  - NICE (1993)
  - SMC (Scottish Medicines Consortium) (2002)
  - All Wales Medicines Strategy Group (AWMSG) (2002)
- Differences in appraisal topic selection, processes, stakeholders and timescales
- Independent academic involvement including National Coordinating Centre for HTA (Un. Of Southampton)

## Approach used

- Search of NHS evidence service provided under the NICE umbrella <http://www.evidence.nhs.uk>
- Search parameters
  - Registry
  - Health Technology Assessment
  - Drugs and technologies

# Overview of findings from the review of the NHS Evidence Service

- 424 documents retrieved (including 33 non-UK)
- 100 documents selected at random for review
- Most recommendations based on data from systematic reviews (>60%) with registries frequently included in search terms, suggesting recognition of their potential relevance
- Multiple references made to the need for registry data to complete an adequate appraisal

But.....

- Use of registry data as main source of data was very limited

## Example of where Registry data provided important input to HTA - 1

- Modelling the cost effectiveness of TNF-alpha antagonists in the management of rheumatoid arthritis: results from the British Society for Rheumatology Biologics Registry *Brennan et al. Rheumatology 2007;46:1345-1354*
- Registry data used to assess response to treatment over three years including HRQoL measured using SF-36
- Influential in determining guidance on the use of etanercept and infliximab for treating rheumatoid arthritis

## Example of where Registry data provided important input to HTA - 2

- Modeling the cost effectiveness of thrombolysis with recombinant plasminogen activator for acute ischemic stroke assessed by a model based on UK NHS costs. *NHS Economic Evaluation database (NHS EED) December 2005*
- Registry set up as part of NHS Health Technology Assessment programme
- Data from registry were used to evaluate cost effectiveness and determine whether and to what extent the UK NHS should implement thrombolytic treatment

## Cross-linkage of datasets – UK [Carl G]

- General Practice Resource Database (GPRD) links GP-level patient data with the following National Health Service (NHS) information:
  - Death data
  - Full hospitalisation records
    - ICD9 disease code
    - operation codes
  - Disease registries (selected)
  - Socioeconomic class data
- Such linkages handled by NHS third party to ensure that confidentiality is not breached

## Sir Michael Rawlins' statements to Royal College of Physicians October 2008

- '... we need a new approach to analysing clinical evidence'
- '... RCTs' appearance at the top of the hierarchy of evidence is inappropriate... need a diversity of approaches that involve the totality of the evidence base'
- '...observational studies are also useful... can provide an important source of evidence about both the benefits and harms of therapeutic interventions'



# **Recent EMEA Experience**

(Sally Thompson and Gabriel Sandblom)

# Registries typically proposed by the EMEA as part of pharmacovigilance planning

- EMEA pharmacovigilance planning document from 2005 defines a registry and outlines potential uses
  - A registry is a list of patients presenting with the same characteristic(s). This characteristic can be a disease (disease registry) or a specific exposure (drug registry).
  - Disease registries can
    - Collect data on drug exposure and other factors associated with a clinical condition
    - Serve as a base for a case-control study comparing the drug exposure of cases identified from the registry and controls selected from either patients with another condition within the registry, or patients outside the registry
  - Exposure (drug) registries address
    - Populations exposed to drugs of interest (e.g., registry of rheumatoid arthritis patients exposed to biological therapies) to determine if a drug has a special impact on this group of patients
    - Some exposure (drug) registries address drug exposures in specific populations, such as pregnant women
  - Registry methods
    - Patients can be followed over time and included in a cohort study to collect data on adverse events using standardised questionnaires.
  - Use of registry data by the EMEA
    - Single cohort studies can measure incidence, but, without a comparison group, cannot provide proof of association.
    - However, registries can be useful for signal amplification, particularly for rare outcomes. This type of registry can be very valuable when examining the safety of an orphan drug indicated for a specific condition.

## Paediatric label extensions may attract recommendations from the EMEA for a registry: The case of etanercept (Enbrel) for the treatment of juvenile idiopathic arthritis

- 3 Clinical studies formed part of the Paediatric Investigation Plan for etanercept (Enbrel) one of which was an open-label non-randomized multicentre registry study of children with polyarticular course or systemic onset JIA from 1 year to less than 18 years for evaluation of long-term safety of etanercept compared to patients receiving methotrexate (Study 20021626).
- Date of decision: 16 June 2009
- Requested date of completion of the paediatric investigation plan: By October 2011

## Registry data may be used to confirm appropriate product use: The case of Xigris (activated drotrecogin alfa) for sepsis patients with multiple organ dysfunction

- Date: 21 April 2005
- After assessing the Expert Group's conclusions, the majority of the CHMP acknowledged that there is a strong need to ensure that Xigris is used exclusively in high-risk patients, as an add-on to best standard care.
- Postmarketing experience shows that as of November 2004, approximately 2000 patients have been treated across the EU, supporting the idea that Xigris is not being used broadly outside the indication.
- Additionally, data from the Belgium Xigris Reimbursement Registry shows that Xigris is only being used within the indicated population, and that 73% of patients even had three or more organ dysfunctions, indicating that Xigris use is largely confined to the more severely ill subset of the MOD population. Data from a similar registry in Italy supports these conclusions. Such observations do not suggest that further clarification of the indication would benefit the severe sepsis patients in the EU.

## Registry data can support a label extension: The case of Crohn's Therapy, Resource Evaluation and Assessment Tool Registry (TREAT) and infliximab (Remicade)

- The TREAT Registry is a prospective, observational, multicenter, long-term registry featuring clinical, economic, and humanistic measures characterizing the treatment of Crohn's disease.
- The objective is to track treatments and patient outcomes over at least a 5-year period.
- Of the 6273 adult CD patients enrolled as of February 2006, a total of 3300 (around 53%) were treated with infliximab
- Infliximab was originally approved in August 1999 for a 2nd-line indication in CD, i.e. for use in patients after failure of corticosteroids and/or immunosuppressant therapies or in patients who are intolerant to or have medical contraindications for such therapies.
- In 2001, the indication was restricted to 3rd line based on considerations of the total benefit/risk profile, particularly taking the limited knowledge of the safety profile of infliximab that was available at that time
- Based on data from clinical trials (ACCENT 1, GETAID2), registry (TREAT3) and other post-marketing data, the MAH applied for a variation to extend the indication from 3rd to 2nd line in CD

## Risk management, post-marketing studies and pharmacovigilance: The case of Factor VIII products

- The need for risk management plan, registries, large observational studies, and reporting of adverse events are important aspects in the pharmacovigilance setting for FVIII products.
- Long-term data can be obtained from the post-authorisation setting, via post-marketing studies or registries.
- A network of national registries with common criteria for data collection is desirable to ensure that data are compatible between national registries in order to allow pooling of anonymised data.



# Recent Canada Experience (Dimitris Polygenis)

# Canada – Registry Characteristics

Total of 32 registries were reviewed

- 18 Canadian; 14 International with Canadian collaboration
- 1 to 35 sites per registry
- Patient registration from 25 (IRESSA) to over 10,000 (Stroke Network, Health and Aging)
- Therapeutic Areas : Schizophrenia, Dementia, Osteoporosis, Stroke, Arthritis, Cardiovascular, Obesity, Aging, Cancer
- Sponsors: Federal and Provincial Governments, Academia, Pharmaceutical Industry , Not-for-profit (Arthritis Society, Stroke Network, Fabry Research Consortium)
- 50% of registries are jointly sponsored, 40% Pharmaceutical Industry-only sponsored
- Administration and management: CROs and Academic Institutes/Hospitals (Lawson,UHN, Hamilton Health Centre, Ottawa Heart Institute)

# Canada – Utility and Impact

- **e-STAR** - Has demonstrated high retention rates with Risperdal® Consta in a real life setting and significant decreases in patient hospitalization supporting full listing in some provinces.
- **Canadian Multicentre Osteoporosis Study** - Data has been used to provide resource utilization/health related quality of life data for modeling studies in CDR/provincial health plan submissions. Developed and validated a clinical tool to help clinicians identify which women are at increased risk for osteoporosis - Osteoporosis Risk Assessment Instrument (ORAI).
- **University of Toronto Psoriatic Arthritis Clinic Database** - Advances in treatment of psoriatic arthritis and identification of patients who need to be treated aggressively.
- **Canadian Study of Health and Aging**- Has provided data on prevalence, incidence and progression of dementia and the caregiver burden of the disease.
- **Registry of the Canadian Stroke Network** - Has provided data for the evaluation of provincial stroke strategies so that health providers and policymakers can improve quality of care. Demonstrated that organized stroke care can prevent nine out of 10 in hospital deaths in the first week following stroke based on the results from the registry.
- **IRESSA patient registry program** - Ensures that only patients who are currently benefiting from IRESSA continue to receive the drug.
- **RESTORE** - CDR recommended a registry as a prerequisite for reimbursement. Registry targeted at effectiveness, health outcome and safety information.

## Canada – Observations/Conclusions

- Less emphasis on patient reported outcomes
- Canadian Agency for Drugs and Technologies in Health (CADTH) is hesitant in considering post-marketing observational study data when conducting health technology assessments
- Fragmented national health care system (i.e., >10 Federal/Provincial payers) impact interpretation and generalizability of resource utilization data (e.g., assigning payer responsibility for specific resources)
- To date, data has been collected on more than 50,000 strokes in Canada; one of the few larger and comprehensive databases

# Canada – Key Trends & Developments

- Over the last decade there has been a sharp increase in the number of Canadian registries
- Likely that more reimbursement decisions will be conditional on establishment of a registry to monitor patient outcomes
- Data collected from some have helped to develop key therapeutic advances e.g., Osteoporosis Risk Assessment Instrument (ORAI) and advances in stroke care
- Privacy issues are being addressed - The Stroke Registry moved from a consent-based approach to being given special privileges under Ontario's Personal Health Information Protection Act to collect data through chart abstraction
  - As a result information can be collected faster and more accurately on a wider range of patients.

# Canada – Key Challenges

- Electronic data capture not widespread; therefore validity of data from multiple sites can vary
- Non-academic access to government data is limited
- Registries may concentrate on drug plan priorities (e.g., drug utilization) rather than effectiveness and other aspects of the disease



# Recent US Public Stakeholders' Experience (Eric Gemmen)

# FDA

- Reviewed all new drug approval letters for the period March 2008 through September 2009 that included a request from the FDA for post-marketing commitment (PMC) studies.
  - From these post-marketing commitment studies, 15-20% drugs required sponsors to conduct Observational Studies/Registries.

All approvals	N=114	
Products with PMC studies	<b>35</b>	31% of all approvals
Observational Studies*	6	5% of all; 17% of PMCs
Safety as objective	6	
Effectiveness as objective	1	
Profiling patient use as objective	1	

\* Crohn's Disease, malaria, fibromyalgia, dyslipidemia, pulmonary arterial hypertension, atrial fibrillation and atrial flutter

## Example of FDA's Use of Registry Data: CASES-PMS

### Carotid Artery Stenting with Emboli Protection Surveillance Post-Marketing Study (CASES-PMS)

- In 2004, sponsor received FDA approval for carotid stent procedure following SAPPHIRE clinical trial
- However, FDA (and CMS) questioned whether outcomes of the trial were generalizable to physicians without prior carotid artery stenting experience
- CASES-PMS was undertaken in response to FDA and CMS requests, to confirm safety and efficacy of carotid artery stenting in a variety of settings and to confirm effectiveness of training
  - Endpoint: non-inferiority vs. MACE rate in SAPPHIRE
  - Contained same patient inclusion/exclusion criteria as Phase III SAPPHIRE trial, but variety of settings and sites (e.g., stenting experience, volume, geography)

## Centers for Medicare & Medicaid Services (CMS): Coverage with Evidence Development

The purpose of coverage with evidence development (CED) is to generate data on the utilization and impact of the item or service evaluated for national coverage determinations, so that Medicare can:

1. Document the appropriateness of use of that item or service in Medicare beneficiaries under current coverage;
2. Consider future changes in coverage for the item or service;
3. Generate clinical information that will improve the evidence base on which providers base their recommendations to Medicare beneficiaries regarding the item or service.

## Example of CMS's Use of Registry Data

- The ICD (implantable cardioverter defibrillator) Registry
- The Heart Rhythm Society and the American College of Cardiology Foundation
- Tracks the relationship between physician training and in-hospital patient outcomes following ICD implantation
- Assesses effectiveness and safety in elderly (65+) population
- All hospitals implanting ICDs for primary prevention in Medicare beneficiaries required, as a condition for coverage, to routinely submit data to the registry

Source: AHRO

Published Reference: Hammill S, Phurrough S, Brandis R. The National ICD Registry: now and into the future. *Heart Rhythm* 2006 April; 3(4):470-3.

# DHHS Effort in Response to ARRA

- ARRA signed into law by President Obama in February 2009
- Comparative Effectiveness initiative: US\$1.1 billion
  - National Institutes of Health
  - Agency for Health Research and Quality (AHRQ)
- Patient registries also considered alongside database/claims analyses, medical record reviews and meta-analyses as comprising the evidence base for comparative effectiveness research
- Initial guidance has prescribed observational studies, but does not specify whether the research is to be conducted retrospectively or prospectively

# Compendia of Patient Registries

- [www.clinicaltrials.gov](http://www.clinicaltrials.gov) is a service of the U.S. National Institutes of Health (NIH)
  - search on 'observational' yielded 14,122 studies
  - search on 'registry' yielded 718 studies
  - search on 'patient registry' yielded 509 studies
- Agency for Healthcare Research and Quality (AHRQ) is developing a registry of patient registries
  - Part of AHRQ's *Effective Health Care Program* and its ongoing project on Registries for Evaluating Patient Outcomes
  - AHRQ has commissioned four white papers:

## U.S. Government Stakeholders Overview

- The FDA uses (pre-requested) evidence from observational studies and registries to confirm safety, but rarely to confirm efficacy
  - When it does, it is more common in medical devices, procedures and in life threatening conditions, e.g., heart disease
- CMS uses evidence from observational studies and registries as economic support (e.g., resource utilization, natural history of disease)
  - In some cases, requires the collection and submission of registry data as a condition of coverage
- Comparative effectiveness research under AHRQ and NIH will involve patient registries, but the extent of patient registries (vs. retrospective research) is yet to be seen



# Recent US Private Payors Experience

(Chris Blanchette, Alex Exuzides)

# US Commercial Payer Markets

- Segmented payers made up of health insurance plans, prescription benefit management programs, and integrated healthcare delivery systems
- Methodologies
  - Independent field notes from communication with managers from all three facets of those mentioned above
  - Review of published technical reports

# US Commercial Payers Reviewed

- Blue Cross Blue Shield ~100 million
  - 39 independent, community-based and locally operated companies
- Aetna ~20 million
  - one of the US's largest health insurers, covering 19 million medical members, 14 million dental members, and 11 million pharmacy members
- United Healthcare ~70 million
  - Network includes 595,000 physicians and health care professionals, 80,000 dentists and 4,965 hospitals. Pharmaceutical management programs provide access to drugs for 13 million people.
- WellPoint ~35 million
  - Health benefits company spanning over 14 states of the US
- America's Health Insurance Plans ~ 1,300 members (200 million)
  - National association representing nearly 1,300 member companies providing health insurance coverage to more than 200 million Americans
- HMO Research Network (HMORN) ~8 million
  - 16 integrated healthcare delivery systems from the US and Israel

<http://www.aetna.com/about/aetna/aag/facts.html>

<http://www.bcbs.com/>

[http://www.uhc.com/about\\_us.htm](http://www.uhc.com/about_us.htm)

<http://www.wellpoint.com/business/default.asp>

<http://www.hmoresearchnetwork.org/about.htm>

# Blue Cross Blue Shield

- SMART Registry developed to identify patients who need routine tests and screenings and to help physicians better monitor and manage chronic conditions.
- Blue Cross Blue Shield of Michigan Cardiovascular Consortium (BMC2) - consortium of health care providers in the Michigan which developed a longitudinal multicenter registry of consecutive percutaneous coronary interventions (PCI): currently includes data on more than 200,000 PCI collected from over 30 hospitals.
- A review of 20 TEC assessments from Blue Cross - Blue Shield Technology Evaluation Center revealed:
  - 3 incorporated registry data; 1 other used observational data from prospective cohort studies.
  - Only 1 assessment (metal-on-metal total hip resurfacing) concluded that the technology evaluated had met TEC criteria.
  - In 2 reports authors mentioned the lack of direct evidence from RCTs on patient outcomes as rationale for considering registry data in the review.
  - In 2 other reports, registry data were used to provide event rate estimates (safety, need for revision surgery) based on a more robust number of patients and longer followup than was available in clinical trials.

# Aetna

- National Kidney Registry

- Aetna Foundation provided support for The National Kidney Registry, a non-profit group dedicated to improving the lives of people facing kidney failure by helping patients with incompatible donors receive kidney transplants through the Registry's national matching system.

- Active Health Management

- An Aetna subsidiary developed to take clinical information from available administrative data, aggregate that data for each member, and apply rules drawn from the medical literature to identify members whose care could be improved. Currently cover millions of members nationwide in a range of health management and data analytics products and services – all to reduce medical errors and medical costs.

# United Healthcare

## ■ *BridgingCare Planner*

- In collaboration with GlaxoSmithKline (GSK), offered patient registry technology to UnitedHealthcare participating physicians to help manage their patients with chronic disease.
- The *BridgingCare Planner*® is a web-based patient registry designed to support improvement in health outcomes for patients with certain diseases, including diabetes and cardiovascular disease.

[https://www.unitedhealthcareonline.com/ccmcontent/ProviderII/UHC/en-US/Assets/ProviderStaticFiles/ProviderStaticFilesPdf/Tools%20and%20Resources/NetworkBulletin\\_July2006.pdf](https://www.unitedhealthcareonline.com/ccmcontent/ProviderII/UHC/en-US/Assets/ProviderStaticFiles/ProviderStaticFilesPdf/Tools%20and%20Resources/NetworkBulletin_July2006.pdf)

<http://appliedclinicaltrialsonline.findpharma.com/appliedclinicaltrials/Industry+news/Drug-Safety-Registry-Introduced-by-Ingelix3-Aperi/ArticleStandard/Article/detail/168025>

<http://www.i3global.com/Solutions/TherapeuticSpecialty/Cardiology/>

# WellPoint

- HealthCore's Integrated Research Network (IRN™)
  - a collaborative research community of clinical consultants including physicians, ancillary providers, facilities, pharmacies, health plans, and HealthCore.

# AHIP

## Vaccine Safety Datalink (VSD) and Clinical Immunization Safety Assessment (CISA) network

- America's Health Insurance Plan (AHIP) was selected in (2002) by the Centers for Disease Control and Prevention (CDC) to provide overall management and coordination for their Vaccine Safety Surveillance and Assessment Projects and Clinical Immunization Safety Assessment network.

# HMORN

## HMORN Multi-center collaborations:

- Cancer Care Outcomes Research and Surveillance Consortium
- Cancer Research Network
- Cardiovascular Research Network
- Center for Education and Research on Therapeutics
- Coordinated Clinical Studies Network
- Developing Evidence to Improve Decisions about Effectiveness (DEcIDE)
- FDA Epidemiologic Studies of Adverse Effects of Marketed Drugs
- Integrated Delivery System Research Networks
- Vaccine Safety Datalink

## HMORN Participating HMOs:

- Meyers Primary Care Institute UMass /Fallon Community Health Plan
- Group Health Cooperative
- Kaiser Permanente: Hawaii, Northwest, Colorado, Southern California, Northern California and Georgia regions
- HealthPartners Research Foundation
- Henry Ford Health System
- Lovelace Clinic Foundation
- Scott & White Health System
- Marshfield Clinic Foundation
- Maccabi Institute for Health Services Research
- Harvard Pilgrim Health Care
- Geisinger Health System

<http://www.hmoresearchnetwork.org/about.htm>

## US Private Payers Overview

- As in UK, use of registry data as primary data source is limited
- May be included in reviews to supplement safety, long-term effectiveness (time to event measures), connection of intermediate efficacy endpoints to clinically meaningful outcomes
- Registry evidence considered mostly for devices, procedures
- No explicit cost-effectiveness use found



# Patient Registries of Disease-Focused Organizations (Chris Pashos)

# The Sonya Slifka Study: Tracking the Real-World Implications of Multiple Sclerosis in the US (1 of 2)

- Sponsored by the not-for-profit United States National Multiple Sclerosis Society (NMSS)
- Tracks a US nationally representative sample of people with multiple sclerosis (MS)
- Provides a unique and multifaceted resource utilized by researchers, advocates, and healthcare providers to improve the quality of life of people with the disease
- Unlike prior MS research undertaken at hospitals and through clinical trials that tended to be narrowly focused, the Slifka Study provides comprehensive data on a range of issues — no other comparable data source exists.

# The Sonya Slifka Study: Tracking the Real-World Implications of Multiple Sclerosis in the US (2 of 2)

- Study findings have been associated with specific NMSS actions
- More than half of all respondents reported cognitive problems and two-thirds talked about feeling depressed or anxious. NMSS supported efforts to develop a curriculum to help mental health providers understand MS. The curriculum included education on drug interactions, the types of cognitive problems that may develop, and the emotional challenges of the disease.
- Although more than 96 percent of participants had health insurance coverage, more than 10 percent of them reported difficulty in obtaining prescription medication. Further, out-of-pocket healthcare expenditures were twice those found in the general population. The NMSS provided the Centers for Medicare and Medicaid Services (CMS) with these data to help demonstrate the need to cover DMAs in the federal prescription drug plan.

# The CF Registry of the United Kingdom's Cystic Fibrosis Trust

- Contains a substantial amount of clinical and demographic data from 2002 to present, and less data going back to 1999.
- Tracks the health of people with Cystic Fibrosis throughout the UK; its primary purpose is to help drive up the standard of clinical care.
- Clinical data is collected in specialist CF Centres and network shared care clinics at the patient's Annual Review
- An annual report identifies new trends in the health of people with CF and provides important information that contributes to creating standards of care, designing clinical trials to test new therapies, and improving the delivery of care.

# The Cystic Fibrosis Patient Registry (1 of 2)

- More than 40 years ago, the Cystic Fibrosis Foundation created the Cystic Fibrosis Patient Registry to track the health of people with CF in the United States.
- The information in this registry allows caregivers and researchers to identify new health trends, recognize the most effective treatments and design clinical trials for potential therapies.

# The Cystic Fibrosis Patient Registry (2 of 2)

- The registry anonymously reports patient data from more than 24,000 CF patients who receive care at CF Foundation-accredited centers. The information collected includes:
  - patient demographic and clinical characteristics
  - treatment patterns and resource use
  - clinical outcomes
- By providing care center data through the patient registry, the Foundation educates and fosters stronger partnerships among people with CF, their families and care center staff.

# The International Spinal Muscular Atrophy Patient Registry (1 of 2)

- Created in 1986 at Indiana University, the Registry connects patients and families interested in participating in research and researchers interested in studying SMA.
- The Registry contains information from over 1,600 families and over 1,700 individuals with SMA from all over the world and continues to grow.
- The Registry helps centralize information on this rare genetic disease.
- Participants provide information on symptoms, treatment, medications, and other experiences.

# The International Spinal Muscular Atrophy Patient Registry (2 of 2)

- In 2008 the Registry joined the group, Translational Research in Europe for the Assessment and Treatment of Neuromuscular Diseases (TREAT-NMD) in a global collaboration to further the research goals of the neuromuscular disease community.
- The SMA Registry is supported by the Patient Advisory Group of the International Coordinating Committee for SMA Clinical Trials which includes Families of SMA, Fight SMA, Muscular Dystrophy Association, SMA Foundation, and other SMA advocacy groups.

# Diseased-Focused Organizations Overview

- Organizations created to support patients with particular diseases, notably rare diseases, have found patient registries to be an effective means to better understand the relevant clinical, economic and humanistic outcomes
- Registry findings have been used to foster greater support from multiple constituencies for meeting unmet needs
- National organizations are increasingly collaborating worldwide

# Conclusions

**Our qualitative assessment suggests that geographic similarities outweigh differences:**

- Multiple stakeholders are involved
- Regulatory authorities are interested almost exclusively in safety, with effectiveness and appropriate use sometimes being adjunct concerns
- Payers use registry data, but actual use lags behind calls for its use

A comprehensive “registry of registries”, as envisioned by AHRQ, would facilitate a quantitative assessment of the uses of registries in the evidence knowledge base.



# Questions and Discussion



## Additional Topics

# Additional Topics

1. Patient registries to support comparative effectiveness
2. Prospective registration of observational studies (e.g., [www.clinicaltrials...gov](http://www.clinicaltrials.gov))
3. Linking public and private health care databases
4. Possible impact of personalized medicine