ADVANTAGES AND DISADVANTAGES OF DISEASE VS. DRUG REGISTRIES: INDUSTRY PERSPECTIVE

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CONTENT

- Registries during medicine’s lifecycle: industry process
- Drug (product) registries: example, advantages / disadvantages
- Disease registries: example, advantages / disadvantages
- Summary
REMINDER: WHAT IS A PATIENT REGISTRY?

- Organized system
- Epidemiology study methods (observational)
- Collect uniform and standardized data
- Evaluate specified outcomes
- Population defined by disease or exposure (drug / product)
- Serve predetermined scientific, clinical and/or policy purpose

REGISTRIES IN INDUSTRY

DURING DRUG DEVELOPMENT PHASE

- Describe the patient population
- Create or expand disease awareness
- Aid the plan of clinical programs
  - size and location of patient population
  - identify high risk sub-populations
  - new indications
- Secure regulatory approval with Risk Management
  - negotiate and design post approval studies
Inform post-launch use of new product
- fulfilment of post approval commitments
  (Risk Management Plan)
- real world - safety
- real world - effectiveness
- drug use patterns & patient management
- new indications
OPUS: US REGISTRY OF OPSUMIT NEW USERS

INDUSTRY EXAMPLE OF DRUG REGISTRY

- US Post-marketing requirement for Opsumit (macitentan) 10mg tablets, under section 505 (o) of the Food, Drug and Cosmetic Act

- Drug exposure registry of Opsumit new users
  - US-based only, multicenter
  - Real-world, observational study
  - Prospective, long-term follow-up

- Objective:
  - Characterize the safety profile, and describe clinical characteristics and outcomes of patients treated with Opsumit in the post-marketing setting

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DRUG REGISTRIES

 Advantages

- Focused study population
- Cost / budget efficient
- Often clear safety objectives, defined stopping rules
- Often short timeline to meet Health authority request, quicker to start then disease registry
- Voluntary participation may offer flexibility
- Insights in potential off-label use in product approved countries

 Disadvantages

- Less generalizability – narrower study population, fragmented picture of entire disease spectrum
- Less scientific interest by academia/patients – could limit recruitment and/or bias selection
- No comparator treatment group
- Voluntary participation may present selection bias
DUO: DIGITAL ULCER OUTCOMES

INDUSTRY EXAMPLE OF DISEASE REGISTRY

- Post-approval commitment towards EMA for the DU indication in Tracleer
- Design: international, multi-centre, prospective, observational, non-interventional
- Disease specific registry:
  - ALL: Tracleer treated patients and non-Tracleer treated patients
  - Patients undergo clinical assessments and receive standard medical care as determined by the patient’s physician.
- Patient population: DU associated with SSc
- Objectives:
  - Describe, clinical characteristics, disease course and management over time in real-world ALL patients
  - Document adherence to Tracleer SMPC (liver function testing, pregnancy prevention) in Tracleer treated only patients
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DISEASE REGISTRIES

Advantages
- Broad study population – enhances generalizability
- Often scientific objectives beyond safety
- Broad stakeholders (incl. academic interest)
- Quicker recruitment (esp. Voluntary & larger pool)
- Real-world, e.g. long term outcomes, off-label use
- Rare disease area when little is known or published – creating datasets for long term future use
- May offer comparator treatment groups

Disadvantage
- Rare disease often require many centers, many countries – all with different requirements and logistics
- May be more costly if larger in scope
- For rare disease stopping rules, statistical precision around an estimate may not be realistic
- Recruitment bias if multiple disease registries competing for same sites/patients
Choice of study design, Drug versus Disease registry may depend on
- Purpose / Questions (objectives) being asked
- Timeline for decision (during New Drug approval process)
- Number of drugs/availability in the marketplace
- Rarity / Poor understanding of disease area

Disease registries offer more flexibility in objectives and may require cooperation amongst industry partners, academic interests and patient organizations

Disease registries which start during drug development may be useful starting point for subset analyses of drug specific observations during post-marketing setting
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