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An Industry Perspective on Clinical Outcomes using Real World Data

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Setting the scene

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The Obvious:

Real World Data (RWD) ≠ Clinical Trial

- Environment
- Patients
- Objectives

But, not opposites … similarities exist

Should clinical trial endpoints be used in the RWD setting?

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- Yes and No
- Endpoints are needed that correspond to traditional, clinical trial endpoints
- Alternate and/or novel endpoints are also needed that may be more meaningful/appropriate/practical in an RWD setting
  - Especially given different objectives of clinical trials and studies using RWD

Endpoint Characteristics

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- Meaningful to regulators, payers, healthcare providers, and patients
  - Different endpoints for different end-users?
- Ability to measure and analyze in a valid, reliable/replicable, and methodologically robust manner
- Ability to measure in multiple data sources?
  - Within a given type of RWD (registry, electronic medical records [EMR], claims)
  - Perhaps across types of RWD
  - Linkage of RWD → opportunity to develop new endpoints

Endpoints corresponding to standard clinical trial endpoints

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- Overall Survival
  - Ultimate endpoint for benefit/risk decisions
  - Relevant and measurable in clinical trial and RWD settings
  - Not always feasible and/or timely
  - Impacted by cross-over
  - Doesn’t account for patient experience

Endpoints corresponding to standard clinical trial endpoints

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- Progression-free survival (PFS)
  - Surrogate for overall survival and/or measure of clinical benefit
    - Overcomes cross-over challenge
  - RECIST vs Clinician-assessed
    - Feasibility of formal RECIST?
    - Non-standardized assessment schedules

RECIST: Response Evaluation Criteria in Solid Tumors

Endpoints corresponding to standard clinical trial endpoints

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- Response rate
  - Evidence of biological activity
  - RECIST vs Clinician-assessed
    - Feasibility of formal RECIST?
  - Applicability beyond biological activity?
    - Time to response
    - Duration of response

Endpoints not typically used in clinical trial setting

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- Time to next treatment
  - with reason for change in treatment?
- Loss of clinical benefit
- Symptomatic endpoints
  - Patient-reported outcomes

Validation

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- Comparison to gold standard(s)
- Triangulation
- Correlation to other endpoints
  - Time to next treatment and Overall survival

Critical Next Steps

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- Opportunities (such as today) to hear perspectives of various interested parties
- Partnership on endpoint development and validation efforts
- Development of guidance documents/white paper on what endpoints are acceptable and when
- Demonstration projects

Final Thoughts

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- All of the above approach to endpoints in RWD setting
  - Overall Survival, Progression free survival, Response
  - Time to next treatment (with reason for change)
  - Opportunity to innovate (e.g., loss of clinical benefit)
- Collaboration will be key
  - Data owners
  - Users of data
  - Decision-makers (regulators, payers, healthcare providers, patients)
- Adapt - Leverage changing technology
  - human abstraction → Natural language processing (NLP)

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Doing now what patients need next