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New pathways towards evidence generation
In nephrology

CADTH Symposium
April 5, 2011
Vancouver, BC
Overview

1) Environmental scan – nephrology 2010
   - Are we lost?

2) An evidence generation program
   - Risk sharing
   - Conditional funding
   - Field evaluations

3) Summary, conclusions, next steps

Mendelssohn DC and Manns BJ
A Proposal for Improving Evidence Generation in Nephrology
AJKD (in press)
Evidence Based Nephrology Practice
Disney: Where Dreams Come True?
Framing our dilemma

- Dialysis is an imperfect therapy, with unacceptably bad outcomes and high cost
  - Minimal progress over the past 25 years
- Strictly applied EBM and CER are a challenge and a barrier to progress
- How can more and better evidence be generated, and how can the evidence that exists be used more effectively to advocate for better care and better outcomes?
- Can we find a way forward?
Consider an example: Biocompatible PD solutions

- Based on non-validated surrogate end points, these may preserve the peritoneal membrane, extend technique survival, and improve Q of L and hard clinical outcomes
- There is a steep price premium
- No formal large scale RCT is planned
- How can we achieve earlier access and a full evaluation?
Preclinical studies → Clinical studies → License

License → Field evaluation

Post regulatory period

Strictly applied evidence based medicine
Comparative effectiveness research
Public Funding
Post Regulatory Decisions

Strictly applied evidence based medicine

Comparative effectiveness research

Health Technology Assessment

Funding decisions by payers (government, CMS, insurers)

DENIAL OF PROMISING NEW THERAPIES
NEGATIVE IMPACT ON POTENTIAL INDUSTRY PARTNERS
TRADITION

JUST BECAUSE YOU’VE ALWAYS DONE IT THAT WAY DOESN’T MEAN IT’S NOT INCREDIBLY STUPID.

www.despair.com
New and costly diagnostics, drugs and therapies

- ESA’s
- Non calcium based PO4 binders
- Calcimimetics
- Drugs for glomerulonephritis
- Daily hemodialysis at home or in-centre
- Bicarbonate based PD solutions
- NGAL assay
- Future therapies?
What is the goal?

- To create an environment that fosters advances in CKD care
- To stimulate RCT’s and to increase evidence based nephrology practice
- To encourage industry to partner in this quest
- To satisfy national and provincial governments that we are reasonable in terms of access/cost tradeoffs
Darwin

It is not the strongest of the species that survives, nor the most intelligent that survives. It is the one that is the most adaptable to change.
The agency (CMS) has experimented with a policy of “coverage with evidence development,” which enables Medicare to cover the use of promising technologies for patients enrolled in studies that will better determine a technology’s risks and benefits.
In rare instances, for some items or services, CMS may determine that the evidence is very preliminary and not reasonable and necessary for Medicare coverage under section 1862(a)(1)(A), but, if the following criteria are met, CSP might be appropriate:

- a. The evidence includes assurance of basic safety;
- b. The item or service has a high potential to provide significant benefit to Medicare beneficiaries; and
- c. There are significant barriers to conducting clinical trials.
Ontario Health Technology Advisory Council (OHTAC)

- Internationally recognized leaders in this field
- Funded by MOHLTC
- Conditionally funded field evaluations
- External content experts (THETA, PATH, ICES)
- Observational studies, micro-economic models, randomized controlled trials, registries, and others
Performance-Based Schemes Between Health Care Payers and Manufacturers

Non-outcomes based schemes

Population Level
- Market Share
- Price Volume
- Utilization Caps
- Manufacturer Funded Treatment Initiation

Patient Level

Health Outcomes-Based Schemes

Conditional Coverage
- Coverage with Evidence Development (CED)

Performance-Linked Reimbursement (PLR)
- Conditional Treatment Continuation (CTC)
- Outcomes Guarantee
- Pattern or Process of Care

Non-outcomes based schemes

Population Level
- Market Share
- Price Volume
- Utilization Caps
- Manufacturer Funded Treatment Initiation

Patient Level

Coverage with Evidence Development (CED)
- Only in Research
- Only With Research

Conditional Treatment Continuation (CTC)
- Clinical Endpoint
- Intermediate Endpoint

Outcomes Guarantee
- Pattern or Process of Care

JJ Carlson et al. Health Policy 2010
Ontario Renal Network: Research and Innovation approaches?

Observational studies
- ORRS, DMAR
- ICES
- DOPPS

Field evaluations

Interventional studies
- RCT – patient level
- RCT – facility level

Comparative (Cost) effectiveness evaluations
Consider an example: Biocompatible PD solutions

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- There is a steep price premium
- No formal large scale RCT is planned
- How can we achieve earlier access and a full evaluation?
  - Field evaluation seems a promising route
Conclusions

1) Environmental scan – nephrology 2010
   - Innovation is well below what is needed, and seems likely to be even further constricted in the future

2) An evidence generation program (risk sharing and/or conditional funding and/or field evaluations)
   - This might align stakeholders and stimulate an environment that fosters innovation
   - CMS already has such a program in place
     - Ontario is a world leader in these methods, but as applied to technology, not drugs
     - Can this model be adapted and internationalized?

   - Will governments share the risks of innovation?
   - Will industry see the value in this approach?

Mendelssohn and Manns: A proposal to improve evidence generation in nephrology AJKD (in press)
Now let’s build a better mousetrap!
Feedback, please?