

Ethnomics, identity and agency in haemophilia care

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Health resources and impossibility

- Post WW-II promise of Utopia: human rights, social consciousness, civil rights, universal health care, rapid scientific advances and a new principlism.

BUT

- Dystopian reality
- Widening disparities, technology delivered little and delivered this to the wealthy well, collapse of society and rise of the multinational company

Dystopia, inequity and the rush of the future

- Inequity between and within countries
- Between: 90/10 in research and care
- Within: Aboriginal care (moral Katrina), elderly, black, women, chronic illness, public hospitals
- The rush of the future: genome mapped, 50% cancer pts alive at 5 yrs, diminishing returns, falling drugs, life-expectancy plateauing
- We cannot afford ourselves
- Despite progress all stakeholders dissatisfied and CAM increasing

Resource allocation

- All countries facing same challenge
- Increasing GDP expenditure on health does not resolve issues
- No successful resolution: UK, USA, Sweden, NZ, Norway
- Intractable
- We are now approaching the point beyond which we ought not go: Big expenditure for minimal gain (BMT in some situations) and the point at which we can not go (Society will not support no funding for environment, education etc)

Ethnomics

- To do nothing is untenable, HMOs don't work (supply-side), cannot stop research
- Ethnomics:
 - Establish community values
 - Establish discourse about costs, risk, limits
 - Accept economic principles: opportunity cost, rule of rescue, finite budgets
 - Reaffirm resource allocation as moral

Haemophilia care in the developed world

- Comprehensive care clearly established as a the best model of care
- Coordinated expertise better than individual care
- Strong government support (esp within systems of public care)
- Genetics
- Research
- Centrality of patient/advocacy groups
- Question of whether we need to reverse centralisation (AutoSCT)

Comprehensive care in a global context: I

- 18 million people die prematurely each year from curable or preventable medical conditions: resp infection, perinatal conditions, diarrhoea, TB, maternal conditions, meningitis and malnutrition
- 50,000 deaths each day
- >95% of these in developing world and among poor in these countries

Comprehensive care: II

- 800 million undernourished
- 1000 million lack safe water
- 2400 million lack access to basic sanitation
- 880 million lack access to basic health care
- 1000 million lack adequate housing
- 2000 million have no electricity
- Inverse care rule: those with the greatest burden of illness have the lowest level of care

Haemophilia Care in Developing World

- Failure to see the other
- Failure of globalisation as a moral concept
- Needs genuine consideration of global responsibilities
- Just because resources do not permit everything does not mean do nothing SO
- Stepwise (blood supply, infrastructure, generics, education, diagnosis, cryo)
- Recognise limits of evidence (context specific)

The rising cost of care

- 15 fold increase in FVIII expenditure over 15 years (5 million - 45 million)
- 5-6 fold increase in usage of FVIII

Why?

- Increased prophylaxis, more aggressive tolerising, more procedures, using more.
- Generally supported by Govt BUT as life expectancy normalises, incremental benefit falls restriction of usage and expenditure is inevitable and desirable (in communitarian terms).

Ethics of economic evaluation: I

- In determining cost effectiveness one must be clear what the outcome measures are and what the length of evaluation will be (whole life?)
- Outcomes: mortality, joints, QoL, happiness, sense of agency?
- Available studies suggest limited benefit of prophylaxis, some benefit of inhibitor management
- Effect of ‘outliers’ on data appears substantial (esp in studies of inhibitors)

Ethics and economics: II

- If life expectancy is increasing and QoL ‘normalising’ and resources finite.....then.....rationing will have to happen.
- Questions then become:
 - Mediating this discourse in the community
 - Preventing discrimination
 - Role of Haem Community and HCPs in setting limits, saying no, mediating anxiety and advocating for own interests.
 - ?Place of resource discussions in the clinical dyad?

Blood, infectious diseases and the limits of the Precautionary Principle

- Post-WWII belief that infectious diseases could be controlled.
- Antibiotic resistance, HIV, H5N1, SARS, vCJD have all dispelled this myth
- Likely emergence of new pathogens in the future
- Parvovirus mutations, circoviruses, zoonoses and prios all appear on the horizon
- BUT: Vigilance is expensive, cost-QALY ration is falling and risk seems unavoidable

Precautionary Principle

Precautionary Principle:

- Where one can reduce risk or avoid risk one should
- Public health concerns should take precedence over economic issues
- Decision-making should involve public
- Need to establish burden of proof (and decide at what point one should respond)
- Decision-making may be complicated by scientific uncertainty

Precautionary Principle, Ethics and Risk

- There is a point at which further expenditure will not reduce risks or anxiety about risk
- There is a point past which we CAN NOT or SHOULD NOT go
- Medicine is approaching these points (diminishing returns)
- Need a dialogue about the definition and measurement of risk, expectations of risk, the goals of health care and public health and the prioritisation of resource allocation.

Risk

How can risk be understood?

- As numerical estimate eg vCJD, HIV
- As uncertainty
- As risk factors
- As a possibility of harm
- As an unavoidable danger (inherent risk of blood)
- As narrative

Expression of risk may be:

- Expert vs lay
- Real vs perceived
- Quantitative vs qualitative (Objective vs subjective)

Somatic Cell Gene Therapy

- Intense interest in GT in Haemophilia
- Success of GT in SCID led to hopes for similar success in Haemophilia

BUT

- Development of ALL in 2 of 10 children on GT trial (1 dies) in 2002 (activity of LMO2 oncogene)
- Death of Jesse Gelsinger

Marked reduction in studies/clinical trials

Revision of eligibility criteria

Ethical review: utility? coercion? Private science? Safety data? Animal studies validity? Latency and F/Up

GT and the promisory note of hESC and SCNT

- hESC and SCNT provides possibility of valid disease models, drug screening, genomic screening NOW
- Possibility of GT and matched HSCT
- Legal in Australia 2006/7
- Role of disease/advocacy groups
- Role of church (Hx of objection to transfusion)
- Role of some scientists/clinicians

Prevention of haemophilia: PND/TOP and PGD/IVF

- Genetic diagnosis, reproductive counseling and ARTs now an accepted part of comprehensive care
- Prevention via TOP or selective implantation of embryos has great economic appeal. (?Preferred approach in developing world?)
- Not value neutral
- Identity (personal, community and familial)
- Stigmatisation
- Loss of narrative and heterogeneity
- ?Normal ?Ab/normal ?Dis/ease

Quality of life

- Quality of life clearly established as a goal of medicine
- QoL improved by treatment and prophylaxis in haemophilia
- Philosophical questions regarding definition, criteria and measurement
- Generic and Haem-specific measures
- Few studies properly assess QoL
- Limited Follow-up
- Absence of Qualitative data (relationships, existential concerns, agency, love, fulfillment, emergent concepts)

QoL and the Narrative

- Haemophilia patient, family and community has been central to change in treatment of haemophilia.
- Advocacy, foundations, consultancy
- Much involvement/advocacy has been related to blood safety and HIV/HCV
- These events have had a profound effect on the experience of illness and health care

Narrative(s)

Two trends:

1. Increasing recognition of importance of elucidating the narrative (esp as it is likely dynamic and influenced by the history of therapy, discovery and genetics and the progressive normalisation of people with haemophilia)
2. Increasing likelihood of restriction of choice (cost, HMOs)

MUST DO NARRATIVE RESEARCH: fear, control, difference, identity, genetic reduction, safety etc