Difficult decisions and clinical ethics support - paediatrics

- Case presentation – Jill Sewell
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‘Henry’ aged 3.5 years

- Rare progressive degenerative neurological disease
- No current treatment effective in reversing or delaying progression – symptom management only
- Ultimately fatal in later childhood (age of death 8-14); somewhat longer survival in children mechanically ventilated long-term (uncommon)

- Parents desperate to find a way to alleviate the disease progress and hopefully cure their son

Parents want neurologist to treat with new drug

Parents have been doing their research – have found out about a new drug

- 'Enzymeinase'
- Requires regular intrathecal injection
- Case reports of therapeutic effect in adults with similar disease (similar symptoms, but much later onset and less severe)
- No published evidence of such effect in children – but one doctor in US has a website reporting slowing of progression in 4 children (ages 5-8)
- Drug company commencing a Phase 1/2 clinical trial in US
- Very expensive – company information says US$600,000 per year

- Parents demand treatment
- Neurologist not convinced that there is any reasonable chance of therapeutic effect and is concerned about side effects eg fever, ECG abnormalities, vomiting, seizures, hypersensitivity
- Not part of established international practice

Parents request application for Special Access Scheme.

SAS requires medical practitioner application to TGA for individual patient eg:

- Critically ill patients requiring urgent care
- Drug initially provided in a clinical trial whilst awaiting marketing approval
- Drug available overseas, not in Australia

Application based on clinical justification, not monetary reasons

- Parents indicate they will try to find other ways to the drug themselves, if SAS approach doesn't work – will pay for it themselves if they have to

Clinical ethics referral

Presenting ethical questions

- Should neurologist agree to put in application to SAS, even though not personally believing that drug has good chance of benefit?

- If SAS application is not done or refused, and parents raise the money to pay for the drug themselves, should neurologist agree to administer it at this hospital?

Question to audience 1

Should neurologist agree to put in application to SAS, even though not personally believing that the drug is appropriate for the child?

A. Definitely not

B. Agree to put in application, but framed relatively negatively

C. Yes, agree and frame in strongly positive terms

D. Not sure

Question to audience 2

If SAS application is not done or refused, and parents raise the money to pay for the drug themselves, should neurologist agree to administer it at this hospital?

A. Yes, this is the solution!
B. Yes, but only if parents pay for costs of hospitalisation as well as drug
C. No – at a public hospital, this is not appropriate, because of equity considerations
D. Not sure

Late-breaking complication

- Drug company offers parents compassionate supply (no charge) for 6 months – just need hospital to agree to administer the drug
- After 6 months, if evidence of benefit, drug company will continue supply at discounted price, if hospital pays 50%.

Question to audience 3

Should the drug company offer be accepted?

A. Yes, if neurologist thinks appropriate
B. Yes, but only if hospital executive agrees
C. No
D. Not sure

Ethical issues

- Treatment with unproven therapy
- Family may suffer eg bankrupt
- Equity of access to care (in public hospital setting)
- Individual child rights
- Opportunity costs to other patients with use of hospital resources
- ? drug company manipulation

The Children's Bioethics Centre provides

Education and training

Specialised training to help clinicians talk with parents about their child's life-threatening illness.

We provide clinical ethics education to doctors, nurses and allied health workers.

Research

An evidence base about respectful decision making, best clinical practice, how children and families react in difficult situations, the best ways to help families, children and the medical team, and what children want to know about their health care.

Clinical Ethics Service

Timely advice to clinicians faced with difficult decisions.

Quality

Expert advice, ethical opinions, position statements, recommendations, procedures and guidelines involving clinical ethical issues.

Leadership

Leadership in clinical ethics issues involving children – locally, nationally and internationally.

Clinical ethics service

- **Informal clinical ethics advice** – patient or professional matters
- **Clinical ethics case consultation** – formal documented ethics guidance, via meeting with Clinical Ethics Response Group (CERG)
- **Facilitated ethics discussions** – teams and departments – aim to resolve conflict, facilitate communication, ease moral distress

Clinical Ethics Case consultation

- Referred by any clinician, usually senior medical consultant
- CERG convened (can be within 24 hrs)
- Collaborative discussion over 1 hour
- Clinical team, CBC team, members of CERG from across the campus
- Medical background and ethical issues of concern in referral document

CERG

- Clarify medical issues
- Draw out nature of ethical issues
- Identify range of options
- Identify, weigh up ethical pros and cons of each option
- Identify ethically appropriate option(s)
- Advise clinician/team (non binding)
- Written report to referring clinician