WECAN SmartStart in Cancer Patient Advocacy

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How to INFLUENCE reimbursement decisions as a patient advocate?

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Who makes what decision: Regulator

Regulator: European Medicines Agency (EMA)

- Aims to establish if **benefits > risks**

  Regulation (EC) No. 726/2004

- Demonstrate **safety** (e.g. toxicity) and **efficacy** (e.g. survival, quality of life)
- No need to show the best choice (or relative efficacy)
- Economics not considered
- Approved indication – a specific population where benefit has been shown

Who makes what decision: HTA/Payer

HTA/Payer (e.g. NICE)

- Reimbursement decisions made on a national level
- Different challenges across Europe – based on healthcare budgets, GDP, political willingness to pay
- As a result different treatments are available to patients in different EU countries (because they are unaffordable if not reimbursed)
- HTA: Is it a cost-effective use of resources?
- Payer: Willingness and ability to pay? What is the budget impact?

NICE
National Institute for Health and Care Excellence

Payer v HTA: Affordability v Cost-Effectiveness

- Cost-effectiveness: value for money (cost v benefit)
  - QALY = Quality-adjusted life year

- Affordability: budget impact (total cost)

Example: Zolgensma (spinal muscular atrophy)

- Potentially curative treatment, offers significant QALY gains (many years of potential benefit)
- “world’s most expensive drug” - $2.1 million
- It may be cost-effective, but is it affordable?
- How does the system afford to pay for 275+ cell and gene therapies in development?

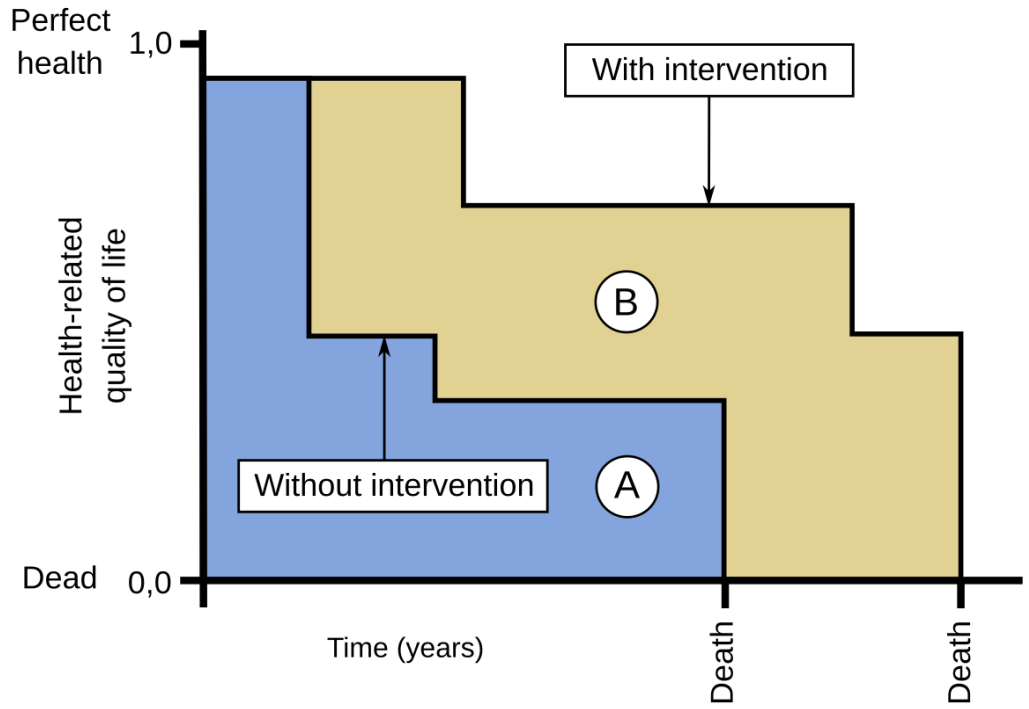
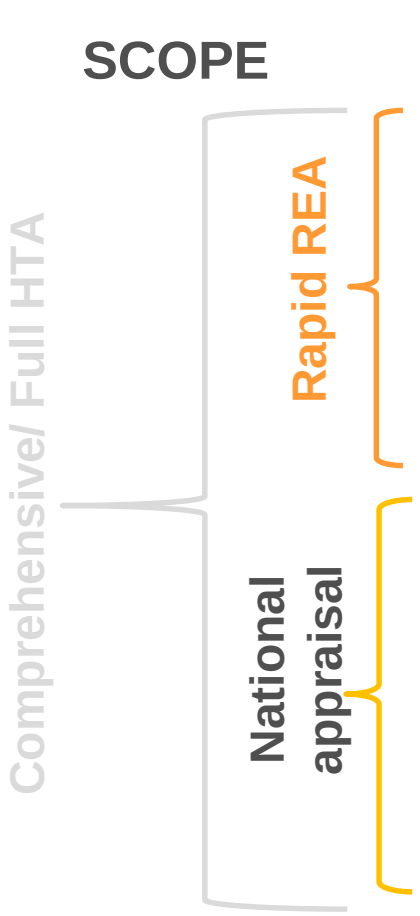


Image: www.publichealthnotes.com/qaly-quality-adjusted-life-years/ (Accessed 12.06.19)

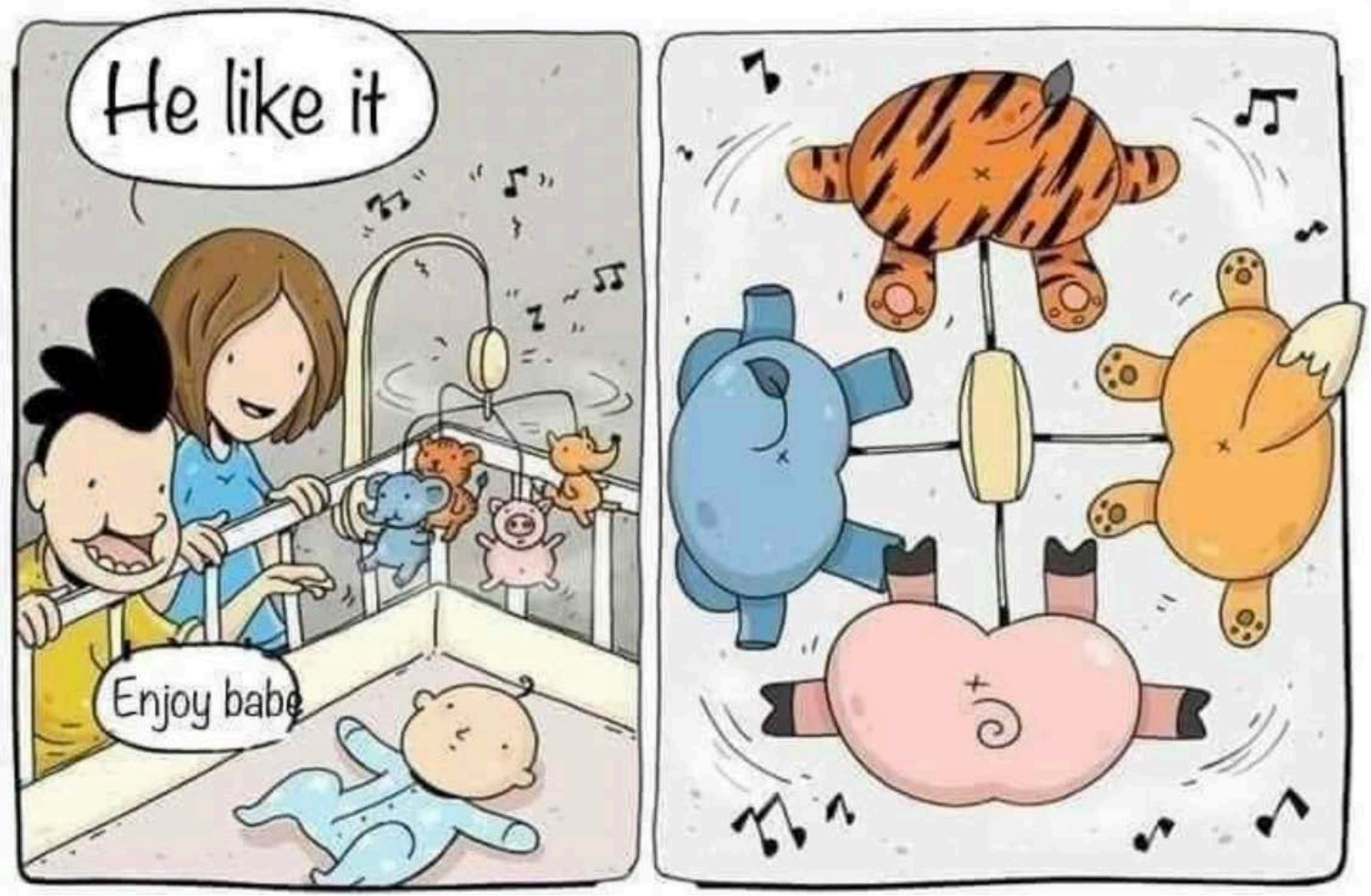
What do they consider in HTA? EUnetHTA HTA Core Model



HTA Core Model DOMAINS
1. Health problem and current use of technology
2. Description and technical characteristics
3. Safety
4. Clinical effectiveness
5. Costs and economic evaluation
6. Ethical analysis
7. Organisational aspects
8. Patient and social aspects
9. Legal aspects

Slide adapted from EUnetHTA talk at EHA, June 2019

Why do we need patient involvement in HTA?



What is the role of patient advocates in HTA?

From the perspective of the HTA agency:

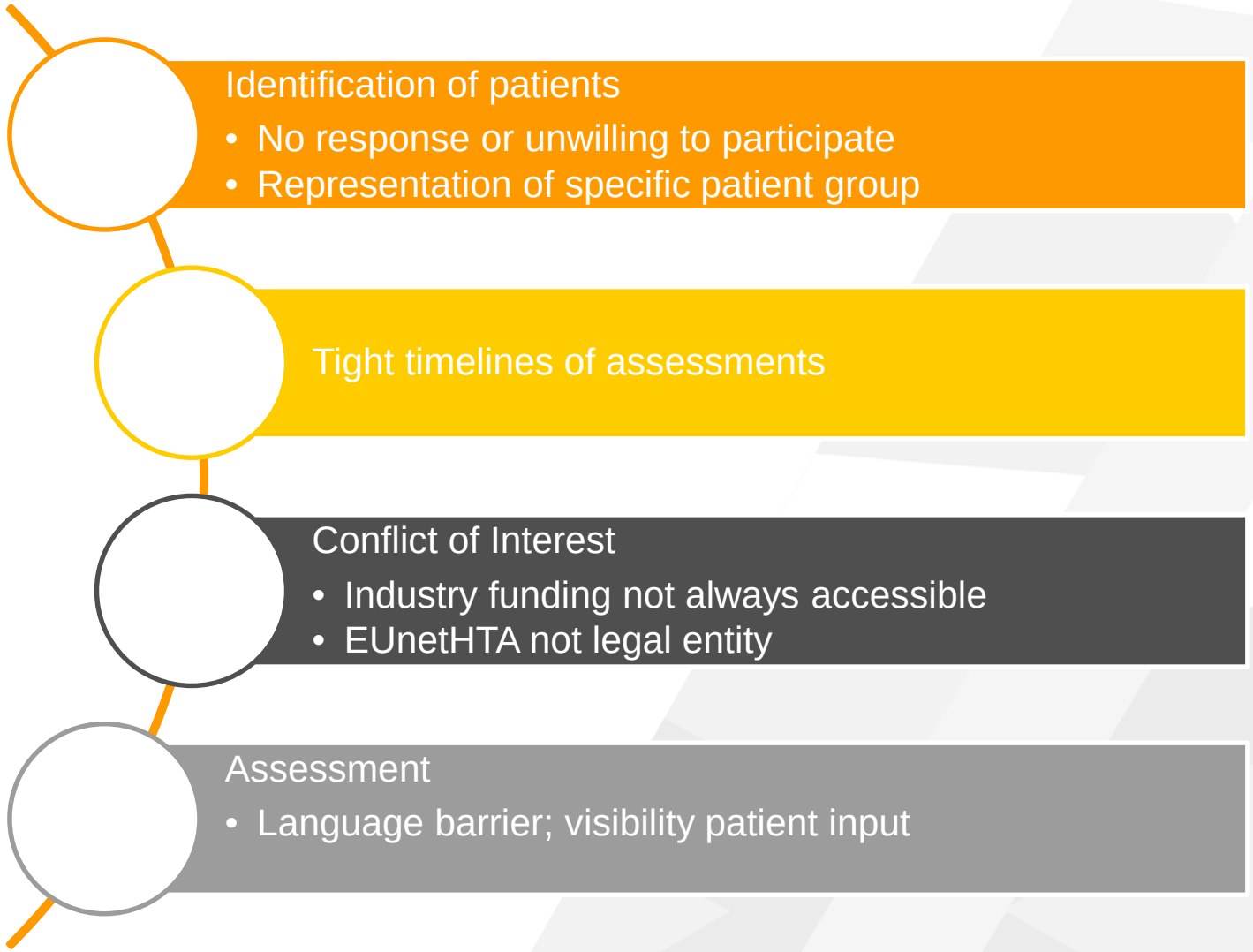
"NICE's approach to patient and public involvement is based on two key principles:

- that lay people, and organisations representing their interests, have **opportunities to contribute** to developing NICE guidance, advice and quality standards, and support their implementation, and
- that, because of this contribution, our guidance and other products have a **greater focus and relevance** for the people most directly affected by our recommendations."

https://www.nice.org.uk/about/nice-communities/nice-and-the-public/public-involvement/public-involvement-programme/patient-public-involvement-policy (Accessed 12.06.19)

Why do we as patient advocates want to be involved in HTA?

Challenges with patient involvement (from an HTA perspective)



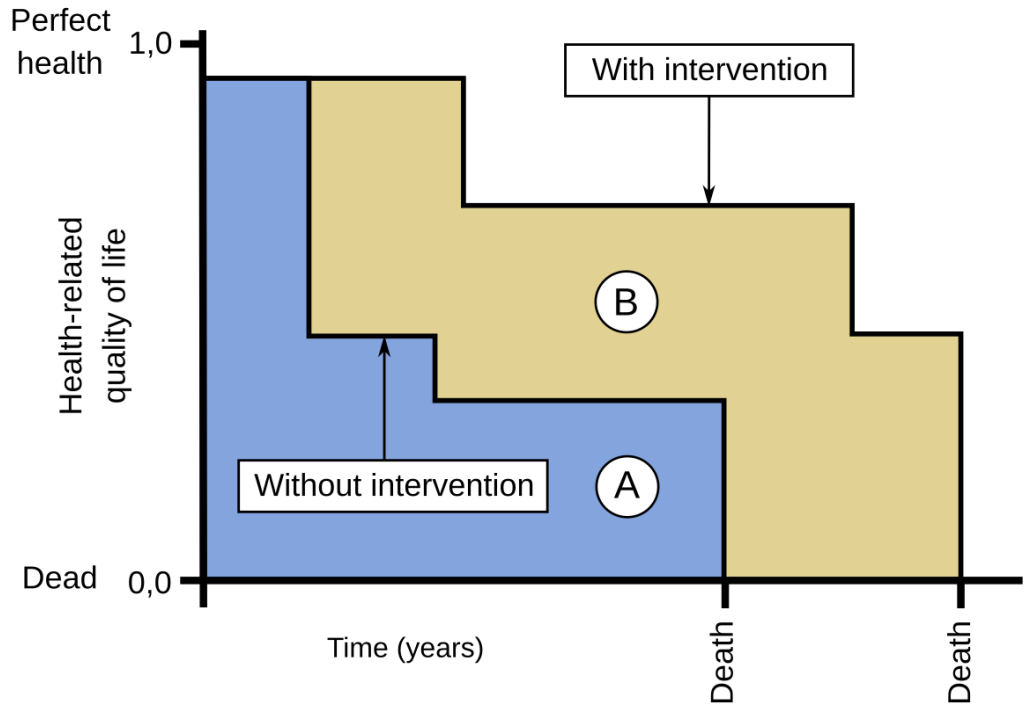
A comparison of the different UK processes

	NICE	SMC	AWMSG
Scoping	✓	X	X
Technical Engagement	✓ (NEW)	X	X
Evidence Submission	✓	✓	✓
Patient Focused Meeting	X	✓ PACE (For rare and end of life medicines)	✓ CAPIG (For rare diseases only)
Committee Meetings	✓	✓	X (Public Gallery)
Opportunity to Appeal	✓ (ACD and FAD)	X	X
Publication	✓	✓	✓

NICE – National Institute for Health and Care Excellence; SMC – Scottish Medicines Consortium; AWMSG – All Wales Medicines Strategy Group

Does patient involvement have an impact?

- Patient Organisations and Patient Experts “do not feel that their efforts to contribute to the process are seen as being credible by NICE committees” (NICE Patient Group Workshop, Jan 19)
- Patient testimony is usually qualitative (e.g. patient testimony), so the impact on decision making is not usually obvious (NICE and Myeloma UK, Measuring Patient Preferences, June 19)
- Where is the opportunity to impact in a QALY based system?
  - Survival X
  - Quality of Life ?



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Case Study: The UK Process

NICE TA541 - Inotuzumab ozogamicin for treating relapsed or refractory B-cell acute lymphoblastic leukaemia

Case Study:

NICE TA541 Inotuzumab ozogamicin

Scoping: Reviewed (November 2016)

Submission: Evidence of patient views (February 2017)

Committee Meeting: Patient Expert (May 2017)

ACD: Responded (June 2017)

Committee Meeting: No opportunity for involvement (July 2017)

National Institute for Health and Care Excellence, TA 541: Inotuzumab ozogamicin for treating relapsed or refractory B-cell acute lymphoblastic leukaemia (19 September 2018) https://www.nice.org.uk/guidance/ta541

Case Study:

NICE TA541 Inotuzumab ozogamicin

FAD: Not recommended (August 2017)

Appeal: Submitted Documentation (September 2017)

Appeal: Hearing (November 2017)

Appeal: Successful (December 2017)

Committee Meeting: Postponed for further evidence (February 2018)

National Institute for Health and Care Excellence, TA 541: Inotuzumab ozogamicin for treating relapsed or refractory B-cell acute lymphoblastic leukaemia (19 September 2018) https://www.nice.org.uk/guidance/ta541

Case Study:

NICE TA541 Inotuzumab ozogamicin

Committee Meeting: No opportunity for involvement (April 2018)

ACD: Responded (May 2018)

Committee Meeting: No opportunity for involvement (July 2018)

FAD: Recommended for use (December 2017)

Guidance: Recommended for use (September 2018)

National Institute for Health and Care Excellence, TA 541: Inotuzumab ozogamicin for treating relapsed or refractory B-cell acute lymphoblastic leukaemia (19 September 2018) https://www.nice.org.uk/guidance/ta541

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Case Study: Unmet Need

Group Task

Case Study: Unmet Need

- A new drug has been developed to treat a rare cancer, for newly diagnosed patients with a specific mutation. Median age at diagnosis is 68.
- The mutation is hard to treat, a predictor of poor prognosis (aggressive disease and shorter survival)
- High chance of relapse, five-year survival of 20% in historical trials
- This drug is an oral treatment that is added to standard of care chemotherapy (very intensive, highly toxic, inpatient treatment)
- For newly diagnosed patients in the clinical trial expected overall survival (with standard of care) is 25 months compared to 75 months with the new drug
- Common side-effects of the new drug (in more than 25% of patients): nausea, vomiting, headache, bleeding, muscle and bone pain, nosebleeds, infection and high blood sugar.

Case Study: End of Life – Task (10 minutes)

- How would you argue the benefits to make this treatment available to patients?
- What **topics** should you consider?
- As a group, prepare a 1 - 2 minute argument for **why this drug is needed**

Case Study: End of Life – Task – Example Topics

Condition

- Incidence, Prevalence and Mortality
- Common symptoms
- Diagnosis – Emergency, What is it like
- Impact – Emotional, Financial, Practical (Pain, Mobility, Self-Care)

Treatments

- Views on current treatments (and their side-effects) and prognosis
- Advantages of the new treatment
- Disadvantages of the new treatment (e.g. side effects)
- Other – Innovation

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Case Study: End of Life

Group Task

Case Study: End of Life - Task

- A new drug has been developed to treat a rare cancer, for relapsed/refractory patients with a specific mutation
- In this setting expected survival (with standard of care) is 4.7 months, there have been no new treatments for decades (but there are lots in development).
- The new drug has a survival benefit of 2.5 months (total of 6.2 months)

Problem

- NICE has a standard approval threshold of £20,000 - £30,000 per QALY
- NICE has a higher threshold of up to £50,000 for End of Life treatments, with criteria:
  - *Short life expectancy – normally less than 24 months*
  - *Extension to life – normally at least a further three months*
- It may only be cost effective if it meets End of Life criteria

Case Study: End of Life – Task (10 minutes)

- **Assume** that this new treatment is of value to patients
- How would you argue the benefits to make this treatment available to patients?
- As a group, prepare a 1 - 2 minute argument for **why this drug should meet End of Life criteria**

Case Study: End of Life – Example Argument

- **How would you argue the benefits to make this treatment available to patients?**

1) ***Acknowledge Issue*** *– survival benefit falls short of the normal requirement*
2) ***Explain*** *– relative and absolute survival benefits. In this setting an increase of 2.5 months equates to a survival improvement of 30%, compared to 12.5% (3 months improvement in a setting where expected survival is 24 months).*
3) ***Precedence*** *- from TA476/paclitaxel (pancreatic cancer) where the guidance states that "the survival gain was particularly important relative to the average survival of people with this condition, and therefore this criterion could be accepted as met in this circumstance" (in TA476 there was a 2.4 month mean improvement).*
4) ***Policy and Process -*** *relapsed/refractory AML is clearly an end of life setting, it would be* ***unfair*** *and* ***unreasonable*** *if the criterion was not applied in this circumstance.*
5) ***Unmet Need*** *- There is an urgent need for access to treatment options available to improve survival for these patients.*