Ethical and Social Policy Considerations of Novel Techniques for Prevention of Maternal Transmission of Mitochondrial DNA Diseases: Workshop

Toleration of Uncertainty

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April 1, 2015
Conflicts of interest

• No financial relationships with any drug or medical device companies
• Research funded by FDA Office of Generic Drugs and Division of Health Communications
• Greenwall Faculty Scholar in Bioethics
Uncertainty tolerance in new product approval

- Uncertainty vs. severity of disease being treated
  - Parallel track
  - Coronary artery stents

- Uncertainty vs. systems to promote reasonable use of product
  - Humanitarian Device and local IRB approval
  - Tysabri and risk management plan

- Uncertainty vs. underlying science
  - Lack of knowledge in one area addressed by better knowledge in other areas
Uncertainty and trials of MRT

• 1. Importance of disease context

• 2. Ensure systems in place to manage uncertainty and quickly adapt once new knowledge is available

• 3. Clarity about underlying science
Promoting autonomous decision-making

• Important to take patients’ views into account at each step of this discussion
• Individual vs. societal goals
• Hard to make informed decisions with limited data on experimental technologies
Conclusions

• Address uncertainty with rigorous testing with FDA oversight
• Sliding scale approach to uncertainty tolerance
• Promote autonomy as much as possible but understand it’s in no one’s interest to have unfettered access to ineffective and potentially unsafe technology
Thank you
Q9. As I said before, the techniques to avoid mitochondrial disease would involve altering the make-up of an egg or embryo, specifically the mitochondria. The donated healthy mitochondria would replace the intended mother’s faulty mitochondria and would then be passed down to the child and, in turn, to that child’s children and beyond. This is called germline gene therapy, because the change goes down through the generations (the germline). Assuming that scientists could show that this is safe, what is your reaction to this?

Very positive
Fairly positive
Neither positive nor negative
Fairly negative
Very negative
Don’t know

Q10. Currently, these techniques cannot be offered to couples as the law only allows them to be carried out in research. However, Parliament may have an opportunity to change the law to allow these techniques to be offered to couples. If Parliament did change the law, who do you think should decide whether individual couples should have the treatment?

An expert regulator should decide on individual cases
An expert regulator should approve particular clinics to offer the treatment, with medical specialists deciding who to offer it to
Couples themselves should make this decision (in consultation with their doctor), without the involvement of an expert regulator
Figure 3. Awareness of mitochondrial disease: overall, by social class and educational level
Figure 5. Attitudes to the regulation of genetic treatments

- Couples themselves should make this decision (in consultation with their doctor) without the involvement of an expert regulator: 36%
- An expert regulator should decide on individual cases: 20%
- An expert regulator should approve particular clinics to offer the treatment, with medical specialists deciding who to offer it to: 19%
- Don't know: 25%