Lessons from pediatric gene transfer research: Implications for novel mitochondrial techniques

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Pediatric gene transfer research lessons for novel biological interventions

• Oversight of clinical trial transition
• Consider the ideal condition/population
  • Limited options
• Minimize expectations of benefit for participants
• Expect harms will occur, even with efforts at minimization
• Involvement of community in planning research
• Anticipate professional and financial conflicts of interests
• Consider direct assessment of parental understanding as part of consent process
• Consider a name for the research that addresses concerns about expectations
Specific questions

• Balancing scientific uncertainties and potential benefits and harms
  • Oversight of transition and study design, population selection, and assessment of consent

• Avoiding problematic down stream applications
  • The somatic/germline distinction was used as simple way to provide reassurance but may not be conceptually appropriate

• Ensuring fairness, equity and access to interventions
  • Existing clinical interventions after 25 years but this challenge is generic