



**A short survey of perceptions of gene
therapy in FA-affected individuals & families**

Mr Thomas Carroll
Fanconi Hope Charitable Trust UK

- Clinical trial design best practice includes user involvement/consultation.
- Fanconi Hope in conjunction with the US-based Fanconi Anaemia Research Fund set up an online survey to explore FA families' perceptions of gene therapy.
- The survey was publicised through US, UK, German and other FA groups in Sept/Oct 2012 with a resulting 69 respondents.
- The data collected will provide Researchers with 'user' derived material to demonstrate consideration of users opinions in clinical trial design and in drafting of clinical trial participant information.
- This presentation and full survey results can be found with these QR Codes or at www.fanconihope.org (at <http://goo.gl/vF8fq>).



This Powerpoint
Presentation



Full Survey
Results



Survey Questions

- Q1: To what extent are you aware of the success/failure of gene therapy in other genetic disorders?
- Q2: What issues would you/your child want clarified if you were to consider participation in a gene therapy clinical trial for Fanconi Anaemia?
- Q3: Do you/your child have any worries about gene therapy in Fanconi Anemia?
- Q4: What are your thoughts about having some bone marrow stored while you/your child is well and years ahead of needing treatment for bone marrow failure?
- Q5: What do you think about a viral vector with the corrected gene being directly injected into you/your child in contrast to the current clinical trials where the bone marrow cells are taken out, corrected in the lab, and then put back?
- Q6: Are you aware of any possible side effects of gene therapy where it has been tried in other conditions?



Introduction: 'Experimenting with patients, not on patients'

- Clinical trial design best practice includes user involvement/consultation.
- UK
 - 'Public Involvement in Clinical Trials'...INVOLVE project, National Institute of Health Research
- EU
 - 'Eurordis Charter for Clinical Trials in Rare Diseases'

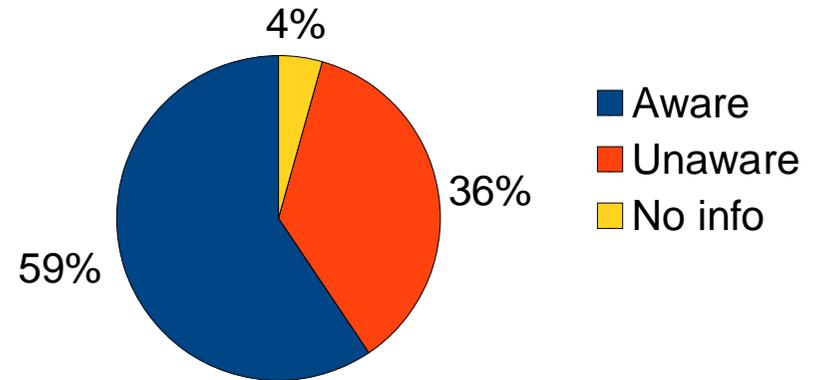


Objective

- To explore FA families perceptions of gene therapy
- And thus to provide Researchers with 'user' derived material to demonstrate consideration of users opinions in clinical trial design and in drafting of clinical trial participant information

- Non-validated questionnaire
- Six questions
- Deliberately open-ended questions with free text fields
- Online Survey Monkey Questionnaire
- Publicised through US, UK, and German and other FA groups...Sept/Oct 2012
- 69 respondents

- **To what extent are you aware of the success/failure of gene therapy in other genetic disorders?**



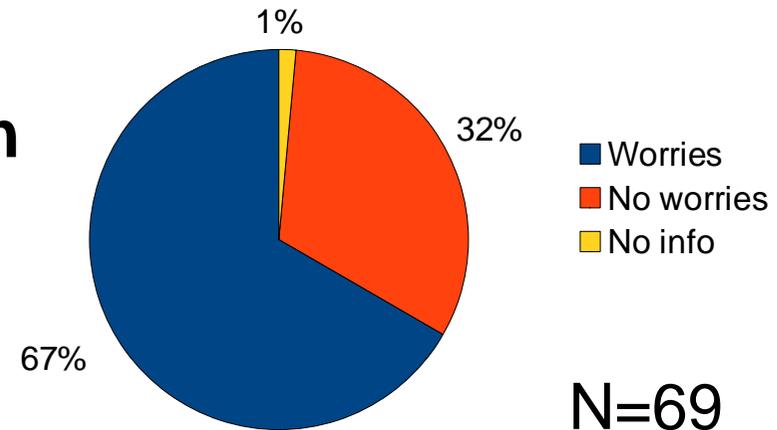
N=69

- **Selected comments:**

- 'I track progress through the use of Google Alerts'
- 'I am not aware of any successful gene therapy'
- 'My children completed gene therapy and it was unsuccessful in FA but worked in other diseases'
- 'Have been following gene therapy since the first successful treatment of SCID'

- **What issues would you/your child want clarified if you were to consider participation in a gene therapy clinical trial for Fanconi Anaemia?**
- **Selected comments:**
 - 'does it simply cure the bone marrow failure or the disorder itself?'
 - 'if the gene therapy did not work, would that prevent later treatment with a bone marrow transplant?'
 - 'the probability of side effects, the risks of cancer, ...the timing of the trial for my son's condition'
 - 'not have to travel halfway across the bloody world to do it'
 - 'risks, costs, time away from home, benefits'
 - 'pain, longevity, intentions, cost, reimbursement'
 - 'would any bone marrow ablation be performed prior to gene therapy...to create space in the bone marrow?'
 - 'would you still have to use immune suppression drugs...would this help with cancer issues later on in life?'

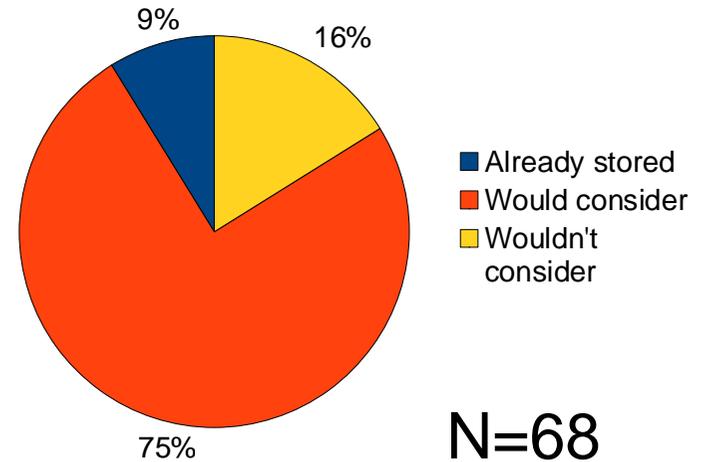
- **Do you/your child have any worries about gene therapy in Fanconi Anemia?**



- **Selected comments:**

- 'I am more worried that you won't have sorted out this gene therapy business by the time my son needs his bone marrow transplant'
- 'Will it cause cancer, will it work, will it be too expensive?'
- 'the future problems caused by gene therapy'
- 'this seems to be in theory (at least for now) far safer than a bone marrow transplant'
- 'previously cancer has been reported when using gene therapy. FA patients are particularly prone to develop cancer. What if gene gene therapy increases this risk?'

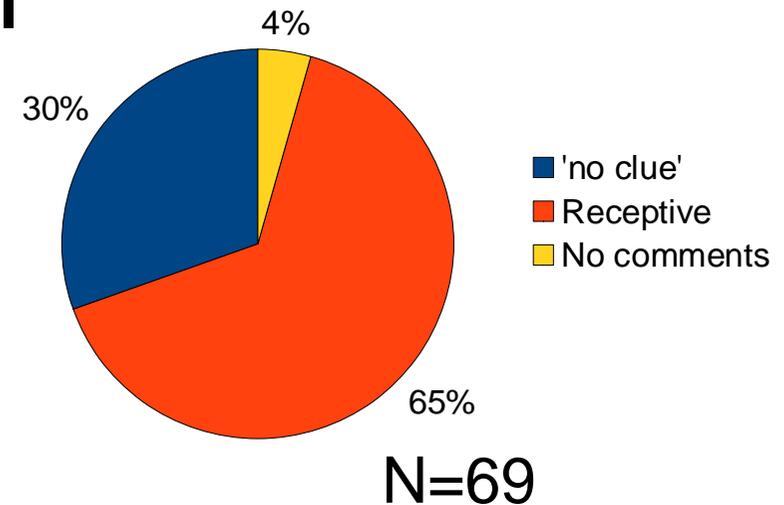
- **What are your thoughts about having some bone marrow stored while you/your child well and years ahead of needing treatment for bone marrow failure?**



- **Selected comments:**

- 'We were told...that although marrow freezes well, most FA cells don't survive the thawing process...it is worth the risk of harvesting marrow especially if the patient needs all of the marrow they can use at that time'
- 'sounds great but expensive'
- 'we have done this already'

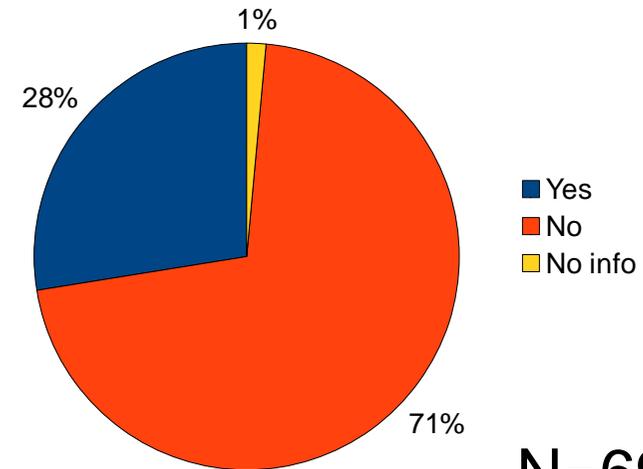
- **What do you think about a viral vector with the corrected gene being directly injected into you/your child in contrast to current clinical trials where bone marrow cells are taken, corrected in the lab, and then put back?**



- **Selected comments:**

- 'No clue to answer this question'
- 'scarier simply because the idea of a viral vector being injected directly into my body sounds a bit dramatic'
- 'it's a little unsettling but the basic science does make sense. It's a risk worth taking'

- Are you aware of any possible side effects of gene therapy where it has been tried in other conditions?



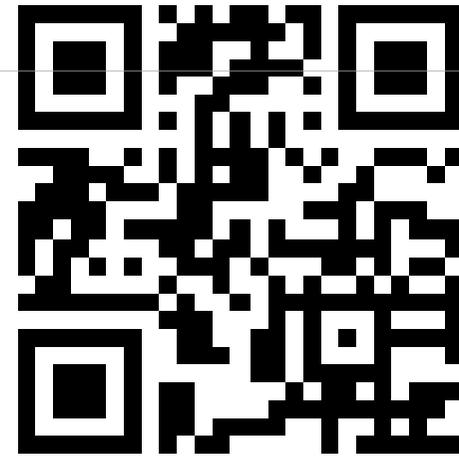
- **Selected comments:**

- 'I don't have sufficient knowledge'
- 'not long term cure, had to keep redoing treatments'
- 'leukaemia in the SCID trials'
- 'cancer and leukaemia'
- 'I am not aware of any'
- *9 free text comments specifically listed leukaemia*

- Full Survey Results are freely obtainable from Mr Thomas Carroll, tac@fanconihope.org or via our website at www.fanconihope.org (at <http://goo.gl/vF8fq>) or via these QR Codes



This Powerpoint Presentation



Full Survey Results