Title

Motivations and Decision Making Processes of Men With X-linked Retinoschisis Considering Participation in an Ocular Gene Therapy Trial

Authors

Turriff, Amy
Blain, Delphine
Similuk, Morgan
et al.

Publication Date

2019-08-01

DOI

10.1016/j.ajo.2019.03.009

Peer reviewed

HHS Public Access

Author manuscript

Am J Ophthalmol. Author manuscript; available in PMC 2020 August 01.

Published in final edited form as:

Am J Ophthalmol. 2019 August ; 204: 90–96. doi:10.1016/j.ajo.2019.03.009.

Motivations and Decision-Making Processes of Men with X-linked Retinoschisis Considering Participation in an Ocular Gene Therapy Trial

Amy Turriff[1,a], **Delphine Blain**[1], **Morgan Similuk**[2], **Barbara Biesecker**[3,b], **Henry Wiley**[4], **Catherine Cukras**[4], and **Paul A. Sieving**[5]

[1]Ophthalmic Genetics & Visual Function Branch, National Eye Institute, National Institutes of Health, 10 Center Drive MSC 1860, Building 10, Room 10N109, Bethesda, MD 20892, USA

[2]Laboratory of Immunology, National Institute of Allergy & Infectious Diseases, National Institutes of Health, 10 Center Drive, Building 10, Room 11C207, Bethesda, MD 20892, USA

[3]Social & Behavioral Research Branch, National Human Genome Research Institute, National Institutes of Health, 31 Center Drive, Building 31, Room B1B36, Bethesda, MD 20892, USA

[4]Division of Epidemiology & Clinical Applications, National Eye Institute, National Institutes of Health, 10 Center Drive MSC 1204, Building 10, 10CRC, Room 3-253, Bethesda, MD 20892, USA

[5]Director, National Eye Institute, National Institutes of Health, 31 Center Drive MSC 2510, Bethesda, MD 20892, USA

Abstract

Purpose: To describe the motivations, expectations, and other factors men with X-linked retinoschisis (XLRS) consider when making decisions to participate in an early-phase ocular gene therapy clinical trial.

Design: Qualitative interview study

Methods: Men with XLRS who were considering participation in a Phase I/IIa ocular gene therapy clinical trial at the National Eye Institute were eligible for this study. Trial participants (n=9) were interviewed prior to receiving the gene transfer and then 3 and 12 months later. Trial participation decliners (n=2) were interviewed at an initial visit and 12 months later. Those screened for the trial and found ineligible (n=2) were interviewed at an initial visit only. Interviews were transcribed, coded, and analyzed thematically.

[a]Correspondence: Amy Turriff, NIH, National Eye Institute, 10 Center Drive MSC 1860, Building 10, Room 10N109, Bethesda, MD 20892 USA, Phone: 301-402-4175, Fax: 301-402-1214, turriffa@mail.nih.gov.
[b]Now at RTI International, 701 13th Street NW, Suite 750 Washington, DC, 20005, USA

B. Financial Disclosures: None of the authors has financial disclosures.

Results: Interview participants described decision-making factors as risk-benefit assessments, personal intuition, trust in the study team, and religious faith. Altruism and the potential for therapeutic benefit were the main motives for trial participation, whereas the uncertainty of risks and benefits was the reason two men declined participation. While most participants hoped for direct benefit, no one expected to benefit. Almost all interview participants considered their decision straightforward and were satisfied with their decision when interviewed over time. Meaningful relationships with the study team and perceived secondary benefits to participation contributed to positive trial experiences.

Conclusions: Engaging prospective research participants in discussion about their hopes, expectations, and personal factors provides a more complete understanding of patient decision-making and may help support informed choices to participate in clinical trials for XLRS.

Introduction

Significant advances in ophthalmic research have led to a rapid increase in the number of clinical trials for inherited eye conditions, as well as the first approved gene therapy treatment in the United States for an early-onset retinal degeneration[1]. In our experience, patients are keenly aware of and hopeful about clinical trials. The main purpose of most early-phase studies is to determine the safety of an intervention rather than efficacy; therefore, participation in an early-phase trial is usually a preference-based, patient decision rather than a medical recommendation. For decades there has been discussion as to why research participants would voluntarily choose to enroll in studies in which there may be considerable risks but very little chance for direct therapeutic benefit[2–4]. Although studies have been conducted in a variety of disease contexts[5–10], little is known about the motivations and decision-making processes of individuals with inherited eye conditions who are considering participation in clinical trials. A prior study of five patients with *RPE65*-associated retinal degeneration who were potential candidates for a gene therapy trial found that patients demonstrated a range of expectations for benefit, high informational needs, and a desire for shared decision-making[11].

X-linked retinoschisis (XLRS) typically presents as reduced visual acuity in boys. In many cases, it is slowly progressive, and adults maintain functional vision throughout their lifetime[12]. However, activities that rely on central visual acuity, such as reading and driving, are usually impacted and are a significant source of concern for individuals with XLRS. The purpose of this study was to understand the motivations, expectations, and decision-making processes of men with XLRS who were considering participation in an early-phase ocular gene therapy clinical trial. Understanding individuals' motivations and decision-making processes to participate in clinical trials will help investigators facilitate recruitment, enhance shared decision-making and the informed consent process, and improve trial experiences.

Methods

Men with XLRS who were considering participation in a Phase I/IIa *RS1* ocular gene therapy clinical trial (www.clinicaltrials.gov:) at the National Eye Institute (NEI), National Institutes of Health (NIH), were invited to participate in a prospective, longitudinal,

qualitative interview study. Details of the clinical trial have been described previously[13]. This study was approved by the NIH Combined Neuroscience Institutional Review Board and adhered to the tenets of the Declaration of Helsinki. Informed consent was provided by all interview participants.

Two investigators unaffiliated with the gene therapy trial (DB and MS) conducted semi-structured interviews between February 2015 and December 2016. Clinical trial participants ("Joiners") were interviewed at an initial visit, and then at 3 months and 12 months after the gene transfer injection. Individuals who declined trial participation ("Decliners") were interviewed at an initial visit and 12 months later, and those who were screened but ineligible for the trial ("Ineligibles") were interviewed at an initial visit only. All of the initial interviews took place after participants had reviewed the informed consent document with the clinical trial team and had decided whether to participate in the trial or had learned about their ineligibility. The interview guide (available upon request) was adapted from Peay *et al*'s[7] approach to exploring clinical trial decision-making and explored motivations, hopes, expectations, decision-making processes, and trial experiences.

Interviews were audio-recorded and transcribed for analysis. Transcripts were checked for accuracy. A preliminary codebook was developed based on *a priori* topics, including understanding of trial purpose, perceived risks and benefits, motivations, expectations, and influences on decision-making. Two investigators (AT and DB) reviewed all transcripts thoroughly and added to the codebook based on topics emerging from the interviews. A subset of initial transcripts was independently coded (AT and DB) using QSR International's NVivo 11 qualitative analysis software. Discrepancies in coding were discussed between the investigators until successfully reconciled and minor revisions to the codebook were made. The remaining transcripts were coded by investigators (AT and DB) using the revised codebook. Thematic analysis was used to interpret coded findings. Data on each code was analyzed to find repeated patterns of meaning and to sort codes into potential themes[14].

Results

This study included nine Joiners, two Decliners, and two Ineligibles. The median age was 47 (range 23-72 years). Most participants had a college (n=4) or post-graduate degree (n=7). Twelve participants were white and non-Hispanic, and one participant was Hispanic. The median best-corrected visual acuity was 20/63 (range 20/50-20/160) in participants' better-seeing eye and 20/100 (range 20/63-20/320) in participants' worse-seeing eye. All men invited to participate consented to the interview study. The results of a subset of the data analysis are summarized in Tables 1 and 2.

Understanding of Trial Purpose and Motivations for Participation

Most participants relayed that the purpose of the trial was to evaluate the safety and potential efficacy of gene transfer. Three Joiners, one Decliner, and one Ineligible mentioned efficacy only as the trial's purpose. However, these responses reflected participants' understanding of the ultimate purpose of conducting a clinical trial, as opposed to the purpose of the initial phase I/IIa. None of the participants misunderstood the trial's purpose as offering a proven treatment.

Altruism and the potential for benefit were the main motivations for trial participation. Men wanted to help future generations in their family and others with XLRS, especially those who may be more severely affected. “I’ve got this condition. I’ve lived with it, and I also understand I’m in a much better position than a lot of other people…so if I can give back in some way it would be great” (Joiner, 007). Participants were also motivated to help advance research for XLRS. Several men described the personal meaning derived from their altruism as outweighing potential risks to participation. “Even if I went blind in my eye…I would be helping the NIH move forward in helping other people [with XLRS] and learning from mistakes” (Joiner, 008). Most men were motivated by potential therapeutic benefit, which they defined as an improvement or stabilization of their vision. “Honestly, I hope that there’s benefit to myself personally” (Joiner, 005).

The inherent uncertainty of an early-phase clinical trial was the primary reason two men declined trial participation. “It’s simple. [There is] uncertainty of the potential for a positive result and also uncertainty in terms of risks” (Decliner, 012).

Hopes and Expectations

Those considering participation (nine Joiners and two Ineligibles) were asked about their hopes and expectations for trial participation. Participants provided responses that reflected distinct concepts; hopes were considered the best possible outcome of participation, whereas expectations were the most likely outcome. All but one person hoped for therapeutic benefit. “I hope for a complete home run scenario where the degeneration stops relatively quickly. I have regeneration…I can read menus. I can see people’s faces more clearly. I can drive” (Ineligible, 010).

The expectations for the likelihood of benefit ranged from very unlikely to very likely. These determinations were often nuanced. Those who thought benefit was likely cited the success of the pre-clinical animal studies, the merit of the research team, and trust in the clinical trial approval process. “If I had to put a percentage on it, I’d be willing to go 70 to 80. Not that I’m a mouse, but I know it was successful in the mice” (Joiner, 009). Individuals did not think they were more likely than other people with XLRS to benefit; rather, some people thought their age or specific eye findings made them less likely to benefit relative to others.

Although most hoped for therapeutic benefit and several people were optimistic about their chances, no one expected to benefit. The incongruence between hopes and motivations for participation and expectations and their understanding of the study’s purpose left some participants feeling conflicted, even guilty for hoping to benefit. “I hope for a change, but my expectations are low…The real [purpose] of the trial is to advance the research towards the ultimate goal of finding a treatment. I struggle with my desire for it to be an effective treatment now versus being part of the process. I have to remind myself that I’m part of the process” (Joiner, 004). In general, participants thought it was personally beneficial and motivating to be hopeful, although people anticipated that hopefulness could also result in disappointment if not balanced by reasonable expectations.

Decision-Making Process

Participants most often described decision-making based on risk/benefit assessments, personal intuition, trust in the study team, and religious faith. While all but one interview participant described benefit as direct therapeutic benefit, people also considered the advancements in research (n=6) and their own personal fulfillment (n=4) as benefits to participation. At the initial interview, people most often mentioned the risk of infection from the intravitreal injection and loss of vision in the study eye as the risks of trial participation (Table 3). Interview participants did not express concern about the risks, and all thought the risks were unlikely to happen. Ultimately, Decliners decided there was too much uncertainty regarding potential benefit to undertake any risk, whereas Joiners decided the potential for benefit outweighed the potential for risk and participating in the trial was a chance worth taking. “I have relatively good vision. I can function on a daily basis fine. Looking at the risk versus reward, I perceive the risk to be relatively low, but I don’t know what the success is” (Decliner, 012). “It’s a calculated gamble…There is a chance of my eyesight getting better or stabilizing and so that’s on one side of the scale. On the other side are the other negative factors. But you know for me that chance is worth it, it’s a calculated risk that I’m willing to take” (Joiner, 003).

While almost all participants articulated considerations of risk and benefit, many participants described making “gut decisions” rather than systematically considering all information provided. “I never questioned whether or not I wanted to participate. I knew from the very beginning that I was going to participate no matter what” (Joiner, 004). “I kind of made this decision from the moment I heard about the study” (Joiner, 008). Trustworthiness of the study team was an important decision-making factor. All but two participants had a patient-physician relationship with the trial team prior to the trial onset, and many described a sense of partnership and commitment to advancing shared goals. “I trust the doctors here, and I trust the results they’ve gotten from A to Z” (Joiner, 008). Religious faith was an important decisional factor for seven participants. “I’m a guy with a lot of faith. I think I’m looked out for either way. That’s it. It’s an easy decision for me. I didn’t have to ponder over it at all” (Joiner, 006).

All but two participants considered their decision straightforward, if a decision at all. Upon hearing of the plans for a trial, one Joiner felt an initial reluctance followed by two years of deliberation before ultimately choosing to enroll in the trial. “I was just weighing all these different possible risks and benefits and considering my age. There was no information that caused me to change my thinking. It was just—I had to make a decision eventually” (Joiner, 003). One person, prior to learning he was ineligible for the trial, was still deliberating and ambivalent about his decision to participate. Even so, he expressed feelings of frustration and disappointment upon learning of his ineligibility. All other participants were confident in their decision, and few anticipated situations in which they might regret their decision. “People make the best decisions at the time, they make it with the best information they have. So right now I’m satisfied with my decision to participate. If something would -- if there would be a negative outcome, it’s very easy in life to look back and say I shouldn’t make that decision, but if there’s a positive outcome then you’re thrilled you made that decision. So as I sit here right now, I’m making the best decision I can based on all of the

information that I have and based on where the medical science for my condition is at this point" (Joiner, 007).

Trial Experiences

Data from the 3 months and 12 months follow-up interviews are summarized in Table 4. Three months after the gene transfer injection, all Joiners were pleased with their decision to participate in the trial and would choose to participate again. Most people felt it was "too soon to tell" if they would benefit personally. Even so, all but two men said their hopes were met, although these responses tended to reflect previous descriptions of expectations, rather than hopes. "I would say I got more [than I hoped for]. I wasn't hoping for a whole lot, so I think my bottom line expectation was that the NIH would get something useful out of the study, and I'm sure they are" (Joiner, 008).

One year after the gene transfer injection, people felt even more certain about their personal outcome of trial participation. All but two men thought their experience was consistent with their expectations. Five people said their experience was less than they hoped for in terms of therapeutic benefit. However, Joiners still identified secondary benefits, such as positive feelings associated with advancing research, feeling this is part of one's legacy, and having greater knowledge and acceptance of the condition. "What I didn't expect was this kind of psychological portion of it, which I find very positive actually. I really never had the chance to talk about my condition, because it has been something that's really been a huge factor in my life for a long time. And so it's nice being able to talk about it. So this part is unexpected and very positive" (Joiner, 003). All Joiners associated their relationship with the study team with a positive trial experience. "What I've really enjoyed most about this whole experience is that I feel so much a familial relationship with everybody here" (Joiner, 004).

Regardless of personal outcome, all Joiners were pleased with their decision to participate and would choose to participate again. "It wasn't a hesitation for me when I decided to do it. And I would say that if I were here and if this was my very first visit, it would still not be a hesitation, even if I knew everything that I was going to go through. I would not hesitate to say, 'yes I'll do it again'" (Joiner, 009).

Discussion

As the number of ophthalmic clinical trials continues to increase, ophthalmologists will more frequently facilitate shared decision-making for trial participation. This study contributes to the understanding of how prospective research participants make decisions about enrolling in early-phase clinical trials, and it is the first study to explore the experiences of men with XLRS considering participation in a Phase I/IIa ocular gene therapy clinical trial.

In this study, we found that most clinical trial participants were motivated by and/or hoped for direct therapeutic benefit, even though they had an accurate understanding of the trial's purpose and had realistic expectations that their participation primarily would generate knowledge to advance research to benefit men with XLRS in the future. Whether therapeutic optimism poses a problem to informed consent has been debated[15–17]. Participants in this

study were not under therapeutic misconception, nor did they unrealistically perceive themselves as more likely to benefit relative to other participants; rather, they were simply "hoping for the best"[17]. This finding adds to a growing body of literature showing that therapeutic optimism is present among many trial participants and is more likely to be an expression of optimism or faith rather than a reflection of understanding or knowledge[7,8,18–20].

Almost all participants perceived their decisions to be straightforward. This "non-decision" to participate in clinical trials has been reported in studies ofparents of children with Duchenne muscular dystrophy[7] and patients with cancer[5], but it is an interesting finding in XLRS considering the non-fatal, minimally progressive nature of the condition. This could reflect participants' altruism and perceptions that benefits of participation extend beyond that of direct therapeutic benefit alone.

This study reinforces the findings from numerous previous studies demonstrating that prospective research participants frequently respond intuitively to research information, rather than considering and weighing all available information[5,8,21,22] Moreover, many people come to the informed consent conversation having already made initial decisions about their participation[5,7,18]. Once a person is committed to participation, perceptions of benefit often are accentuated while perceptions of risk are minimized[23]. While all participants understood that there were risks associated with trial participation, they reported the nature of the risks inconsistently. The more familiar and concrete risks, such as the risk of infection, were mentioned most frequently, whereas the more abstract and theoretical risks, such as the potential risk of the gene transfer vector, were reported less often, especially among clinical trial Joiners. Those already committed to participation had decided that the risks were tolerable, and they trusted the study team to manage any potential side effects. Other studies have found that research participants interpret and use risk information subjectively, and perceptions of risk are influenced greatly by familiarity and personal experience[22]. While further research would illuminate ways to improve communication of risk and benefit information to prospective research participants,[24] this study's findings suggest that efforts aimed at understanding individuals' perceptions and how this is incorporated into a person's decision-making may be more meaningful than efforts aimed at improving comprehension.

A strength of this interview study is the prospective, longitudinal study design, allowing us to capture the experiences of men actively engaged in a non-hypothetical decision about participating in a clinical trial and to assess the psychosocial impact of hopes and expectations over time. This study found no negative consequences to participants' hopefulness or potential misestimation of risks and benefits over the time that participants were interviewed. While this could reflect participants' unwillingness to voice disappointment or other negative sentiments, interviews were conducted by people unaffiliated with the clinical trial to reduce this possibility. Over the 12 months, participants made less distinction between their hopes and expectations, and they often used these terms interchangeably. This was a shift from the initial interview and represents a re-framing of hopes and expectations that aligned with perceptions of secondary benefits to trial participation. Regardless of personal outcome, all of the Joiners reported positive trial

experiences that, similar to other studies[6,18,25], emphasized their meaningful relationship with the study team. Secondary benefits were highly valued by participants and contributed to feelings that hopes were realized and participation was worthwhile.

This study has several limitations. The study population was largely white, non-Hispanic, highly educated, and most were established patients of the clinical trial team. All participants were men affected by an X-linked condition. As such, the results of this study may not be generalizable to all people with inherited eye conditions, which also may have different natural histories and degrees of resulting disability. We were limited in our ability to interview Decliners and people who were ineligible for the trial because most people invited to be screened for the clinical trial were eligible and interested in enrolling. All interviews took place after participants had reviewed the informed consent document with the clinical trial team; it is unknown how this may have influenced participants' responses.

This study highlights the importance of engaging prospective participants in discussion about their hopes, expectations, and decisional influences to have a more complete understanding of patient decision-making and to help support informed choices. Many research participants hope for direct therapeutic benefit. Investigators should facilitate a nuanced consideration of the risks, benefits and possible trial outcomes to help people anticipate and avoid situations in which they might experience regret over their decision. Future research in a more diverse population would be beneficial to replicate these findings and to understand better the experiences of individuals with vision loss who are considering clinical trial participation.

Acknowledgments

A. Funding Source: This research was funded by the Intramural Research Program of the National Eye Institute, National Institutes of Health, Bethesda, MD, USA. The funder had no role in the study design; in the collection, analysis, and interpretation of the data; in the writing of the paper; or in the decision to submit the paper for publication.

C. All authors attest that they meet the current ICMJE criteria for authorship. We thank the men who participated in this study, and we thank Celeste D'Amanda, ScM, CGC (University of Rochester Medical Center, Rochester, NY) and Laryssa Huryn, MD (National Eye Institute, Bethesda, MD) for feedback on an initial draft.

References

1. Administration USFD. FDA approves novel gene therapy to treat patients with a rare form of inherited vision loss [Press release]. Available at https://www.fda.gov/newsevents/newsroom/pressannouncements/ucm589467.htm Accessed December 19, 2018.
2. Appelbaum PS, Lidz CW, Grisso T Therapeutic misconception in clinical research: frequency and risk factors. IRB. 2004;26(2):1–8.
3. Daugherty C, Ratain MJ, Grochowski E, et al. Perceptions of cancer patients and their physicians involved in phase I trials. J Clin Oncol 1995; 13(5): 1062–1072. [PubMed: 7738612]
4. Lidz CW, Appelbaum PS. The therapeutic misconception: problems and solutions. Med Care. 2002;40(9 Suppl):V55–63. [PubMed: 12226586]
5. Dellson P, Nilsson K, Jernström H, Carlsson C. Patients' reasoning regarding the decision to participate in clinical cancer trials: an interview study. Trials. 2018;19(1):528–536. [PubMed: 30268150]

6. Henderson GE, Peay HL, Kroon E, et al. Ethics of treatment interruption trials in HIV cure research: addressing the conundrum of risk/benefit assessment. J Med Ethics. 2018;44(4):270–276. [PubMed: 29127137]
7. Peay HL, Scharff H, Tibben A, et al. "Watching time tick by…": Decision making for Duchenne muscular dystrophy trials. Contemp Clin Trials. 2016;46:1–6. [PubMed: 26546066]
8. Kim SY, Schrock L, Wilson RM, et al. An approach to evaluating the therapeutic misconception. IRB. 2009;31(5):7–14.
9. Zolkipli-Cunningham Z, Xiao R, Stoddart A, et al. Mitochondrial disease patient motivations and barriers to participate in clinical trials. PloS One. 2018;13(5):e0197513. [PubMed: 29771953]
10. McCann SK, Campbell MK, Entwistle VA. Reasons for participating in randomised controlled trials: conditional altruism and considerations for self. Trials. 2010;11:31. [PubMed: 20307273]
11. Nelles M, Stieger K, Preising MN, Kruse J, Lorenz B. Shared decision-making, control preferences and psychological well-being in patients with RPE65 deficiency awaiting experimental gene therapy. Ophthalmic Res 2015;54(2):96–102. [PubMed: 26278420]
12. Cukras CA, Huryn LA, Jeffrey BG, Turriff A, Sieving PA. Analysis of anatomic and functional measures in X-Linked retinoschisis. Invest Ophthalmol & Vis Sci 2018;59(7):2841–2847. [PubMed: 30025115]
13. Cukras C, Wiley HE, Jeffrey BG, et al. Retinal AAV8-RS1 gene therapy for X-Linked Retinoschisis: initial findings from a phase I/IIa trial by intravitreal delivery. Mol Ther 2018;26(9): 2282–2294. [PubMed: 30196853]
14. Braun V, Clarke V Using thematic analysis in psychology. Qual Res Psychol 2006;3(2):77–101.
15. Weinfurt KP, Seils DM, Tzeng JP, et al. Expectations of benefit in early-phase clinical trials: implications for assessing the adequacy of informed consent. Med Decis Making. 2008;28(4):575–581. [PubMed: 18378940]
16. Jansen LA. The problem with optimism in clinical trials. IRB. 2006;28(4):13–19. [PubMed: 17036434]
17. Jansen LA. Two concepts of therapeutic optimism. J Med Ethics. 2011 ;37(9):563–566. [PubMed: 21551464]
18. Peay HL, Tibben A, Fisher T, Brenna E, Biesecker BB. Expectations and experiences of investigators and parents involved in a clinical trial for Duchenne/Becker muscular dystrophy. Clin Trials. 2014;11(1):77–85. [PubMed: 24311736]
19. Sulmasy DP, Astrow AB, He MK, et al. The culture of faith and hope: patients' justifications for their high estimations of expected therapeutic benefit when enrolling in early phase oncology trials. Cancer. 2010;116(15):3702–3711. [PubMed: 20564120]
20. Weinfurt KP, Castel LD, Li Y, et al. The correlation between patient characteristics and expectations of benefit from Phase I clinical trials. Cancer. 2003;98(1):166–175. [PubMed: 12833469]
21. Gigerenzer G, Gaissmaier W. Heuristic Decision Making. Annu Rev Psychol 2011 ;62(1):451–482. [PubMed: 21126183]
22. Reynolds WW, Nelson RM. Risk perception and decision processes underlying informed consent to research participation. Soc Sci Med 2007;65(10):2105–2115. [PubMed: 17689846]
23. Jansen LA. Mindsets, informed consent, and research. Hastings Cent Rep 2014;44(1):25–32.
24. Nusbaum L, Douglas B, Damus K, Paasche-Orlow M, Estrella-Luna N. Communicating risks and benefits in informed consent for research: a qualitative study. Glob Qual Nurs Res 2017;4:2333393617732017.
25. Kost RG, Lee LM, Yessis J, Coller BS, Henderson DK, The Research Participant Perception Survey Focus Group Subcommittee. Assessing research participants' perceptions of their clinical research experiences. Clin Transl Sci 2011;4(6):403–413. [PubMed: 22212221]

Table 1:

Understanding, Motivations, Expectations, and Decisional Influences of Individuals Considering Trial Participation

Participant	Understanding of Purpose	Motivations for Participation	Chances of Personal Benefit	Expectations	Decisional Influences
Joiner 001	Safety Efficacy	Interest in Research	Very Unlikely	No Benefit	Intuition
Joiner 002	Efficacy	Altruism	Very Likely	Advances Research	Intuition Hope Religious Faith Trust in Study Team
Joiner 003	Safety	Direct Benefit	Unlikely	No Benefit	Hope Risk<Benefit Insider's Advantage
Joiner 004	Safety Efficacy	Altruism Direct Benefit	Unlikely	No Benefit No Adverse Effects	Intuition Hope Risk<Benefit Religious Faith
Joiner 005	Safety	Altruism Direct Benefit	50-50	No Benefit	Intuition Hope
Joiner 006	Safety Efficacy	Altruism Direct Benefit	50-50	No Benefit Advances Research	Intuition Hope Risk<Benefit Religious Faith Faith in Research Process
Joiner 007	Efficacy	Altruism Direct Benefit	Unknown	No Benefit	Hope Risk<Benefit Religious Faith Trust in Study Team
Joiner 008	Efficacy	Direct Benefit	Likely	No Benefit Advances Research	Intuition Hope Risk<Benefit Religious Faith Faith in Research Process Trust in Study Team Insider's Advantage
Joiner 009	Safety	Altruism	Likely	Advances Research	Intuition Hope
Ineligible 010	Efficacy	Altruism Direct Benefit	Unlikely	No Benefit Advances Research	Hope Risk<Benefit
Ineligible 011	Safety	Altruism	50-50	No Benefit No Adverse Effects	Intuition Trust in Study Team

Table 2:

Understanding, Motivations, and Decisional Influences of Individuals Who Declined Trial Participation

Participant	Understanding of Purpose	Reasons Declined Participation	Chances of Personal Benefit	Decisional Influences
Decliner 012	Efficacy	Uncertainty of Direct Benefit Uncertainty of Risks	Unknown	Intuition Risk>Benefit
Decliner 013	Safety Efficacy	Uncertainty of Direct Benefit	Likely	Risk>Benefit

Table 3:

Perceived Risks of the Clinical Trial

Perceived Risk	Joiners N =9	Decliners N=2	Ineligibles N=2
Infection	7	2	0
Vision loss	6	2	0
Inflammation	1	1	2
Death	3	0	0
Retinal tear or hemorrhage	1	0	1
Unknown risks of the gene transfer vector	0	1	1
The gene transfer does not work	2	0	0
Exclusion from future studies	1	0	0

Table 4:

Trial Experiences at 3 Months and 12 Months after Gene Transfer

		3 months N[a]	12 months N
Feelings about Decision to Participate	Positive	6	8
	No regrets	4	3
Thoughts about Trial Experience	Too soon to determine efficacy	7	1
	What I thought it would be	8	7
	Different from what I thought it would be	2	1
	Unsure	0	1
More or Less than Hoped for	More	2	4
	Less	2	5
	What I hoped for	4	2
	What I expected	2	4
Perceived Secondary Benefits	Learning more about XLRS	1	0
	Altruism	2	5
	Legacy	0	1
	Helped with accepting my condition	0	1
Would you choose to participate again	Yes	9	9
	No	0	0

[a]The total number of responses may not add to 9 since an individual may have responded in more than one way