

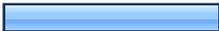
1. To what extent are you aware of the success/failure of gene therapy in other genetic disorders?

	Very Aware	Somewhat Aware	Unaware	No information	Rating Average	Response Count
	4.3% (3)	55.1% (38)	36.2% (25)	4.3% (3)	1.59	69
	Please explain:					48
	answered question					69
	skipped question					0

2. What issues would you/your child want clarified if you were to consider participation in a gene therapy clinical trial for Fanconi anemia?

	Response Count	
	67	
	answered question	67
	skipped question	2

3. Do you/your child have any worries about gene therapy in Fanconi Anemia?

		Response Percent	Response Count
Yes		67.6%	46
No		32.4%	22
	Please explain:		55
	answered question		68
	skipped question		1

4. 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?

	Response Count
	68
answered question	68
skipped question	1

5. 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?

	Response Count
	66
answered question	66
skipped question	3

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

		Response Percent	Response Count
Yes		27.9%	19
No		72.1%	49
	Please explain:		32
answered question			68
skipped question			1

Q1. To what extent are you aware of the success/failure of gene therapy in other genetic disorders?

1	We were at Camp Sunshine in August 2012, and Dr. Tolar spoke to us of his involvement in the gene therapy work - this is the first I had really heard of it.	Oct 12, 2012 3:30 PM
2	First trials were disappointing (nineties) Gene therapy for SCID children gave a lot of hopes some years ago. But after 1 child died of leukemia thereafter, doctors stopped and went back to work to find better vectors Recently, there was a successful gene therapy for beta-thalassemie. But I don't follow closely this topic.	Oct 7, 2012 11:54 AM
3	I try to follow press news	Sep 30, 2012 4:31 AM
4	I am not aware of the status of gene therapy in any other disorders than FA	Sep 17, 2012 11:20 AM
5	I think I have heard of gene therapy being done for EB (fragile skin syndrome) and a form of hemophilia (in eurpoe)	Sep 15, 2012 8:17 PM
6	I have heard about various trial, but only through what I read or hear in the news.	Sep 14, 2012 1:06 PM
7	what is the danger of this therapy how successfull is it.	Sep 14, 2012 5:53 AM
8	I track progress through the use of Google Alerts	Sep 13, 2012 10:09 PM
9	We have heard of gene therapy but are not aware of it's success/failure.	Sep 13, 2012 7:32 PM
10	I heard a presentation a couple of years ago by Dr. Wagoner at Camp Sunshine, and I've read some of the literature in the FA newsletter.	Sep 11, 2012 6:37 PM
11	I understand the basics of how researchers have used a virus to deliver a corrected gene into a cell nucleus and the corrected gene may supplant or replace the defective gene in that cell, then when cell division takes place, the newly created cell contains the corrected gene. In this way, with time, many corrected cells can be created, and with attrition of older, defective cells, the organism becomes comprised of non-defective cells, essentially "cured" of the genetic defect. I am excited that this process is taking place in a clinical trial setting or settings, with FANC being the defect.	Sep 11, 2012 5:16 AM
12	Aware of deaths in patients on gene therapy trials for other diseases during 1999-2009.	Sep 6, 2012 8:34 AM
13	Want to keep current in case it will help my child with FA.	Sep 6, 2012 7:50 AM
14	No he tenido a un ningun tipo de informacion me gustaria q me la hagan saber .	Sep 3, 2012 5:21 PM
15	I know very little about gene therapy trials in other populations. My interest in treatment for FA has been primarily in the realm of BMT, but I am now becoming excited about the potential of gene therapy in FA. I remember hearing about this possibility when I was much younger, but the trials then were unsuccessful and ultimately deemed unsafe...As per the last meeting at Camp Sunshine, it sounds like the methods have been significantly refined and are promising for the future. Yay!	Sep 3, 2012 12:24 PM
16	Have not been following the gene therapy. It does sound very interesting and if they could correct the non working gene, without side effects to the patient I think it is a wonderful thing.	Sep 3, 2012 5:44 AM

Q1. To what extent are you aware of the success/failure of gene therapy in other genetic disorders?

17	I have read research materials over the past 10 years that this research has been ongoing.	Sep 2, 2012 1:27 PM
18	Heard it was happening, some progress were made. I do not know what Gene therapy "really" means concretely and the actual results and progress in general.	Sep 1, 2012 11:34 PM
19	son newly diagnosed with FA	Sep 1, 2012 1:11 PM
20	I know many attempts have been made in multiple disorders but have not heard how the outcomes have been a few years down the road.	Aug 31, 2012 4:08 PM
21	my daughter was only diagnosed with fanconi in jan	Aug 31, 2012 2:55 PM
22	We have heard at Camp Sunshine of some success. I somewhat recall gene therapy being curative for "boy in the bubble" disease?	Aug 31, 2012 1:34 PM
23	I have read some articles on internet	Aug 31, 2012 8:28 AM
24	I know of gene therapy but have not heard that it has been successful or that it has failed to help in other genetic disorders.	Aug 31, 2012 5:10 AM
25	Have been following gene therapy since the first successful treatment of SCID.	Aug 30, 2012 11:07 PM
26	I have read about updates concerning gene therapy in genetic disorders from time-to-time. Sources include news articles and the FA newsletter.	Aug 30, 2012 10:57 PM
27	I have read about some of the other attempts, and also know two people have were involved in trials.	Aug 30, 2012 9:54 PM
28	I dont understand what gene therapy really is	Aug 30, 2012 8:13 PM
29	I have heard and read about some other gene therapy trials, but have not heard of any real success stories about FA.	Aug 30, 2012 4:00 PM
30	I have no idea how gene therapy works for any disorder	Aug 30, 2012 11:31 AM
31	I'm not aware of any successful gene therapy	Aug 30, 2012 8:33 AM
32	it's something we've heard of but don't know much about.	Aug 30, 2012 7:54 AM
33	Know it is successful in some diseases but not yet with FA	Aug 30, 2012 7:17 AM
34	As a scientist I often discuss the application of gene therapy to other disease entities, not necessarily genetic diseases	Aug 30, 2012 6:26 AM
35	Don't know of any disorders that have been successful or have failed.	Aug 29, 2012 11:50 PM
36	Read alot ..very curious..research what i can - so somewhat aware....	Aug 29, 2012 9:42 PM
37	Not into reading that type of material.	Aug 29, 2012 8:06 PM
38	I know there's research being done but you never hear updates or when it actually gets to be available for the general public	Aug 29, 2012 7:11 PM

Q1. To what extent are you aware of the success/failure of gene therapy in other genetic disorders?

39	I have heard that gene therapy has been used for other genetic disorders, but that it is hard to find a virus (vector?) that can be controlled when entering the body.	Aug 29, 2012 6:22 PM
40	I don not no much about genetic disorders and gene therapy.	Aug 29, 2012 4:28 PM
41	I do read some of the FA newsletters, I also have attended 2 of the adult meetings, I hear what they say although I've been categorized differently. I've had 2 skin biopsies, neither one resulting in learning my gene, I even spent time years ago with Dr Aurbough, I'm sure that's wrong but as far as I know I'm the healthiest of everyone.	Aug 29, 2012 3:54 PM
42	My children completed gene therapy and it was unsuccessful in FA but worked in other diseases.	Aug 29, 2012 3:39 PM
43	I have no idea.	Aug 29, 2012 3:30 PM
44	I know in "bubble boy" it has caused leukemia	Aug 29, 2012 3:12 PM
45	Only read about it.	Aug 29, 2012 3:02 PM
46	I am aware of limited success but also the potential side effects, including leukaemia.	Aug 29, 2012 2:46 PM
47	I know that there is experimental work still being done and was told it is "not quite ready".	Aug 29, 2012 2:39 PM
48	Have heard of some in-utero gene therapies that have been done for other diseases.	Aug 29, 2012 1:04 PM

Q2. What issues would you/your child want clarified if you were to consider participation in a gene therapy clinical trial for Fanconi anemia?

1	- eligibility criteria and the rationale behind the criteria - assessment of the potential success rate for my child's future bone marrow transplant (i.e. given his current state of health, fact that there is only one 9/10 unrelated donor available in the database) - all potential side effects of the gene therapy trial	Oct 12, 2012 3:30 PM
2	Risk of failure Side effects Toxicity of vectors and drugs needed Length of the treatment What happens if none effective.	Oct 7, 2012 11:54 AM
3	the chance for success	Sep 30, 2012 4:31 AM
4	What is the alternative for the patient to trying gene therapy? For instance, my son has currently an ok life when using Oxymetholone. If he should try gene therapy he would need to stop with Oxy. Then there is always a risk that Oxy will not work in the same efficient manner when starting up again. Key is therefore also what is the downside? Is there a risk that the treatment can put you in a worse place than you were before you started? If there is little downside, but uncertain upside it would be easier to try gene therapy. For my son the likely treatment when/if oxy doesn't work is to undergo unrelated BMT. The risk associated with this treatment is high and there are significant sideeffects. Faced with this alternative my son would probably want to do gene therapy before doing the BMT. You would always want to optimise on your alternatives.	Sep 17, 2012 11:20 AM
5	Risks, likely outcomes, weather or not it would decrease the risk for cancer. Long term outcomes.	Sep 15, 2012 8:17 PM
6	The risk factors involved in the trails.	Sep 14, 2012 1:06 PM
7	all the information, had none at this stage	Sep 14, 2012 5:53 AM
8	Is it an actual cure of FA, removing the propensity to cancers as well as restoring the BM function? Is it likely to lead to a different set of problems which are just as serious? Participation in an experimental trial suggests there are no other less risky options otherwise one would not be accepted for the trial. Is this the case? Once the technique is proven will it be available and useful to people who have already had BMT, or has the damage really been done through the BMT process? Have previous trials been carried out on my child's particular complementation group? If not, is it seen that different outcomes can be expected for different complementation groups or is this still unknown. If the trial doesn't 'work', can I then revert to a more conventional treatment ie BMT?	Sep 13, 2012 10:09 PM
9	The potential health risks and pros and cons of being involved in the trial.	Sep 13, 2012 7:32 PM
10	los riesgos y posibles complicaciones o mejora	Sep 12, 2012 1:24 PM
11	The risks. If it failed, whether BMT still be an option.	Sep 11, 2012 6:37 PM
12	An evidence-based, comprehensive list of the things that could go "wrong", their likelihood of occurring, the outcomes in terms of how is the patient would be affected, the timeframes in which these affects would become measurable / noticable / problematic to the patient, and the number of of patients or other subjects (animals) on which this evidence is based and a scientific explanation of why this number is sufficient to warrant a sense of "trust" that the projections or	Sep 11, 2012 5:16 AM

Q2. What issues would you/your child want clarified if you were to consider participation in a gene therapy clinical trial for Fanconi anemia?

	"likelihoods" can be considered valid.	
13	Safety. Potential benefit to my child. Implications for other potential treatments.	Sep 6, 2012 8:34 AM
14	What are the benefits, what are the risks. What work was done previously to ensure safety and effectiveness of the therapy.	Sep 6, 2012 7:50 AM
15	Does it simply cure the Bone Marrow failure or the disorder itself??	Sep 5, 2012 1:44 PM
16	success rate and risks.	Sep 4, 2012 4:59 PM
17	I wouldn't want to participate unless it was my child's choice but at this time he is too young to decide for himself. Mainly I would want to know the risk factors, and the success rate for other Fanconi kids if the trial was to be a success.	Sep 3, 2012 8:48 PM
18	what possible side affects from treatment-success rate-is it a long term actual cure-	Sep 3, 2012 7:21 PM
19	Mi hijo a un tiene un año y dos meses de edad a un no sabe nada de esto.	Sep 3, 2012 5:21 PM
20	Safety. I would like to know more about the vector utilized for this purpose and any risks posed by the procedure. I would be curious as well about the addition of any conditioning prior to the therapy. My understanding is that this trial does not include a conditioning regimen. If it is unsuccessful, what dosage of chemo/radiation might be considered at some point in the future?	Sep 3, 2012 12:24 PM
21	First of all, is the gene therapy for kids that have already had transplant? If so , I wouldn't want him to have any problems. Like I said, any side effects that would shorten his life in any way other than the fanconi itself. I know we have come a long way but I want the test to be perfective before they start testing on children. I want to know whats involved and than we'll consider it.	Sep 3, 2012 5:44 AM
22	Long term / death related issues if the therapy fails.	Sep 2, 2012 1:27 PM
23	What are side effects Is it a permanent cure	Sep 2, 2012 2:27 AM
24	- Actual details of results and test on animals or human until now - Real risks and so what is the moment to consider to do it (last resort because too risky or rather soon than later as the risks so low) - Does it hurt - Other side effects : other disease, blood cancer, etc....., - What are the long term effects ? Would it be necessary to have another run ? once is enough if it works - Will there be a need for medication afterwards - What are the medical care/control needed afterwards - At what age can you undergo a Fanconi gene therapy - As of when will this treatment be available - Where is it the best to do in Europe	Sep 1, 2012 11:34 PM
25	location/travel issues, risks and benefits	Sep 1, 2012 1:11 PM
26	The risks and at what point gene therapy would be most advantageous.	Aug 31, 2012 4:08 PM
27	is it at all dagerous? is it painful?	Aug 31, 2012 2:55 PM
28	We would want to understand the risks. Given that Fanconi anemia is a cancer predisposition syndrome, there's something unsettling about manipulating	Aug 31, 2012 1:34 PM

Q2. What issues would you/your child want clarified if you were to consider participation in a gene therapy clinical trial for Fanconi anemia?

	"healthy" (not yet transplant-reliant) cells.	
29	Risks and benefits compared to traditional/current treatments. Long term risks.	Aug 31, 2012 9:46 AM
30	That the treatment is safe and if it appears not be succesfull, that it causes no damage to the body and there will still be the possibility of a BMT.	Aug 31, 2012 8:28 AM
31	I would want to know that using it would not preclude using other treatments if it failed. My child has already had a bone marrow transplant and I don't know whether he would qualify for treatment, although at one stage, I thought his BMT had not been successful.	Aug 31, 2012 5:10 AM
32	1. If the gene therapy did not work, would that prevent later treatment with a bone marrow transplant? 2. Would any bone marrow ablation be performed prior to the gene therapy? Saw one study where a short course of chemotherapy was used to create space in the bone marrow for transplanted cells.	Aug 30, 2012 11:07 PM
33	I would like to know about the side-effects, if there is adequate information about them, will the effects (good and bad) be short-lived, will the good effects be stable, would the therapy increase the chances of the child developing a tumor (if so, a family would probably decline volunteering if the patient is stable to begin with), would the volunteer/participant be able to lead an otherwise normal life during the course of the trial, will the therapy involve painful injections for the participant.	Aug 30, 2012 10:57 PM
34	I would want to learn as much as I possibly could.	Aug 30, 2012 9:54 PM
35	everything	Aug 30, 2012 8:13 PM
36	Side effects	Aug 30, 2012 4:48 PM
37	My daughter had an unrelated cord blood transplant in 2008, so I do not know that she would qualify for gene therapy - but I would like to know for sure if this would or would not be an option for her.	Aug 30, 2012 4:00 PM
38	risks chances of success	Aug 30, 2012 12:32 PM
39	As having no knowledge I would need everything explained. At this point nothing would need to be clarified because I don't know enough to be able to ask questions	Aug 30, 2012 11:31 AM
40	The risks and benefits and when if the best time to try it.	Aug 30, 2012 8:41 AM
41	desired outcome, side effects, follow-up plans, costs/stipends, etc	Aug 30, 2012 8:33 AM
42	Eli has already had a BMT, is this something that only prevents bone marrow failure, or is it being used to also prevent cancers?	Aug 30, 2012 7:54 AM
43	side effects harm it can do to good cells long term side effects	Aug 30, 2012 7:17 AM
44	That if anything were to show up in the tests that we and our doctor would be notified.	Aug 30, 2012 6:43 AM

Q2. What issues would you/your child want clarified if you were to consider participation in a gene therapy clinical trial for Fanconi anemia?

45	what are the risks and potential benefits and how do they compare to standard therapeutic options? what is the risk of using viruses? what can we expect?	Aug 30, 2012 6:26 AM
46	What it have any effects on my son?	Aug 30, 2012 4:00 AM
47	How easy/difficult would it be to control the therapy while ongoing? I think I would be 'scared' of the 'what have we started to grow in my FA-child'. If it seems to have gone 'out of control' can we then recorrect this? Most of this caution I think stems from a general feeling of not wanting to experiment too much with an already genetically unstable body/system.	Aug 30, 2012 2:04 AM
48	side effects and potential treatment options. is this something that is intended to treat bone marrow failure? Unclear.	Aug 30, 2012 12:44 AM
49	The probability of side effects, the risks of cancer, the cost of the trial, and the timing of the trial for my son's condition.	Aug 29, 2012 11:50 PM
50	side effects...	Aug 29, 2012 9:42 PM
51	Risks, cost, time away from home, benefits for me.	Aug 29, 2012 8:06 PM
52	The only thing I'd want is to be able to do it at HOME and not have to travel halfway across the bloody world to do it. The other thing is I would like to know the risks and entire procedure at each and every stage and have the option to remove myself from the trial if it becomes unsafe.	Aug 29, 2012 7:11 PM
53	Would not be interested at this time because her counts are stable. Would possibly consider it in the future if there was some proven promise of success.	Aug 29, 2012 6:22 PM
54	Risks vs benefits	Aug 29, 2012 6:04 PM
55	What would be the side effects be if unsuccessful? How long of a recovery process? Would you have to use immune suppression drugs? If it was unsuccessful would you still be a candidate for a bone marrow transplant? Would this help with cancer issues later in life or just the bone marrow failure part?	Aug 29, 2012 4:30 PM
56	pain, longevity, intentions, cost, reimbursement	Aug 29, 2012 4:28 PM
57	possible negative effects	Aug 29, 2012 4:07 PM
58	I would want to hear the successes and failures.	Aug 29, 2012 3:58 PM
59	Social Security, it seems to be for anyone who is has not as sever problems as we, the biggest thing I can think of & heard, SSI is more than horrible, I think that is what you should worry about most for us adults, otherwise we can't have a house, car, job, money, health insurance.	Aug 29, 2012 3:54 PM
60	None	Aug 29, 2012 3:39 PM
61	Would need to know the risks chances of success	Aug 29, 2012 3:34 PM
62	Whether or not it's safe and what the side effects are	Aug 29, 2012 3:30 PM

Q2. What issues would you/your child want clarified if you were to consider participation in a gene therapy clinical trial for Fanconi anemia?

63	How will it affect my child?	Aug 29, 2012 3:02 PM
64	Safety, including long term implications for health.	Aug 29, 2012 2:46 PM
65	What happens before getting the new gene, do kids have to undergo chemo and/or radiation? What is the success rate? What are concerns and risks that we should know about? How long will the child have to be hospitalized? Will medications will a child have to have during and after? What limitations will a child have afterwards (isolation at home)?	Aug 29, 2012 2:39 PM
66	More details regarding the actual procedure, recovery time, and monitoring process.	Aug 29, 2012 2:27 PM
67	Risks involved Success rates Short term/ long term effects Cost	Aug 29, 2012 1:04 PM

Q3. Do you/your child have any worries about gene therapy in Fanconi Anemia?

1	Of course there is always worry about something that is not yet proven, and potential side effects that are not even on the radar at the moment. i.e. you don't know what you don't know But 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! i.e. for some reason I have confidence that the risks will be mitigated and this will be a viable option in the very near future (I think this is somewhat because of the passion and knowledge with which Dr. Tolar spoke to us at Camp).	Oct 12, 2012 3:30 PM
2	I have worries about long term effects I heard about gene therapy for FA since maybe 20 years. I hope it will work but I think we are not so close of a real alternative treatment for bone marrow failure.	Oct 7, 2012 11:54 AM
3	the future problems caused by gene therapy	Sep 30, 2012 4:31 AM
4	He doesn't want to be the first one. As he has time to wait, see how others are doing when trying gene therapy. It is always safer not to be the first one; if you have the luxury of deciding when to do it. If there are any success stories, please let us know!	Sep 17, 2012 11:20 AM
5	she is too young to understand the concept.	Sep 15, 2012 8:17 PM
6	Naturally, until any therapy is tried and tested it is wise to be cautious.	Sep 14, 2012 1:06 PM
7	will it be successfull	Sep 14, 2012 5:53 AM
8	If GT is only now being trialled then the long term effects if any will not be known for a number of years (My child does not know about gene therapy and is going through BMT at present)	Sep 13, 2012 10:09 PM
9	Generally worried that the therapy won't be successful for FA.	Sep 13, 2012 7:32 PM
10	Untested, or limited testing thus far.	Sep 11, 2012 6:37 PM
11	It's not like you can "take it back" if things go wrong. The thought of introducing modifications into the patient's cells breeds a fear that, since the science (or the practical applications in humans) is relatively new, we don't have a lot of experience and past results that we can go on to predict the possible outcomes. What happens if things are replicated that are harmful? How often have unexpected results occurred. (What happens if "things go haywire", and is there a chance of that?)	Sep 11, 2012 5:16 AM
12	As above.	Sep 6, 2012 8:34 AM
13	Will it cause cancer. Will it work. Will it be too expensive.	Sep 6, 2012 7:50 AM
14	How would it affect any children in the future??	Sep 5, 2012 1:44 PM
15	success rate and risk.	Sep 4, 2012 4:59 PM
16	possible side effects from treatment-there are a lot of unknowns	Sep 3, 2012 7:21 PM
17	Este tratamiento tiene algun tipo de reaccion adversa con mi hijo .	Sep 3, 2012 5:21 PM

Q3. Do you/your child have any worries about gene therapy in Fanconi Anemia?

18	I have worries about everything :). But excitement for the future certainly weighs out. My concerns are simply about safety, and frankly, this seems to be in theory (at least for now) far safer than a bone marrow transplant, and have the potential to correct far more. It is simply new and very little is known about the effect of this procedure in the FA population. But of course, that's why we need to find out.	Sep 3, 2012 12:24 PM
19	Well, I'm not up to date with all the details. There is so much that can go wrong in a disease as complex as Fanconi Anemia.	Sep 3, 2012 5:44 AM
20	Would consider once all the "kinks" have been ironed out of the research.	Sep 2, 2012 1:27 PM
21	Side effects, type of treatment	Sep 2, 2012 2:27 AM
22	Earlier tests of a few years back, were not successful (as far as I can understand). Patient got sick and died from the therapy. Very high risks. What is the rationale today to do gene therapy rather than a transplant ?	Sep 1, 2012 11:34 PM
23	hopeful, but concerned about any negative consequences	Sep 1, 2012 1:11 PM
24	Just what to expect and the again the actual stats on the risk factors.	Aug 31, 2012 4:08 PM
25	pain, side effects	Aug 31, 2012 2:55 PM
26	See above	Aug 31, 2012 1:34 PM
27	Because it has not been tested on human beings yet, we do not know what complications could appear rightaway or after a periode of time (5 years, 10 years)	Aug 31, 2012 8:28 AM
28	As FA patients usually have delicate constitutions, I wonder whether FA patients would be able to withstand the therapy used in gene therapy.	Aug 31, 2012 5:10 AM
29	The durability of the therapy. Will the corrected cells have a competitive advantage over the native cells and therefore persist long term? Or will they 1) maintain a constant ratio the native cells, with the patient becoming chimeric, or 2) die out and fade away.	Aug 30, 2012 11:07 PM
30	If I understand correctly, it is a double-edged sword. There are chances that the virus may recover its ability to cause disease. There may be increased chances for the patient to develop a tumor.	Aug 30, 2012 10:57 PM
31	Is it possible post transplant?	Aug 30, 2012 4:00 PM
32	Can't have any worries when I don't know anything about gene therapy	Aug 30, 2012 11:31 AM
33	Am not considering it at the moment because blood count is fine for now	Aug 30, 2012 8:41 AM
34	I worry about FA period, new therapies and new risks would be a concern but not to the extent I would not want it.	Aug 30, 2012 7:54 AM
35	successfulness	Aug 30, 2012 7:17 AM
36	previously cancer has been reported when using gene therapy. FA patients are	Aug 30, 2012 6:26 AM

Q3. Do you/your child have any worries about gene therapy in Fanconi Anemia?

particularly prone to develop cancer. What is gene therapy increases this risk?

37	Too young	Aug 30, 2012 2:04 AM
38	side effects and potential outcome vs. time spent participating in study.	Aug 30, 2012 12:44 AM
39	Sure there always of worries with Eli's health. Will the therapy cause other unknown issues?	Aug 29, 2012 11:50 PM
40	Same as above	Aug 29, 2012 8:06 PM
41	Of course, we're messing with DNA here, what if "fixing" it isn't really fixing it? or what if something else goes wrong or it doesn't take and makes it worse?	Aug 29, 2012 7:11 PM
42	We have not considered it at this time.	Aug 29, 2012 6:22 PM
43	Risks of failure and potential side effects	Aug 29, 2012 6:04 PM
44	not sure what it is	Aug 29, 2012 4:28 PM
45	possible negative effects	Aug 29, 2012 4:07 PM
46	I think this could be a wonderful break through	Aug 29, 2012 3:58 PM
47	I have no worries about gene therapy since they don't know my gene, but what about those who do know their group, trying to have a job, it doesn't work, we need help on the gene level & school/job issue.	Aug 29, 2012 3:54 PM
48	Already completed it	Aug 29, 2012 3:39 PM
49	that it wont be developed in time for use...or that it will be used and considered "safe" when there in fact are long term negative consequences.	Aug 29, 2012 3:34 PM
50	We would love it to become an option, but getting to the point of it being successful (tests, trials) is the scary part	Aug 29, 2012 3:30 PM
51	Is there anything to worry about?	Aug 29, 2012 3:02 PM
52	If gene therapy were successful and hsct not required, what are the long term implications?	Aug 29, 2012 2:46 PM
53	There is the worry that it is not 100% successful. It is relatively new, there may be problems that have not yet been discovered. If chemo and/or radiation is needed is a worry.	Aug 29, 2012 2:39 PM
54	Risks & side effects of gene therapy, particularly in relation to a subsequent bone marrow transplant.	Aug 29, 2012 2:27 PM
55	Hoping it will work. It would be the best chance towards at cure that most would have.	Aug 29, 2012 1:04 PM

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?

1	I don't know much about this process, but provided that there are no adverse effects, I wouldn't have an issue with it.	Oct 12, 2012 3:30 PM
2	I know nothing about the possible effects of taking some bone marrow from someone whom we know does not or will not product enough cells. For the time being, I would be reluctant, unless for a very small quantity.	Oct 7, 2012 11:54 AM
3	I like this idea	Sep 30, 2012 4:31 AM
4	We did this for our son 10 years ago. I don't know whether the marrow will still work but this is something I would support. Almost like a lifeline.	Sep 17, 2012 11:20 AM
5	I am not sure I understand this. Why would you want to store bone marrow that would eventually fail anyway?	Sep 15, 2012 8:17 PM
6	I discussed with my child's consultant when they were first diagnosed and was surprised to hear that this could not be arranged. This would seem to be a sensible consideration.	Sep 14, 2012 1:06 PM
7	good idea	Sep 14, 2012 5:53 AM
8	I am very supportive of this, but understand that this is not something that is available via the NHS in the UK. I think this needs to be reconsidered now in the light of progress in gene therapies.	Sep 13, 2012 10:09 PM
9	I would be open to learning more about how this would benefit our child and the risks of putting him through the procedure.	Sep 13, 2012 7:32 PM
10	estamos completamemte deacuerdo y lo consideramos un acierto,en lo que nosotros conocemos	Sep 12, 2012 1:24 PM
11	Absolutely fine with that request.	Sep 11, 2012 6:37 PM
12	Great idea. I have confidence this could be done by competant professionals and facilities. Makes sense to me. Who pays?, because it sounds costly and I'm poor.	Sep 11, 2012 5:16 AM
13	Not applicable but have no problem with this approach.	Sep 6, 2012 8:34 AM
14	Too late for us. Sounds like a good idea for others unless cost prohibitive.	Sep 6, 2012 7:50 AM
15	Great idea!	Sep 5, 2012 1:44 PM
16	WE have done this already.	Sep 4, 2012 4:59 PM
17	I don't have a prOblem with it as long as my child is a healthy candidate for that option.	Sep 3, 2012 8:48 PM
18	do not have a problem with that	Sep 3, 2012 7:21 PM
19	Seria genial por que yo se que en algun momento la va a necesitar .	Sep 3, 2012 5:21 PM
20	It's a fantastic idea. I have some stored from many years ago and understand	Sep 3, 2012 12:24 PM

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?

that methods for freezing and thawing have advanced tremendously since then. I would actually recommend this idea to any family today, FA or not (well, cord blood storage, anyway).

21	I'm thinking it's good to have something put aside if you need it. So I'm thinking that this gene therapy isn't for kids that have already had transplants, correct? But if the procedure wasn't so painful for getting this bonemarrow out, I'm sure Craig would have something saved for the future, just in case	Sep 3, 2012 5:44 AM
22	YES !!!	Sep 2, 2012 1:27 PM
23	Good idea	Sep 2, 2012 2:27 AM
24	I think this is a great idea. It gives garanty of a possible treatment with the unique, up to day, treatment to extend FA patient lives.	Sep 1, 2012 11:34 PM
25	similar to cord blood? sounds like a good idea	Sep 1, 2012 1:11 PM
26	for sure	Aug 31, 2012 4:08 PM
27	well i don't know how far away transplant is for her but i would do it at her next bone marrow biopsy	Aug 31, 2012 2:55 PM
28	We did an autologous harvest at the U of MN in 2004, with hopes of gene therapy being available when our daughter's marrow failed. Our daughter went to transplant (successfully, at the U of MN) in 2011, with a matched unrelated donor. She has FANC, Q13x.	Aug 31, 2012 1:34 PM
29	I am in agreement with this. If this was an option for my son and something that would help improve the success of treatment for bone marrow failure, I would do this for him.	Aug 31, 2012 9:46 AM
30	A great idea	Aug 31, 2012 8:28 AM
31	I think that is a good idea. We did this before our son had his transplant. The sample could be useful in the future for research.	Aug 31, 2012 5:10 AM
32	Sounds like a good idea.	Aug 30, 2012 11:07 PM
33	It is difficult to say how many years ahead is "years ahead", isn't it? If there was a way to pinpoint approximately when bone marrow failure may occur (with a reasonable probability), then we would be willing to have the marrow stored (my child is well at the moment, but I am unaware of how much time he has before his marrow eventually fails).	Aug 30, 2012 10:57 PM
34	That seems prudent!	Aug 30, 2012 9:54 PM
35	Is that safe and for how long can it be stored?	Aug 30, 2012 4:48 PM
36	See #2	Aug 30, 2012 4:00 PM
37	Would have done this but my child is now post transplant.	Aug 30, 2012 2:48 PM

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?

38	My son had a bone marrow harvest about six years ago. We were told at the time that although the marrow freezes well, most FA cells don't survive the thawing process. I would be hesitant to believe that the storage would actually work. I also have to wonder, if this is still the case, if it is worth the risk of harvesting marrow, especially if the patient needs all of the marrow they can use at that time. I do believe in research, though!	Aug 30, 2012 12:32 PM
39	In theory I think that is a good idea	Aug 30, 2012 11:31 AM
40	Great idea	Aug 30, 2012 8:41 AM
41	My daughter already had her transplant, but I would have supported storing some bone marrow	Aug 30, 2012 8:33 AM
42	Wouldn't apply to us	Aug 30, 2012 7:54 AM
43	Good idea before they are in bm failure....	Aug 30, 2012 7:17 AM
44	I think as long as it is shown to be just as good as fresh then there would be no concerns.	Aug 30, 2012 6:43 AM
45	Good idea	Aug 30, 2012 6:26 AM
46	I'm more than ok with it.	Aug 30, 2012 4:00 AM
47	I sounds like a great idea. We have tried out PGD in order to have a HLA match in 'the backhand' and the feeling could be somehow similar: we have 'stored' something which potentially could be of great help for the FA-child. It could give hope.	Aug 30, 2012 2:04 AM
48	positive, depending on cost.	Aug 30, 2012 12:44 AM
49	Even that possibility of this is exciting. The cost, the retrieval of the bone marrow, and the risks of taking it would be things that I would have to be comfortable with.	Aug 29, 2012 11:50 PM
50	Have questions but would be open to the opportunity if the circumstances were right and the outcome had possibilities..	Aug 29, 2012 9:42 PM
51	It is a thought, if you have FA, are you considered well?	Aug 29, 2012 8:06 PM
52	Moot point here, I went through bone marrow failure 21 years ago, but still haven't had a transplant or anything. I'm treated with Oxymetholone 25mg per week currently and my counts are great.	Aug 29, 2012 7:11 PM
53	We have already done this. I wanted to do a second batch of marrow, in the event that the marrow we have harvested 8 plus years ago was "too old" for use...but it was not something the doctor would consider.	Aug 29, 2012 6:22 PM
54	Good idea.	Aug 29, 2012 6:04 PM
55	It really wouldn't matter to me just don't understand why you wuldnt do the harvest closer to the time.	Aug 29, 2012 4:30 PM

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?

56	sounds great, but expensive	Aug 29, 2012 4:28 PM
57	N/A. post transplant	Aug 29, 2012 4:07 PM
58	I am all for it.	Aug 29, 2012 3:58 PM
59	I'm 31, the biggest thing I have been through is scoliosis, my back was bad, I have 2 rods on either of my spine, hooks & screws in there, I was given lot's of meds, I remember the pain, I haven't had any other major things that hurt me like that so yes, I'm scared to death to have bone marrow taken out, I've heard it's horrible!!!!	Aug 29, 2012 3:54 PM
60	The frozen cells typically do not survive with good quality, so no questions.	Aug 29, 2012 3:39 PM
61	fine with it	Aug 29, 2012 3:34 PM
62	I don't think that's a bad idea	Aug 29, 2012 3:30 PM
63	We have done it for our daughter	Aug 29, 2012 3:12 PM
64	If possible that would be great!	Aug 29, 2012 3:02 PM
65	Great idea. We would if we could.	Aug 29, 2012 2:46 PM
66	I would very seriously consider it if there was a good purpose for it and a doctor recommended it.	Aug 29, 2012 2:39 PM
67	As long as the amount removed wouldn't cause any major complications, I'd be comfortable with this.	Aug 29, 2012 2:27 PM
68	Great idea	Aug 29, 2012 1:04 PM

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?

1	I am not that knowledgeable about the pros and cons of each option. I believe they have used viral vectors successfully in gene therapy for other diseases, so I am not particularly worried that my child would pick up some unwanted virus as a result. I would want you to do the procedure where the benefit to cost ratio is the highest (i.e. with the 'cost' being the potential side effects, and the 'benefit' being the chance of a successful outcome).	Oct 12, 2012 3:30 PM
2	No clue to answer this question	Oct 7, 2012 11:54 AM
3	I am not expert, I would rely on expert's opinion	Sep 30, 2012 4:31 AM
4	Strange to ask "amateurs" such a question. I have no views on this and would need to trust the experts. Any added risk by this procedure?	Sep 17, 2012 11:20 AM
5	Seems like it would be safe so long as the viral vector doesn't pose a risk of actually getting a virus. Also, I would want to make sure the chances of success using both types of treatment are equal.	Sep 15, 2012 8:17 PM
6	I don't feel qualified to judge these and compare them as I don't have enough specific information.	Sep 14, 2012 1:06 PM
7	sound good	Sep 14, 2012 5:53 AM
8	I don't have the medical understanding to comment on this.	Sep 13, 2012 10:09 PM
9	I would need to know more about the gene therapy. It seems like gene therapy may be less invasive and we wouldn't need to find a potential bone marrow donor.	Sep 13, 2012 7:32 PM
10	quizas la utilizacion del vector viral,consista en un tratamiento menos traumatico,e incluso mas comodo tanto para el paciente como para la familia.	Sep 12, 2012 1:24 PM
11	Really above my head to answer. I'd want the different approaches and risks clearly explained to me by my child's doctor.	Sep 11, 2012 6:37 PM
12	Wow, I don't know, I guess having them be corrected outside sounds safer on the surface, but I'm pretty ignorant about this. It seems to me it may be a lot slower, piecemeal process rather than having the virus "go to work on the entire ocean rather than desalinizing one gallon of seawater at a time"	Sep 11, 2012 5:16 AM
13	Depending on the safety record of the vector I would not be opposed to viral vector vs. bone marrow were my child pre-transplant.	Sep 6, 2012 8:34 AM
14	If it is proven safe and effective it would make no difference. Bad things can happen either way.	Sep 6, 2012 7:50 AM
15	Whatever works...	Sep 5, 2012 1:44 PM
16	Unknown. What are risks of procedure?	Sep 4, 2012 4:59 PM
17	I think that it seems a little more dangerous but also maybe not for a healthy Fanconi candidate.	Sep 3, 2012 8:48 PM

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?

18	if it is the long term correction for the problem-and it replaces the defective genes with corrected version and keeps working-that would be tremendous	Sep 3, 2012 7:21 PM
19	Yo creo que todo tipo de tratamiento es bueno , ahora por que la ciencia cada día avansa mas .	Sep 3, 2012 5:21 PM
20	Without a solid background in science, it is very difficult for me to judge. It sounds scarier simply because the idea of a viral vector being injected directly into my body sounds a bit dramatic, but I honestly would want to learn more about this. It's possible that this would be entirely safe and perhaps more effective. I should have taken genetics and biology in college!	Sep 3, 2012 12:24 PM
21	Amazing! Look how far they have come. Now if you are born with a defect it can be corrected. It's wonderful. But can they do it successfully? That is the question.	Sep 3, 2012 5:44 AM
22	I absolutely am for the a gene injection rather than the risk of removing, transporting, replacing method. I would imagine much less chance of infection or other complications.	Sep 2, 2012 1:27 PM
23	No idea about what the difference is medically	Sep 2, 2012 2:27 AM
24	Well, I do not have a problem with it, as long as it has been tested, re-tested, confirmed, re-tested and again tested and that it works and the risk to do gene therapy that way is safer than transplant!	Sep 1, 2012 11:34 PM
25	don't know enough about it	Sep 1, 2012 1:11 PM
26	Seems that the risks would be less tested outside of the body vs inside but do not know actual facts to base opinion on.	Aug 31, 2012 4:08 PM
27	i would like all that explained to me	Aug 31, 2012 2:55 PM
28	We are uneducated as to the difference and they sound equal in risk. We would seek a professional's opinion as to the advantages and disadvantages to both approaches.	Aug 31, 2012 1:34 PM
29	The same answer as question 3: Because it has not been tested on human beings yet, we do not know what complications could appear rightaway or after a periode of time (5 years, 10 years)	Aug 31, 2012 8:28 AM
30	I don't feel qualified to give an opinion on this.	Aug 31, 2012 5:10 AM
31	Would consider that to be less traumatic. Also, it avoids reducing cell death due to handling in the lab.	Aug 30, 2012 11:07 PM
32	It frightens me. I would need to do a lot of background reading in order to convince myself otherwise. Even if I were convinced to allow my child to participate, my spouse would probably object, unless we had no option and the inevitable was imminent.	Aug 30, 2012 10:57 PM
33	I would want to know more about the trade off between the two approaches.	Aug 30, 2012 9:54 PM

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?

34	?	Aug 30, 2012 4:48 PM
35	Not sure.	Aug 30, 2012 4:00 PM
36	Worry about later affects.	Aug 30, 2012 2:48 PM
37	I know it sounds horrible, but I would like to see that it actually works before it is tried on my child! Of course, I feel the same way about the other trials.	Aug 30, 2012 12:32 PM
38	Would need to know more information to make a judgement on this idea	Aug 30, 2012 11:31 AM
39	No idea what this means	Aug 30, 2012 8:41 AM
40	I don't have enough information to judge	Aug 30, 2012 8:33 AM
41	I would want more info.	Aug 30, 2012 7:54 AM
42	Which would work the best? Which is safer? Which one works the quickest with the best results overall?	Aug 30, 2012 7:17 AM
43	It would be great if that would not only correct bone marrow cells but also all the other cells in the body	Aug 30, 2012 6:26 AM
44	I would need to be certain it wouldn't harm my son in any way.	Aug 30, 2012 4:00 AM
45	Anything viral sounds bad to me. But If it was proven as a safe way to treat my FA-child, I would of course be less hesitant.	Aug 30, 2012 2:04 AM
46	worth a try!	Aug 30, 2012 12:44 AM
47	Until we have scientific studies that show that this really works, the bone marrow cells would probably be better done in the lab. Once it works, I think that the direct injection might be better since you would not be taking the bone marrow out of the body.	Aug 29, 2012 11:50 PM
48	Do not know enough information to provide an informed answer..	Aug 29, 2012 9:42 PM
49	Not sure	Aug 29, 2012 8:06 PM
50	if the viral vector is 100% safe and will NOT cause any illness or other kind of damage then I think that would be better and less intrusive than stressful painful bone marrow harvests!	Aug 29, 2012 7:11 PM
51	As noted above, I have heard that it is difficult to control a viral vector.	Aug 29, 2012 6:22 PM
52	That would be great if the risks were less than the other procedure	Aug 29, 2012 6:04 PM
53	I would want to know all side effects up front. I would want to know more info.	Aug 29, 2012 4:30 PM
54	don't like the idea of something being injected in me or my child	Aug 29, 2012 4:28 PM
55	It is a bit disconcerting. Would need more info about the vector.	Aug 29, 2012 4:07 PM

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?

56	I would want to hear how that is worked in other studies	Aug 29, 2012 3:58 PM
57	I am not sure about this, I have don't know they entire procedure, I would be open to hear about it but like I said, I don't want anymore pain.	Aug 29, 2012 3:54 PM
58	Depends on proof. What would be the difference in previous trials I which all of the cells that were removed fresh and survived the lab and reinsertion.	Aug 29, 2012 3:39 PM
59	I would be very hesitant but could be convinced if we were sure it is truly safe	Aug 29, 2012 3:34 PM
60	It's a bit scary. I'm not very aware of the current trials so I wouldn't know how to compare.	Aug 29, 2012 3:30 PM
61	I would want to read the science behind it.	Aug 29, 2012 3:12 PM
62	Sounds good	Aug 29, 2012 3:02 PM
63	I am not sure that I know enough about the procedures or potential risks to answer this. If there were no difference in success rates and potential risks then I would support direct injection.	Aug 29, 2012 2:46 PM
64	If it is more successful, or less invasive, or upkeep it would be good. As long as there is a benefit.	Aug 29, 2012 2:39 PM
65	It's a little unsettling, but the basic science does makes sense. It's a risk worth taking.	Aug 29, 2012 2:27 PM
66	Fine as long as its shown to work and the risk to benefit ratio is the same or better.	Aug 29, 2012 1:04 PM

Q6. Are you aware of any possible side effects of gene therapy where it has been tried in other conditions?

1	Cancers and leukemia	Oct 7, 2012 11:54 AM
2	I try to follow all news and I am afraid of it	Sep 30, 2012 4:31 AM
3	I know that patients have developed tumours and that some have died of leukaemia, but that was several years ago.	Sep 14, 2012 1:06 PM
4	got no information	Sep 14, 2012 5:53 AM
5	I am unclear of the side effects and am unclear whether much is known by the medical community about the short/long term side effects, given that GT trials are a relatively recent development more generally.	Sep 13, 2012 10:09 PM
6	I am not aware of any.	Sep 13, 2012 7:32 PM
7	Duh, I dunno. (sorry, it's early in the morning.)	Sep 11, 2012 5:16 AM
8	Obviously death. Leukemia.	Sep 6, 2012 8:34 AM
9	Understand it caused immune reactions, cancer and sometimes death.	Sep 6, 2012 7:50 AM
10	don't remember exactly-but created problems-with unaffected genes-and was not long term cure-had to keep redoing treatments	Sep 3, 2012 7:21 PM
11	Por que esto es algo que recién se está estudiando todo en la vida tiene su pro y contra .	Sep 3, 2012 5:21 PM
12	What are the side effects?	Sep 3, 2012 5:44 AM
13	Gene therapy resulting in death, but this was quite a few years ago.	Sep 2, 2012 1:27 PM
14	Like I said in another question : my understanding is that trials done a few years back, the patients got sick and got leukemia and died from it. So as much as I have a lot of hope for my kids with Gene therapy, it needs to be safer than transplant.	Sep 1, 2012 11:34 PM
15	This we would research heavily, if the trial applied to us. We wish you the best of luck, and await your results with breath held and great hope. Thank you for your efforts.	Aug 31, 2012 1:34 PM
16	I'm not aware.	Aug 31, 2012 5:10 AM
17	1. Death. Although it appears that was due to error, not the therapy per se. 2. Leukemia in the SCID patients	Aug 30, 2012 11:07 PM
18	An old article from 1999, I think, reported two deaths for patients being who had volunteered for gene therapy for treatment of liver cancer. I have read some positive reports for gene therapy for Parkinson's as well.	Aug 30, 2012 10:57 PM
19	Once again can't comment on an issue I have no knowledge of	Aug 30, 2012 11:31 AM
20	Again, I don't have sufficient knowledge	Aug 30, 2012 8:33 AM
21	the only ones I know of were it caused leukemia in some of the trials....	Aug 30, 2012 7:17 AM

Q6. Are you aware of any possible side effects of gene therapy where it has been tried in other conditions?

22	cancer	Aug 30, 2012 6:26 AM
23	i havent done enough research in this area...	Aug 30, 2012 12:44 AM
24	leukemia happened.	Aug 29, 2012 11:50 PM
25	Aware of possible side effects in any new therapy...	Aug 29, 2012 9:42 PM
26	No, you only ever hear that they are doing a trial of gene therapy for so and so. You never hear of what could happen they just say oh this will fix that and this is how it will be done, but side effects are rarely mentionned.	Aug 29, 2012 7:11 PM
27	I remember sev eral years ago when some children got leukemia from a gene therapy regiment for a disease other than FA.	Aug 29, 2012 6:22 PM
28	don't have info on the gene therapy Was hoping for another question/comment box: Looks like this survey is for people directly affected. My niece has FA and I do not know much about it. Not sure if you wanted the survey to be filled out by only FA parents, but I just tried to help!	Aug 29, 2012 4:28 PM
29	I have been very lucky, I've had a few things to deal with but compared to the ones I know, I've been lucky.	Aug 29, 2012 3:54 PM
30	I had no idea gene therapy was an option until my children were diagnosed with FA	Aug 29, 2012 3:30 PM
31	Leukaemia	Aug 29, 2012 2:46 PM
32	Not that knowledgeable on the topic	Aug 29, 2012 1:04 PM