Online Supplement

Original Research
Gene Therapy: Knowledge, Attitudes, and Preferences Among Individuals with Alpha-1 Antitrypsin Deficiency

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SUPPLEMENTAL TABLES

Supplemental Table 1: Information about gene therapy desired by participants

Information About Gene Therapy (GT)	%	(n)
General or Non-Specific/Uncertain Desire for More Information	24	(66)
Safety, Risks, Side Effects, Limitations	23	(63)
Efficacy/Expected Benefits	17	(48)
Results of GT Research and Clinical Trials	13	(37)
Details of Administration and Logistics of Receiving GT	13	(35)
Current Status/Timeline for Availability of GT	11	(29)
GT Methodology/Mechanism	10	(28)
Eligibility for GT	8	(21)
Compatibility with Current Treatment/Augmentation Therapy	7	(19)
None	4	(11)
Duration of the Benefits of GT	3	(9)
Heritability of GT	2	(6)
Eligibility for Future Treatments/Clinical Trials	1	(5)
Cost	1	(4)
Ethics	1	(4)
Impact/Benefit for Future Generations	1	(3)
How to Participate in a GT Clinical Trial	1	(3)
GT Manufacturer Profits	1	(3)

Data are from 275 responses with 94 responses listing two or more types of information. Additional types of information that were each listed in less than 1% of response included (n): positions of scientific foundations on GT (1), alternatives to GT (1), monitoring health after GT (1), and sources of funding for GT (1).

Supplemental Table 2: Sources of information about gene therapy trusted by participants

Information Source	%	(n)
Alpha-1 Foundation, AlphaNet, Alpha-1 Community	63	(207)
Doctor/Providers seen for AATD	28	(92)
Internet	10	(33)
Unsure	9	(31)
Scientific Literature/Research(er)	7	(24)
Government Agencies (NIH, FDA, CDC, Clinical Trials.gov) or NORD	5	(18)
University, Research Institute, Hospital/Care Center	3	(11)
Gene Therapy Specialist, Geneticist	2	(5)

Data are from 331 responses with 75 responses listing two or more information sources. Additional sources that were each listed in less than 1% of responses include (n): own research (3), artificial intelligence (2), library/books (2), pharmaceutical/biotechnology company (1), and conferences (1).

Supplemental Table 3: Additional factors that would most increase likelihood of participation in a gene therapy clinical trial

Factor	%	(n)
Low Burden of Participation (Time, Travel, Discomfort, Cost)	30	(150)
Additional Information/Knowledge (Including Safety Profile)	20	(102)
Evidence of Efficacy (Including Ability to Replace Augmentation Therapy Replacement, Potential to be Curative)	14	(71)
Ability to Continue Augmentation Therapy and Other Current Treatments During Trial	8	(42)
Evidence of Safety Including No Side Effects or Health Risks	8	(42)
Current Age or Health Status (Young Enough, Well Enough to Participate, Sick Enough to Need a Different Treatment Option)	7	(34)
None	7	(33)
Approval, Support, or Involvement of Healthcare Providers or Other Trusted Entities	6	(31)
Expectation of the Trial Benefiting Others	5	(24)
Unsure	3	(17)
Guarantee Not to Receive Placebo	2	(12)
Eligibility (Genotype and Lung Transplant Recipient Status)	2	(11)
Provision of Access to Healthcare and Health Monitoring	1	(5)

Data are from 498 responses with 79 responses listing two or more factors. Additional factors that were each listed in less than 1% of responses include (n): considered (personally) ethically acceptable (3), FDA approval (3), if not having (or potentially having) children in the future (2), the trial being large or knowing other individuals who are participating (2), guarantee of privacy (1), involvement of international researchers (1), late phase trial (1), no impact on insurance coverage (1), open-label provision included (1), positive reviews/word-of-mouth from other participants (1).

Supplemental Table 4: Additional factors that would most decrease likelihood of participation in a gene therapy clinical trial

Factor	%	(n)

High Burden of Participation (Inconvenience, Travel, Discomfort, Cost)	33	(145)
Known Safety Concerns Including Side Effects and Health Risks	25	(112)
Having to Pause Augmentation Therapy or Other Current Treatments During Trial	12	(52)
Current Age or Health Status (Being Too Old, Too Sick, or Too Healthy)	9	(42)
None	8	(34)
Insufficient Evidence of Efficacy	7	(31)
Lack of Information or Knowledge	5	(22)
Lack of Trust	4	(19)
Unsure	3	(12)
Chance of Receiving Placebo	2	(11)
Participation Discouraged or Not Encouraged by Healthcare Provider(s)	2	(10)
Ineligibility (Genotype, Other Trial Participation, Language Spoken)	1	(6)
Possible Ineligibility for Future Treatments/Trials (Including Lung Transplant)	1	(6)

Data are from 444 responses with 63 responses listing two or more factors. Additional factors that were each listed in less than 1% of responses include (n): prior lung transplant (4), possible loss of insurance benefits (3), early-phase trial/treatment not yet approved (3), religious reasons (2), negative reviews/word-of-mouth from other participants (1).

SUPPLEMENTAL METHODS

Research Instrument Development

The novel research instrument employed in this study (available below) allowed for the domains of attitudes, preferences, and knowledge to be assessed independently and for the examination of relationships between domains (example: relationship between knowledge and attitudes). The collection of demographic and health data allowed for additional analyses of subpopulations of participants defined by self-reported demographic and/or health characteristics. Participants were also asked a set of four open-ended questions regarding what additional information about gene therapy they would like to know, where they would seek information about gene therapy, and what additional factors would increase or decrease their likelihood of participating in a gene therapy clinical trial.

Individuals with AATD participated in each stage of the development of the study. Early participation included interviews to discuss the study design, anticipated outcomes and impact, and the assessment strategy for each domain. Later participation included multiple rounds of feedback on the structure of the research instrument as well as the inclusion/exclusion and wording of individual items.

Participants and Recruitment

Participants were recruited through AlphaNet, a not-for-profit organization with a mission to "provide innovative health management and customized care to individuals with AATD while funding research for a cure." AATD-affected individuals using augmentation therapy receive structured peer support through the Alpha-1 Disease Management and Prevention (ADMAP) program, while the Risk Evaluation to Achieve Continued Health (REACH) program offers disease management for those who are not currently on augmentation therapy or who are caregivers of children with AATD. Members in both the ADMAP and REACH programs were invited to participate in the study. Additional inclusion criteria were age 18 and older, documented consent to allow use of data for research, English-speaking, and an email address on file with AlphaNet. The invitation to participate was sent to 4,452 individuals meeting these criteria.

Recruitment of participants was multi-tiered and utilized AlphaNet Coordinators, who are individuals with AATD who receive training to provide support and information to

subscribers in the AlphaNet programs. Coordinators complete monthly structured phone conversations with the members they serve. Prior to the opening of the study, a brief presentation was given during the regular monthly online meeting of AlphaNet Coordinators to introduce the study and invite Coordinators to discuss study participation with their members during the next monthly call. Alpha-Net Coordinators were also provided with an FAQ document to aid in answering questions from members (available below). Survey invitations were then sent via email, and a follow-up reminder was sent two weeks after the initial invitation (available below).

Data Collection

The research instrument was administered, and data were collected through AlphaNet's online patient portal. Participants who were unable to complete the survey through the online portal completed it on the phone with the help of their AlphaNet Coordinator who then entered their responses into the portal (n = 32, 2.9% of respondents). The age of all participants 90 or older was recorded as 90 to protect anonymity.

Qualitative Data Analysis

Responses to open-ended questions were evaluated using a thematic analysis workflow to identify the most common themes included within the responses to each of the four open-ended questions. This included initial theme identification and coding by M.S. followed by a second round of coding by S.M.. The lists of themes present in each collection of

responses were agreed upon, and the themes present in each response were assigned by consensus.

RESEARCH INSTRUMENT

Introduction to the Gene Therapy Survey

Thank you for your participation in our study! This survey consists of four parts. Based on our pilot study, the total completion time is expected to be 15-20 minutes.

As you may know, gene therapies for alpha-1 antitrypsin deficiency (AATD) have entered clinical trials and have the potential to transform how AATD is treated or potentially cured. However, there is a lack of research exploring the Alpha-1 Community's preferences and attitudes towards gene therapies or the Alpha-1 Community's collective knowledge of genetics and gene therapies. The purpose of this study is to fill these gaps.

Understanding the preferences and attitudes of members the Alpha-1 Community towards gene therapies can help inform clinical trial design that meets the needs of potential participants. Gaining a better understanding of the Alpha-1 Community's collective knowledge of genetics and gene therapies can help identify gaps or misconceptions that exist and guide efforts to ensure that members of the Community are fully informed when making decisions about participating in gene therapy clinical trials.

Part 1: Demographic and Health Characteristics

We understand you have likely provided some of the information requested below for prior AlphaNet surveys. We are asking for this important information here to ensure we have the most current information possible for our project. We sincerely appreciate your willingness to provide this information.

How long has it been since you were diagnosed with alpha-1 antitrypsin deficiency (AATD)?

- ◯ Less than 1 year
- ◯ More than 1 year but less than 3 years
- ◯ More than 3 years but less than 5 years
- ◯ More than 5 years but less than 10 years
- ◯ More than 10 years

What is your AATD genotype?

- ◯ ZZ
- ◯ SZ
- ◯ MZ
- ◯ Z/null
- ◯ SS
- ◯ MS
- ◯ S/null
- ◯ Null/Null
- ◯ Other (including F or I or other rare alleles) Please specify_____
- ◯ Unknown

Have you ever been diagnosed with lung disease by a healthcare provider?

- ◯ Yes
- ◯ No

In the past year, how many times have you experienced exacerbations or flare-ups of your lung problems?

◯ 0
◯ 1-2
◯ 3-4
◯ 5 or more

In the past year, how many times have you been hospitalized for lung disease?
◯ 0
◯ 1-2
◯ 3-4
◯ 5 or more

Do you currently use supplemental oxygen?
◯ Yes
◯ No

Have you ever been diagnosed with liver disease by a healthcare provider?
◯ Yes
◯ No

In the past year, how many times have you been hospitalized for liver disease?
◯ 0
◯ 1-2
◯ 3-4
◯ 5 or more

How many in-person or virtual conferences or learning days on AATD have you attended?
◯ 0
◯ 1
◯ 2-3
◯ 4-5
◯ 6 or more

What is the highest level of education or degree you have completed?
◯ Less than high school
◯ High school diploma, GED, or equivalent
◯ Some college credit without degree
◯ Trade school, technical certification, or equivalent
◯ Associate's degree
◯ Bachelor's degree
◯ Master's degree
◯ Doctoral degree

Part 2: Attitudes Towards Gene Therapies

This portion of the survey is designed to help us better understand current attitudes of members of the Alpha-1 Community towards gene therapies.

Gene therapy = any treatment that adds, removes, and/or modifies genetic material.
AATD = alpha-1 antitrypsin deficiency
FDA = The Food and Drug Administration of the Unites States of America is the regulatory body that oversees clinical trials.

Please rate each of the following as:

	very low 1	somewhat low 2	intermediate 3	somewhat high 4	very high 5
Your willingness to participate in a clinical trial for AATD that does NOT use gene therapy.					
Your willingness to participate in a gene therapy clinical trial for AATD.					
Your willingness to receive a gene therapy to treat AATD if it is approved by the FDA.					
Your concern about the safety of gene therapy clinical trials for AATD.					
Your concern about the safety of (future) FDA-approved gene therapies for AATD.					
Your support of research and development of gene therapies for AATD.					
Your interest in learning more about gene therapy specifically for AATD.					
Your interest in learning more about the limitations of gene therapies.					
Your interest in learning more about the known risks associated with gene therapies.					
Your level of trust in the Alpha-1 Foundation and AlphaNet.					
Your level of trust in the healthcare provider(s) you see for AATD.					
Your level of trust in the scientific research community.					
Your level trust in the healthcare system.					
Your level of trust in the FDA.					
Your level of trust in pharmaceutical and biotechnology companies.					

Part 3: Gene Therapy Preferences and Priorities

This portion of the survey is designed to help us better understand the preferences and priorities of members of the Alpha-1 Community regarding participation in a gene therapy clinical trial for AATD.
Please rate how each of the following would influence your likelihood of participating in a gene therapy clinical trial for AATD.

Please consider each item independent from all others.

Gene therapy = any treatment that adds, removes, and/or modifies genetic material.
AATD = alpha-1 antitrypsin deficiency

How would each item influence your decision to participate in an AATD gene therapy clinical trial?

	Strongly discourage participation 1	Discourage participation 2	Neutral (would not affect decision to participate) 3	Encourage participation 4	Strongly encourage participation 5
The clinical trial is the first time the gene therapy has been studied in humans.					
The clinical trial is unlikely to provide a health benefit to you. However, it is likely to benefit future individuals with AATD.					
The gene therapy is only designed to address lung problems in AATD.					
The gene therapy is only designed to address liver problems in AATD.					
The gene therapy is designed to address BOTH lung and liver problems in AATD.					
The gene therapy is NOT expected to require any re-dosing to maintain health benefits.					
The gene therapy is expected to require YEARLY re-dosing to maintain health benefits.					
The gene therapy is expected to require MONTHLY re-dosing to maintain health benefits.					
The gene therapy is expected to require WEEKLY re-dosing to maintain health benefits.					
There is a chance you could be assigned to a placebo group.					
Participation in this gene therapy clinical trial may exclude you from participating in future gene therapy clinical trials.					
You are confident that you understand the risks associated with participating in the clinical trial.					
You are NOT confident that you understand the risks associated with participating in the clinical trial.					
You are told that your personal information, biological samples, and genetic information will be secured/protected using best practices, but security cannot be guaranteed.					
A trusted member of your healthcare team has reviewed the trial and encouraged you to participate.					

A trusted member of your healthcare team has reviewed the trial and expressed concerns about your participation.					

Free Response (optional)
We recognize that these questions do not address all of the factors that are considered important when deciding whether or not to participate in a gene therapy clinical trial. Below, you have the opportunity to list/describe additional factors that would influence your decision to participate.

What additional factors would most increase the likelihood of you participating in an AATD gene therapy clinical trial?

What additional factors would most decrease the likelihood of you participating in an AATD gene therapy clinical trial?

Part 4: Genetics and Gene Therapy Knowledge

This portion of the survey is designed to help us better understand the current collective understanding of genetics and gene therapies within the Alpha-1 Community. We hope you will see this part of the survey as an opportunity to help us identify how we can further empower the Alpha-1 Community to make informed decisions regarding gene therapy clinical trial participation through improvements in how information on genetics and gene therapies is provided to the Community.

Based on your current understanding of AATD genetics and gene therapy, select the best ending to each of the statements below. You can also select "unsure" if you are unsure which choice is best.

We request that you <u>do not</u> look up the answers to these questions while completing the survey so that our data will most accurately reflect the current collective knowledge of the Alpha-1 Community. The choices that we consider to be best, along with brief explanations of why, will be emailed to you after you complete and submit this survey.

Genes contain information that directs the production of…
- ◯ proteins.
- ◯ chromosomes.
- ◯ Unsure

A human cell typically has…
- ◯ one copy of each gene.
- ◯ two copies of each gene.
- ◯ Unsure

A person's lung cells and liver cells…
- ◯ have entirely different genes.
- ◯ have exactly the same genes but use some genes differently.
- ◯ Unsure

It is intended that the addition, removal, or modification of genetic material that results from a gene therapy being given to an adult…
- ◯ will not be inherited by their future biological children.
- ◯ will be inherited by their future biological children.
- ◯ Unsure

Viral vectors, which are commonly used to deliver a gene therapy to target cells…
- ◯ cannot cause a viral infection.
- ◯ can potentially cause a viral infection.
- ◯ Unsure

The health benefits of a gene therapy in an individual…
- ◯ are never lost no matter which type of gene therapy is used.
- ◯ can be lost over time depending on which type of gene therapy is used.
- ◯ Unsure

The earliest phase of a clinical trial is known as phase I. The purpose of a phase I clinical trial is to…
- ◯ determine the safe dosage range and identify side effects of a treatment in a small number of

participants.
◯ determine the safety and effectiveness of a treatment in a large number of participants.
◯ Unsure

A patient who has previously participated in a gene therapy clinical trial…
◯ can be excluded from participating in a current gene therapy clinical trial because of their participation
in the previous clinical trial.
◯ cannot be excluded from participating in a current gene therapy clinical trial because of their
participation in the previous clinical trial.
◯ Unsure

A gene therapy clinical trial for AATD…
◯ cannot require participants to pause their augmentation therapy during the trial.
◯ can require participants to pause their augmentation therapy during the trial.
◯ Unsure

Most of the alpha-1 antitrypsin found in the lungs of a healthy individual (MM) is produced by cells in…
◯ the liver.
◯ the lungs.
◯ Unsure

The liver problem that can occur in a ZZ individual with AATD is caused by…
◯ not making enough alpha-1 antitrypsin in the liver.
◯ making a faulty form of alpha-1 antitrypsin that gets stuck inside liver cells.
◯ Unsure

If an MM individual and a ZZ individual have a child together, the child is expected to be…
◯ MM
◯ MZ
◯ ZZ
◯ Unsure

Free Response (optional)

What would you most like to know more about regarding gene therapies for AATD?

Where would you go to find trustworthy information about gene therapies for AATD?

FOLLOW UP EMAIL: BEST ANSWER EXPLANATIONS

Thank you for participating in the gene therapy survey. The final section of the survey focused on knowledge of genetics and gene therapy. Your responses to this section will help us improve the information we provide to Alphas about genetics and gene therapies. The ultimate goal is to empower Alphas to make informed decisions about gene therapy clinical trial participation.

Below are the answers that we consider to be best and a brief explanation for each answer. Please do not share this information with other Alphas, as they may still plan to complete the survey. We want to get accurate information about current knowledge among Alphas so we can develop educational materials that address gaps in knowledge.

1. Genes contain information that directs the production of…**proteins**.

   In each of our cells, genetic information (DNA) is packaged into 46 chromosomes. Each chromosome contains a long molecule of DNA. A gene is a segment of that DNA molecule that codes for something. Thus, genes do not direct the production of chromosomes. Rather, they are contained within chromosomes.

   Most of our genes code for a specific protein. That is, the information contained in genes is used by our cells to direct the production of the proteins that they need. For example, the *SERPINA1* gene (sometimes called the *AAT* gene) directs the production of the alpha-1 antitrypsin (AAT) protein.

2. A human cell typically has…**two copies of each gene**.

   The 46 chromosomes typically present in each of our cells can be thought of as two sets of 23 chromosomes. We inherit one set of 23 chromosomes from our mother and another set of 23 chromosomes from our father. Thus, we typically have two copies of each chromosome. Because we have two copies of each chromosome, we have two copies of each gene.

3. A person's lung cells and liver cells…**have exactly the same genes but use some genes differently**.

   All of our cells are the descendants of a single original cell whose genetic information was established at fertilization. The complete set of genes present in that cell is passed on to each of the trillions of cells in our body. This means that each of our cells has the same set of genes (around 20,000 genes). However, not all of those genes are used by all of our cells.

   Some genes that are important for basic functions of all cells are used by all types of cells, but genes that are important for the unique functions of a particular cell type may only be used by that cell type. For example, a gene that is important for a unique function of liver cells would not be used in lung cells, and vice versa. Thus, the reason different types of cells are different from each other is not because they have different genes. It is because they use some of the genes differently.

4. It is intended that the addition, removal, or modification of genetic material that results from a gene therapy being given to an adult…**will not be inherited by their future biological children**.

   Unless the addition, removal, or modification of genetic material occurs in egg or sperm cells (or cells that can produce or become egg or sperm cells), it will not be inherited by future children. Currently, gene therapies given to adults are typically targeted to the specific types of cells affected by the condition being treated.

   For example, a gene therapy for AATD would likely target liver cells and/or lung cells. Any changes that result from the gene therapy would remain within those cells and (potentially) the cells that they produce. One of the important goals for most gene therapies is to avoid any change to egg or sperm cells, so any changes produced by the gene therapy would not be passed on to future children.

5. Viral vectors, which are commonly used to deliver a gene therapy to target cells…**cannot cause a viral infection**.

   Viral vectors are a useful way to deliver gene therapies to target cells because they use the ability of viruses to get into human cells. The viral vectors used in gene therapy cannot cause a viral infection because the genes needed for the virus to cause harm have been removed or inactivated. Essentially, the outer envelope or shell of the virus is used to deliver the therapeutic components of the gene therapy to the cell type(s) being targeted.

   This does not mean that viral vectors have zero risk. Any specific risks known to be associated with the use of a particular viral vector in a gene therapy clinical trial will be disclosed to participants. Common side effects are chills and flu-like symptoms that are difficult to distinguish from an infection. This risk is also like some vaccination side effects.

6. The health benefits of a gene therapy in an individual…**can be lost over time depending on which type of gene therapy is used.**

   Not all gene therapies work in the same way. Some gene therapies are expected to produce benefits that last for a lifetime. Others are expected to produce benefits that are lost over time, which may make re-dosing necessary.

7. The earliest phase of a clinical trial is known as phase I. The purpose of a phase I clinical trial is to…**determine the safe dosage range and identify side effects of a treatment in a small number of participants**.

   Phase I clinical trials are designed to determine the safe dosage range and identify side effects of a treatment (such as a gene therapy) in a relatively small number of participants. In gene therapy clinical trials, phase I participants are typically individuals with the condition/disease, while in many other types of clinical trials, phase I participants are healthy volunteers. Later phases of clinical trials include more participants and focus on safety and effectiveness.

8. A patient who has previously participated in a gene therapy clinical trial…**can be excluded from participating in a current gene therapy clinical trial because of their participation in the previous clinical trial**.

   A patient who has previously participated in a gene therapy clinical trial can be excluded from participating in a current gene therapy clinical trial because it may be difficult for the research team to know if any observed effects are due to the previous trial or the current trial. Some gene therapy clinical trials might not exclude individuals who have previously participated in a different gene therapy clinical. Each clinical trial will have a clear description of eligibility criteria.

9. A gene therapy clinical trial for AATD…**can require participants to pause their augmentation therapy during the trial**.

   A gene therapy clinical trial can require participants with AATD to pause their augmentation therapy during the trial in order to participate. Individuals can always choose to end their participation in a clinical trial (and resume augmentation therapy) at any time. Some gene therapy clinical trials may not require participants with AATD to pause their augmentation therapy.

10. Most of the alpha-1 antitrypsin found in the lungs of a healthy individual (MM) is produced by cells in…**the liver**.

    The *SERPINA1* gene (sometimes called the *AAT* gene) directs the production of the alpha-1 antitrypsin (AAT) protein. The M variant of the *SERPINA1* gene directs production of the normal form of AAT protein.

    The *SERPINA1* gene is highly used by liver cells and only minimally used by lung cells. The AAT protein produced by liver cells is transported out of the liver cells, into the bloodstream, and to the lungs where it helps to maintain lung health.

    Because lung cells don't use the *SERPINA1* gene nearly as much as liver cells do, only a small amount of the AAT protein found in the lungs is actually produced by cells in the lungs. Thus, most of the AAT protein found in the lungs is produced by liver cells.

11. The liver problem that can occur in a ZZ individual with AATD is caused by…**making a faulty form of alpha-1 antitrypsin that gets stuck inside liver cells**.

    The Z variant of the *SERPINA1* gene directs the production of a faulty form of the AAT protein that clumps together and cannot be transported out of liver cells properly. This has two important consequences:

    First, because ZZ individuals only make the faulty Z form of the AAT protein, these proteins clump together and accumulate in liver cells. This increases risk for liver disease.

Second, because the Z form AAT proteins remain stuck in liver cells, the lungs do not receive the supply of AAT that they need. This results in a deficiency of AAT in the lungs that puts ZZ individuals at significantly increased risk for lung disease.

12. If an MM individual and a ZZ individual have a child together, the child is expected to be…**MZ**.

    An MM individual has two M variants of the *SERPINA1* gene (sometimes called the *AAT* gene). Each individual only passes one of their two variants on to their child, so the MM individual will pass on an M variant. A ZZ individual has two Z variants of the *SERPINA1* gene—so the ZZ individual will pass on a Z variant.

    The child will receive an M variant from one parent and a Z variant from the other. Thus, the child will be MZ.

    As an MZ individual, the child's liver cells will produce both the normal M form of the AAT protein (from their M variant of the *SERPINA1* gene) and the faulty Z form of the AAT protein (from their Z variant of the *SERPINA1* gene). The M form of the AAT protein is expected to get out of the liver cells and be transported to the lungs through the blood, while the Z form of the AAT protein is expected to remain stuck in the liver cells.

    This means the MZ child will end up having some M form AAT in their lungs but not as much as their MM parent. They will also have some Z form AAT accumulation in their liver cells but not as much as their ZZ parent. Thus, the child has more risk of developing lung and liver disease than their MM parent, but less risk of developing lung and liver disease than their ZZ parent.

INITIAL ELECTRONIC INVITATION

ALREADY PORTAL-ENABLED
SURVEY INVITATION EMAIL TEMPLATE

Hi [Subscriber name will appear here],

Gene therapies have the potential to transform how alpha-1 antitrypsin deficiency is treated or potentially cured. These therapies have started to be investigated in clinical trials.
We have very little understanding of Alphas' preferences and concerns about gene therapies, or Alphas' general knowledge about genetics and gene therapies. AlphaNet has developed a one-time survey to learn about these topics.

Most of the survey questions ask about your opinions, so there are no right or wrong answers to these questions. Some of the survey questions ask about knowledge of genetics and gene therapy. We will send you the best answers to these questions after you complete and submit the survey.

Survey findings will help us develop educational materials that are targeted to the information needs of Alphas, and hopefully will contribute to the development of gene therapy clinical trials that are more appealing to potential participants.
It will take approximately 15-20 minutes to complete the survey.

Please complete this survey, which is available at https://subscriber.alphanet.org. This survey is only available in the portal. It is not available by phone, email, or US mail.

Thanks,

[The AlphaNet Coordinator's name, email address, and phone number will appear here]

REMINDER ELECTRONIC INVITATION

ALREADY PORTAL-ENABLED
SURVEY INVITATION EMAIL TEMPLATE

Hi [Subscriber name will appear here],

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