

THE PUBLIC AND GENETIC EDITING, TESTING, AND THERAPY

January 2016



HARVARD
T.H. CHAN
SCHOOL OF PUBLIC HEALTH

STAT

INTRODUCTION

A new poll of adults in the United States conducted by *STAT* and Harvard T.H. Chan School of Public Health shows that Americans have mixed views on emerging genetic technologies, including changing the genetic characteristics of unborn babies (germline editing), genetic testing, and gene therapy treatments. This survey was conducted to understand the public's views on emerging technologies in human genetics, and whether or not the public supports federal government funding and regulation of these technologies. It was conducted in response to the current scientific debate over gene editing, as well as the recent National Academy of Sciences' (NAS) International Summit on Human Gene Editing.

According to the National Academies of Sciences, Engineering, and Medicine, powerful new gene-editing technologies, such as CRISPR-Cas9, have the potential to advance science and treat diseases, but they also raise concerns and present complex scientific, ethical, and governance challenges.¹ In particular, these new technologies may potentially be used to make genetic changes that could be passed on to future generations, thereby changing the human gene pool. At the end of the NAS Summit, the organizing committee concluded that while gene editing for clinical use in somatic cells holds great promise, it would be irresponsible to proceed with any clinical use of germline editing.² This corresponds with the findings in our poll: a majority of Americans favor gene therapy for clinical use among patients with serious diseases, but they largely oppose genetic editing of unborn babies, even to reduce their risk of developing serious diseases. The organizing committee of the Summit also called on the NAS to create an ongoing international forum to engage a wide range of perspectives, including members of the general public.

This poll was conducted to ask Americans about some of the issues involved in the emerging public dialogue on genetic technologies. It sought the public's views on seven main questions, in response to this summit: (1) What does the public think about changing the genetic characteristics of unborn babies? (2) Does the public think the government should fund research on changing the genes of unborn babies? (3) Who does the public think should decide whether or not to allow the genes of unborn babies to be changed? (4) Who has undergone genetic testing, and why? (5) Is the public interested in taking a genetic test in the future? (6) What does the public think about gene therapy? (7) Does the public think the government should fund research on gene therapy?

¹ For more information, see the National Academies of Sciences, Engineering, and Medicine. "Human Gene-Editing Initiative." Available at: <http://www.nationalacademies.org/gene-editing/index.htm>.

² National Academies of Sciences, Engineering, and Medicine. International Summit on Human Gene Editing. "On Human Gene Editing: International Summit Statement." December 3, 2015. Available at: <http://www8.nationalacademies.org/onpinews/newsitem.aspx?RecordID=12032015a>.

WHAT DOES THE PUBLIC THINK ABOUT CHANGING THE GENETIC CHARACTERISTICS OF UNBORN BABIES?

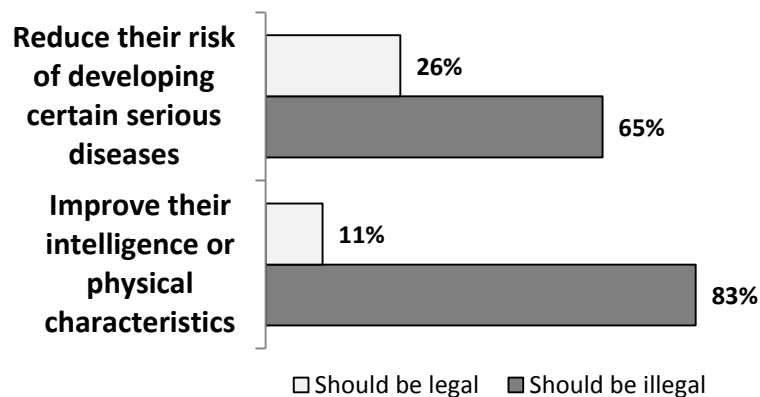
Recently, there have been discussions among international experts on the potential for changing the genetic characteristics of unborn babies to improve their health, physical traits, or intelligence (known as “germline editing”). About three in ten Americans (31%) have heard quite a lot or some about discussions on this issue, while almost seven in ten (69%) have heard not much or nothing at all.

There is an ongoing controversy over these new human gene-editing technologies, where some scientists argue that parents should have the right to change the genes of unborn babies to prevent certain serious diseases such as Huntington’s disease, cystic fibrosis, or some types of muscular dystrophy. In addition, some also believe parents should be able to change their unborn babies’ genes to select desired characteristics such as intelligence, or physical traits such as athletic ability or appearance. On the other hand, some scientists argue that changing the genes of unborn babies is an unsafe experiment with uncertain consequences, these genetic changes will be passed onto future generations, they may cause irreversible harm, and they could lead to a society of genetic “haves” and “have nots.”

Figure 1 depicts how Americans feel about changing the genes of unborn babies in two circumstances. Roughly one in four Americans (26%) think changing the genes of unborn babies should be legal to reduce their risk of developing certain serious diseases, while 65 percent think it should be illegal. When asked about changing the genes of unborn babies to improve their intelligence or physical characteristics, only 11 percent of Americans think that it should be legal, while the vast majority (83%) of adults think it should be illegal.

FIGURE 1: Americans’ Views on Changing the Genes of Unborn Babies

Q. Do you think that changing the genes of unborn babies to ... should be legal or should be illegal?



The International Summit on Human Gene Editing highlighted the controversies among experts on whether it is responsible to proceed with any clinical use of germline editing, and the results of this survey align with scientists’ views about the need for caution in proceeding with experimental techniques to change the genes of unborn babies. However, although a majority of the public did not express support for changing the genes of unborn babies, those who said they had heard or read about these discussions (41%) were twice as likely as those who had not (20%) to say they thought it should be legal to change the genes of unborn babies to reduce their risk of developing certain serious diseases.

DOES THE PUBLIC THINK THE GOVERNMENT SHOULD FUND RESEARCH ON CHANGING THE GENES OF UNBORN BABIES?

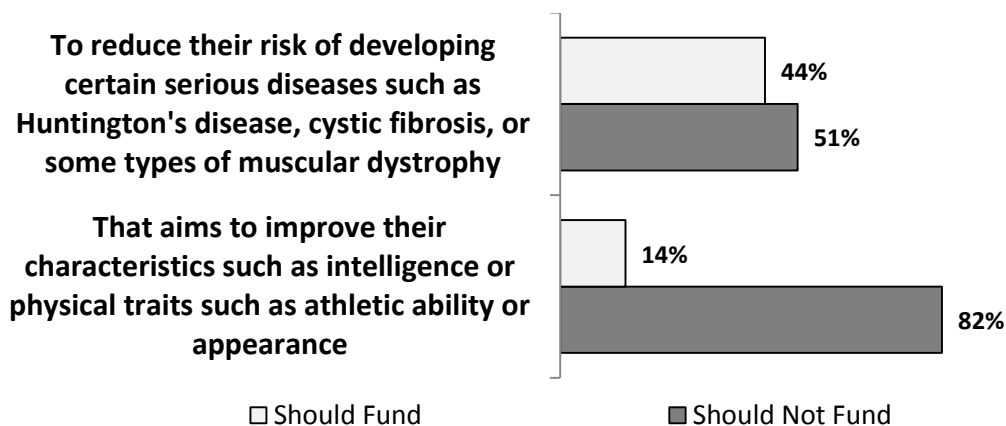
While Americans largely oppose changing the genes of unborn babies, their attitudes toward federal government funding of research on changing the genes of unborn babies to reduce the risk of developing diseases is more positive. More than four in ten Americans (44%) think that the federal government should fund scientific research on changing the genes of unborn babies to reduce their risk of developing certain serious diseases such as Huntington’s disease, cystic fibrosis, or some types of muscular dystrophy (*Figure 2*). Fifty-one percent of Americans think the federal government should not fund this type of research.

Only 14 percent of Americans think the federal government should fund scientific research on changing the genes of unborn babies that aims to improve their characteristics such as intelligence or physical traits such as athletic ability or appearance. More than eight in ten adults (82%) think the federal government should not fund this type of research.

Although a majority of the public did not express support for federal research funding for changing the genes of unborn babies, a majority (54%) of those who said they had heard or read about these discussions supported funding for changing the genes of unborn babies to reduce their risk of developing certain serious diseases, compared to 39 percent of those who had not heard or read much about this issue.

FIGURE 2: Americans’ Views on Federal Funding for Research On Changing the Genes of Unborn Babies

Q. Do you think the federal government should or should not fund scientific research on changing the genes of unborn babies...

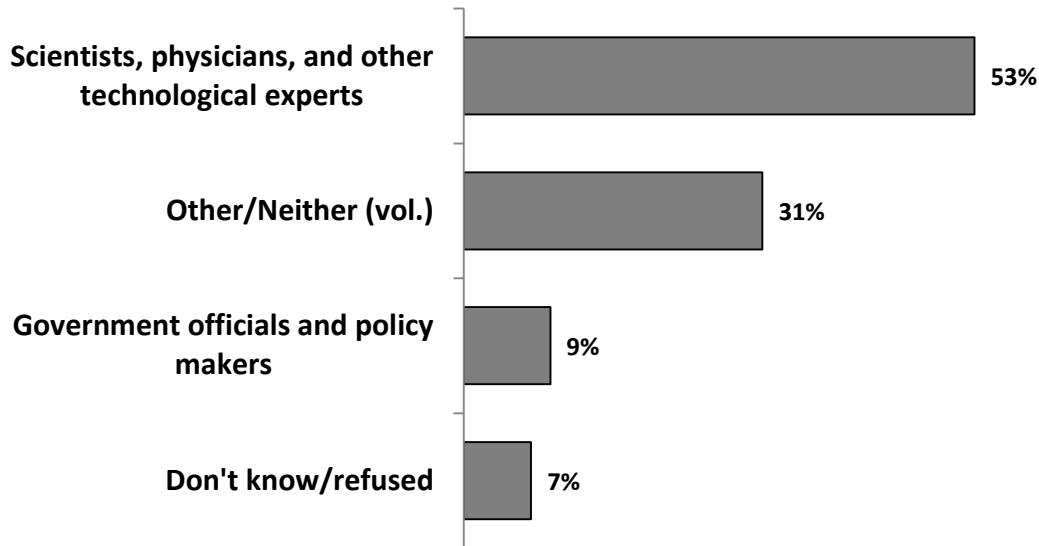


CHANGING THE GENES OF UNBORN BABIES: WHO SHOULD DECIDE?

When asked about who should make decisions on whether or not to allow changing the genes of unborn babies to improve their health, physical traits, or intelligence, a majority of adults (53%) said we should leave it up to scientists, physicians, and other technological experts. Only 9 percent said we should leave it up to government officials and policy makers, while 31 percent said we should leave it up to someone else. Only a small percentage of those who self-identified as either Democrats (16%) or as Republicans (6%) thought we should leave it up to the government to make these decisions.

FIGURE 3: Who Should Decide Whether or Not to Allow Changing the Genes of Unborn Babies?

For decisions on whether or not to allow changing the genes of unborn babies to improve their healthy, physical traits, or intelligence, do you think we should leave it up to...



WHO HAS UNDERGONE GENETIC TESTING, AND WHY?

Recently, there have been some discussions in the news about genetic tests, which use DNA to predict and diagnose certain diseases, or can identify where a person's ancestors come from. Half (50%) of Americans say they have heard or read some or quite a lot about recent discussions on genetic testing, while half have either not heard much (24%) or anything at all (26%). A majority (57%) of Americans believe these tests are mostly accurate and reliable, while a substantial portion believes they are not reliable (24%) or remains unsure (19%).

Only 6 percent of adults say they have personally ever had genetic testing done, and the main reasons for doing so are split between concerns about health problems for future children (35%), a desire to learn more about heritage or family history (25%), concerns about future health problems for themselves (18%), and some other reason (18%). The vast majority (81%) of people who have had genetic testing done say the results were helpful to them.

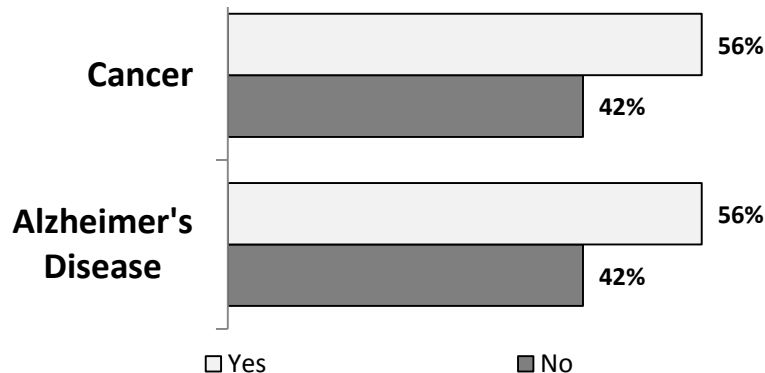
INTEREST IN TAKING A GENETIC TEST IN THE FUTURE

Despite the low percentage of Americans who have been tested in the past, a majority (56%) of adults would be interested in taking a genetic test that indicated whether or not they were likely to develop Alzheimer's disease or cancer in the future. In particular, adults ages 30-64 are more likely to say they would be interested in taking a test for Alzheimer's disease (62%) compared to both younger adults (50% of those 18-29) and older adults (47% of those 65+). Adults ages 30-64 were also more likely to say they would be interested in taking a test for cancer (63%) compared to both younger adults (48% of those 18-29) and older adults (47% of those 65+).

Almost one in five adults (19%) say they or someone in their immediate family has ever been told that they carried a gene predisposing them to certain serious diseases, such as Alzheimer's disease, cancer, heart disease, or sickle cell anemia.

FIGURE 4: Interest in taking a genetic test in the future to know your likelihood of developing diseases

Q. If a genetic test was available that could tell you whether or not you were likely to develop ... in the future, would you want to take the test or not?



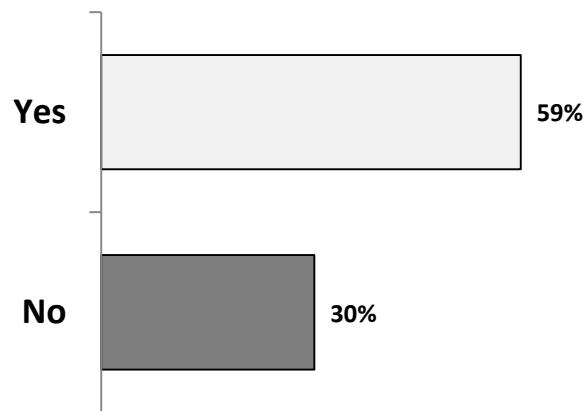
WHAT DOES THE PUBLIC THINK ABOUT GENE THERAPY?

In addition to genetic testing, scientists are developing experimental ways to change human genes to treat diseases through gene therapy. Gene therapy involves replacing a person's genes to treat specific diseases, with the potential to treat diseases such as HIV/AIDS, sickle cell anemia, and some types of cancers.³ Some oppose gene therapy because it has a risk of health complications, or they believe it may lead to unethical use or it is unnatural.

Despite low familiarity with genetic technologies, a majority (59%) of Americans think the Food and Drug Administration (FDA) should approve gene therapy treatments for use in the United States, while 30 percent think they should not. Democrats and Republicans do not differ in their support for FDA approval of gene therapy treatments.

FIGURE 5: Government Regulation of Gene Therapy in the U.S.

Q. Do you think the Food and Drug Administration should or should not approve gene therapy treatments for use in the United States?



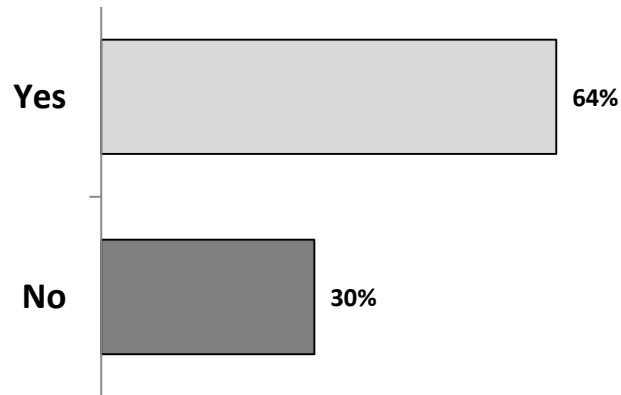
³ Carroll D. 2014. "Genome Engineering with Targetable Nucleases." *Annual Review of Biochemistry* 83: 409–439.

DOES THE PUBLIC THINK THE GOVERNMENT SHOULD FUND RESEARCH ON GENE THERAPY?

More than two-thirds (64%) of adults say they think the federal government should fund scientific research on developing new gene therapy treatments. A majority of both Republicans (59%) and Democrats (74%) support federal funding for scientific research on gene therapy. This finding shows bipartisan support for gene therapy research and treatments, which may be included in the 21st Century Cures Act in Congress that passed the House of Representatives in July 2015.

FIGURE 6: Americans' Views on Federal Research Funding for Gene Therapy Treatments

Q. Do you think the federal government should or should not fund scientific research on developing new gene therapy treatments?



CONCLUSION

Even though the scientific community has yet to reach a consensus regarding the risks and potential benefits of genetic technologies, our poll finds that at this point in time, the public has definite views on these issues. While many in the public are largely unfamiliar with genetic technologies, they are generally interested in genetic testing, supportive of research and FDA approval of new gene therapy treatments, and opposed to changing the genetic characteristics of unborn babies, even to reduce their risk of developing certain serious diseases. Americans who say they are more familiar with these issues show higher approval for their research applications and government funding.

A majority of Americans believe that scientists, physicians and other technological experts should decide whether or not to allow changing the genes of unborn babies, while less than one in ten Americans believe that government officials and policy makers should decide.

The views of the public are largely in line with the conclusions of the organizing committee of the recent National Academy of Sciences' (NAS) International Summit on Human Gene Editing: while a majority favor gene therapy for clinical use among patients with serious diseases, they generally oppose genetic editing of the unborn. It is important to note that these findings could change over time, as national and international discussions on these issues continue to evolve.

METHODOLOGY

The results are based on polling conducted by *Stat* and Harvard T.H. Chan School of Public Health. Representatives of the two organizations worked closely to develop the survey questionnaire and analyze the results of the poll. *Stat* and Harvard T.H. Chan School of Public Health paid for the survey and related expenses.

The project team was led by Robert J. Blendon, Sc.D., Richard L. Menschel Professor of Health Policy and Political Analysis at Harvard T.H. Chan School of Public Health, and Gideon Gil, Managing Editor, Enterprise and Partnerships of *Stat*. Harvard research team also included John M. Benson, Mary T. Gorski, and Justin M. Sayde.

Interviews were conducted with a nationally representative sample of 1,000 randomly selected adults, ages 18 and older, via telephone (including cell phones and landlines) by SSRS of Media, Pennsylvania. The interviewing period was January 13 – 17, 2016. The questions about ever having had a genetic test were also asked of a supplemental sample, January 20 – 24, 2016. For those questions, the combined sample was 1,489 U.S. adults. The data were weighted to reflect the demographics of the national adult population as described by the U.S. Census.

When interpreting these findings, one should recognize that all surveys are subject to sampling error. Results may differ from what would be obtained if the whole U.S. adult population had been interviewed. The margin of error for the total sample in the main poll is ± 3.7 percentage points; for the combined sample, the margin of error is ± 3.0 percentage points.

Possible sources of non-sampling error include non-response bias, as well as question wording and ordering effects. Non-response in telephone surveys produces some known biases in survey-derived estimates because participation tends to vary for different subgroups of the population. To compensate for these known biases and for variations in probability of selection within and across households, sample data are weighted by household size, cell phone/landline use and demographics (sex, age, race/ethnicity, education, and region) to reflect the true population. Other techniques, including random-digit dialing, replicate subsamples, and systematic respondent selection within households, are used to ensure that the sample is representative.



STAT/Harvard T.H. Chan School of Public Health Poll

The Public and Genetic Editing, Testing, and Therapy

This survey was conducted for *STAT* and Harvard T.H. Chan School of Public Health via telephone by SSRS, an independent research company. Interviews were conducted via telephone (cell phone and landline) January 13 – 17, 2016, among a nationally representative sample of 1,000 U.S. adults. The margin of error for total respondents is +/- 3.7 percentage points at the 95% confidence level. Questions 3 through 5 were also asked of a supplemental sample, January 20 – 24, 2016. For those questions, the combined sample was 1,489 U.S. adults, with a margin of error for total respondents of +/- 3.0 percentage points at the 95% confidence level. More information about SSRS can be obtained by visiting www.ssrs.com

GENETIC TESTING AND GENE THERAPY

(Asked of split sample; n = 480)

ST-01. Recently, there have been some discussions in the news on genetic testing. Scientists have developed tests that can use the DNA sequence of people’s genes to help predict whether an individual, or their future children, might develop certain diseases, as well as to diagnose whether they have certain diseases. Similar tests can also be used to identify where a person’s ancestors came from.

How much have you heard or read about these discussions on genetic testing?

Quite a lot	Some	Not much	Nothing at all	Don’t know/ Refused
19	31	24	26	--

(Asked of split sample; n = 480)

ST-02. Do you believe tests that use genes to predict diseases are mostly accurate and reliable, or not?

Yes, accurate and reliable	No, not accurate and reliable	Don’t know/ Refused
57	24	19

(Asked in main poll and supplement; n = 1489)

ST-03. Have you personally ever had any type of genetic testing done, or not?

Yes	No	Don't know/ Refused
6	93	1

(Asked of those who have had genetic testing; n = 107)

ST-04. What is the main reason you did this? Was is mainly because of...?

	%
A concern about health problems for your future children	35
A desire to learn more about your heritage or family history	25
A concern about future health problems for yourself	18
Some other reason	18
Don't know/Refused	4

(Asked of those who have had genetic testing; n = 107)

ST-05. Were the results of your genetic testing helpful to you, or not?

Yes, helpful	No, not helpful	Don't know/ Refused
81	10	9

(Asked of split sample; n = 480)

ST-06. If a genetic test was available that could tell you whether or not you were likely to develop Alzheimer's disease in the future, would you want to take the test, or not?

Yes, would want to take the test	No, would not want to take the test	Don't know/Refused
56	42	2

(Asked of split sample; n = 480)

ST-07. If a genetic test could tell you whether you or not you were likely to develop cancer in the future, would you want to take the test, or not?

Yes, would want to take the test	No, would not want to take the test	Don't know/Refused
56	42	2

(Asked of split sample; n = 480)

ST-08. In addition to genetic testing, scientists are developing experimental ways to change human genes to treat diseases, often called gene therapy. Gene therapy involves replacing a person's genes to treat specific diseases, with the potential to treat diseases such as HIV/AIDS, sickle cell anemia, and some types of cancers. Some people oppose gene therapy because it has a risk of health complications, or they believe it may lead to unethical use or it is unnatural.

Do you think the Food and Drug Administration should or should not approve gene therapy treatments for use in the United States?

Yes, they should	No, they should not	Don't know/Refused
59	30	11

(Asked of split sample; n = 480)

ST-09. Do you think the federal government should or should not fund scientific research on developing new gene therapy treatments?

Yes, they should	No, they should not	Don't know/Refused
64	30	6

GENETIC CHANGES TO UNBORN BABIES

(Asked of split sample; n = 520)

ST-10. Recently, there have been discussions among experts on the potential for changing the genetic characteristics of unborn babies to improve their health, physical traits, or intelligence.

How much have you heard or read about these discussions?

Quite a lot	Some	Not much	Nothing at all	Don't know/ Refused
8	23	27	42	*

As you may know, there is an ongoing controversy over these new scientific discoveries. Some scientists argue that parents should have the right to change the genes of their unborn babies to prevent certain serious diseases such as Huntington's disease, cystic fibrosis, or some types of muscular dystrophy. In addition, some also believe parents should be able to change their unborn babies' genes to select desired characteristics such as intelligence, or physical traits such as athletic ability or appearance.

On the other hand, some scientists argue that changing the genes of unborn babies is an unsafe experiment with uncertain consequences, these genetic changes will be passed on to future generations, they may cause irreversible harm, and they could lead to a society of genetic "haves" and "have nots."

(Asked of split sample; n = 520)

ST-11a. Do you think that changing the genes of unborn babies to reduce their risk of developing certain serious diseases should be legal or should be illegal?

Should be legal	Should be illegal	Don't know/ Refused
26	65	9

(Asked of split sample; n = 520)

ST-12. Do you think that changing the genes of unborn babies to improve their intelligence or physical characteristics should be legal or should be illegal?

Should be legal	Should be illegal	Refused/ Refused
11	83	6

(Asked of split sample; n = 520)

ST-13. For decisions on whether or not to allow changing the genes of unborn babies to improve their health, physical traits, or intelligence, do you think we should leave it up to scientists, physicians, and other technological experts, or do you think we should leave it up to government officials and policy makers?

Scientists, physicians, and other technological experts	Government officials and policy makers	Other/ Neither (vol)	Don't know/ Refused
53	9	31	7

(Asked of split sample; n = 520)

ST-14. Do you think the federal government should or should not fund scientific research on changing the genes of unborn babies to reduce their risk of developing certain serious diseases such as Huntington's disease, cystic fibrosis, or some types of muscular dystrophy?

Should	Should not	Don't know/ Refused
44	51	5

(Asked of split sample; n = 520)

ST-15. Do you think the federal government should or should not fund scientific research on changing the genes of unborn babies that aims to improve their characteristics such as intelligence or physical traits such as athletic ability or appearance?

Should	Should not	Don't know/ Refused
14	82	4

(Asked of total sample; n = 1000)

ST-16. Have you, or has anyone in your immediate family, ever been told that you carried a gene that predisposed you to certain serious diseases, such as Alzheimer's disease, cancer, heart disease or sickle cell anemia?

Yes, told	No, not told	Don't know/ Refused
19	80	1