

Patients and Patient Representatives – Preliminary Findings from a Review of Responses to the Common Rule NPRM

There were 245 responses in this category. Responses from those identifying as patients predominantly included individuals with Desmoid Tumors, Leiomyosarcoma and other rare disorders and their parents, spouses, children, siblings, friends, and caregivers. The responses focused solely on the issue of biospecimens.

Biospecimens (97% oppose, 2% support, 1% support with qualifiers)

We reviewed three major proposals specific to biospecimens including the proposal to change the definition of “human subject” to include non-identified biospecimens, to mandate broad consent for secondary research use of biospecimens and to restrict institutional review board (IRB) waiver of consent for secondary research use of biospecimens. Ninety-four percent (230 of 245) of responses included comments on at least one of the three proposed changes. Of these, 97% (224 of 230) opposed the proposed changes 2% (5 of 230) supported them and 1% (1 of 230) offered qualified support.

This group was particularly concerned about the implications for rare diseases given how few biospecimens there are presently and that their own samples might be rendered unavailable for future research due to broad consent requirements and proposed restrictions on waiver of consent. Those responding expressed concern that the proposed changes will reduce the number of biospecimens, could delay basic and translational research on rare diseases and would inhibit scientific breakthroughs.

Definition of “Human Subject” (90% oppose, 10% support)

Nine percent (21 of 245) of responses included comments on the proposal to expand the definition of “human subject” to include non-identified biospecimens. Among the responses, 90% (19 of 21) were opposed and 10% (2 of 21) supported the proposed change. Of those opposed to changing the definition, three (1%) suggested that if a change were made they would prefer Alternative A – expanding the definition to include whole genome sequencing.

“I sympathize with the family of Helen [Henrietta] Lacks, but this bill [regulation] is a knee-jerk reaction to that situation. It would create problems for people with rare diseases and their families. Please don't put the remote chance of invading the privacy of some patients ahead of the lives of others.”

“Please consider the human impact of your decision.”

“We need more research, not a new rule or law that hinders the already painfully slow progress being done on this orphan disease. What would you do if it was your child? Please work with us!”

“I am against this regulation in that it will make research into my ailment much more difficult. It only makes for good management to use these otherwise discarded tissues for the betterment of mankind. No one will be harmed by it. Where MANY PEOPLE WILL BE HARMED BY ANY RESTRICTIONS ON RESEARCH!”

“I was diagnosed in 1996 with a desmoid tumor. The doctors knew very little about the best way to treat the tumor. Two surgeries and radiation for 36 treatments. This is a rare tumor and we still have very little data on treatment.”

“We need this continued research. I would challenge any person who discovers this disease exists within themselves or a loved one to deny the continued research with any and all tumor tissue available. There is no good reason to deny access to all discarded tissue.”

Broad Consent (85% oppose, 9% support, 6% support with qualifiers)

Thirteen percent (33 of 245) of responses included comments specific to the proposal to require broad consent for future unspecified research use of biospecimens. Of these, 85% (28 of 33) opposed the proposed change, 9% (3 of 33) supported it and 6% (2 of 33) offered qualified support.

“If you don't have a rare disease then you don't understand the ramifications of no studies because of no samples because of a consent form. Please it's diseased dead tissue, nobody wants to keep, we as patients need research and testing done.”

“I am a uterine Leiomyosarcoma patient with a limited number of treatment options. For this reason, I want to express by objection to changes in the Common Rule (1991) re: Federal Policy of the Protection of Human Subjects which will require consent for all research on biospecimens even when the specimens are de-identified or anonymized. I believe changes to the rule will offer less flexibility for researchers needing biospecimens that are collected and archived at the time of a patient's surgery...often long before researchers are in need of the specimens. Changes would burden medical centers with the task of maintaining and following up on consents making many specimens ineligible for use in research. Those of us with rare medical conditions for which there has been limited research do not want extra layers of regulation that would impede research for future treatment options. I am asking for life saving policy not life ending policies.”

Most comments in support of the change were submitted following the publication of a New York Times opinion piece by Rebecca Skloot, author of the book *The Immortal Life of Henrietta Lacks*, which provided a link to the proposed regulations, and mention the article, Lacks or compensation.

“I recently was made aware of this proposed change after reading a New York Times article. In the article it made the statement, ‘...but so far most comments are coming from scientists, research institutions, bioethicists and industry groups who strongly oppose the new consent requirements. Many favor the status quo; others want changes, but disagree

with the ones proposed. So [‘some’] question whether people even care what happens to a vial of blood or bits of a tumor after they leave the doctor. .’. Indeed, as patients we do care! I find it highly offensive that any Doctor would make such an assumption on my behalf. This attitude reminds that years ago some Doctors did play God by not fully informing their patients about impending death or the seriousness of an illness. Are we still in that era again. I certainly hope not.”

“As a Cancer patient, I was asked if I would consent to having my tissue and blood samples submitted for research. I consented. I believe it to be crucial to have patients give informed consent if their tissue or blood are to be used. I also believe that if their cells end up being used in a profitable manner such as HeLa or the Moore cells, that families should be compensated.”

Waiver of Consent (100% oppose)

Eighty-four percent (205 of 245) of responses commented on the proposed restrictions to IRB waiver of consent. Of these, 100% (205 of 205) opposed the proposed restrictions. Among the responses, 132 explicitly oppose waiver and 73 are not explicit but their response is consistent with those of others from their patient cohort and advocacy group. Responses from patients, family and caregivers often utilized language that may have been included in a notice from their advocacy group but personalized them with their stories and often provided their name or signed them.

“As a healthcare provider and a person with a rare disease, I strongly believe that the waiver of informed consent by IRBs should continue to be permitted for archival tissues using the waiver criteria in the current Common Rule. Without this waiver, research for many diseases would be difficult, and may become impossible for rare diseases. I do not believe that this current waiver puts patients at risk.”

“The result of this new regulation will be a drastic decrease in the number of samples available for research. Many important studies that have been performed in the past would have been impossible with this new regulation in place. PLEASE DO NOT PASS THIS IN ITS CURRENT FORM. The waiver of informed consent by IRBs should continue to be permitted for archival tissues using the waiver criteria in the current Common Rule.”

“I am a patient with the rare disease of having desmoid tumors. It is a painful and aggressive disease with no effective treatment to slow, stop, or remove the tumors. Progress has been made in research that is in process now. But the disease has crippled me and others who are unfortunate enough to have it. I am eager to provide archival tissue samples of my own for research purposes if it could help someone else. The waiver of informed consent by IRBs should continue to be permitted for archival tissue using the waiver criteria in the current Common Rule.”

Additional Areas of Concern

Patients and caregivers did not respond to most of the other areas we queried, including mandated use of a single IRB, extending the Common Rule to all clinical trials, and proposed data security safeguards. One response offered qualified support for the proposal to post clinical trial consent forms to a federal website.