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Secretary's Advisory Committee on Human Research Protections

Comments regarding the Draft Guidance on Disclosing Reasonably Foreseeable Risks in Research Evaluating Standards of Care

My name is Sharon Terry, and I am President & CEO of Genetic Alliance. Our mission is to empower individuals, families and communities to transform health. We are a network of more than 10,000 health advocacy organizations, 1,200 of which are disease advocacy organizations. We were founded in 1986, and have always sought to recognize, promote, and empower the voice of the public in health. I only became involved in this advocacy because in 1994 my two children were diagnosed with a genetic condition that will lead to blindness and cardiovascular disease. I am basically a mom with a mission.

We appreciate the opportunity to provide public comment today at this meeting, and in the future, as relevant stakeholders engage in dialogue about the Draft Guidance on Disclosing Reasonably Foreseeable Risks in Research Evaluating Standards of Care. We will ask HHS for an extension to the comment period so that we can appropriately engage the communities this will impact.

There are a number of significant issues raised in, or as a result of, this guidance. First, as the guidance illustrates, it is critical that research that evaluates standards of care be encouraged and supported. A learning healthcare environment requires it. The continuum from research to services must become an integrated and functional system. Only then will we have a true healthcare 'system'.

Next, it is certainly true that, in order to optimize health status, individuals, their families and their clinicians must have access to the best information possible about the risks and benefits of diagnostic strategies and health interventions. These interventions are varied. Across the health services spectrum they will include behavioral changes, diets, procedures, tests, devices, drugs, and/or biological treatments. In some cases there are a plethora of 'standards' from which to choose, and in other cases, there is no alternative treatment at all. In most situations, more than one intervention is known to have a favorable balance of risk and benefit, but no one is certain whether one intervention is better than the other or whether they should be used together. Because most treatments produce an array of possible benefits and risks, the situation becomes even more complex for individual decision making because different people respond differently to interventions. We are already randomized by virtue of the clinician we chose, his or her observations, our

compliance, or the lack thereof, placebo effect, a variety of data points that have greater or lesser influence on an option, and occasionally our own preferences as patients.

There are major national efforts that are now poised to begin to understand when the balance shifts in favor of a proposed intervention. I am part of PCORnet, as the PI of a funded network within the network, also serve on its executive committee. In this position, I am keenly aware of the promise of evaluating standards of care. With about 1,000 other dedicated souls, we are ~~dedicated to~~ creating a system whereby this can be done with foresight and coordination. This is a moment in which we need a guidance that addresses the future of comparative effectiveness research in which patients, clinicians and researchers are all participants.

More Americans than ever before have an electronic health record and increasing numbers of registries are being kept by professional groups of clinicians and patients interested in particular diseases and medical conditions. Despite the large amount of data available, in many cases simply analyzing available data will not provide enough insight because most interventions have a modest effect on outcomes. When the effect is modest, observational analysis often gives the wrong answer because clinicians can only pick interventions based on assessment; and therefore randomization is needed to make sure the comparisons are evaluating people at the same risk when they start the intervention.

Those of us who long for the promise of participant-centric research to be realized believe that this draft guidance will discourage that science. In the practice of medicine, clinicians can recommend and prescribe with no oversight of their discussion of risks and benefits, but the researcher, who is not responsible for the clinical care, now has to be responsible for describing the risk of the underlying condition. The guidance states that when an intervention is assigned in a clinical trial the researcher will have to explain the risks of the underlying health issue and the disease and the risks of all interventions assigned in the trial. The current mechanisms in wide use for informed consent are already overly burdensome, and this will only make them more so.

The guidance does not address the world in which we live in today, with its many new options for engagement, let alone the future. New ways of engaging individuals are upon us already. For example, the cross-condition registry system Genetic Alliance has built as part of PCORnet uses novel participant engagement mechanisms that provide dynamic and granular data sharing, privacy and access controls. Local, trusted, guides describe the risks and benefits of participation. We who live with illness and disease know only too well that <u>we don't know</u>. We know that we need a health care system in which risks and benefits of interventions are discussed as part of routine clinical care and the researchers are accountable for discussing the extra risks caused by the research.

Following the SUPPORT trial, one mother said: "In the time we lived in the NICU, we learned to accept risk. Just to enter that place is to embrace terror and uncertainty. There may be risk to participating in a study, but there is also risk to not participating. I don't believe that ethicists and doctors at two dozen institutions conspired to hurt babies. If they decide to tweak the language in their paperwork, so be it. But second-guessing and finger wagging should not hamstring further studies. At the frontier of human possibility, no form can make medicine a safe or predictable endeavor." Every moment we are not learning deeply what works and what doesn't work, for

populations and for individuals, is a wasted moment. Now is not the time to further burden an arcane system. Learning in healthcare and biomedical research is already painfully slow compared to the rapid rate of learning by other systems around us. Let's work together to build a visionary system for the 21st century to match the promise of other systems around us. We promise to engage multiple stakeholders and to provide productive comments and recommendations to OHRP. We thank the committee for your start down this path today, and ask you to use your deliberative and convening capacity as you advise on this topic. Your job is often a tough one. It just got tougher – millions of Americans, and the researchers and clinicians who serve them, are depending on you, and us, to get this right.

Sincerely,

Sharon F. Terry
President & CEO