Thank you, Chairman Augustine. I am James Jorkasky, Executive Director of the National Alliance for Eye and Vision Research, or NAEVR, which serves as the privately funded “‘Friends of the National Eye Institute (NEI).’” NAEVR is a research advocacy organization that does not speak for NEI, but about its accomplishments. I appreciate the opportunity to listen to these initial discussions about a comprehensive NIH plan for clinical and translational research.

Although the TMAT’s discussions are in their infancy and will develop further during tomorrow’s planned discussions, I did want to inform you about clinical and translational research initiatives in the vision space. I provide these comments for three reasons: none of the panelists scheduled to appear represent the vision space; although the NEI is a relatively small Institute, it has conducted numerous translational collaborations that have smartly and effectively expanded its research dollars; and NEI’s translational research has resulted in drugs and devices—and combinations thereof—as well as diagnostics and gene therapy approaches, reflecting what Dr. Varmus just stated about the promise of translational research to offer a “rich repertoire of patient solutions.”

As the TMAT proceeds, I hope that it works with staff from all Institutes to become aware of the novel and effective translational collaborations. I wish to offer just a few examples of NEI collaborations within the NIH, across the Department of Health and Human Services (DHHS), with other government agencies, with private funding organizations, and internationally.

This past year the NEI celebrated its 40th anniversary. In June, it hosted a Translational Research and Vision Symposium on the NIH campus as the last of its educational celebratory events. At that meeting, Dr. Collins provided keynote remarks stating that, “The NEI has been central to advances in translational research. NEI’s vision has allowed us to see farther and better and has enabled the NIH to attain its best vision. Most importantly, the best is yet to come.” Within the meeting, several of NEI’s collaborations were described, including:

### Trans-NIH

- NEI’s collaboration with the National Cancer Institute (NCI) and the National Heart, Lung and Blood Institute (NHLBI) into factors that inhibit new blood vessel growth has resulted in the first generation of Food and Drug Administration (FDA)-approved ophthalmic drugs to treat the “wet” form of Age-related Macular Degeneration (AMD).

- NEI has worked closely with the National Institute of Diabetes and Digestive and Kidney Disorders (NIDDK) through its Diabetic Retinopathy Clinical Research (DRCR) Network to study the best treatment practices for diabetic retinopathy.
• Both of these collaborations have resulted in ongoing Comparative Effectiveness Research (CER), which is an NIH priority. NEI is currently conducting the Comparison of AMD Treatment Trials study on AMD drug therapies, while the DRCR Network recently released results of a study which confirmed that laser treatments for diabetic macular edema, when combined with injections of the FDA-approved drug Lucentis, are more effective than laser treatment alone.

Across DHHS

• On September 24, the NEI and FDA will jointly host the fourth of a series of symposia to consider endpoints appropriate for use in clinical trials that support approvals for new ophthalmic drugs and devices. This meeting will be the second regarding clinical endpoints for Glaucoma, and previous meetings have addressed endpoints for AMD and Diabetic Retinopathy, as well as the use of Patient Reported Outcomes. This collaboration is very much in the spirit of the new NIH/FDA Joint Leadership Council to incorporate the latest science into the regulatory review process.

With Other Government Agencies

• NEI and the National Aeronautics and Space Administration (NASA) have collaborated on a diagnostic device for cataracts, which is the clouding of the natural lens. The compact fiber optic probe uses dynamic light scattering to measure the amount of the anti-cataract protein alpha-crystalline—the less light scattering from the protein, the more likely the individual is to develop cataracts.

• The Department of Energy, along with the National Science Foundation and the NEI, are supporting research into the development of an Artificial Retina—in initial versions of which have enabled individuals completely blind to navigate their homes and community.

With Private Funding Organizations

• The collaboration between NEI and private funding organization Foundation Fighting Blindness (FFB) has resulted in successful gene therapy to treat the retinal degenerative disease Leber Congenital Amaurosis (LCA). In his April and May testimony before hearings of the House and Senate Labor, Health and Human Services, and Education (LHHS) Appropriations Subcommittees, respectively, Dr. Collins played a video entitled “Corey’s Story,” which featured a recipient of the gene therapy navigating a maze unsuccessfully prior to the procedure, then successfully after the procedure.

Internationally

• NEI recently established the International AMD Genetics Consortium to share information globally from Genome-Wide Association studies (GWAS) to determine the increased risk of developing AMD from gene variants. Once these pathways are understood, researchers can develop appropriate diagnostics and therapies. On September 23, my organization is sponsoring a Capitol Hill briefing acknowledging International AMD Awareness Week in which we will update Congressional staff on all of the basic, clinical, and translational research developments into AMD.

Thank you for this opportunity to comment so early in your discussions of this issue.