Written Comment

Generation Patient

Submitted to the U.S. House of Representatives
Committee on Energy and Commerce

March 17, 2022

“The Future of Medicine: Legislation to Encourage Innovation and Improve Oversight”

Generation Patient represents young adults with chronic and rare conditions across the United States. Generation Patient is one of the first and only organizations supporting this unique demographic transitioning into adulthood with lifelong conditions. Our organization is independent and we do not accept any funding from the pharmaceutical, insurance, hospital, or other industries. We appreciate the opportunity to submit a statement for the record and speak to crucial legislation impacting our young adult patient community.

Generation Patient was created and is currently led *entirely* by young adult patients. Our work has touched regions across the U.S. from South Texas to Indiana to California. Through our programs, the Health Policy Lab and the Crohn's and Colitis Young Adults Network, we work to increase the health literacy, patient activation, self-management, and advocacy skills of young adult patients. We have facilitated over 200 virtual meetings over the last couple of years in addition to a variety of programming in higher education, peer support, and health policy education.

As an organization, we are concerned about the lack of inclusion of adolescents and young adults with chronic and rare conditions in clinical trials. For all pieces of legislation discussed today, efforts to diversify clinical trials must explicitly include adolescents and young adult patients. Even with limited research, we know that there are substantial differences among pediatric, adolescent, and adult patient populations in drug disposition and response (Kapogiannis & Mattison, 2008). We appreciate the focus on equity within these bills but would encourage greater specificity for the inclusion of adolescents and young adults.

We are also recommending that any post-market reported data of adolescents and young adults be stratified to include narrower age ranges given this is a distinct developmental period. For example, the FDA Adverse Event Reporting System currently has 18-64 as one demographic, which does not allow for the understanding of how a medical product affects young adults versus adults. We recommend narrowing the range to 18-26. This ensures the inclusion of developmentally different ages, as an 18-year-old body is physically different from that of a 60-year-old. The FDA should develop enrollment target numbers that adequately represent the age demographic spread of the patient population for a particular condition. Further, the FDA should require more stringent enrollment criteria as we believe this will expedite the enrollment of underrepresented demographics rather than relying on the sponsors alone which may take longer.

As part of the Accelerated Approval Integrity Act (H.R. 6963), we are in concordance with post-approval progress report requirements, and without a doubt, these should be

made publicly available. The FDA should also ensure the prompt public release of postapproval information. Patients need and deserve to see this information. We appreciate the authority of the FDA to quickly withdraw an accelerated approval drug to prevent patients from being harmed longer than we should.

We are in full support of the development of a Rare Disease and Drug Advisory Committee under the Speeding Therapy Access Today Act (H.R. 1730**)**. The majority of us at Generation Patient live with a rare disease, so the value to us of patient voices as leading the conversation cannot be understated. We recommend council members maintain the integrity of this important advisory committee by disclosing any conflicts of interest and receiving under $5000 from the pharmaceutical industry. We would appreciate the explicit inclusion of independent patient leaders who will serve in the advisory capacity. We would also like to emphasize the need to include diverse ages as part of this committee. Most of the approximate 7,000-10,000 rare diseases disproportionately impact children, adolescents, and young adults (Tisdale et al., 2021). Thus, we believe that a young adult voice is critical to the advisory committee.

For young adult patients living with potentially lifelong conditions, the development of new therapeutic options is extremely important. Many of us are diagnosed with rare and complex conditions for which limited treatment options are available. Our life and quality of life depend on genuine innovation and the prompt development of alternative treatments. While we need new therapeutics, we also need to ensure the integrity of the clinical trial and post-market process. We hope that all discussions today prioritize patient safety.

Thank you for your time and to all parties for contributing perspectives. We applaud the work of the House Committee on Energy and Commerce in facilitating discussions that directly impact patients. We hope that our perspectives will be weighed heavily as the next generation of independent patient advocates.

Kapogiannis, B. G., & Mattison, D. R. (2008). Adolescents in clinical trials. *Clin Pharmacol Ther*, *84*(6), 655-659. https://doi.org/10.1038/clpt.2008.191

Tisdale, A., Cutillo, C. M., Nathan, R., Russo, P., Laraway, B., Haendel, M., Nowak, D., Hasche, C., Chan, C.-H., & Griese, E. (2021). The IDeaS initiative: pilot study to assess the impact of rare diseases on patients and healthcare systems. *Orphanet journal of rare diseases*, *16*(1), 1-18.