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October 31st, 2024

Robert M. Califf, M.D, Commissioner
Food and Drugs Administration
Department of Health and Human Services
Attn: FDA-2024-N-3528
Rockville, MD 20852

RE: GHLF Comments on Creation of Rare Disease Innovation Hub (FDA-2024-N-3528)

Dear Commissioner Califf,

Thank you for the opportunity to comment on the proposed creation of a Rare Disease Innovation Hub and it's possible priorities. The Global Healthy Living Foundation supports the access and growth of the rare disease treatment market and we are encouraged that FDA is committed to aiding those patient communities.

By way of background, the Global Healthy Living Foundation (GHLF) is a 501(c)(3) patient group that works to improve the quality of life for people with chronic disease, often focusing on those least able to advocate for themselves. Through our websites, social media channels, and conventional media, GHLF reaches more than 10 million chronically ill people monthly in the United States – in English and Spanish. Rare disease patients within our community face many different issues around the country, including but not limited to: limited research into both the disease and possible treatments, diagnostic delays that can lead to significant disease progressions, lack of specialized expertise which can increase already long delays in diagnosis and treatment, and a general lack of coverage and treatment access further increasing the burden on patients.

Rare diseases, due to their limited impact on a small number of people, are often not given high priority for research and development efforts. The lack of funding and interest in studying these diseases can hinder the development of new treatments and diagnostic tools, leaving patients with few options. Pharmaceutical companies may be less willing to invest in research and development due to the small potential market for any new treatments. As a result, there is a significant gap in medical knowledge and therapeutic options for individuals with rare diseases. This lack of research can lead to a sense of neglect and hopelessness among patients and their families. Finding ways to increase funding and research into rare diseases and possible treatments should be a main priority for the Rare Disease Innovation Hub.

Patients with rare diseases often face long and emotionally challenging journeys to obtain a correct diagnosis. Identifying these conditions can be difficult, leading to misdiagnosis, delayed treatment, and significant emotional distress. The symptoms of rare diseases can be nonspecific

or similar to those of more common conditions, making it difficult for healthcare providers to accurately recognize and diagnose them. Patients may undergo numerous tests and consultations before receiving a correct diagnosis, leading to unnecessary suffering, anxiety, and treatment delays. This process can take a toll on the mental and physical well-being of patients and their families. Additionally, due to the rarity of these conditions, many healthcare providers may lack the necessary expertise to diagnose and treat rare diseases. This can result in inadequate care, poor patient outcomes, and further delays in diagnosis and treatment. Addressing the lack of specialized expertise should be another priority for the Innovation Hub.

Obtaining insurance coverage for rare disease treatments can be challenging due to the high costs and the lack of established evidence. This can lead to financial hardship for patients and their families. The high cost of treatments for rare diseases, combined with the lack of established evidence supporting their effectiveness, can make it challenging for insurance companies to justify coverage. As a result, patients may face significant financial burdens in order to access the treatments they need, adding to the emotional and physical strain caused by the diseases themselves. Patients often face significant challenges in accessing specialized care, including the need to travel long distances or seek treatment outside of their state. Specialized care may only be available at a limited number of medical centers due to the rarity of these conditions, requiring patients to travel long distances or relocate to access necessary treatment. This imposes significant burdens on them and their families, affecting their overall well-being and quality of life. Additionally, the lack of specialized care providers in certain regions can limit the availability of treatment options for patients with rare diseases, further complicating their healthcare journey. We hope this also becomes a priority focus for the Innovation Hub as it moves forward.

As always, we believe that the best way to accomplish any of these goals is to work with patient and caregiver groups to aid in increasing patient participation at these meetings and raise the profile of the patient voice at the stakeholder table. Partnering with the people and organizations that already have a foothold in those patient communities will ensure that best practices are utilized, and patients are not left behind.

Thank you again for the opportunity to provide input on this topic and we look forward to a strong working relationship with the Innovation Hub.

Sincerely,

Steven Newmark, JD
Director of Policy
Global Healthy Living Foundation