GLI GLOBAL LIVER INSTITUTE

Impacts of Patient-Focused Drug Development Meetings Across Liver Diseases

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On behalf of Global Liver Institute (GLI), a nonprofit dedicated to improving the lives of individuals affected by liver disease, we appreciate the opportunity to provide feedback on the impact of Patient-Focused Drug Development (PFDD).

GLI has conducted two externally-led PFDD (EL-PFDD) meetings in this space, one on primary biliary cholangitis (PBC) on February 4, 2022 and one on NASH/MASH, now broadly recognized under the umbrella of steatotic liver disease (SLD) on November 4, 2021, with corresponding Voices of the Patient reports. These meetings have substantially shaped how the patient experience is understood and how sponsors prioritize therapeutic development.

In liver disease, Patient-Focused Drug Development (PFDD) has been effective in integrating patient experience into drug development and regulatory science. Its contribution has been improving the characterization and measurement of outcomes that matter most to patients, ensuring that clinical development reflects the real-world burden of disease in addition to traditional clinical and laboratory endpoints.

Primary biliary cholangitis (PBC) is a clear example. Following the approval of ursodeoxycholic acid (UDCA) in 1994, there were limited therapeutic advancements for more than two decades. During GLI’s EL-PFDD engagement efforts, patients consistently identified pruritus and fatigue as among the most burdensome and life-limiting aspects of PBC. These symptoms significantly affect sleep, daily functioning, and quality of life, yet were historically underemphasized in clinical development compared to biochemical markers. PFDD helped elevate these symptoms as critical components of the disease experience and reinforced their importance as meaningful and measurable outcomes in clinical research.

The impact of this shift is evident in subsequent development programs. In 2024, FDA granted accelerated approval to both elafibranor (Iqirvo®) and seladelpar (Livdelzi®), marking an important advancement in the PBC treatment landscape. Both programs incorporated patient-relevant symptom measures, including pruritus, alongside traditional biochemical endpoints. In 2026, FDA approved linerixibat (Lynavoy®), the first therapy in the United States specifically indicated for cholestatic pruritus in adults with PBC. This represents a notable example of how patient-reported experience can help elevate a symptom from a secondary consideration to a defined therapeutic target. Continued activity in the PBC pipeline, including the priority review of saroglitazar, further reflects a development landscape in which patient-prioritized outcomes are defined and consistently incorporated into clinical evaluation.

The impact of patient-focused engagement is also evident in steatotic liver disease (SLD). Historically, research and clinical assessment focused largely on liver enzymes, fibrosis staging, and histologic findings, while the patient experience remained less well characterized.

Through PFDD and related patient-engagement efforts, patients highlighted burdens extending beyond traditional clinical measures, including fatigue, stigma, uncertainty surrounding diagnosis, cognitive challenges, emotional distress, and the day-to-day impact of living with a chronic disease that was frequently misunderstood by both the public and healthcare professionals.

Importantly, patients also emphasized persistent gaps in early detection and disease education. Many described years of living with progressive disease before diagnosis, often despite the presence of multiple risk factors, and expressed frustration with inconsistent screening practices and limited awareness among healthcare professionals. Participants further highlighted challenges associated with existing diagnostic approaches, including the burden of liver biopsy, and the lack of effective treatment options beyond lifestyle interventions that often produced variable results. These insights helped address important gaps in understanding disease burden and reinforced the need to incorporate patient-reported outcomes into both research and clinical care.

Patient perspectives additionally informed broader discussions regarding disease stigma and communication. Concerns raised by patients about how the terminology "nonalcoholic fatty liver disease" influenced perceptions of disease seriousness, causation, and blame became an important consideration in ongoing efforts to improve disease awareness and understanding. While the transition from NAFLD/NASH to MASLD/MASH resulted from a broad international, multi-stakeholder consensus process, it reflects a core principle of PFDD: that the lived experience of patients should help inform how diseases are described, studied, and addressed.

These evolutions demonstrate that patient-focused engagement can contribute not only to drug development, but also to a more complete understanding of disease burden and the barriers patients face. The progress observed across liver disease demonstrates the value of PFDD. Symptoms that were once overlooked are now routinely measured and patient-reported outcomes are increasingly incorporated into development programs.

About Global Liver Institute

Global Liver Institute (GLI) is a 501(c)3 nonprofit organization founded in the belief that liver health must take its place on the global public health agenda commensurate with the prevalence and impact of liver illness. GLI promotes innovation, encourages collaboration, and supports the scaling of optimal approaches to help eradicate liver diseases. Operating globally, GLI is committed to solving the problems that matter to liver patients and equipping advocates to improve the lives of individuals and families impacted by liver disease. GLI holds Platinum Transparency with Candid/GuideStar, is a member of the National Health Council and NORD, and serves as a Healthy People 2030 Champion. Follow GLI on Facebook, Instagram, LinkedIn, and YouTube or visit www.globalliver.org.