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**FORUM**  
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## **Announcements and Upcoming Events from the Forum and Partners**

**June 19th, 2017**

### **The Forum for Collaborative Research Launches New Project Focused on Developing Treatment for the Rare Disease PSC**

Washington D.C., June 19, 2017- The [Forum for Collaborative Research](#) is launching the [PSC Forum](#) to advance the regulatory science for the development of therapeutics for PSC and its associated morbidities.

Primary Sclerosing Cholangitis (PSC) is an immune-mediated disease of the liver, caused by chronic inflammation and fibrosis of the bile ducts inside and outside the liver, leading to abdominal discomfort, fatigue, severe itching, weight loss, and in later stages, cirrhosis, and liver failure, as well as bile duct, colon and liver cancers. Itching (known as pruritus) can be so extreme that it leads to excoriation and frequently requires a (or multiple) liver transplant(s) to help patients cope with this unbearable condition. PSC is a rare disease, affecting approximately 32,000 people in the United States. Liver transplant free survival ranges from an average of 13-21 years and PSC is the fifth most frequent indication for liver transplantation in the United States. Despite the severity of the disease, there are no effective medical treatments for halting the progression of PSC, and liver transplant is the only therapeutic modality available.

The PSC Forum meets the needs of patients with this disease by promoting the efficient and safe development of new drug candidates and therapeutic strategies. “We are excited to offer The Forum for Collaborative Research’s unique model of cross-stakeholder collaboration in a non-competitive, neutral and safe space as a

mechanism to increase clarity and reduce uncertainty in the regulatory path for new treatments for this greatly unmet medical need,” stated Dr. Veronica Miller, Executive Director of the Forum for Collaborative Research. “Dialogue and deliberation among patients, regulators and clinical researchers from both sides of the Atlantic will ensure that we maximize the efforts of all the organizations already involved around one common mission.”

A Steering Committee guides the work of the PSC Forum, with the goal of synergizing work across the community and identifying gaps and barriers in current research, allowing consensus regarding regulatory issues to evolve and facilitating collaborative research where needed. Keith Lindor, past-president of the American Association for the Study of Liver Diseases (AASLD) and Dean, College of Health Solutions in Arizona and the academic co-chair of the Steering Committee states “The field has been struggling too long on the question of how to successfully address the urgent need for treatment for this dire medical condition, with a complex clinical presentation, heterogeneity among patients, lack of adequate diagnostic tools and no treatment whatsoever. Bringing everyone together around the same table with a joint mission of working out a solution with partners in the U.S. and across the Atlantic is the only sensible way to go”. Patient advocates are represented on the Steering Committee by PSC Partners Seeking a Cure and PSC Support. Ricky Safer, the CEO of PSC Partners Seeking a Cure, explains, “As family members and patients who have lost too many in our community to this disease, we are thrilled to be involved and help guide the efforts of the PSC Forum through our active engagement, representing the priorities of the patients and their families. We appreciate the safe space the PSC Forum provides for us to bring our perspectives and priorities to the regulatory agencies (FDA and EMA), the pharmaceutical and diagnostic developers, and the academic researchers, many of whom have been taking care of us over many years”.

The impetus for this program was the *Trial Design and Endpoints for Clinical Trials in Adults and Children with PSC* meeting sponsored by the U.S. Food and Drug Administration (FDA) and AASLD. While this meeting highlighted the complexities of establishing a treatment for PSC, ongoing work is needed to further facilitate advancement in the field. The PSC Forum was developed to build on this foundation and will continue to coordinate the collaborative efforts between many parties moving forwards.

The PSC Forum will hold its first in-person meeting in October 2017. The agenda will be set by the Steering Committee and will be responsive to the needs of the PSC community.

### **About the Forum for Collaborative Research**

The Forum for Collaborative Research was founded in 1997, as a public/private partnership and operates under the auspices of the University of California, Berkeley. The Forum's mission is to facilitate and enhance research by addressing cutting edge science and policy issues through a process of stakeholder engagement and deliberation. Its impetus was a request from Vice President Al Gore to convene all stakeholder groups to promote dialogue and collaboration with a focus on improving treatment for HIV, specifically in relation to antiretrovirals. The Forum's goal is to address regulatory hurdles in order to advance safe and efficient drug development. The success of this model has changed the way clinical trials are conducted, established broader and fairer access to trials and expanded access programs, and accelerated the delivery of new drugs. While the Forum began working in HIV, the organization has expanded to include projects in the areas of NASH, HBV, HCV, CMV, and most recently PSC.

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