



PSC Partners Statement about the Use of Medications Not Approved by the FDA for PSC

At this time, there are no FDA-approved medications for the treatment and cure of primary sclerosing cholangitis (PSC). PSC Partners Seeking a Cure recognizes the urgent need for treatments to stop or slow the progression of PSC. We support the development of clinical trials testing the safety and efficacy of new drugs for PSC, including the investigation of drugs currently given off-label, as well as new drugs in the pipeline.

In a rare and progressive disease such as PSC with a potential risk of liver failure, patients are more prepared to take risks based on anecdotal evidence. Opinions and anecdotal experiences have many important roles in drug development, including generating hypotheses for future research and identifying meaningful biomarkers. In the absence of clinical trials, the potential side effects and other risks associated with these drugs are not captured and may be overlooked. While some positive reports may suggest efficacy, this may not be the case for an individual PSC patient, and the potential side effects and other risks are a very real concern.

PSC Partners is working with researchers, regulators, and pharmaceutical/biotech companies to facilitate and accelerate research leading to a cure. PSC Partners and PSC Partners Canada have dedicated more than \$4 million to research and will continue to support important research until treatments and a cure are found.

To learn more about studies and clinical trials, PSC patients can join the PSC Partners Patient Registry, which maintains a list of updated clinical trials. Your participation in the Registry will inform researchers about your most important concerns and support the translation of anecdotal evidence into scientifically approved treatments. Through participation in the Registry, PSC patients clearly show researchers their urgency and willingness to be part of the solution. PSC Partners encourages all PSC patients and the parents/guardians of PSC patients to join the 1,900 participants in the Registry at PSCPartnersRegistry.org or by writing to registrycoordinator@pscpartners.org.

PSC Partners encourages everyone to research and discuss all potential medications with their medical providers. Every patient's situation is unique given their health status and tolerance of unknown risks. While there are currently no FDA-approved treatments for PSC, some PSC patients choose to take prescribed off-label medications. Others may not feel comfortable accepting potential unknown risks. All PSC patients have the right to request a consultation or an additional opinion from another doctor.

PSC Partners supports scientific research to discover treatments and a cure for PSC, while prioritizing the safety and well-being of all PSC patients. Together, we are creating a world where a PSC diagnosis comes with a cure.

No written material from PSC Partners should ever be used as a substitute for direct medical advice from your doctor or other qualified healthcare provider. PSC Partners is not recommending or endorsing any opinions, products, medications, or treatments, even though they may be mentioned on the website or on social media.

At this time, some off-label medications prescribed to PSC patients include: ursodeoxycholic acid (ursodiol, UDCA, Urso), vancomycin (Vancocin), and rifaximin (Xifaxan).

This information is up to date as of July 1, 2021.

