

Board of Directors

John J. Seng

Chair
Spectrum Communications
Washington, D.C.

Susan Alpert, PhD, MD

Immediate Past Chair
Minneapolis, MN

~~~

**Gail L. Daubert, JD**

Reed Smith  
Washington, DC

**Roberta Gartside, MD**

New Image Plastic Surgery Associates, PLC  
Reston, VA

**Nieca Goldberg, MD**

Joan H. Tisch NYU Langone Center for  
Women's Health  
New York, NY

**Carol Kelly, MPA**

National Association of Chain Drug Stores  
Arlington, VA

**Bruce McEwen, Ph.D.**

The Rockefeller University  
New York, NY

**Donnica L. Moore, MD**

DrDonnica.com  
Sapphire Women's Health Group LLC  
Chester, NJ

**David C. Page, MD**

Massachusetts Institute of Technology  
Cambridge, MA

**Stephanie Pincus, MD, MBA**

Golden Seeds  
New York, NY

**Judith K. Wolf, MD, MS**

Vermillion, Inc.  
Austin, TX

~~~

Florence P. Haseltine, PhD, MD

Emerita Scientist, National Institutes of
Health
Founder
Society for Women's Health Research
Alexandria, VA

June 9, 2016

Steven Pearson, MD

President

Institute for Clinical and Economic Review

2 Liberty Square, Ninth Floor

Boston, MA 02109

Dear Dr. Pearson,

On behalf of the Society for Women's Health Research (SWHR®), we appreciate the opportunity to comment on the Institute for Clinical and Economic Review's (ICER) draft evidence report titled "Obeticholic Acid for the Treatment of Primary Biliary Cholangitis: Comparative Clinical Effectiveness and Value," in preparation for the meeting of the Comparative Effectiveness Public Advisory Council (CEPAC) in July.

SWHR, a national non-profit organization, located in Washington D.C., is widely recognized as the thought-leader in promoting research on biological differences in disease. For more than 25 years, our organization has brought attention to the variety of diseases and conditions that disproportionately or predominately impact women and is dedicated to transforming women's health through science, advocacy, and education.

Primary Biliary Cholangitis (PBC) is a chronic disease of the liver that slowly destroys the organ's medium-sized bile ducts. Research suggests that it is a rare autoimmune disease which appears to have a genetic component, but the underlying cause of the disease is currently unknown.

Additionally, PBC disproportionately affects women in the prime of their lives. Approximately 90 percent of PBC patients are women and the disease usually presents between the ages of 30-65; however, patients as young as 22 have been diagnosed. It is our hope that as the field of personalized medicine and genetics advances, and more attention and research is directed

at this disease, the underlying genetic components and causes can be identified, leading to improved diagnosis and treatments for patients.

Since the U.S. Food and Drug Administration (FDA) recently granted approval for Ocaliva (obeticholic acid) for the treatment of PBC in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA, or as a single therapy in adults unable to tolerate UDCA, SWHR believes that ICER should consider this approval as they are evaluating the comparative effectiveness and value of this drug because of the significant benefit this therapy could have for women who suffer from this disease.

PBC is difficult to diagnose early, as patients are often asymptomatic in the initial stages of the disease. As the disease progresses, the most common symptoms are fatigue, itching, and dry mouth and eyes. Some individuals may experience jaundice, a condition that causes the skin and whites of the eyes to turn yellow. PBC is diagnosed in approximately 60 percent of people before symptoms begin and often ultrasounds and liver biopsies are required to aid in diagnosis. The hallmark trait of PBC is the presence of antimitochondrial antibodies (AMAs) in the blood. These AMAs can be found in 90-95 percent of PBC patients.

UDCA has been shown to slow the progression of the disease, and is presently the only treatment option available for PBC patients. In fact, there have not been any novel treatments for this disease in over three decades. Many patients experience resistance or intolerance to UDCA, leaving them no treatment option for managing this chronic condition, which may lead to life-threatening complications or require a transplant. Innovative therapies are desperately needed to improve the health and quality of life for PBC patients.

SWHR strongly believes that with FDA's recent approval of Ocaliva (obeticholic acid), ICER should evaluate the benefits that this therapy could have for PBC patients – especially women at the CEPAC meeting in July.

Thank you for the opportunity to provide comments on this draft evidence report. If you have any questions or would like to discuss this issue further, please contact me at heather@swhr.org or (202) 496-5003.

Sincerely,



Heather Boyd, MPP
Director of Public Policy
Society for Women's Health Research (SWHR)