

PRESS STATEMENT: October 2021

The BPA is delighted by the recent announcement that NICE (the National Institute for Care and Excellence) has recommended the use of Givosiran (GIVLAARI®) on the NHS in England for the treatment of recurrent attacks of acute porphyria.

The acute porphyrias are a group of complicated, debilitating and potentially life-threatening genetic disorders that have a severe impact on the quality of life of those affected. The burden of illness is far reaching and covers all areas of a patient's life, as well as that of family, friends and carers.

The positive recommendation offers a new treatment option for patients experiencing severe, recurrent attacks of porphyria (4 or more attacks in a 12-month period). That this small group of patients may be able to benefit from the immense benefits of this life-changing new treatment is a welcome step change in acute porphyria care.

For decades, people with this life-threatening condition have had limited options to control the relentless and debilitating pain, nausea, and fatigue that dominate virtually all aspects of their lives. The decision today from NICE is therefore a very welcome and important one. It will mark real change for patients and families in future, allowing more people to live without the constant fear of when the next agonising attack may strike.

John Chamberlayne (Chair of Trustees)

For further information on the porphyrias, please do not hesitate to visit our website: www.porphyria.org.uk.

Patient quote: Salman (AIP) age 28

Givosiran gave me faith that miracles really do happen and that there is hope to live a somewhat normal life, and everyone deserves that chance!

Patient quote: Liz (AIP) age 44

Most of my twenties disappeared in a blurry haze of hospital admissions and all that comes with them – pain, sickness, paralysis, breathing assistance, humiliation of being unable to care for personal needs or feed self, sickness, pain, PAIN, PAIN! And repeat!

AIP robbed me of so much: to take part in normal life, the chance to work and develop a career, to walk, run and exercise, to travel. Finally, in my mid-40s, Givosiran has given me the chance to start experiencing life as it should be.

Patient quote: Sue (AIP) age 41

I suffered a progressive deterioration in health with each attack: increased weakness, more pain, more fatigue, disturbed sleep, more haem, more sickness, more use of the wheelchair, escalating levels of medications, anxiety around earnings, lost independence, lost job and company car, downsized home as couldn't afford on one wage, complete dependence on partner for physical care, such as bathing, cooking and cleaning, as well as complete financial dependence on my partner and the benefit system. One of the hardest things was also having to admit that I couldn't do things that I was previously able to do.

Givosiran has been completely life-changing. I am able to contribute to family life, to my life, to think of the future. I feel able to take on responsibilities as a mother, a wife, and an employee – as everyone should be able to! I don't have to continuously plan. I'm able to live a life that I did before and for the first time in a long time ... I'm excited for what my future could be again.