



## PRESS RELEASE

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# Last Patient Last Visit for landmark Tourette Syndrome study

(Stockholm, 2 February 2023.) **The Last Patient's Last Visit took place on 1 February less than 12 months after the study's initiation in Asarina Pharma's Phase IIa clinical study in Sepranolone for the treatment of Tourette Syndrome. All patient examination and treatment in the study has now been completed and the patient dropout rate has remained unexpectedly low. Topline results are expected on schedule at the end of March 2023.**

A total of 28 patients took part in the study: a 'control group' of 9 patients who received their standard treatment, plus an 'active group' of 17 who received their standard treatment plus 10 mg injections of Sepranolone twice weekly for 12 weeks. Only two patients dropped out of the study – one from the active group and one from the control group. Patients ranged from 12 to 45 years old. Adult patients were treated at the Bispebjerg University Hospital in Copenhagen, teenage patients at the Danish National Center for Tourette at Herlev University Hospital in Copenhagen.

The active group was significantly larger than required in the Study protocol. The protocol is designed to detect a 25% difference in the Yale Global Tic Severity Scale (YGTSS), the study's clinical endpoint, based on an active group of 10 patients. The YGTSS is the standard clinical rating instrument used in Tourette and other tic disorder studies. It measures and evaluates the number, frequency, intensity, complexity, and interference of motor and phonic symptoms.

Peter Nordkild, CEO: "With an unusually low dropout rate and a large active group we hope to be able to deliver clear and detailed findings of Sepranolone's potential impact on Tourette. We look forward to releasing the study findings at the end of March 2023. I'd like to thank the fantastic teams at Bispebjerg and Herlev, and our volunteer patients and carers for their contribution and engagement."

### TREATING TOURETTE: A SIGNIFICANT UNMET NEED

The unmet need for a safe, effective pharmaceutical treatment of Tourette is well-established. A 2018 survey by the Tourette Association of America (1) found that 29% of children with Tourette had tried five or more different medications, and 44% of parents felt that current Tourette treatments fail to adequately control their child's symptoms. Current treatments like haloperidol (Haldol) can involve extremely severe side effects.

The compound being trialled in the study, Sepranolone (*isallopregnanolone*), has been found to reduce tics without inducing any off target motor side effects in several preclinical studies (2) and has demonstrated a strong safety profile in multiple clinical studies. Over 300 women have taken Sepranolone in previous trials with no other side effects than mild and reversible local skin irritation.

### A NEW TREATMENT MODALITY FOR COMPULSION-RELATED CONDITIONS

Sepranolone is one of the first *neuroendocrinological* compounds to be tested for the treatment of Tourette. It is an endogenous neurosteroid produced by the body specifically to target and modulate the effects of Allopregnanolone - a powerful neurosteroid implicated in the exacerbation of tics, and a range of other stress- and compulsion-related indications. 86% of Tourette patients have at least one additional disorder co-morbid with Tourette, with OCD being particularly prevalent. In March 2021, a US patent was granted for the use of Sepranolone for the treatment of OCD.

(1)  
2018, the Tourette Association of America, Impact Survey

(2)  
-*Preliminary study of finasteride in Tourette syndrome*, Moroni, Bortolato et al, 2011  
-*Behavioral fragmentation in the D1CT-7 mouse model of Tourette's syndrome*, Bortolato et al 2018  
-*The other side of the coin: the neuropsychiatric side effects of allopregnanolone*, presentation, Prof Marco Bortolato, Allopregnanolone and its synthetic analogues: from bench to clinical strategies for neuropathology Conference, Torino, Italy, February 11 2021

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**ABOUT ASARINA PHARMA AB**

We are a Swedish biotech company developing Sepranolone for allopregnanolone-induced stress- and compulsivity-driven disorders. Our product pipeline is built on over 40 years of research into allopregnanolone-related neurological disorders. With our new family of GAMSAs (GABA-A Modulating Steroid Antagonists) we aim to deliver a new generation of safe, efficacious drugs for neurological conditions from Tourette syndrome to Obsessive Compulsive Disorder that still lack safe, efficacious pharmaceutical treatments.