



PRESS RELEASE

## Servier and Neurochlore launch phase 3 studies in Europe to evaluate bumetanide in children suffering from Autism Spectrum Disorders

**Paris, 23 January 2019 – Servier, an independent, international pharmaceutical company, and Neurochlore, a French biotechnology, announce the launch in Europe of phase 3 studies to evaluate the use of bumetanide in children suffering from Autism Spectrum Disorders (ASD).**

The pediatric investigation plan, which involves the use of an oral liquid formulation for children, has been approved by the Pediatric Committee of the European Medicines Agency. Two multicenter, randomized, double-blind, placebo-controlled clinical trials conducted by Servier are underway and will evaluate the efficacy and safety of bumetanide administered twice a day to children suffering from ASD aged 2 to 6 years and 7 to 17 years.

The main aim of these trials is to evaluate the efficacy and safety of bumetanide, compared with placebo, on the core symptoms (persistent impairment in reciprocal social communication and interaction and restricted and repetitive patterns of behavior) of ASD after a 6-month treatment. Overall the total duration of each of these two trials will be one year. Following the 6-month evaluation period versus placebo all the patients will be treated with bumetanide for a period of 6 months. Each patient will thus have the opportunity to be on active treatment for at least 6 months.

Servier has obtained the required authorizations to conduct these clinical trials in some European countries (France, Spain, Hungary, Germany, Italy, Poland and United Kingdom). Following additional regulatory approval, other European countries and countries outside Europe should be included as well in these trials. The objective is to recruit approximately 200 patients in each study.

For each trial, the design, the outcome measures, the eligibility criteria and the contacts and locations of the clinical centers are available at <https://clinicaltrials.gov/ct2/show/NCT03715153> (study of 2- to 6-year-olds) and <https://clinicaltrials.gov/ct2/show/NCT03715166> (study of 7- to 17-year-olds).

Lode Dewulf, Chief Patient Officer at Servier, declared: *“Autism spectrum disorders affect about one in every hundred children and are a great source of suffering for the patients and their families. Medication may not be the ideal solution for many of those affected, but it is much needed by other sufferers, who eagerly await drug treatment innovations that will improve their everyday lives. This is why Servier is very keen to help develop a pediatric drug for inclusion in effective multidisciplinary treatment, thereby bringing hope to patients and their families.”*

Bumetanide reduces the high concentrations of chloride observed in neurons in certain neurodevelopmental disorders like ASD. In 2017, Neurochlore published results supporting efficacy and safety from its phase 2b study with bumetanide in 6 centers in France in almost 90 children (aged



2-18 years). Bumetanide's favorable effects on several aspects of ASD allow to envision management of autism, particularly of its core symptoms.

Professor Ben-Ari, President of Neurochlore, commented: *"We are pleased with the launch of these phase 3 studies which are in the continuum of our ongoing research into autism, for which there is still no drug treatment."*

These clinical studies are in the frame of [a partnership agreement signed in 2017](#) in which Servier will develop and commercialize bumetanide in Europe while Neurochlore will retain the rights for the rest of the world.

#### **About Servier**

Servier is an international pharmaceutical company governed by a non-profit foundation, with its headquarters in France (Suresnes). With a strong international presence in 149 countries and a turnover of 4.152 billion euros in 2017, Servier employs 21 700 people worldwide. Entirely independent, the Group reinvests 25% of its turnover (excluding generics) in research and development and uses all its profits for development. Corporate growth is driven by Servier's constant search for innovation in five areas of excellence: cardiovascular, immune-inflammatory and neurodegenerative diseases, cancer and diabetes, as well as by its activities in high-quality generic drugs. Servier also offers eHealth solutions beyond drug development.

More information: [www.servier.com](http://www.servier.com)

Follow us on Social Media:   

#### **Press contacts:**

Sonia Marques : [media@servier.com](mailto:media@servier.com) – Tel. +33 (0)1 55 72 40 21 / + 33 (0) 7 84 28 76 13

Karine Bousseau : [media@servier.com](mailto:media@servier.com) – Tel. +33 (0)1 55 72 60 37 / + 33 (0) 6 49 92 16 05

#### **About Neurochlore**

Created 8 years ago by Yehezkel Ben-Ari, Neurochlore is a biotechnology company. Its goal is to study molecular and cellular changes in animal models of neurological and psychiatric diseases in relation to the ionic exchanges of chloride. It is located within the Luminy Science Park in Marseille. A founder of several biotechnology companies, Prof. Ben-Ari has received numerous awards for his contributions on cerebral development, including the Grand Prix of INSERM Research and the Belgian FNRS, as well as the American foundations and European epilepsy. Neurochlore conducted a Phase 2 study with bumetanide in children suffering from ASD. The company has about 20 employees (researchers and technicians). Neurochlore invests its funding in basic research, especially on intra-uterine alterations related to autism during work and birth.

More information: [www.neurochlore.fr](http://www.neurochlore.fr) <https://leblogdebenari.com/>

#### **Contact:**

Elise Gauthier : [gauthier@neurochlore.fr](mailto:gauthier@neurochlore.fr) - Tel +33 (0)6 88 42 86 93