

French drugmakers join forces on drug with potential to treat autism in children

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Servier, France's largest independent pharma company, has entered into a partnership to accelerate the development of a medicine that could become the first treatment for the core symptoms of autism.

It has signed an exclusive agreement with Marseille-based Neurochlore to develop and market bumetanide in pediatric autism in Europe.

The terms of the agreement state that Servier will develop and market the product in Europe and Neurochlore will retain these rights for the USA. Rights in relation to other countries are still under negotiation. The development plan includes three Phase III trials with an oral liquid form designed for children. Filing for marketing authorization is envisaged for the end of 2021.

Neurochlore president, acting chief executive and co-founder Professor Yehezkel Ben-Ari has been studying bumetanide's mechanism of action for a number of years. In 2014, his research, published in *Science* magazine, demonstrated in animal models that it was possible to prevent autistic behavior in the offspring of females treated with bumetanide prior to giving birth. Bumetanide acts on the high chloride levels in neurons, which are observed in certain neurodevelopmental disorders such as autism, thus paving the way for research in humans.

A Phase IIa clinical trial was first performed by Dr E Lemonnier, then a Phase IIb multicentric trial was sponsored by Neurochlore in six centers in France including almost 90 children (from 2 to 18 years old). This study has recently produced promising results, published today, in favor of bumetanide based on several autism evaluation scales. An overall management of the disorder and particularly of its core symptoms of social deficit and repetitive behavior can thus be considered.

Prolonged use of bumetanide in children can be envisaged as it has many years of well-documented safety data among the adult population through its use to treat heart failure, as well as renal and hepatic edema.

Now off patent, bumetanide is the active ingredient of Roche's Bumex, a treatment for edema associated with congestive heart failure, hepatic and renal disease.