



Bumetanide Oral Liquid Formulation for the Treatment of Children and Adolescents with Autism Spectrum Disorder: Design of Two Phase III Studies (SIGN Trials)

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Click the links to watch the video and find out more.

Autism spectrum disorder (ASD)

A neurodevelopmental condition with early **deficits in communication and social interaction**, and the presence of **restrictive, repetitive behaviors**.



~1% of the population



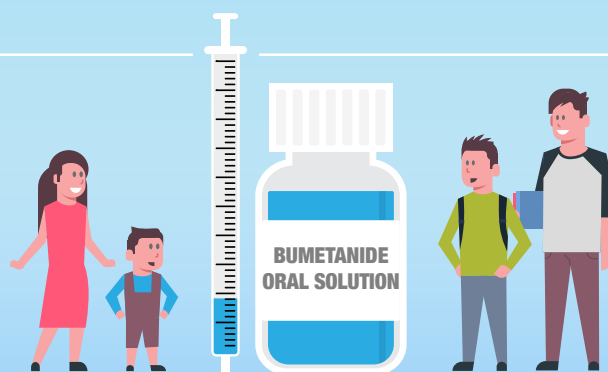
3:1 male to female ratio

No approved medication for these symptoms

Bumetanide

In ASD, bumetanide is hypothesized to act by inhibiting the **NKCC1 cotransporter** in the brain and promoting inhibitory GABA signaling through normalization of intracellular chloride levels.

An **oral solution** of bumetanide is being assessed as a potential treatment for children and adolescents with ASD with a dedicated formulation.



The SIGN trials

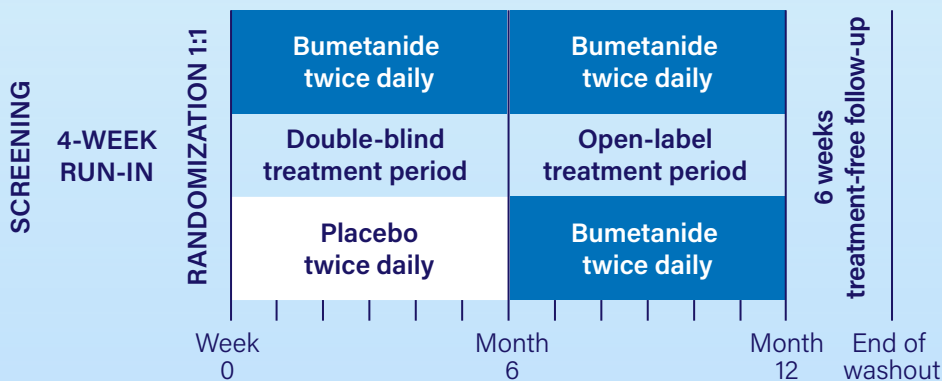
13 countries

2 trials

400 total patients

200 autistic children aged 2-6 years

200 autistic children and adolescents aged 7-17 years



Patients <25 kg: oral solution of bumetanide 0.02 mg/kg twice daily, or placebo
Patients ≥25 kg: oral solution of bumetanide 0.5 mg twice daily, or placebo

Primary endpoint



Secondary Endpoints



Conclusion

Positive results would herald bumetanide oral solution as the first and only pharmacological treatment to **enhance social reciprocity and limit repetitive behavior**, thereby helping patients and families living with ASD to improve their **social integration and quality of life**.