aV1ation

A Phase 2 24-week study of balovaptan compared with placebo in children and young people with autism with an optional 52-week open-label extension (NCT02901431)

Roche

WHAT WAS THE AV1ATION STUDY?

- **aV1ation** looked at the **safety, tolerability, and effectiveness** of balovaptan* compared with placebo in autistic children and young people
- **The placebo** looked the same as balovaptan but did not contain real medicine so **had no medicine-related effect on the body**
- The study measured changes in **socialisation and communication** skills 24 weeks after starting the medicine

339* children and young people (aged 5 to 17 years of age) with moderate-to-severe characteristics of autism†

41 sites across the USA

WHAT WERE THE RESULTS?

- At Week 24, people taking balovaptan did not improve more in their socialisation and communication skills than people who took placebo, as measured by the Vineland™-II two-domain composite score‡

WHAT WERE THE SIDE EFFECTS?

- Balovaptan was **well-tolerated** with **no safety concerns**
- There were **no significant differences** in the number of side effects experienced by people taking balovaptan compared with people taking placebo
- Overall, 24% of people taking balovaptan and 19% of people taking placebo had side effects that were not considered serious
- No one in this study had serious side effects in either the balovaptan or the placebo groups

Four most common side effects§	People taking 10 mg balovaptan (86 people total)	People taking placebo (81 people total)
Being irritable	6%	4%
Headache	5%	2%
Being aggressive	5%	0%
Accidental overdose	2%	2%

Your meaningful contribution to our research efforts

We would like to thank all of the participants and families who took part in the aV1ation study and gratefully acknowledge the efforts of everyone involved

Although there was no medicine-related improvement in social communication, this study was very valuable for researchers to learn more about autism and of the ways to improve health outcomes and quality of life for people with autism

What will happen to my data?

- The anonymised aggregated data from this study have been published in the scientific journal 'JAMA Psychiatry', DOI: *10.1001/jamapsychiatry.2022.1717*.

Where can I find more information?

You can find more information about this study on the websites listed below:

- https://clinicaltrials.gov/ct2/show/results/NCT02901431
- https://forpatients.roche.com/en/trials/neurodevelopmental-disorder/autism-spectrum-disorder/a-study-to-investigate-the-efficacy-and-safety-of-ro5285119-in-p.html
- https://medinfo.roche.com/

Any further questions?

- Please contact a representative at your local Roche office

*339 people were assigned to receive either placebo, or 4 mg or 10 mg of balovaptan.172 people who enrolled in the trial received a lower-than-expected dose of the medicine, which led to a lower-than-expected amount of the medicine in their bloodstreams. Therefore, the data from these people were not included in the main analysis. Overall, 86 people taking 10 mg balovaptan and 81 people taking placebo were included in the main analysis;

†According to the Social Responsiveness Scale™, second edition (SRS-2) ≥66;

‡The Vineland™-II two domain composite score assesses aspects of socialisation and communication, such as how well a person interacts with others and how well they understand language;

§The most common side effects are described across both balovaptan and placebo groups.

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