ForPatients
by Roche

Autism Spectrum Disorder

A Study to Investigate the Efficacy and Safety of RO5285119 in Participants With Autism Spectrum Disorder (ASD)

Trial Status	Trial Runs In	Trial Identifier
Terminated	1 Country	NCT02901431 BP30153

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

A Phase II Multi-Center, Randomized, Double-Blind, 24-Week, Parallel Group, Placebo-Controlled Study to Investigate the Efficacy and Safety of Balovaptan (RO5285119) in Children and Adolescents Age 5-17 With Autism Spectrum Disorder (ASD)

Trial Summary:

For participants enrolled prior to Version 6 of the protocol: This was a Phase II multi-center, randomized, double-blind, 24-week, 3-arm, parallel group, placebo-controlled study to investigate the efficacy, safety, and pharmacokinetics of balovaptan in children and adolescents aged 5-17 years with ASD who are high functioning (intelligence quotient [IQ] greater than or equal to [>=] 70). For participants enrolled according to Version 6 of the protocol: This was a Phase II multi-center, randomized, double-blind, 24-week, parallel group, placebo-controlled, 2-arm study with participants assigned either to a 10 milligram (mg) or equivalent dose of balovaptan, or placebo. All other study parameters remained as stated above. There are three parts to this study: PK Part (Study part 1) included up to 8 weeks of treatment, Main Treatment Part (Study part 2) included 24 week of treatment, and the Open Label Extension Part (Study part 3) included Week 24 to Week 76 of treatment. All participants that completed the 24-week treatment period were eligible to participate in an optional 52-week open-label extension (OLE) during which they received balovaptan treatment.

Hoffmann-La Roche	Phase 2
Sponsor	Phase

NCT02901431 BP30153
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	# 5 Years & # 17 Years	No

Inclusion Criteria:

- Fluent in English
- Diagnostic and Statistical Manual of Mental Disorders, 5th Edition (DSM-5) criteria for ASD or International Statistical Classification of Diseases and Related Health Problems, 10th revision (ICD10) criteria for Autism diagnosis confirmed by Autism Diagnostic Observational Schedule (ADOS-2) criteria
- Social Responsiveness Scale, second edition (SRS-2) (T-score) >= 66
- Clinical Global Impressions of Severity (CGI-S) >= 4 (moderately ill) at screening
- IQ >= 70 as assessed by Wechsler Abbreviated Scale of Intelligence Scale: Second Edition (WASI-II) or Wechsler Preschool and Primary Scale of Intelligence: Fourth Edition (WPPSI-IV) intelligence test
- Language, hearing, and vision compatible with the study measurements as judged by the investigator

Inclusion Criteria for the OLE:

- Have completed the blinded treatment phase of the study OR were required to stop dosing at or before Week 8
- Have no adverse events that would prohibit starting the OLE

Exclusion Criteria:

- Initiation of a major change in psychosocial intervention (including investigational) within 4 weeks prior to screening
- Unstable or uncontrolled clinically significant psychiatric and/or neurological disorder that may interfere with the safety or efficacy endpoints
- Known personal or family history of cerebral aneurysm
- Risk of suicidal behavior
- Seizure within the past 6 months
- Medical history of alcohol or substance abuse/dependence
- Concurrent cardio-vascular disease not considered well controlled by the Investigator
- Clinically significant abnormality on electrocardiogram at screening
- Concomitant disease or condition (pulmonary, gastro-intestinal, hepatic, renal, metabolic, immunological system, or obesity that could interfere with the conduct of the study
- Evidence for current gastro-intestinal bleeding, e.g., active stomach ulcer disease
- History of coagulopathies, bleeding disorders, or blood dyscrasias
- Positive serology for hepatitis B (HBV), hepatitis C (HCV), human immunodeficiency virus (HIV) 1, or HIV 2
- Confirmed clinically significant abnormality in parameters of hematology, clinical chemistry, coagulation, or urinalysis
- Medical history of malignancy if not considered cured
- Participation in an investigational drug study within 90 days or 5 times the half-life of the investigational molecule (whichever is longer) prior to randomization
- Loss of blood over 250 milliliters within three months prior to screening
- Allowed medications have not been stable since 4 weeks before screening, and allowed medications for treatment of epilepsy have not been stable since 3 months before screening
- Use of prohibited medications within 2 weeks prior to screening visit or 5 times the half-life prior to randomization (whichever is longer)