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## **FDA grants Breakthrough Therapy Designation for Roche's balovaptan in autism spectrum disorder**

- **Balovaptan has the potential to be the first pharmacotherapy to help improve core social interaction and communication in people with autism spectrum disorder**
- **Second FDA Breakthrough Therapy Designation for a Roche neuroscience medicine**

Roche (SIX: RO, ROG; OTCQX: RHHBY) announced today that the US Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for its investigational oral medicine balovaptan (previously known as RG7314), a vasopressin 1a (V1a) receptor antagonist for individuals with autism spectrum disorder (ASD). Balovaptan has shown the potential to improve social interaction and communication in people with ASD. Evidence from both human and animal studies implicate the V1a receptor in mediating and modulating key social behaviours that are challenging for individuals with ASD.<sup>1</sup> FDA Breakthrough Therapy Designation is intended to expedite the development and review of medicines with early evidence of substantial potential clinical benefit to patients, or benefit patients without current treatment options.<sup>2</sup>

“We are very pleased that the FDA has granted Breakthrough Therapy Designation for balovaptan, in recognition of its early promise for individuals with ASD,” said Sandra Horning, MD, Roche’s Chief Medical Officer and Head of Global Product Development. “We look forward to working closely with the FDA in the hope that we can bring this medicine to these individuals as quickly as possible.”

ASD is a lifelong developmental condition that affects how an individual behaves, communicates and interacts with others.<sup>3</sup> It is referred to as a ‘spectrum disorder’ because of the broad range and severity of symptoms that individuals with autism have, all of which can significantly impact their daily functioning.<sup>4</sup> No pharmacological treatment currently exists to help improve the challenges that individuals may experience with social skills, repetitive behaviours, restrictive interests and communication. These core symptoms of ASD may be persistent and difficult to overcome.<sup>4</sup>

FDA Breakthrough Therapy Designation for balovaptan is primarily based on efficacy findings in the VANILLA (Vasopressin ANtagonist to Improve social communication in Autism) study, a Phase II trial of balovaptan in adults with ASD.<sup>1</sup> Trial results were presented at the International Congress for Autism Research (IMFAR) in May 2017, and also showed that balovaptan was safe and well tolerated by the subjects in the study.<sup>1</sup> A Phase II trial (aV1ation) investigating balovaptan in children and adolescents with ASD is ongoing and other trials in ASD are being planned.<sup>5</sup>

### **About balovaptan**

Balovaptan is an investigational small molecule currently in clinical development by Roche for the treatment of ASD. It acts as a vasopressin (V1a) receptor antagonist. Evidence from both human and animal studies implicate the V1a receptor in mediating and modulating key social behaviours that are challenging for individuals with ASD.<sup>1</sup> If results from the VANILLA study are confirmed in further studies, balovaptan has the potential to be the first pharmacotherapy to help improve core socialisation and communication symptoms of ASD.

### **About autism spectrum disorder**

ASD is associated with a wide range of symptoms, which can be grouped into two main categories: ‘core’ and ‘associated’.<sup>4</sup>

- Core symptoms are impairments of social interaction and communication, as well as repetitive behaviour and restricted interests, all of which can significantly impact daily functioning.<sup>4</sup>
- Associated symptoms can include anxiety, seizures, language disability, sensory issues, attention deficits, mood alterations, sleep deficits, angry outburst and self-injury.<sup>4</sup>

ASD is approximately four times more commonly diagnosed in boys than in girls. In USA, it is estimated that 1 in 42 boys and 1 in 189 girls have ASD.<sup>6</sup> The World Health Organization estimates that the global prevalence of ASD is approximately one in every 160 people and 0.3% of the global burden of disease.<sup>7</sup> In the European Union, it is estimated that the prevalence of ASD can range from 57 to 67 per 10,000 children.<sup>8</sup>

### **About Roche in neuroscience**

Neuroscience is a major focus of research and development at Roche. The company’s goal is to develop treatment options based on the biology of the nervous system to help improve the lives of people with chronic and potentially devastating diseases. Roche has more than a dozen investigational medicines in

clinical development for diseases that include multiple sclerosis, Alzheimer's disease, spinal muscular atrophy, Duchenne muscular dystrophy, Huntington's disease and autism.

### **About Roche**

Roche is a global pioneer in pharmaceuticals and diagnostics focused on advancing science to improve people's lives. The combined strengths of pharmaceuticals and diagnostics under one roof have made Roche the leader in personalised healthcare – a strategy that aims to fit the right treatment to each patient in the best way possible.

Roche is the world's largest biotech company, with truly differentiated medicines in oncology, immunology, infectious diseases, ophthalmology and diseases of the central nervous system. Roche is also the world leader in in vitro diagnostics and tissue-based cancer diagnostics, and a frontrunner in diabetes management. Founded in 1896, Roche continues to search for better ways to prevent, diagnose and treat diseases and make a sustainable contribution to society. The company also aims to improve patient access to medical innovations by working with all relevant stakeholders. Thirty medicines developed by Roche are included in the World Health Organization Model Lists of Essential Medicines, among them life-saving antibiotics, antimalarials and cancer medicines. Roche has been recognised as the Group Leader in sustainability within the Pharmaceuticals, Biotechnology & Life Sciences Industry nine years in a row by the Dow Jones Sustainability Indices (DJSI).

The Roche Group, headquartered in Basel, Switzerland, is active in over 100 countries and in 2016 employed more than 94,000 people worldwide. In 2016, Roche invested CHF 9.9 billion in R&D and posted sales of CHF 50.6 billion. Genentech, in the United States, is a wholly owned member of the Roche Group. Roche is the majority shareholder in Chugai Pharmaceutical, Japan. For more information, please visit [www.roche.com](http://www.roche.com).

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