

A Potential TREATMENT FOR AUTISM

► *Kevin Sanders, M.D., Principal Medical Director of the Autism Program at Roche, talks about the company's research program for balovaptan, which is being investigated to help improve social interaction and communication in people with autism.*

Autism, a lifelong developmental condition, affects how an individual behaves, communicates, and interacts with others. The mutual give-and-take nature of typical communication and interaction is often particularly challenging for autistic children, who may fail to respond to their names, avoid eye contact with other people, and only interact with others to achieve specific goals. They also may find it difficult to understand other people's feelings or talk about their own feelings.

No pharmacological treatment currently exists to help improve the challenges that individuals with autism experience with social skills, repetitive behaviors, restrictive interests, and communication.

Currently, in the United States two antipsychotic medications, aripiprazole (Otsuka's Abilify) and risperidone (Janssen's Risperdal) are approved for associated symptoms of autism, such as irritability. These medications have a multitude of side

effects and are usually only used in more challenging cases.

Roche is conducting a trial for balovaptan, which would be the first pharmacotherapy to help improve core socialization and communication symptoms of autism spectrum disorder (ASD). In January 2018, the FDA granted Roche breakthrough therapy designation for balovaptan.

"Evidence from both human and animal studies implicate the V1a receptor and find it plays a role in mediating and modulating key social behaviors that are challenging for individuals with ASD," says Kevin Sanders, M.D., principal medical director of the autism program at Roche. "Balovaptan works by modulating vasopressin in the brain."

In the last decade, there have been a number of studies exploring the roles of oxytocin, a hormone released by the pituitary gland, and vasopressin in autism. Vasopressin is a neuropeptide that helps neurons in the brain communicate with each other and seems to play a role in social bonding. In the case of autism, higher levels of vasopressin in the blood correlate with better social functioning.

In Roche's trial in adults with autism, 223 men on the spectrum took 1.5, 4, or 10 milligrams of the drug or a placebo every day for 12 weeks.


Caregivers rated the men's social abilities on the Social Responsiveness Scale 2 (SRS) — the trial's primary outcome measure — before and after the trial. Investigators also evaluated study participants using the Vineland Adaptive Behavior Scales, which measures how an individual adapts to social changes and navigates life's day-to-day demands.

The trial showed no benefit for the drug on the SRS, but the men who took the drug scored significantly higher on the Vineland Adaptive Behavior Scales than those who received the placebo. Vineland is one of six scales recommended by experts for measuring social behavior changes in people with autism.

Roche is currently recruiting patients for a pediatric Phase II trial and started an adult Phase III trial in July 2018.

Dr. Sanders says the hope is that balovaptan will show

improvement in social and communication function across the spectrum, and if approved the drug could be a tool to help patients function better in certain situations.

"Think of this like reading glasses," he says. "You can see better with them, and it's your choice to wear them or not. If this program is successful and the medication approved, there will be some people who this treatment will help and they'll choose to use it." 

About Autism Spectrum Disorder

Autism Spectrum Disorder (ASD) is associated with a wide range of symptoms, which can be grouped into two main categories: core and associated.

Core symptoms are impairments of social interaction and communication, as well as repetitive behavior and restricted interests, all of which can significantly impact daily functioning.

Associated symptoms can include anxiety, seizures, language disability, sensory issues, attention deficit, mood alteration, sleep deficit, angry outbursts, and self-injury.

About 1.7% or 1 in 59 children were identified with ASD, based on data reported from 11 communities across the United States in 2014. Autism is about four times more commonly diagnosed in boys than in girls. In the United States, it is estimated that 1 in 42 boys and 1 in 189 girls have autism.

The World Health Organization estimates that the global prevalence of autism is about one in every 160 people and 0.3% of the global burden of disease.

Dr. Kevin Sanders



© PharmaLinx LLC. Rights do not include promotional use. For distribution or printing rights, contact mwals@pharmaVOICE.com

Join the HBA's United Force for Change in the Nation's Capital

where more than 1,000 leaders from all sectors of healthcare will gather to

- build business connections for a sustainable leadership advantage
- learn from thought leaders to broaden perspectives
- expand their business acumen

2018 HBA ANNUAL CONFERENCE | 5 & 6 NOVEMBER | OMNI SHOREHAM HOTEL | WASHINGTON, D.C.

Tentative Program

Monday, 5 November

PRE-CONFERENCE SEMINARS*

50 is the New 30—How to Stay in the Game (or Get Back In) with Fawn Germer

Communicate with Influence with Stacey Hanke

Navigating Conflict and Tricky Conversations with Deborah Riegel

Five Powerful Lenses to Personal Development, Leadership and Teamwork with Christopher Lindberg

*Pre-registration and an additional fee required

Tuesday, 6 November

BREAKOUT SESSIONS - LEARN FROM THE EXPERTS

Unique 60-minute sessions with issues running the spectrum from *Mission Critical: The First 100 Days in Your Leadership Role* to *Finding Your Mojo* and *Super Powers*



Closing Keynote Speaker

Lisa Bodell

An author and **futurist** who's convinced everyone has the power to **innovate** – if they simply know how. She's the founder and CEO of futurethink, an innovation research and training firm that's helped industry companies—like GE, Pfizer and Johnson & Johnson—**eliminate barriers** in order for them to innovate successfully. Come and learn from Lisa “**Why Simple Wins.**”

Early-Bird
Registration Deadline:
14 September

#HBAAC18
HBA.net.org/2018AC

HBA Healthcare
Businesswomen's
Association