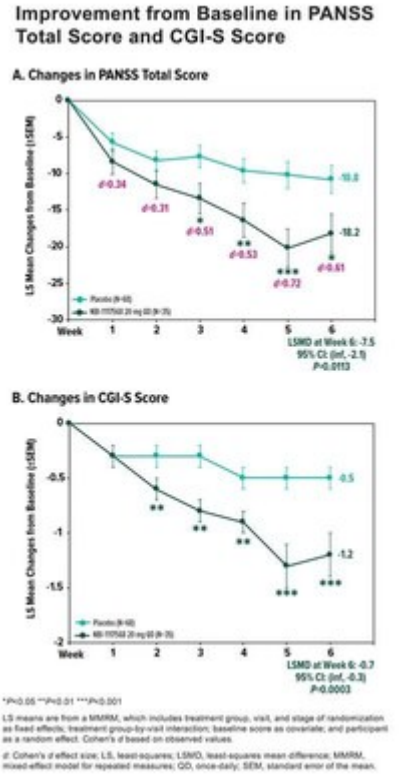
# Neurocrine Biosciences Presents New Positive Data from Phase 2 Study of NBI-1117568 in Adults with Schizophrenia at American Society of Clinical Psychopharmacology 2025

May 28, 2025

SAN DIEGO, May 28, 2025 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced the presentation of data from the Phase 2 study of NBI-1117568 in adults with schizophrenia, which showed a significant improvement in symptoms and overall severity and highlighted new data on the safety and tolerability of the treatment. NBI-1117568 is the first and only investigational oral muscarinic M4 selective orthosteric agonist in clinical development as a potential treatment for schizophrenia. These results were shared as an oral presentation and poster at the American Society of Clinical Psychopharmacology 2025 Annual Meeting in Scottsdale, Arizona.




"Traditional treatment approaches for schizophrenia can lead to significant short- and long-term challenges and often result in discontinuation of therapy. Given these challenges, there is a continued need for new, effective and tolerable treatment options," said Eiry W. Roberts, M.D., Chief Medical Officer, Neurocrine Biosciences. "This compound is promising as it is a direct and selective muscarinic M4 receptor agonist, which is believed to be a key regulator of neurotransmitters impacted by schizophrenia, and we look forward to advancing its development in the Phase 3 registrational program."

In this dose-finding study, adults aged 18 to 55 years with schizophrenia were randomized (2:1) to either NBI-1117568 (dose arms: 20 mg, 40 mg, 60 mg once daily; 30 mg twice daily) or placebo. Other antipsychotics were not allowed during the study. The study consisted of a six-week, double-blind, placebo-controlled period and a two-week safety follow-up.

NBI-1117568 was generally safe and well tolerated at all doses studied, with treatment discontinuation rates due to adverse events similar between NBI-1117568 and placebo. Adverse events with the highest incidence for NBI-1117568 compared with placebo were somnolence (10.7% vs 2.9%, respectively) and dizziness (9.3% vs 1.4%). Increases in heart rate were transient, attenuated over the course of treatment, and not clinically meaningful. No weight gain was associated with the NBI-1117568 treatment groups relative to placebo.

The primary endpoint was the change in total Positive and Negative Syndrome Scale (PANSS) score from baseline to Week 6. The study showed statistically significant improvements in PANSS total score with 20 mg of NBI-1117568 once daily by Week 3 and at all subsequent visits through Week 6. A statistically significant improvement was also observed by Week 2 in the Clinical Global Impression of Severity (CGI-S) scale, with continued improvement seen at all following visits through Week 6.

For all other doses (40 mg and 60 mg once daily, 30 mg twice daily), mean decreases from baseline at Week 6 in PANSS total and CGI-S scale scores were greater with NBI-1117568 than with placebo, but not statistically significant.

Based on these positive Phase 2 results, a Phase 3 registrational program was recently initiated to further evaluate the efficacy, safety and tolerability of NBI-1117568 as a potential treatment for schizophrenia. The Phase 3 study is a global double-blind, placebo-controlled trial evaluating NBI-1117568 in adults with a primary diagnosis of schizophrenia who are experiencing an acute exacerbation or relapse of symptoms. The study is expected to enroll approximately 280 patients. The primary endpoint of the study is a reduction from baseline in the PANSS. The key secondary endpoint is improvement in the CGI-S scale. For more information about the Phase 3 NBI-1117568 study, please visit ClinicalTrials.gov.

Additional poster presentations at the American Society of Clinical Psychopharmacology 2025 annual meeting include:

- Valbenazine Improves the Impacts and Symptoms of Tardive Dyskinesia: Topline Results from the Phase 4 KINECT-PRO™ Study (Poster #W79)
- Remission of Tardive Dyskinesia in Patients Receiving Long-Term Valbenazine Treatment (Poster #T47)

**About NBI-1117568**
NBI-1117568 is the first and only investigational oral muscarinic M4 selective orthosteric agonist in clinical development for the treatment of schizophrenia. There are five muscarinic acetylcholine receptors involved in neurotransmission. Muscarinic receptors are central to brain function and validated as drug targets in psychosis and cognitive disorders. As an M4 selective orthosteric agonist, NBI-1117568 offers the potential for a novel mechanism with an improved safety profile without the need for combination therapy to minimize off-target pharmacology-related side effects, while not being dependent on the presence of acetylcholine for efficacy.

**About the NBI-1117568-SCZ2028 Phase 2 Clinical Study**
The Phase 2, multicenter, randomized, double-blind, placebo-controlled, multiarm, multistage, inpatient dose-finding study was designed to assess the efficacy, safety, tolerability and pharmacokinetics of NBI-1117568 compared with placebo in adult subjects with a primary diagnosis of schizophrenia who experience an acute exacerbation or relapse of symptoms. The study enrolled 210 participants. For more information about this study, visit ClinicalTrials.gov.

**About Neurocrine Biosciences' Muscarinic Portfolio**
In addition to NBI-1117568, Neurocrine has a broad portfolio of assets in clinical development that selectively target muscarinic receptors. The company's muscarinic agonist portfolio also includes NBI-1117567, NBI-1117569, and NBI-1117570, which the company acquired the rights to develop and commercialize from Nxera Pharma. Neurocrine also is developing NBI-1076986, an investigational, selective M4 antagonist that was discovered and is being developed internally at Neurocrine.

| Compound | Primary Mechanism (M1-M4) | Phase of Development | Therapeutic Areas | Potential Areas for Development |
|---|---|---|---|---|
| NBI-1117568 | M4 agonist | 3 | Psychosis<br>Cognition | Alzheimer's Disease<br>Bipolar Disorder<br>Lewy Body Dementia<br>Parkinson's Disease<br>Schizophrenia |
| NBI-1117567 | M1 agonist | 1 | | |
| NBI-1117569 | M4 agonist | 1 | | |
| NBI-1117570 | M1/M4 dual agonist | 1 | | |
| NBI-1076986 | M4 antagonist | 1 | Movement Disorders | Dystonia<br>Parkinson's Disease Tremor |

**About Schizophrenia**
Schizophrenia is a serious and complex syndrome with heterogeneous symptoms. The World Health Organization estimates that the disorder impacts approximately 24 million people worldwide. Annual associated costs for schizophrenia are estimated to be more than $150 billion in the United States. As one of the leading causes of disability worldwide, it often results in significant emotional and functional burden for those who experience symptoms, as well as their family and friends. This chronic and disabling mental health condition is thought to result from a complex interplay of genetic and environmental risk factors. Traditional treatment approaches for schizophrenia rely on the use of antipsychotic medications that can lead to considerable short- and long-term health impacts.

**About Neurocrine Biosciences, Inc.**
Neurocrine Biosciences is a leading neuroscience-focused, biopharmaceutical company with a simple purpose: to relieve suffering for people with great needs. We are dedicated to discovering and developing life-changing treatments for patients with under-addressed neurological, neuroendocrine and neuropsychiatric disorders. The company's diverse portfolio includes FDA-approved treatments for tardive dyskinesia, chorea associated with Huntington's disease, classic congenital adrenal hyperplasia, endometriosis* and uterine fibroids,* as well as a robust pipeline including multiple compounds in mid- to late-phase clinical

development across our core therapeutic areas. For three decades, we have applied our unique insight into neuroscience and the interconnections between brain and body systems to treat complex conditions. We relentlessly pursue medicines to ease the burden of debilitating diseases and disorders because you deserve brave science. For more information, visit neurocrine.com, and follow the company on LinkedIn, X and Facebook. (**in collaboration with AbbVie)*



**Forward-Looking Statements**
In addition to historical facts, this press release contains forward-looking statements that involve a number of risks and uncertainties. These statements include, but are not limited to, statements regarding the clinical results from, and our future development plans with respect to, NBI-1117568, as well as the therapeutic potential and clinical benefits or safety profile of NBI-1117568. Factors that could cause actual results to differ materially from those stated or implied in the forward-looking statements include, but are not limited to, the following: data that we report may change following a more comprehensive review of the data related to the clinical study and such data may not accurately reflect the complete results of the clinical study; risks that clinical development activities may not be initiated or completed on time or at all, or may be delayed for regulatory, manufacturing or other reasons, may not be successful or replicate previous clinical trial results, may fail to demonstrate that our product candidates are safe and effective, or may not be predictive of real-world results or of results in subsequent clinical trials; risks that regulatory submissions for our product candidates may not occur or be submitted in a timely manner; our future financial and operating performance; risks associated with our dependence on third parties for development, manufacturing and commercialization activities for our products and product candidates and our ability to manage these third parties; risks that the FDA or other regulatory authorities may make adverse decisions regarding our products or product candidates; risks that the potential benefits of the agreements with our collaboration partners may never be realized; risks that our products and/or our product candidates may be precluded from commercialization by the proprietary or regulatory rights of third parties, or have unintended side effects, adverse reactions or incidents of misuse; risks associated with U.S. federal or state legislative or regulatory and/or policy efforts which may result in, among other things, an adverse impact on our revenues or potential revenue; risks associated with potential generic entrants for our products; and other risks described in the Company's periodic reports filed with the Securities and Exchange Commission, including without limitation the Company's quarterly report on Form 10-Q for the quarter ended March 31, 2025. Neurocrine Biosciences disclaims any obligation to update the statements contained in this press release after the date hereof other than required by law.



View original content to download multimedia:https://www.prnewswire.com/news-releases/neurocrine-biosciences-presents-new-positive-data-from-phase-2-study-of-nbi-1117568-in-adults-with-schizophrenia-at-american-society-of-clinical-psychopharmacology-2025-302467554.html

SOURCE Neurocrine Biosciences, Inc.

Neurocrine Biosciences, Inc. Media: Liz Reynolds, 1-818-749-8778, media@neurocrine.com; Investors: Todd Tushla, 1-858-617-7143, ir@neurocrine.com