SECTION 3

Interventions with potential to improve behavioral health

Emerging Drug Therapies

Medications play a significant role in managing mental health conditions in the U.S. According to the Centers for Disease Control and Prevention (CDC), 20% of adults aged 18 and older had received any mental health treatment in the past 12 months, including 16.5% who had taken prescription medication for their mental health and 10.1% who received any kind of therapy or counseling from a professional. Women were more likely than men to receive any treatment, and non-Hispanic white adults were most likely to have received any mental health treatment in the past 12 months. The use of mental health treatment rose during the COVID-19 pandemic.

Purchasers should have a basic understanding of the role of the Food and Drug Administration (FDA) in evaluating evidence for new medications. The FDA requires randomized clinical trials to show safety and efficacy of a medication to receive its approval. FDA approval also grants market exclusivity protections for new drugs it approves. Medications are often used in combination with psychotherapy, such as cognitive behavioral therapy. Mental health medications can affect people in diverse ways, and it may take multiple tries for patients and clinicians to find one that improves outcomes with tolerable side effects. Some cases and conditions are resistant to treatment, including medication.

Purchasers should have a basic understanding of the role of the Food and Drug Administration (FDA) in evaluating evidence for new medications.

Progress in developing new and better psychiatric drugs has been slow. Some psychiatric medications have developed from serendipitous findings in other drugs. Frustration has motivated some advocates and researchers to look to a class of "psychedelic" drugs for potential therapeutic application for some psychiatric disorders. Clinical trials are underway for some of these drugs with the intent to seek FDA approval.

Ketamine is a drug attracting a lot of interest as a treatment for treatment-resistant depression and other psychiatric conditions. It is a dissociative anesthetic approved decades ago by the FDA for sedation. The FDA has yet to approve ketamine by injection for any mental health condition. It is a controlled substance by the U.S. Drug Enforcement Administration, has serious risks, and must be given under clinical supervision. One form, known as esketamine, has been approved by the FDA for treatment-resistant depression and is administered nasally. The FDA approved esketamine but required the drug only be administered under strict controls of a Risk Evaluation and Mitigation Strategy (REMS) to ensure any benefits of the drug outweigh its safety and abuse risks.

A PCORI-funded study of ketamine comparing it to electroconvulsive therapy (ECT) for treatment-resistance depression was published in The New England Journal of Medicine in 2023. The trial results for ketamine (by injection) were positive but modest. Industry rarely conducts comparative effectiveness studies because the FDA approval process requires showing efficacy compared to a placebo. The financial risk-return calculation for investors to seek FDA approval for a new application of a drug that is in generic status, such as ketamine, may be too low. Moreover, the practice of "off label" use permits prescribing a drug already on the market for more uses. As a public-private entity PCORI can play a vital role filling research gaps and helping find opportunities for existing treatments to improve health outcomes. CHCC produced a brief with a fuller discussion of the issues related to evidence, safety, and FDA approval.

Disclaimer

The statements and views expressed in this report are solely the responsibility of the authors and CHCC and do not necessarily represent the views of the Patient-Centered Outcomes Research, Institute (PCORI), its Board of Governors or Methodology Committee.