Ovid Therapeutics Inc.
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Ovid™ THERAPEUTICS
BOLDMEDICINE

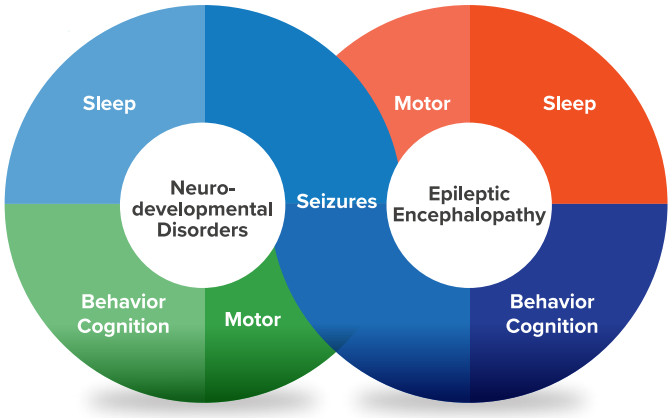
Transforming treatments for rare neurological disorders

Through an innovative approach to both science and business, Ovid Therapeutics is developing impactful medicines for people with rare neurological disorders.

There are over 100 different rare brain disorders for which there are few effective medicines. It's an area of unmet need, and one in which Ovid Therapeutics sees itself as a catalyst for transformation.

"Over a million people in the US are affected by rare brain disorders, which have been largely ignored until now," said Ovid CMO Amit Rakhit, a pediatric cardiologist who, when at Biogen, developed Spinraza (nusinersen) for a rare neuromuscular disorder. "Previously, there was little focus or understanding of rare diseases, particularly in pediatrics, and neurologists didn't have the tools to make meaningful interventions. Now we're experiencing a knowledge explosion—neurology today is becoming the new immuno-oncology."

Over the past ten years there has been a sea change in the scientific understanding of many of these rare disorders, explained Ovid CEO Jeremy Levin, former CEO of Teva Pharmaceuticals. Additionally, tools for unravelling and influencing the molecular pathways and causes of these diseases have blossomed, regulatory agencies attitudes to rare disorders are maturing, and patient advocacy bodies use social media to call attention to these disorders and disseminate research findings. "Consequently, neurological diseases that were previously considered intractable are now potentially treatable," he said.



Ovid's focus on related neurologic disorders allows for a scalable clinical and business development model.

The Ovid approach

Established in 2014, Ovid was built in a very specific and strategic fashion to address a particular opportunity, and its founders hired a highly experienced management team with deep expertise in translational science, drug evaluation, orphan clinical drug development and commercialization.

What further sets the company apart is the targeted approach it takes to select compounds for development. Ovid seeks and secures novel molecules with excellent safety profiles for which new scientific understanding suggests a mechanism of action better suited to treating a rare neurological disorder. The team also goes out of its way to cultivate a full appreciation of the impact of a condition on patients, their families, support organizations, doctors, regulators and payers. This deep understanding enables them to construct a working hypothesis of how the candidate medicine should work, as well as the endpoints that will lead to meaningful and measurable evidence of its impact, thereby driving new therapeutic options for these devastating disorders.

Ovid's lead candidate OV101, for instance, was originally under development by Lundbeck for insomnia but was never brought to market. The actual mode of action, identified after the drug was put aside, speaks to the presumed underlying causes of Fragile X and Angelman syndromes, said Rakhit. Recognizing this, Ovid licensed exclusive rights to the compound in 2015.

Both Angelman and Fragile X syndrome cause a range of physical, intellectual and psychological disabilities. It is believed that in these disorders reduced levels of the neurological transmitter GABA result in neurons that are not properly modulated by the extrasynaptic GABA receptor. OV101 is a potent agonist that selectively targets this receptor and thereby restores neuron function. Its safety profile has been assessed in clinical data from over 4,000 adults, and it is now in phase 2 trials in adults with Angelman syndrome and in phase 1 trials in adolescents with Angelman or Fragile X syndrome.

Building on its experience with OV101 and by 'cloning' its scientific and medical capability, Ovid was able to strike a unique 50:50 partnership in January 2017 to codevelop and commercialize OV935, a compound discovered by Takeda. OV935 had been considered for a number of conditions, but Ovid and Takeda scientists realized that using OV935 to modulate its target, cholesterol 24 hydroxylase (CH24H), an enzyme expressed predominantly in the brain with a central role in neuronal cholesterol homeostasis, might be of great value in helping to alleviate epileptic encephalopathies, such as Dravet and Lennox–Gastaut syndromes and tuberous sclerosis complex. It is thought that increased CH24H levels in these disorders lead to the activation of NMDA receptors and high glutamatergic signaling implicated in seizures and cognitive and behavioral deficits. OV935, a CH24H inhibitor, reduces glutamatergic signaling via modulation of the NMDA receptor. The candidate, which has been tested in over 80 adults, is currently in phase 1b/2a trials in adults with developmental and/or epileptic encephalopathies.

Looking forward and partnering

With two potential first-in-class assets and a proven scalable model allowing expansion into additional patient populations and with novel molecules, Ovid is on the way to becoming a leading neurological disorder company. "Diseases of the brain previously thought to be rare are actually very common in total, representing a compelling medical opportunity," said Levin. "We are on the cusp of a transformation in treating these disorders."

Over time, Ovid plans to in-license other compounds that address rare neuronal disorders. Through each collaboration it aims to build on existing strengths to expand the diseases it can treat, and it is looking for partners with the right scientific, medical, technical and financial fit. "Our programs meet very high standards of science and our investment in patients doesn't rest on only one compound," said Levin. "For each disorder, we are looking to deepen our pipeline and seek partnerships that can bolster that commitment."

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