

ARCADE and ELEKTRA Studies in Rare Developmental and Epileptic Encephalopathies



Ovid Therapeutics, in collaboration with Takeda Pharmaceutical Company Limited, is conducting a broad clinical development program to evaluate TAK-935/OV935 across a range of rare epilepsies.

Currently, the development program is evaluating adults with Dravet syndrome, Lennox-Gastaut syndrome and Tuberous Sclerosis Complex. In the third quarter of 2018, the companies plan to expand the program into two trials in younger patients: ELEKTRA in Dravet syndrome and Lennox-Gastaut syndrome, and ARCADE in Cyclin-Dependent Kinase-Like 5 (CDKL5) deficiency disorder (CDD) and Duplication 15q (Dup15q) syndrome.

About **ARCADE**

STUDY OVERVIEW

Phase 2, multicenter, open-label, pilot study in CDD and Dup15q syndrome



OBJECTIVE

Primary Objective

Assess the frequency of motor seizures in patients treated with TAK-935/OV935

Secondary Objectives

Safety, tolerability and pharmacokinetic (PK) assessments



About **ELEKTRA**

STUDY OVERVIEW

Phase 2, multicenter, randomized, double-blind, placebo controlled study

OBJECTIVE

Primary Objective

Assess the frequency of seizures in patients treated with TAK-935/OV935 versus placebo

Secondary Objectives

Safety, tolerability and PK assessments

ENROLLMENT

Approximately 30 patients
(CDD ~15, Dup15q ~15)

30  **125**
PATIENTS PATIENTS

ENROLLMENT

Approximately 125 patients

PATIENT POPULATION

Pediatric patients, aged 2 to 17 years old, with confirmed diagnosis of CDD or Dup15q syndrome


2-17
YEARS OF AGE

PATIENT POPULATION

Pediatric patients, aged 2 to 17 years old, with Dravet syndrome or Lennox-Gastaut syndrome

TIMING

Ovid and Takeda expect to initiate the study in the third quarter of 2018


THIRD
QUARTER 2018

TIMING

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About the TAK-935/OV935 Broad Clinical Development Program in Rare Developmental and Epileptic Encephalopathies (DEE)

The TAK-935/OV935 clinical program focuses on the development of potential therapies for people living with certain rare epilepsies, known as developmental and epileptic encephalopathies, or DEE, that cause intractable seizures and are often associated with cognitive, neurologic and behavioral problems. Since TAK-935/OV935 is thought to modulate the N-Methyl-D-Aspartate (NMDA) signaling receptor of the brain, conditions evaluated as part of the development program are considered if the NMDA receptor underlies the pathological mechanism of the disorder.

TAK-935/OV935 is a potent, highly-selective, first-in-class inhibitor of the enzyme cholesterol 24-hydroxylase (CH24H). CH24H is predominantly expressed in the brain. CH24H converts cholesterol to 24HC, which then exits the brain into the blood plasma circulation. Glutamate is one of the main neurotransmitters in the brain and has been shown to play a role in the initiation and spread of seizure activity. Recent literature indicates CH24H is involved in over-activation of the glutamatergic pathway through modulation of the NMDA channel, implying its potential role in central nervous system diseases such as epilepsy.



“Developmental and epileptic encephalopathies is a term for a specific group of rare epilepsy conditions associated with severe cognitive and behavioral disturbances. These conditions have few or limited therapeutic options available, which makes this an area of significant unmet need in the treatment of rare pediatric epilepsies. We are excited to further our clinical development program and are now in the process of initiating these important clinical trials.”

– Dr. Amit Rakhit, Chief Medical & Portfolio Officer, Ovid Therapeutics

This fact sheet includes certain disclosures that contain “forward-looking statements,” including, without limitation, statements regarding (i) progress, timing, scope and results of clinical trials for Ovid’s product candidates, and (ii) the potential clinical benefit of TAK-935/OV935 to treat patients with DEE. You can identify forward-looking statements because they contain words such as “will,” “believes” and “expects.”

Forward-looking statements are based on Ovid’s current expectations and assumptions. Because forward-looking statements relate to the future, they are subject to inherent uncertainties, risks and changes in circumstances that may differ materially from those contemplated by the forward-looking statements, which are neither statements of historical fact nor guarantees or assurances of future performance. Important factors that could cause actual results to differ materially from those in the forward-looking statements are set forth in Ovid’s filings with the Securities and Exchange Commission, including its Quarterly Report on Form 10-Q for the period ended March 31, 2018 under the caption “Risk Factors.” Ovid assumes no obligation to update any forward-looking statements contained herein to reflect any change in expectations, even as new information becomes available.

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