- Intelligence Simplified

FDA approves Epidiolex (cannabidiol) [CBD] an active ingredient derived from marijuana to treat rare, severe forms of epilepsy

Approval of Epidiolex offers a fresh solution in pharmaco-treatable epilepsy cases. According to Centers for Disease Control and Prevention (CDC) more than 3 million adults and 470,000 children are affected by epilepsy in United states. As per industry service provider and KOL there is a constant need to have a more efficient medicines to treat epilepsy. Epidiolex is unique because of its novel mechanism of action providing an effective treatment option.

A quick handy summary for Epidiolex:

Product Details: Dose adjustable, Oral Solution 100mg/ml supplied in bottle with dose plunger.

Product Administration: The recommended starting dosage is 2.5 mg/kg taken twice daily (5 mg/kg/day). After one week, the dosage can be increased to a maintenance dosage of 5 mg/kg twice daily (10 mg/kg/day).

Indication: for the treatment of seizures associated with Lennox-Gastaut syndrome and Dravet syndrome in patients 2 and older.

Novel Mechanism: CBD does not act through cannabinoid (CB) receptors or sodium channels - Prime targets are GPR55 and adenosine reuptake, TRPV1

Epidiolex, or purified cannabidiol is indicated for the treatment of seizures associated with Lennox-Gastaut syndrome and Dravet syndrome in patients 2 and older.

US Launch: GW Pharma expects to launch in the last quarter 2018 as cannabidiol (CBD) is currently listed as schedule I category and DEA need to issue an IFR to reschedule it.

Peak Sales Estimates: Analysts have predicted peak annual sales of $600mn in Lennox-Gastaut and $200mn in Dravet, with the remaining indication of Tuberous Sclerosis Complex (TSC) worth an estimated $250mn at peak.

Europe Approval and Launch: EMA submission accepted in Feb 2018 for review, expected approval in Q1 2019.

Next Targeted Indication for approval: Tuberous Sclerosis Complex (TSC).

New Formulations to work on: Solid dose and Intravenous preparations.

Competition: Zogenix could compete with Epidiolex with its ZX008 ZX008 (Low Dose-Fenfluramine Hydrochloride) Oral Solution in combination with Cannabidiol, as an adjunctive therapy in children and young adults with Dravet Syndrome or Lennox-Gastaut Syndrome. ZX008 has received orphan drug designation in the United States and Europe.

Takeda has forged a partnership with Ovid Therapeutics to develop TAK-935 (Oral tablets), which is in early to mid-stage trials and targets Dravet, Lennox-Gastaut and TSC.

Other Regulatory salient features: The FDA granted Priority Review designation for this application. Fast-Track designation was granted for Dravet syndrome. Orphan Drug designation was granted for both the Dravet syndrome and Lennox-Gastaut syndrome indications.

Intellectual Property: 8 patents granted & 2 Notices of Allowance issued by the USPTO. Company plans to file new patent applications based on the new data generated by team.

Orphan Designation

- 7 years in the US plus expected 6-month pediatric extension
- 10 years in the EU plus expected 2 years pediatric extension