

## **Palynziq: A Literature Review Project Resulting in a Scarcity Neurocognitive and Psychiatric Outcome Studies**

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**Background:** Phenylketonuria (PKU) is caused by phenylalanine hydroxylase (PAH) deficiency that results in phenylalanine (PHE) accumulation leading to elevated blood PHE concentrations that are toxic to the brain with associated neurocognitive and psychiatric impairments reducing quality of life. In 2018, the Food and Drug Administration (FDA) approved Palynziq™ from BioMarin Pharmaceutical, as a treatment for PKU. This approval was after a decade of regulated human trials. Although reduced PHE levels with Palynziq treatment results in diet liberalization, studies of neurocognitive and psychiatric improvements are limited. The objective of this project is to explore factors that are limiting studies and to provide a clinical assessment framework that will prompt more research. This framework of neurocognitive and psychiatric measures will allow for more individualized interventions and optimize outcomes for patients with PKU.

**Methods:** As part of a graduate student project, searches of MEDLINE were conducted using the terms pegvaliase, Palynziq, phenylalanine ammonia lyase (PAL), PEG PAL, neurocognitive, neuropsychological assessment, cognitive, and psychiatric. Additional data were obtained from Clinicaltrials.gov.

**Results:** There is currently limited research on the neurocognitive and/or psychiatric outcomes in adults either prior to or after treatment with Palynziq.

**Conclusion:** This literature review indicated that while diet liberalization benefit of Palynziq™ treatment is well understood, studies about neurocognitive and psychiatric improvements in adults are limited. It is important that this investigative project continues as it will contribute to an assessment measure framework to guide clinicians and document the overall benefits of Palynziq™ in adults with PKU using reliable and valid measures.

### **LEARNING OUTCOMES:**

- Describe approval of Palynziq as a treatment for PKU in adults
- Highlight potential neurological effects and benefits of Palynziq treatment
- Propose framework for future neurocognitive and psychological studies