MarketVue® Prion Disease (U.S.) – August 2024

Assessing detection, diagnosis, and emerging therapies in prion disease: Neurologists' insights on implications for novel therapies in clinical practice

BACKGROUND

This syndicated report provides comprehensive commercial insights regarding future treatment of prion disease driven by primary market research with KOLs and physician specialists. It delves into diagnosis dynamics, pipeline analysis, and the potential uptake and impact of novel therapies within this unique and evolving market. By examining the market dynamics and therapeutic advancements, this report helps stakeholders understand the factors influencing product uptake and utilization in prion disease.

QUESTIONS ANSWERED IN THIS REPORT

- **Identification and Diagnosis:**
  - What is the current state of identification and diagnosis of prion disease?
  - How might current diagnosis dynamics impact uptake and efficacy of future therapeutic intervention?
  - What strategies are being implemented to improve time to diagnosis?
  - How are genetic cases currently identified?
  - What is the typical disease progression and outlook for patients following disease onset in the absence of approved therapies?

- **Epidemiology and Patient Population**
  - How many incident cases of prion disease occur annually in the U.S.?
  - What prion disease patient types would be most amenable to therapeutic intervention?

- **Trial Design**
  - What are the clinical trial design and feasibility considerations in recruiting and evaluating different patient types?
  - How have historical and ongoing clinical trials been designed?

- **Product Differentiation**
  - What are neurologists' perceptions of therapeutic benefit among various patient types?
  - What are the perceived product differentiations in emerging therapies?
  - What are neurologists' perceptions of different emerging therapy mechanisms of action for treating prion disease, including antisense oligonucleotide therapy?
  - What level of achievement do neurologists desire regarding key efficacy endpoints, safety, and tolerability from novel therapies?
  - How is the treatment landscape expected to evolve with the introduction of novel therapies?

METHODOLOGY

- Qualitative 60-minute research interviews with 8 U.S. KOL neurodegenerative disease specialists
- Interviews conducted in August 2024

RESEARCH DELIVERABLES

- PowerPoint deliverable with key findings and insights
- Custom analysis of proprietary questions (if applicable)
- Customized presentation tailored to customer priorities

OTHER PUBLISHED NEUROLOGY AND NEUROMUSCULAR STUDIES

- **Angelman Syndrome**
- **Myasthenia Gravis**
- **STXBP1 Encephalopathy**
- **Tardive Dyskinesia**
- **Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)**

About REACH Market Research

REACH is an independent pharmaceutical market research company focused on rare diseases. We specialize in providing market assessments, product profile assessments, and commercial insights for pipeline assets and marketed brands.

PRIMARY MARKET RESEARCH REPORT

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