R&D Trends: Autism Spectrum Disorders

Description: Unknown etiology and low industry interest hinders pipeline progression

Since 2006, two drugs have gained FDA approval for the symptomatic treatment of irritability in autism spectrum disorders (ASDs). However, the pipeline is also characterized by a high attrition rate, with eight pipeline candidates having been discontinued since 2009. CureMark's Phase III candidate, CM-AT, currently represents the most advanced compound in the small ASD pipeline.

Features and benefits

- Overview of drugs in late- and early-stage clinical development for autism spectrum disorders.
- Identification of the target product profile for future autism spectrum disorders treatments.
- Summary of clinical trial design in autism spectrum disorders including exploration of key endpoints and assessment methodology.
- Identification of key challenges associated with the conduct of clinical trials in autism spectrum disorders.
- Exploration of the future treatment in autism spectrum disorders including the development of personalized treatments and the use of biomarkers.

Highlights

- The autism spectrum disorders (ASDs) pipeline is sparse relative to other conditions that are treated with psychotropic medications. The unknown etiology of ASDs poses a fundamental challenge to the therapeutic discovery process and the pipeline is characterized by a high attrition rate.
- The two approved therapies for the management of autism spectrum disorders both target the symptoms of irritability. The comparator therapy identified by Datamonitor is Risperdal (risperidone; Johnson & Johnson) as it was the first to be approved in this indication, demonstrates a strong efficacy profile and is regarded as a first-line therapy.
- Key challenges associated with the conduct of clinical trials in autism spectrum disorders are: the unknown etiology of the disorders and heterogeneous symptomology. Communication and language impairments commonly associated with autism spectrum disorders further compound these challenges.

Your key questions answered

- How many drugs are in clinical development for the treatment of autism spectrum disorders? What deters companies from investing in this area?
- What symptoms and mechanisms of action are most frequently targeted? What key companies are involved in the pipeline?
- What is the target product profile and most appropriate comparator therapy?
- What are the key challenges in the conduct of clinical trials in autism spectrum disorders?
- How is the treatment of autism spectrum disorders likely to evolve over the next 10 to 20 years?

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Executive Summary
- Strategic scoping and focus
- Datamonitor key findings
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OVERVIEW
- Catalyst
- Summary

CLINICAL PIPELINE OVERVIEW
- Overview of the autism spectrum disorders pipeline
- The autism spectrum disorders pipeline is sparse, comprising just four compounds in clinical development
  - Emerging features of the autism spectrum disorders clinical pipeline
  - Classification of pipeline products
  - Each of the four pipeline candidates targets a different mechanism of action
Companies involved in the autism spectrum disorders clinical pipeline
Pipeline activity is limited to small, specialist pharmaceutical companies
Late-stage development compounds recently discontinued
Pipeline failures prevalent due to unknown etiology of autism spectrum disorders

TARGET PRODUCT PROFILE
Clinical targets
Clinical trial data indicate a lack of efficacy of investigated therapies
Although superior to Risperdal, side effects are also prominent with Abilify treatment
Ease of administration is a key consideration in the development of treatments for the pediatric population
Comparator therapy: Risperdal (risperidone; Johnson & Johnson)
Risperdal was the first drug to receive FDA approval for irritability associated with autistic disorder
Clinical trial data for Risperdal
Target product profile versus current level of attainment
Target product for the treatment of irritability should demonstrate superior efficacy and side effect-profile to Risperdal

CLINICAL TRIAL DESIGN IN AUTISM SPECTRUM DISORDERS
Use of clinical trial endpoints varies according to the target behavioral symptom
Core symptoms
Repetitive behavior
Irritability
ADHD symptoms
Social impairment
Typical clinical trial design
Key challenges in the conduct of clinical trials in autism spectrum disorders
Unknown etiology of autism spectrum disorders hinders selection of appropriate targets
Heterogeneity of symptomatology complicates patient subgroup identification
Conduct of trials in children and young adolescents is complicated by communication difficulties
Future developments in clinical trial design
Clinical trials are likely to include adult autism spectrum disorder patients in the future
Increased patient selection will help to increase response rates in clinical trials

INNOVATIVE EARLY-STAGE APPROACHES
Scientific rationale determines targets, but clinical efficacy needs to be demonstrated
Directly reducing glutamate transmission holds promise, but more trials are required
Consequence of low insulin-like growth factor levels in autism spectrum disorders is unknown
Efficacy will need to be particularly high to outweigh safety concerns associated with stem cell treatment

THE FUTURE OF TREATMENT IN AUTISM SPECTRUM DISORDERS
Continued research into the etiology of ASDs is essential for development of treatments
Elucidating the genetic and environmental interactions will aid treatment discovery
Biomarkers will aid the development of personalized treatment options
Promise for identification, earlier intervention, and treatment response
Subpopulation stratification will increase treatment response

BIBLIOGRAPHY
Journal papers and publications
Websites
Datamonitor reports

APPENDIX
Contributing experts
Conferences attended
Report methodology

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