

# US Orphan Drug Pipeline Analysis

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## Abstracts

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In recent years, the pharmaceutical industry has been experiencing a paradigm shift. While a large pool of patients was considered as a major source of revenue for pharma companies in the past, the focus is now gradually shifting to small sections of patients suffering from rare disease. In US, this pool of patients is gradually growing and orphan drugs are becoming an extremely attractive business proposition for the pharmaceuticals industry. With close to 30 million people in the US having some kind of rare disease complaints, the increasing activities around the development of orphan drugs only imply that the appropriate treatments for unmet needs are increasing, thus increasing the profit margins in the future.

“US Orphan Drug Pipeline Analysis” by PNS Pharma gives comprehensive insight on the various orphan designated drugs being developed for the treatment of rare disease. Research report covers all the orphan designated drugs being developed in various development phases. This report enables pharmaceutical companies, collaborators and other associated stake holders to identify and analyze the available investment opportunity in the US Orphan Drug market based upon development process.

**Following parameters for each vaccine profile in development phase are covered in “US Orphan Drug Pipeline Analysis” research report:**

Drug Profile Overview

Alternate Names for Drug

Active Indication

Phase of Development

Mechanism of Action

Brand Name

Patent Information

Orphan Designation by Indication, Country & Organisation

Country for Clinical Trial

Owner / Originator/ Licensee/Collaborator

Administrative Route

Drug Class

ATC Codes

### **US Orphan Drug Pipeline by Clinical Phase:**

Research: 4

Preclinical: 54

Phase-I: 68

Phase-I/II: 62

Phase-II: 169

Phase-II/III: 15

Phase-III: 93

Preregistration: 28

Registration: 18

Marketed: 137

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## About

### **Arbaclofen - Seaside Therapeutics**

(GABA-B) agonist, for the treatment of social withdrawal (and possibly other core impairments) associated with fragile X syndrome (FXS) and autistic disorder. These disorders are believed to be caused by excessive activation of glutamate signalling, and abnormally high ratios of excitatory to inhibitory neurotransmission in the brain. Arbaclofen is designed to inhibit glutamate signalling, thereby inhibiting excessive metabotropic glutamate receptor (mGluR)-mediated protein synthesis. It may also optimise the ratio of excitatory to inhibitory neurotransmission. Studies in preclinical models of fragile X syndrome and autistic disorder have shown this agent to be efficacious. Phase III development of capsule and fast-dissolving tablet formulations are underway in patients with autistic disorder in the US. Phase III clinical development in fragile X syndrome is also in progress in the US.

Autistic disorder (also called autistic spectrum disorders or ASD) is used to describe a group of complex developmental brain disorders that are caused by a combination of genetic and environmental disorders. It is marked by the presence of markedly abnormal or impaired development in social interaction and communication and a markedly restricted repertoire of activity and interest. Manifestations of the disorder vary greatly depending on the developmental level and chronological age of the individual. There are currently no US FDA-approved therapeutics to treat the core impairments of autistic disorder.

### **Acetylcysteine Injection - Cumberland Pharmaceuticals**

Cumberland Pharmaceuticals has developed an intravenously injectable formulation of acetylcysteine (Acetadote®) for the treatment of paracetamol overdose. Acetylcysteine is used in emergency departments to prevent or lessen the liver damage that results from paracetamol overdose. This product was the first injectable treatment available in the US for this potentially lethal overdosing occurrence. It has also been launched in Australia and New Zealand for use in paracetamol poisoning. A supplemental NDA (sNDA) has been submitted to the US FDA seeking approval for use of Acetadote® in patients with non-paracetamol-related acute liver failure. Phase II/III trials of acetylcysteine for the prevention of radiocontrast-induced nephropathy are underway in the US.

In January 2013, the US FDA approved InnoPharma's ANDA for acetylcysteine intravenous injection for the prevention or lessening hepatic injury following paracetamol overdose. Fresenius Kabi USA will sell, market and distribute the product under the agreement with InnoPharma. The ANDA approval provides for the first generic alternative to Acetadote® injection. Acetylcysteine injection was launched by Fresenius Kabi in January 2013. Perrigo also launched a generic version of acetylcysteine.

Cumberland's competitors in the paracetamol overdose market are those companies selling orally administered N-acetylcysteine; this includes (but is not limited to) Geneva Pharmaceuticals, Bedford Laboratories (a division of Ben Venue Laboratories), Roxane Laboratories, and Hospira.

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