

# Orphan Drugs for Cancer Pipeline Analysis

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## Abstracts

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A huge market opportunity is offered by small patient population which suffers from rare or orphan diseases. Among the category of new orphan drugs, Oncology account for the largest disease group in recent years. It has been observed that majority of the orphan drugs in the clinical stages are for rare cancer disease drugs, and are in the late stages of the pipeline. Some of the drugs are being developed for treating rare cancer diseases like solid tumor of the pancreas and thyroid, blood cancer, melanoma, and others.

“Orphan Drugs for Cancer Pipeline Analysis” by PNS Pharma gives comprehensive insight on the various orphan designated drugs being developed for the treatment of multiple cancers. Research report covers all the ongoing vaccines 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 market for orphan designated cancer drugs based upon development process.

**Following parameters for each orphan designated drug profile in development phase are covered in “Orphan Drugs for Cancer Pipeline Analysis” research report:**

Drug Profile Overview

Alternate Names for Drug

Active Indication

## Phase of Development

Detailed Therapeutic Trials

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

## **Orphan Designated Drugs for Cancer Treatment by Clinical Development Phase:**

Preclinical: 11

Phase-I: 25

Phase-I/II: 33

Phase-II: 68

Phase-II/III: 6

Phase-III: 41

Preregistration: 8

Registered: 3

Marketed: 59

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Alternate Names

Originator & Owner

Collaborator

Technology Provider

Licensee

Highest Development Phase

Indications

Class

Mechanism of Action

ATC Code

Designated Brand Name & Orphan Designation

## About

ERYtech Pharma is developing an encapsulated L-asparaginase product called GRASPA for the treatment of cancer indications, with an initial focus on recurrent acute lymphoblastic leukaemia (ALL) in adults and children. Development for ALL is at the phase II/III stage in Belgium, France, Austria and Spain. A phase I trial in pancreatic cancer was completed in France. A phase IIb trial in elderly patients with newly diagnosed acute myeloid leukaemia (AML) is also underway in France.

A companion diagnostic test is in development in collaboration with M. D. Anderson Cancer Center. Asparaginase hydrolyses asparagine to L-aspartic acid and ammonia, causing the depletion of asparagine. This leads to the death of cells that require asparagine to survive, such as leukaemic lymphocytes. Therapeutic enzymes, like asparaginase, commonly cause toxicity in the body and often have a short half-life from days to minutes. Anti-enzyme antibodies can eventually be generated, decreasing the effectiveness of the enzyme. GRASPA (ERYASP) uses the Cleav'ERY System whereby asparaginase is encapsulated into red blood cells, so the enzyme is only active inside the erythrocyte. This increases the half-life of asparaginase and decreases the dose of enzyme required. One single injection enhances the depletion of plasmatic asparagine up to one month, with adverse events being strongly reduced. Anti-enzyme antibodies do not occur as the asparaginase is not extra-cellular.

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