

# Hereditary Tyrosinemia - Pipeline Review - 2019

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## Abstracts

Firstview Insight's Hereditary Tyrosinemia - Pipeline Review-2019 provides an overview of the pipeline landscape of Hereditary Tyrosinemia. It provides comprehensive insights of all the clinical and non-clinical therapeutics in development with detailed description about the collaborations; deals; designations; patent information etc. These reports encourage the clients in distinguishing the upcoming and existing competitors in their separate therapeutic spaces. The report provides detailed description of the competitor profiles with key milestones and evidence along with analysis by mechanism of action; route of administration; molecule type; stage of development. Information obtained from multiple sources will be used to triangulate and update the profiles. The report also provides key events in the last year related to the indication. This report provides detailed analysis of all the products along with the companies involved.

Tyrosinemia is a genetic disorder characterized by disruptions in the multistep process that breaks down the amino acid tyrosine, a building block of most proteins. If untreated, tyrosine and its byproducts build up in tissues and organs, which can lead to serious health problems.

There are three types of tyrosinemia, which are each distinguished by their symptoms and genetic cause. Tyrosinemia type I, the most severe form of this disorder, is characterized by signs and symptoms that begin in the first few months of life. Affected infants fail to gain weight and grow at the expected rate (failure to thrive) due to poor food tolerance because high-protein foods lead to diarrhea and vomiting. Affected infants may also have yellowing of the skin and whites of the eyes (jaundice), a cabbage-like odor, and an increased tendency to bleed (particularly nosebleeds). Tyrosinemia type I can lead to liver and kidney failure, softening and weakening of the bones (rickets), and an increased risk of liver cancer (hepatocellular carcinoma). Some affected children have repeated neurologic crises that consist of changes in mental state, reduced sensation in the arms and legs (peripheral neuropathy), abdominal pain,

and respiratory failure. These crises can last from 1 to 7 days. Untreated, children with tyrosinemia type I often do not survive past the age of 10.

Tyrosinemia type II can affect the eyes, skin, and mental development. Signs and symptoms often begin in early childhood and include eye pain and redness, excessive tearing, abnormal sensitivity to light (photophobia), and thick, painful skin on the palms of their hands and soles of their feet (palmoplantar hyperkeratosis). About 50 percent of individuals with tyrosinemia type II have some degree of intellectual disability.

Tyrosinemia type III is the rarest of the three types. The characteristic features of this type include intellectual disability, seizures, and periodic loss of balance and coordination (intermittent ataxia).

About 10 percent of newborns have temporarily elevated levels of tyrosine (transient tyrosinemia). In these cases, the cause is not genetic. The most likely causes are vitamin C deficiency or immature liver enzymes due to premature birth.

### **Drug Profile Overview:**

The pipeline section provides descriptive drug profiles for the pipeline products including product description, mechanism of action, route of administration, molecule type, technology involved, chemical information.

### **Clinical Trial Overview:**

This section of the report focuses on the clinical activity of the molecule. It includes both clinical and pre-clinical activity which provides detailed information about the safety, efficacy, tolerability, toxicity of pipeline drugs. A graphical representation of the clinical trial landscape of pipeline therapy which includes information about phase of development, trial design, treatment arms, dosage and frequency, formulation of the drug, primary and secondary completion date, enrolment number, exclusion and inclusion criteria, line of therapy. This section also includes the clinical trial results and analysis based on those results.

### **Product Development Activity:**

This section of the report focuses on detail information about designations, exclusivity details, technology, licensing and collaboration, funding and financing, key milestones and various other development activities.

## **Company Overview:**

Company profile includes the detail about type of company, headquarter, global presence, research focus and key financial

## **Scope**

The report provides a competitive landscape

The report also provides clinical trial landscape of the pipeline drugs including status; trial phase; sponsor type and end-point status

The report provides the list of companies which are the most active in the pipeline

The report covers pipeline products based on various stages of development ranging from pre-registration till discovery

The report provides descriptive drug profiles which includes product description; comprising detailed mechanism of action (MoA); route of administration (RoA); Stage of development; clinical trial status; licensing and collaboration details & other developmental activities

The report features comparative analysis of product profiles based on molecule type; mechanism of action (MoA); route of administration (RoA)

The report summarizes all the dormant and discontinued pipeline projects

The report also provides latest news for the past one year

## **Reasons To Buy**

To identifying prominent players in the treatment landscape

To determine the drivers; barriers and unmet need in the treatment space

Gain strategically significant competitor information; analysis; and insights to

formulate effective R&D strategies

Define in-licensing and out-licensing strategies by identifying prospective partners with the most attractive projects to enhance and expand business potential and scope

To understand the composition of the pipeline in terms of molecule type; molecular target; mechanism of action and route of administration

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