

# Barth Syndrome - Pipeline Review - 2019

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## Abstracts

Firstview Insight's Barth Syndrome - Pipeline Review-2019 provides an overview of the pipeline landscape of Barth Syndrome. 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.

Barth syndrome is a rare condition characterized by an enlarged and weakened heart (dilated cardiomyopathy), weakness in muscles used for movement (skeletal myopathy), recurrent infections due to small numbers of white blood cells (neutropenia), and short stature. Barth syndrome occurs almost exclusively in males. In males with Barth syndrome, dilated cardiomyopathy is often present at birth or develops within the first months of life. Over time, the heart muscle becomes increasingly weakened and is less able to pump blood. Individuals with Barth syndrome may have elastic fibers in place of muscle fibers in some areas of the heart muscle, which contributes to the cardiomyopathy.

### 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

## **List of products**

Elamipretide

trihexanoin

digoxin

furosemide

captopril

carvedilol



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