

# Barth Syndrome - Pipeline Review, H2 2018

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## Abstracts

Barth Syndrome - Pipeline Review, H2 2018

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Barth Syndrome - Pipeline Review, H2 2018, provides an overview of the Barth Syndrome (Other Diseases) pipeline landscape.

Barth syndrome is a rare disorder characterized by dilated cardiomyopathy, skeletal myopathy, recurrent infections due to neutropenia, and short stature. It is passed from mother to son through the X chromosome. Symptoms include cardiomyopathy, skeletal muscle abnormalities, weak muscle tone, increased levels of organic acids in blood and urine and frequent bacterial infections such as pneumonia. This occurs exclusively in male. Treatment includes physical therapy to help with reduced muscle tone and antibiotics for bacterial infections.

### REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Barth Syndrome - Pipeline Review, H2 2018, provides comprehensive information on the therapeutics under development for Barth Syndrome (Other Diseases), complete with analysis by stage of development, drug target, mechanism of action (MoA), route of administration (RoA) and molecule type. The guide covers the descriptive pharmacological action of the therapeutics, its complete research and development history and latest news and press releases.

The Barth Syndrome (Other Diseases) pipeline guide also reviews of key players involved in therapeutic development for Barth Syndrome and features dormant and

discontinued projects. The guide covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Phase III, Phase II and Preclinical stages are 1, 1 and 2 respectively. Similarly, the Universities portfolio in Preclinical and Discovery stages comprises 1 and 1 molecules, respectively.

Barth Syndrome (Other Diseases) pipeline guide helps in identifying and tracking emerging players in the market and their portfolios, enhances decision making capabilities and helps to create effective counter strategies to gain competitive advantage. The guide is built using data and information sourced from Global Markets Direct's proprietary databases, company/university websites, clinical trial registries, conferences, SEC filings, investor presentations and featured press releases from company/university sites and industry-specific third party sources. Additionally, various dynamic tracking processes ensure that the most recent developments are captured on a real time basis.

**Note:** Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

## SCOPE

The pipeline guide provides a snapshot of the global therapeutic landscape of Barth Syndrome (Other Diseases).

The pipeline guide reviews pipeline therapeutics for Barth Syndrome (Other Diseases) by companies and universities/research institutes based on information derived from company and industry-specific sources.

The pipeline guide covers pipeline products based on several stages of development ranging from pre-registration till discovery and undisclosed stages.

The pipeline guide features descriptive drug profiles for the pipeline products which comprise, product description, descriptive licensing and collaboration details, R&D brief, MoA & other developmental activities.

The pipeline guide reviews key companies involved in Barth Syndrome (Other Diseases) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Barth Syndrome (Other Diseases) therapeutics based on mechanism of action (MoA), drug target, route of administration (RoA)

and molecule type.

The pipeline guide encapsulates all the dormant and discontinued pipeline projects.

The pipeline guide reviews latest news related to pipeline therapeutics for Barth Syndrome (Other Diseases)

## **REASONS TO BUY**

Procure strategically important competitor information, analysis, and insights to formulate effective R&D strategies.

Recognize emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage.

Find and recognize significant and varied types of therapeutics under development for Barth Syndrome (Other Diseases).

Classify potential new clients or partners in the target demographic.

Develop tactical initiatives by understanding the focus areas of leading companies.

Plan mergers and acquisitions meritoriously by identifying key players and it's most promising pipeline therapeutics.

Formulate corrective measures for pipeline projects by understanding Barth Syndrome (Other Diseases) pipeline depth and focus of Indication therapeutics.

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

Adjust the therapeutic portfolio by recognizing discontinued projects and understand from the know-how what drove them from pipeline.

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### COMPANIES MENTIONED

Cerenis Therapeutics Holding SA  
Mitotech SA  
Stealth BioTherapeutics Inc  
Ultragenyx Pharmaceutical Inc

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