

# Batten Disease - Pipeline Review, H1 2020

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## Abstracts

Batten Disease - Pipeline Review, H1 2020

### SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Batten Disease - Pipeline Review, H1 2020, provides an overview of the Batten Disease (Central Nervous System) pipeline landscape.

Batten disease, a rare genetic disorder, belongs to a group of progressive degenerative neurometabolic disorders known as the neuronal ceroid lipofuscinoses. Symptoms of Batten disease usually become apparent between five and 15 years of age. Symptoms include vision loss, lack of muscle coordination, mental retardation or decreasing mental function, emotional disturbances, seizures, muscle spasms, deterioration of muscle tone and movement problems.

### REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Batten Disease - Pipeline Review, H1 2020, provides comprehensive information on the therapeutics under development for Batten Disease (Central Nervous System), 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 Batten Disease (Central Nervous System) pipeline guide also reviews of key players involved in therapeutic development for Batten Disease and features dormant and discontinued projects. The guide covers therapeutics under Development by

Companies/Universities/Institutes, the molecules developed by Companies in Phase II, IND/CTA Filed, Preclinical and Discovery stages are 3, 2, 16 and 1 respectively. Similarly, the Universities portfolio in Phase I, Preclinical and Discovery stages comprises 1, 4 and 2 molecules, respectively.

Batten Disease (Central Nervous System) 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 Batten Disease (Central Nervous System).

The pipeline guide reviews pipeline therapeutics for Batten Disease (Central Nervous System) 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 Batten Disease (Central Nervous System) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Batten Disease (Central Nervous System) 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 Batten Disease (Central Nervous System)

## **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 Batten Disease (Central Nervous System).

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 Batten Disease (Central Nervous System) 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.

## Contents

|   |
|---|
| Introduction  |
| Global Markets Direct Report Coverage                           |
| Batten Disease - Overview                                       |
| Batten Disease - Therapeutics Development                       |
| Pipeline Overview   |
| Pipeline by Companies   |
| Pipeline by Universities/Institutes                             |
| Products under Development by Companies                         |
| Products under Development by Universities/Institutes           |
| Batten Disease - Therapeutics Assessment                        |
| Assessment by Target  |
| Assessment by Mechanism of Action                               |
| Assessment by Route of Administration                           |
| Assessment by Molecule Type                                     |
| Batten Disease - Companies Involved in Therapeutics Development |
| Abeona Therapeutics Inc   |
| Amicus Therapeutics Inc   |
| Blue Turtle Bio Technologies Inc                                |
| Circumvent Pharmaceuticals Inc                                  |
| Collaborations Pharmaceuticals Inc                              |
| Exicure Inc   |
| Polaryx Therapeutics Inc  |
| Recursion Pharmaceuticals Inc                                   |
| RegenxBio Inc   |
| Retrotope Inc   |
| Seneb BioSciences Inc   |
| Spark Therapeutics Inc  |
| Theranexus SAS  |
| Xonovo Inc  |
| Batten Disease - Drug Profiles                                  |
| (gemfibrozil + tretinoin) - Drug Profile                        |
| Product Description   |
| Mechanism Of Action   |
| R&D Progress  |
| ABO-201 - Drug Profile  |
| Product Description   |
| Mechanism Of Action   |

R&D Progress

ABO-202 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

ATGTX-502 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

BBDF-101 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

DUOC-01 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

gemfibrozil - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate CLN1 for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate CLN6 for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate CLN6 for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate CLN6 for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate CLN8 for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate Palmitoyl Protein Thioesterase 1 for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate TPP1 for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Gene Therapy to Activate TPP1 for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

INI-0602 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Oligonucleotides for Neurology - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

PLX-300 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Recombinant Palmitoyl-Protein Thioesterase-1 Replacement for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

RGX-181 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

RT-001 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

RT-002 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Small Molecule for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Small Molecules for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Small Molecules for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Small Molecules to Inhibit CDC42 for Batten Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

SNB-4050 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

SPKTPP-1 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

XN-001 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Batten Disease - Dormant Projects

Batten Disease - Discontinued Products

Batten Disease - Product Development Milestones

Featured News & Press Releases

Jan 20, 2020: Polaryx Therapeutics receives IND approval for PLX-200 From the FDA for the treatment of late infantile neuronal ceroid lipofuscinosis

Oct 17, 2019: REGENXBIO Announces Presentations at the European Society of Gene & Cell Therapy 27th Annual Congress

Jun 18, 2019: Abeona Therapeutics receives FDA fast track designation for ABO-202 AAV9 gene therapy in CLN1 disease

May 21, 2019: Abeona Therapeutics announces FDA clearance of Investigational New Drug Application for ABO-202 Gene Therapy in CLN1 Disease

Apr 15, 2019: Abeona Therapeutics to present data for ABO-202 in CLN1 disease at the American Society of Gene and Cell Therapy Annual Meeting

Jan 31, 2019: Spark Therapeutics announces presentation of preclinical data in CLN2 disease at 15th annual WORLDSymposium

Jan 31, 2019: REGENXBIO receives Rare Pediatric Disease Designation for RGX-181 Gene Therapy for the Treatment of CLN2 Form of Batten Disease

Dec 06, 2018: Abeona Therapeutics provides update on Infantile Batten Disease drug candidate ABO-202 at 2018 R&D Day

Nov 14, 2018: FDA grants Orphan Drug Designation to RGX-181 gene therapy for the treatment of CLN2 form of Batten disease

Sep 03, 2018: Regenxbio adds new neurodegenerative candidate to pipeline

Aug 30, 2018: REGENXBIO Announces Pipeline Expansion With RGX-181 Gene Therapy For CLN2 Form Of Batten Disease

Apr 20, 2018: Abeona Therapeutics Receives Orphan Drug Designation in the European Union for ABO-202 Gene Therapy Program in Batten Disease

Mar 15, 2018: Abeona Therapeutics Receives FDA Rare Pediatric Disease Designation for ABO-202 Gene Therapy Program in CLN1 Disease

Feb 12, 2018: Abeona Therapeutics Receives FDA Orphan Drug Designation for ABO-202 Gene Therapy Program in Infantile Batten Disease

Dec 11, 2017: Polaryx Therapeutics Receives Orphan Drug Designation From the US FDA for the Treatment of Neuronal Ceroid Lipofuscinoses With PLX-100

Appendix

Methodology

Coverage

Secondary Research

Primary Research

Expert Panel Validation

Contact Us

Disclaimer



## List Of Tables

### LIST OF TABLES

Number of Products under Development for Batten Disease, H1 2020  
Number of Products under Development by Companies, H1 2020  
Number of Products under Development by Universities/Institutes, H1 2020  
Products under Development by Companies, H1 2020  
Products under Development by Companies, H1 2020 (Contd..1), H1 2020  
Products under Development by Universities/Institutes, H1 2020  
Number of Products by Stage and Target, H1 2020  
Number of Products by Stage and Mechanism of Action, H1 2020  
Number of Products by Stage and Route of Administration, H1 2020  
Number of Products by Stage and Molecule Type, H1 2020  
Batten Disease - Pipeline by Abeona Therapeutics Inc, H1 2020  
Batten Disease - Pipeline by Amicus Therapeutics Inc, H1 2020  
Batten Disease - Pipeline by Blue Turtle Bio Technologies Inc, H1 2020  
Batten Disease - Pipeline by Circumvent Pharmaceuticals Inc, H1 2020  
Batten Disease - Pipeline by Collaborations Pharmaceuticals Inc, H1 2020  
Batten Disease - Pipeline by Exicure Inc, H1 2020  
Batten Disease - Pipeline by Polaryx Therapeutics Inc, H1 2020  
Batten Disease - Pipeline by Recursion Pharmaceuticals Inc, H1 2020  
Batten Disease - Pipeline by RegenxBio Inc, H1 2020  
Batten Disease - Pipeline by Retrotope Inc, H1 2020  
Batten Disease - Pipeline by Seneb BioSciences Inc, H1 2020  
Batten Disease - Pipeline by Spark Therapeutics Inc, H1 2020  
Batten Disease - Pipeline by Theranexus SAS, H1 2020  
Batten Disease - Pipeline by Xonovo Inc, H1 2020  
Batten Disease - Dormant Projects, H1 2020  
Batten Disease - Discontinued Products, H1 2020

## List Of Figures

### LIST OF FIGURES

Number of Products under Development for Batten Disease, H1 2020  
Number of Products under Development by Companies, H1 2020  
Number of Products under Development by Universities/Institutes, H1 2020  
Number of Products by Top 10 Targets, H1 2020  
Number of Products by Stage and Top 10 Targets, H1 2020  
Number of Products by Top 10 Mechanism of Actions, H1 2020  
Number of Products by Stage and Top 10 Mechanism of Actions, H1 2020  
Number of Products by Routes of Administration, H1 2020  
Number of Products by Stage and Routes of Administration, H1 2020  
Number of Products by Molecule Types, H1 2020  
Number of Products by Stage and Molecule Types, H1 2020

### COMPANIES MENTIONED

Abeona Therapeutics Inc  
Amicus Therapeutics Inc  
Blue Turtle Bio Technologies Inc  
Circumvent Pharmaceuticals Inc  
Collaborations Pharmaceuticals Inc  
Exicure Inc  
Polaryx Therapeutics Inc  
Recursion Pharmaceuticals Inc  
RegenxBio Inc  
Retrotape Inc  
Seneb BioSciences Inc  
Spark Therapeutics Inc  
Theranexus SAS  
Xonovo Inc

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