

# Retinitis Pigmentosa - Pipeline Insight, 2021

<https://marketpublishers.com/r/RE988738EF5EN.html>

Date: May 2021

Pages: 80

Price: US\$ 2,000.00 (Single User License)

ID: RE988738EF5EN

## Abstracts

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DelveInsight's, "Retinitis Pigmentosa - Pipeline Insight, 2021," report provides comprehensive insights about 45+ companies and 45+ pipeline drugs in Retinitis Pigmentosa pipeline landscape. It covers the pipeline drug profiles, including clinical and nonclinical stage products. It also covers the therapeutics assessment by product type, stage, route of administration, and molecule type. It further highlights the inactive pipeline products in this space.

### Geography Covered

Global coverage

### Retinitis Pigmentosa Understanding

#### Retinitis Pigmentosa: Overview

Retinitis pigmentosa (RP) is an umbrella term for a group of hereditary progressive retinal disorders that cause progressive degeneration of the retina. The disease is inherited as autosomal recessive, autosomal dominant or X-linked recessive traits. Signs of retinitis pigmentosa usually appear during childhood or adolescence. Common symptoms include difficulty seeing at night and a loss of side (peripheral) vision. RP is diagnosed by electroretinography (ERG), visual field testing, and retinal imaging, fundus auto-fluorescence (FAF) and genetic testing. Currently, there is no therapy that stops the evolution of pigmentary retinopathies or restores the vision. Management of retinitis pigmentosa includes dietary supplements, ozone therapy, surgery etc. Gene therapy based treatments are under research focus for the treatment of Retinitis pigmentosa.

'Retinitis Pigmentosa - Pipeline Insight, 2021' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Retinitis Pigmentosa pipeline landscape is provided which includes the disease overview and Retinitis Pigmentosa treatment guidelines. The assessment part of the report embraces, in depth Retinitis Pigmentosa commercial assessment and clinical assessment of the pipeline products under development. In the report, detailed description of the drug is given which includes mechanism of action of the drug, clinical studies, NDA approvals (if any), and product development activities comprising the technology, Retinitis Pigmentosa collaborations, licensing, mergers and acquisition, funding, designations and other product related details.

## Report Highlights

The companies and academics are working to assess challenges and seek opportunities that could influence Retinitis Pigmentosa R&D. The therapies under development are focused on novel approaches to treat/improve Retinitis Pigmentosa.

## Retinitis Pigmentosa Emerging Drugs Chapters

This segment of the Retinitis Pigmentosa report encloses its detailed analysis of various drugs in different stages of clinical development, including phase III, II, I, preclinical and Discovery. It also helps to understand clinical trial details, expressive pharmacological action, agreements and collaborations, and the latest news and press releases.

## Retinitis Pigmentosa Emerging Drugs

### BIIB-112: Biogen

BIIB-112 (formerly called NSR-RPGR) consists of a standard AAV8 vector carrying a codon optimized RPGR gene. BIIB112 is in phase II clinical studies for X-linked retinitis pigmentosa treatment. In September 2018, NSR-RPGR was granted orphan drug designation for the treatment of inherited retinal dystrophies due to defects in the RPGR gene from the U.S. Food and Drug Administration.

### QR 1123 (formerly ISIS RHO 2.5Rx): Ionis Pharmaceuticals

QR 1123 (formerly ISIS RHO 2.5Rx) is an antisense oligonucleotide, designed to specifically target the mutant P23H messenger ribonucleic acid (mRNA) in order to reduce the expression of the P23H protein selectively, while preserving expression of the wild type (WT) rhodopsin (RHO) protein. The therapy is currently in Phase II clinical evaluation for the treatment of Retinitis Pigmentosa. In November 2019, ProQR Therapeutics announced that it received Orphan Drug designation (ODD) from the Food and Drug Administration (FDA) for QR-1123. QR-1123 was in-licensed from Ionis Pharmaceuticals in 2018. QR-1123 has also received Fast Track designation from the FDA.

Further product details are provided in the report

### Retinitis Pigmentosa: Therapeutic Assessment

This segment of the report provides insights about the different Retinitis Pigmentosa drugs segregated based on following parameters that define the scope of the report, such as:

#### Major Players in Retinitis Pigmentosa

There are approx. 45+ key companies which are developing the therapies for Retinitis Pigmentosa. The companies which have their Retinitis Pigmentosa drug candidates in the most advanced stage, i.e. Phase II include, Biogen.

#### Phases

DelveInsight's report covers around 45+ products under different phases of clinical development like

Late stage products (Phase III)

Mid-stage products (Phase II)

Early-stage product (Phase I) along with the details of

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

Retinitis Pigmentosa pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as

Oral

Parenteral

Intravitreal

Subretinal

Topical

Molecule Type

Products have been categorized under various Molecule types such as

Monoclonal Antibody

Peptides

Polymer

Small molecule

Gene therapy

Product Type

Drugs have been categorized under various product types like Mono, Combination and Mono/Combination.

### Retinitis Pigmentosa: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase III, II, I, preclinical and discovery stage. It also analyses Retinitis Pigmentosa therapeutic drugs key players involved in developing key drugs.

### Pipeline Development Activities

The report covers the detailed information of collaborations, acquisition and merger, licensing along with a thorough therapeutic assessment of emerging Retinitis Pigmentosa drugs.

### Retinitis Pigmentosa Report Insights

- Retinitis Pigmentosa Pipeline Analysis

- Therapeutic Assessment

- Unmet Needs

- Impact of Drugs

### Retinitis Pigmentosa Report Assessment

- Pipeline Product Profiles

- Therapeutic Assessment

- Pipeline Assessment

- Inactive drugs assessment

- Unmet Needs

## Key Questions

### Current Treatment Scenario and Emerging Therapies:

How many companies are developing Retinitis Pigmentosa drugs?

How many Retinitis Pigmentosa drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Retinitis Pigmentosa?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Retinitis Pigmentosa therapeutics?

What are the recent trends, drug types and novel technologies developed to overcome the limitation of existing therapies?

What are the clinical studies going on for Retinitis Pigmentosa and their status?

What are the key designations that have been granted to the emerging drugs?

## Key Players

Biogen

Neurotech

Ionis Pharmaceuticals

Novartis Pharmaceuticals

Nacuity Pharmaceuticals

ReNeuron

ID Pharma

Allegro Ophthalmics

SparingVision

Editas Medicine

OiDE OptoEye

### Key Products

BIIB-112

NT 501

QR 1123 (formerly ISIS RHO 2.5Rx)

CPK 850

N-acetylcysteine-amide

ReN 003

DVC1-0401

Risuteganib

SPVN 06

SPVN 20

LCA10 program

GtCCR4 gene therapy

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Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report

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BIIB-112: Biogen

Product Description

Research and Development

Product Development Activities



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