

# Huntingtin (Huntington Disease Protein or HTT) - Pipeline Review, H2 2018

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## Abstracts

Huntingtin (Huntington Disease Protein or HTT) - Pipeline Review, H2 2018

### SUMMARY

According to the recently published report 'Huntingtin - Pipeline Review, H2 2018'; Huntingtin (Huntington Disease Protein or HTT) pipeline Target constitutes close to 16 molecules. Out of which approximately 14 molecules are developed by companies and remaining by the universities/institutes.

Huntingtin (Huntington Disease Protein or HTT) - Huntingtin protein is an encoded by the huntingtin gene, also called the HTT or HD (Huntington disease) gene. Huntingtin up regulates the expression of brain derived neurotrophic factor (BDNF) at the transcription level. Huntingtin is primarily associated with vesicles and microtubules. These indicate an important role in cytoskeletal anchoring or transport of mitochondria. The Htt protein is involved in vesicle trafficking as it interacts with HIP1 to mediate endocytosis. Huntingtin also plays an important role in the establishment in epithelial polarity through its interaction with RAB11A.

The report 'Huntingtin - Pipeline Review, H2 2018' outlays comprehensive information on the Huntingtin (Huntington Disease Protein or HTT) targeted therapeutics, complete with analysis by indications, stage of development, mechanism of action (MoA), route of administration (RoA) and molecule type; that are being developed by Companies/Universities.

It also reviews key players involved in Huntingtin (Huntington Disease Protein or HTT) targeted therapeutics development with respective active and dormant or discontinued

projects. Currently, The molecules developed by companies in Phase II, Preclinical and Discovery stages are 3, 6 and 5 respectively.

Similarly, the universities portfolio in Preclinical and Discovery stages comprises 1 and 1 molecules, respectively. Report covers products from therapy areas Central Nervous System and Genetic Disorders which include indications Huntington Disease, Alzheimer's Disease, Kennedy's Disease (Spinal and Bulbar Muscular Atrophy) and Spinocerebellar Ataxia (SCA).

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

## SCOPE

The report provides a snapshot of the global therapeutic landscape for Huntingtin (Huntington Disease Protein or HTT)

The report reviews Huntingtin (Huntington Disease Protein or HTT) targeted therapeutics under development by companies and universities/research institutes based on information derived from company and industry-specific sources

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

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

The report reviews key players involved in Huntingtin (Huntington Disease Protein or HTT) targeted therapeutics and enlists all their major and minor projects

The report assesses Huntingtin (Huntington Disease Protein or HTT) targeted therapeutics based on mechanism of action (MoA), route of administration (RoA) and molecule type

The report summarizes all the dormant and discontinued pipeline projects

The report reviews latest news and deals related to Huntingtin (Huntington Disease Protein or HTT) targeted therapeutics

## REASONS TO BUY

Gain strategically significant competitor information, analysis, and insights to formulate effective R&D strategies

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

Identify and understand the targeted therapy areas and indications for Huntingtin (Huntington Disease Protein or HTT)

Identify the use of drugs for target identification and drug repurposing

Identify potential new clients or partners in the target demographic

Develop strategic initiatives by understanding the focus areas of leading companies

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

Devise corrective measures for pipeline projects by understanding Huntingtin (Huntington Disease Protein or HTT) development landscape

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

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Exicure Inc

F. Hoffmann-La Roche Ltd

Neurimmune Holding AG

nLife Therapeutics SL

PTC Therapeutics Inc

reMYND NV

Shire Plc

UniQure NV

Voyager Therapeutics Inc

Vybion Inc

WAVE Life Sciences Ltd

Huntingtin (Huntington Disease Protein or HTT) - Drug Profiles

AMT-130 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Antisense Oligonucleotide to Inhibit Huntingtin Protein for Huntington's Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

## Antisense Oligonucleotides to Inhibit HTT for Huntington's Disease - Drug Profile

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Product Description

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R&D Progress

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Product Description

Mechanism Of Action

R&D Progress

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Product Description

Mechanism Of Action

R&D Progress

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Product Description

Mechanism Of Action

R&D Progress

## Small Molecules to Inhibit Huntingtin Protein for Huntington's Disease - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

## Synthetic Peptide to Inhibit Huntingtin Protein for Huntington's Disease - Drug Profile

Product Description

Mechanism Of Action

## R&D Progress

Vaccine to Target Huntingtin for Huntington's Disease - Drug Profile

Product Description

Mechanism Of Action

## R&D Progress

VYHTT-01 - Drug Profile

Product Description

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## R&D Progress

WVE-120101 - Drug Profile

Product Description

Mechanism Of Action

## R&D Progress

WVE-120102 - Drug Profile

Product Description

Mechanism Of Action

## R&D Progress

Huntingtin (Huntington Disease Protein or HTT) - Dormant Products

Huntingtin (Huntington Disease Protein or HTT) - Product Development Milestones

Featured News & Press Releases

Aug 03, 2018: PRIME designation granted by European Medicines Agency for RG6042 for treatment of Huntington's disease

May 17, 2018: Voyager Therapeutics Announces New Data on VY-HTT01 at the American Society of Gene and Cell Therapy 2018 Annual Meeting

Apr 25, 2018: uniQure Delivers Oral Presentation on Broad Set of Preclinical Data on AMT-130 in Huntington's Disease at the 2018 American Academy of Neurology Annual Meeting

Apr 24, 2018: New Data from IONIS-HTT Rx Phase 1/2 Study Demonstrates Correlation Between Reduction of Disease-causing Protein and Improvement in Clinical Measures of Huntington's Disease

Apr 15, 2018: Genentech to Present Phase I/IIa Data of RG6042 at AAN

Apr 03, 2018: Patent Issues for Novel Huntington and Alzheimer's Disease Gene Therapy

Mar 01, 2018: IONIS-HTT Rx (RG6042) Top-Line Data Demonstrate Significant Reductions of Disease-Causing Mutant Huntingtin Protein in People with Huntington's Disease

Jan 22, 2018: uniQure Receives Orphan Medicinal Product Designation in Europe for AMT-130 in Huntington's disease

Dec 13, 2017: New drug could halt progression of Huntington's disease

Oct 18, 2017: uniQure Presents New Preclinical Data on AMT-130 in Huntington's Disease at the ESGCT 25th Anniversary Congress in Berlin

Oct 06, 2017: UniQure Announces FDA Orphan Drug Designation for AMT-130 in Huntington's disease

Jul 17, 2017: Wave Life Sciences Initiates Phase 1b/2a Clinical Trial: PRECISION-HD1 in Patients with Huntington's Disease

Jul 17, 2017: Wave Life Sciences Announces Initiation of Phase 1b/2a Clinical Trial: PRECISION-HD2 in Patients with Huntington's Disease

Jun 22, 2017: Enrollment in Phase 1/2a Study of IONIS-HTT Rx in Patients with Huntingtons Disease Completed and Open-Label Extension Study to Open in 2H 2017

Jun 01, 2017: Voyager Therapeutics Selects Lead Clinical Candidate for Huntington's Disease

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F. Hoffmann-La Roche Ltd  
Neurimmune Holding AG  
nLife Therapeutics SL  
PTC Therapeutics Inc  
reMYND NV  
Shire Plc  
UniQure NV  
Voyager Therapeutics Inc  
Vybion Inc  
WAVE Life Sciences Ltd

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