

Huntingtin (Huntington Disease Protein or HTT) - Pipeline Review, H1 2018

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Abstracts

Huntingtin (Huntington Disease Protein or HTT) - Pipeline Review, H1 2018

SUMMARY

Huntingtin (Huntington Disease Protein or HTT) pipeline Target constitutes close to 15 molecules. Out of which approximately 13 molecules are developed by companies and remaining by the universities/institutes.

The latest report Huntingtin - Pipeline Review, H1 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.

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 molecules developed by companies in Phase II, Preclinical and Discovery stages are 3, 7 and 3 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).

Furthermore, this report also reviews key players involved in Huntingtin (Huntington Disease Protein or HTT) targeted therapeutics development with respective active and dormant or discontinued projects. Driven by data and information sourced from 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.

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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Assessment by Route of Administration

Assessment by Molecule Type

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AFFiRiS AG

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

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

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WVE-120101 - Drug Profile

Product Description

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WVE-120102 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

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

Featured News & Press Releases

Dec 13, 2017: New drug could halt progression of Huntingtons 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

Apr 26, 2017: uniQure Presents New Preclinical Data on AMT-130 in Huntington's Disease at CHDI's 12th Annual Huntington's Disease Therapeutics Conference

Apr 24, 2017: uniQure Announces Presentations at the Upcoming Annual Meeting of The American Society of Gene and Cell Therapy

Apr 04, 2017: uniQure Publishes Data Demonstrating Wide Distribution of AAV5 in the Central Nervous System

Jan 30, 2017: Vybion Huntington's Disease Drug Patent receives Track I Status

Jan 06, 2017: WAVE Life Sciences Provides Update on Lead Huntington's Disease Program WVE-120101

Jan 06, 2017: WAVE Life Sciences Provides Update on Lead Huntington's Disease Program WVE-120102

Aug 24, 2016: Novel Gene Therapy Drug for Huntington Disease

Jun 21, 2016: WAVE Life Sciences Receives Orphan Drug Designation from FDA for its Lead Candidate Designed to Treat Huntington's Disease

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Pipeline by nLife Therapeutics SL, H1 2018

Pipeline by PTC Therapeutics Inc, H1 2018

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COMPANIES MENTIONED

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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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