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Huntingtin - Pipeline Review, H2 2020

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Abstracts

Huntingtin - Pipeline Review, H2 2020

SUMMARY

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.
Huntingtin (Huntington Disease Protein or HTT) pipeline Target constitutes close to 23 molecules. Out of which approximately 20 molecules are developed by companies and remaining by the universities/institutes. The molecules developed by companies in Phase III, Phase II, Preclinical and Discovery stages are 1, 3, 11 and 5 respectively. Similarly, the universities portfolio in Preclinical and Discovery stages comprises 2 and 1 molecules, respectively. Report covers products from therapy areas Central Nervous System and Genetic Disorders which include indications Huntington Disease, Spinocerebellar Ataxia (SCA), Alzheimer's Disease, Kennedy’s Disease (Spinal and Bulbar Muscular Atrophy) and Spinal Muscular Atrophy (SMA).
The latest report Huntingtin - Pipeline Review, H2 2020, 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. It also reviews key players involved in Huntingtin (Huntington Disease Protein or HTT) targeted therapeutics development with respective active and dormant or discontinued projects. The report is built using data and information sourced from proprietary databases,

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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

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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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Sep 25, 2020: uniQure announces recommendations from data safety monitoring board of phase I/II clinical trial of AMT-130 for the treatment of Huntington’s disease
Jun 19, 2020: uniQure announces first two patients treated in phase I/II clinical trial of AMT-130 for the treatment of Huntington’s disease
Jun 03, 2020: Neurobiology of Disease publishes encouraging preclinical results of AFFiRiS’ antibody mAB C6-17 to treat Huntington’s disease
Apr 20, 2020: Ionis and partner announce enrollment completion of global Phase 3 GENERATION HD1 study for Huntington's disease
Feb 27, 2020: Ionis and partner highlight tominersen (formerly known as IONIS-HTTRx and RG6042) data at annual Huntington’s disease drug discovery conference
Feb 27, 2020: uniQure presents multiple new preclinical data on AMT-130 at the CHDI’s 15th Annual Huntington’s Disease Therapeutics Conference
Feb 18, 2020: Wave Life Sciences to present preclinical in vivo and in vitro data for SNP3-targeting Huntington’s Disease program at CHDI Foundation’s 15th Annual HD Therapeutics Conference
Feb 17, 2020: Chugai receives orphan drug designation for RG6042 in Huntington's disease from the MHLW
Dec 30, 2019: Wave Life Sciences announces topline data and addition of higher dose cohort in ongoing Phase 1b/2a PRECISION-HD2 trial in Huntington’s Disease
Dec 19, 2019: uniQure announces publication of preclinical data for AMT-130 in Huntington’s disease supporting non-selective HTT-lowering approach
Sep 27, 2019: uniQure to present update on of AMT-130 at European Huntington Association 2019 Conference
Jul 01, 2019: Sangamo Therapeutics announces Nature Medicine publication detailing the activity of disease allele-selective zinc finger proteins in preclinical models of

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huntington’s disease
May 22, 2019: uniQure strengthens intellectual property portfolio with granted patent claims covering AMT-130 for huntington's disease
May 07, 2019: uniQure announces featured presentations of new data on AMT-130 at the 2019 American Academy of Neurology Annual Meeting
May 06, 2019: Huntington drug successfully lowers levels of disease-causing protein
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