

## ACTIVE SUBSTANCE AND PHARMACEUTICAL COMPOSITION FOR USE IN TREATMENT OF HUNTINGTON'S DISEASE

### Market

Huntington's Disease (HD) is classified as a genetic neurodegenerative disorder. Incidence of disease varies worldwide, with reported prevalence rates of 1 in 15 000 people in developed countries. HD is a serious social and economic burden, mainly due to high costs of care, amounting to approximately 25 000 euro per person per year.

According to report Drug Discovery Services Market by Process (Target Selection, Hit-to-Lead Identification, Lead Optimization), Type (Medicinal Chemistry, Biology Services, DMPK), Drug Type (Small Molecules, Biologics), Therapeutic Areas (Oncology, Neurology) - Forecast to 2022 medicine market is estimated expand to \$ 14,4 billion by 2022.

### Technology

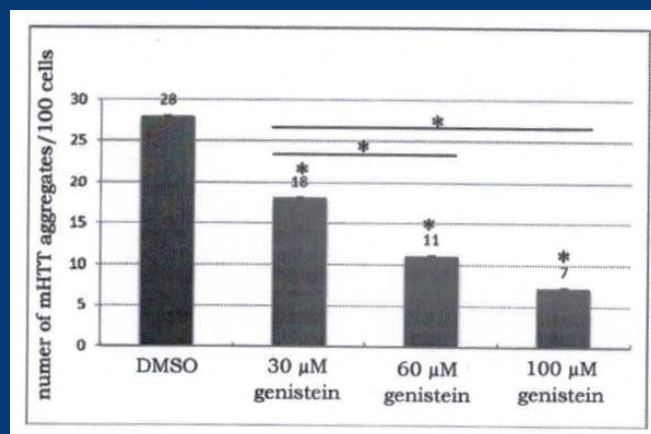
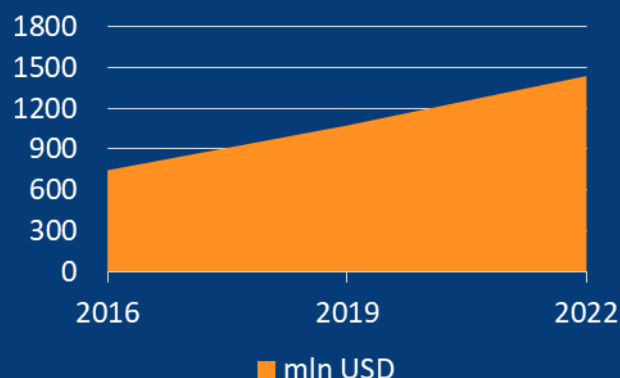
Genistein 5,7-dihydroxy-3-(4-hydroxyphenyl)-4H-1-benzopyran-4-one, i.e. a compound from isoflavone group naturally occurring in plants in free form as aglycone or more often glycosidically bound (genistein) causes a reduction in level of mutant form of huntingtin in cultures of human cells.

Invention relates to medical use of 5,7-dihydroxy-3-(4-hydroxyphenyl)-4H-1-benzopyran-4-one in manufacture of medicament that actively inhibits size and number of protein aggregates.

### Market forecast of medicinal products by 2022



Market of medicinal products.



Number of huntingtin aggregates in human embryonic kidney cells transfected with plasmid pEGFP-Q74 (bearing in 1st exon of the HTT gene with 74 CAG repeats), treated with genistein (30, 60 and 100 μM) or DMSO (control cells), in a volume equal to that of genistein, for 48 h.

## Technology highlights

- 1 5,7-dihydroxy-3-(4-hydroxyphenyl)-4H-1-benzopyran-4-one has medical use for manufacture of medicament that reduction in multiplicity and number of protein aggregates.
- 2 Conducted studies have demonstrated positive effect of genistein in form of increased cell survival.
- 3 Described method causes reduction level of mutant form of huntingtin in cultures of human cells.

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## Commercialization opportunities



- ➔ Licensing agreement
- ➔ Transfer of ownership
- ➔ Spin off

## IP Status



The invention was submitted for patenting according to Polish (P.417983) procedures.

## Implementation progress



TRL 4  
Technology validated in laboratory conditions

## Summary

Huntington's Disease (HD) is classified as a genetic neurodegenerative disorder. The incidence of the disease varies worldwide, with reported prevalence rates of 1 in 15 000 people in developed countries. HD is a serious social and economic burden, mainly due to high costs of care, amounting to approximately 25,000. euro per person per year.

Direct causes of the disease can be traced to a mutation in the gene encoding the huntingtin protein. Mutated proteins tend to form extensive aggregates in cells, leading to neuronal dysfunction and progressive neurodegeneration in certain areas of the brain, with clinical effects including motor coordination disorders and dementia.

Despite ongoing research, no effective treatment for Huntington's disease is currently available. Existing pharmacological therapies and physiotherapies primarily serve to alleviate the symptoms of the disease.

Invention described herein allows for 5,7-dihydroxy-3-(4-hydroxyphenyl)-4H-1-benzopyran-4-one to be used as an active ingredient of novel formulation, which causes a reduction in the size and number of protein aggregates and a decrease in the total number of mutated huntingtin forms. This in turn may result in significant slowdown or even complete inhibition of Huntington's disease progression.

## Technology Transfer Office



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