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A company of the LifeScience Foundation

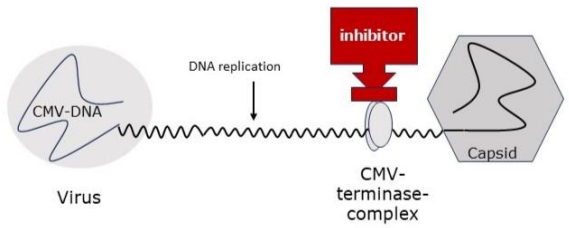
REFERENCE NUMBER
TO 02-00402

NOVEL INHIBITORS OF CYTOMEGALOVIRUS

Keywords: small molecule, first-in-class, CMV, hCMV, solid organ transplantation, SOT, hematopoietic stem cell transplantation, HSCT

INVENTION NOVELTY
Provided are novel, first-in-class, simple to manufacture small molecule inhibitors of cytomegalovirus (CMV) with a new mechanism of action and advantageous toxicological and pharmacological properties as drug candidates for the treatment of CMV infections.

VALUE PROPOSITION
Infection with CMV is very common worldwide, usually does not cause serious health problems, and accordingly, often remains unnoticed. Nevertheless, the virus persists in the body and can easily be transferred throughout life. Transmission of the virus happens via contact with body fluids including breast milk, as well as during pregnancy or via transplanted organs or hematopoietic stem cells. Especially babies infected prenatally often suffer from severe disorders. But also infection of newborn infants or people with a weak or suppressed immune system can turn into serious disease. Although there are drugs available for treatment of CMV infection, these are afflicted with serious adverse side effects or lost efficacy due to development of resistance. Thus, novel treatment options for CMV infections are needed.



Possible mechanisms and pathways of human cytomegalovirus (HCMV) infection in spiral ganglion neurons (SGN)

TECHNOLOGY DESCRIPTION
The invention relates to highly active, highly specific small molecules with a novel structure and new mechanism of action. Starting from a hit compound identified by screening of a compound library a series of novel derivatives were generated. The potential drug candidates show advantageous toxicological and pharmacological properties and can be produced at low cost.

COMMERCIAL OPPORTUNITY
Application as antiviral drug for prophylaxis of hematopoietic stem cell transplantation (HSCT) and solid organ transplant (SOT) recipients at risk of clinically significant CMV infection, particularly for treatment of HCMV infections refractory or resistant to currently used antiviral drugs. The technology is offered for co-development and/or licensing.

DEVELOPMENT STATUS
ADME/Tox data obtained *in vitro* and pharmacokinetic data in a mouse model are available. The molecular target is known. The compounds break Letermovir and Ganciclovir resistance; the frontrunners exceed Letermovir's potency also in Letermovir-sensitive HCMV.

PATENT SITUATION
European priority application was filed in February 2023 (EP23 157 076.3).

FURTHER READING
Khawaja et al. 2023. Cytomegalovirus infection in transplant recipients: newly approved additions to our armamentarium. Clin Microbiol Infect. 2023 Jan;29(1):44-50. doi: 10.1016/j.cmi.2022.07.001.

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