

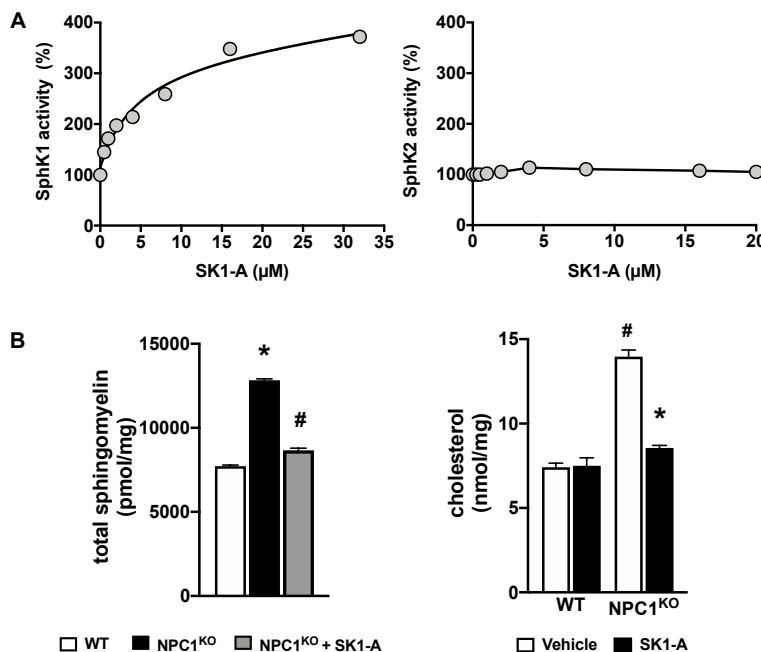
A Novel Treatment for Niemann-Pick Disease

Increasing SphK1 activity to prevent cell death

In Niemann-Pick Type C Disease, the sphingosine kinase 1 enzyme (SphK1) is less active, resulting in a buildup of sphingolipids and cholesterol within the cell leading to cell death. Currently, no treatments exist for lysosomal storage diseases like Niemann-Pick Type C that treat both the sphingolipid and cholesterol buildup that occurs and results in cell death. Recent studies at Virginia Commonwealth University have determined that activation of SphK1 can ameliorate sphingolipid and cholesterol accumulation in lysosomal storage diseases such as Niemann-Pick Type C.

The technology

Researchers at Virginia Commonwealth University have developed a novel compound, known as SK1-A, that specifically activates SphK1. Laboratory testing determined that SK1-A, without impacting SphK2 activity, was able to activate SphK1 and can be seen in part A of the figure below. By doing so SK1-A returned intracellular sphingolipid and cholesterol levels in diseased cells to non-diseased levels, rescuing the diseased phenotype which can be seen in part B of the figure below. Thus, this compound could be developed as a potential treatment option for conditions associated with reduced activity of SphK1, such as Niemann-Pick Type C Disease. This compound will also enable researchers to further study the role of SphK1 in disease pathogenesis.



Benefits

- » Significant reduction in the accumulation of sphingolipid and cholesterol levels in diseased cells
- » Specifically targets SphK1
- » Wide separation between therapeutic and side effect doses

Applications

- » Niemann-Pick Type C Disease
- » Other Lysosomal storage disorders

Patent status:

Patent pending: U.S. and foreign rights are available.

License status:

This technology is available for licensing to industry for further development and commercialization.

Category:

Biomedical

VCU Tech #:

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Investigators:

[Sarah Spiegel](#), PhD
[Jason Newton](#), PhD
[Santiago Lima](#), PhD

Contact us about this technology

Magdalena K. Morgan, Ph.D.
Director of Licensing
mkmorgan@vcu.edu
(804) 827-6095