

Development of lead compounds as a sphingomyelin synthase 1 inhibitor to reduce the hepatocyte lipotoxicity for the treatment of liver cirrhosis

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METABOLIC	Hit
Product Type	Chemical - Small Molecule
Indication	1st indication: Liver Cirrhosis 2nd indication: Non-alcoholic Steatohepatitis (NASH)
Target	Sphingomyelin Synthase 1 (SMS1)
MoA(Mechanism of Action)	In fatty liver models including NASH, overexpression of SMS1 elevates DAG level. As a result, phosphorylation of protein kinase C δ and activation of NLRC4 lead to pyroptosis. At the same time, NLRP3 inflammasome in Kupffer cell is activated and give rise to liver cirrhosis.
Competitiveness	First in Class <ul style="list-style-type: none"> • Control progression of incurable liver cirrhosis • Unique positioning strategy while most global pharma companies are targeting NASH
Development Stage	Hit
Route of Administration	P.O.
Current Progress	<ul style="list-style-type: none"> • In vitro and in silico screening are in progress. • Lead scaffolds as selective inhibitors are identified and validated. • In vivo validation is in progress.