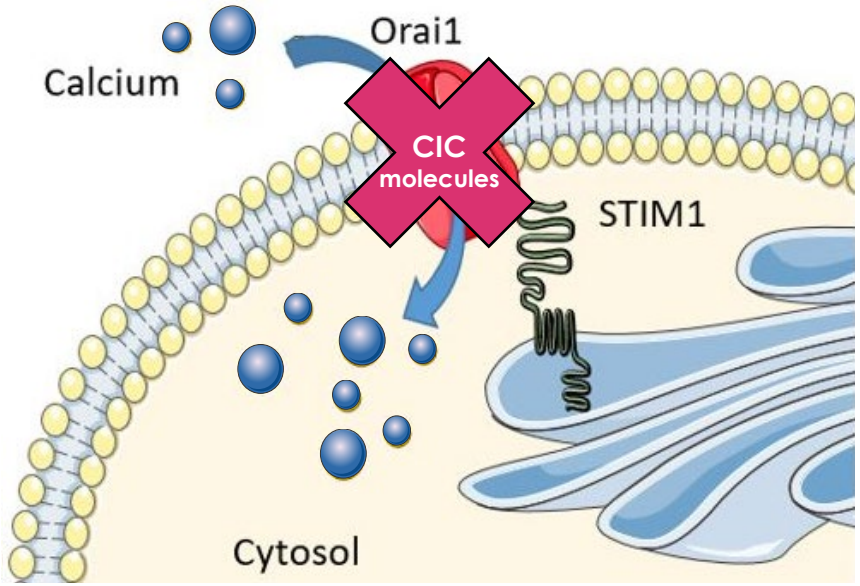
Solution: novel SOCE inhibitors

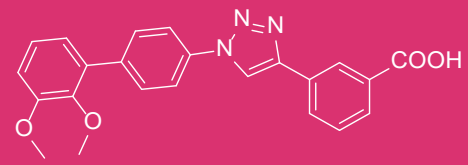
Innovative mechanism of action



2 novel chemical families of small molecules

- IP protected
- High selectivity
- Good safety & PK profile
- Easy manufacturing
- High potency (nM) range

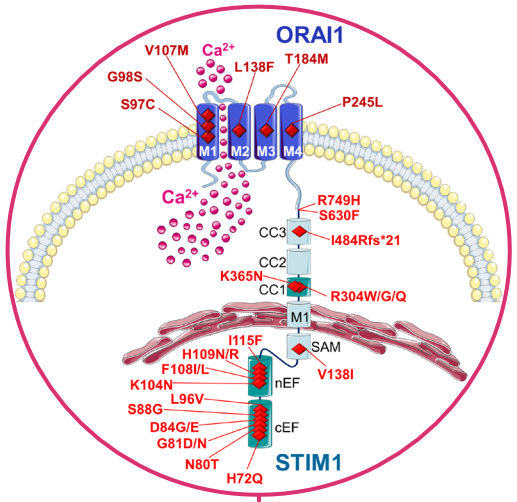
CIC-39



Target pathologies

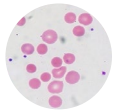
Tubular aggregate myopathies

Tubular aggregate myopathy, Stormorken syndrome
York platelet syndrome

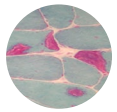


SOCE over-activation

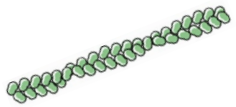
Thrombocytopenia
Abnormal Bleeding



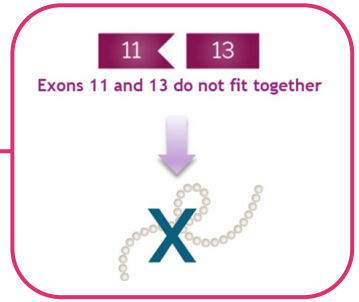
Muscle stifness
Painfull Cramps



Duchenne muscular dystrophy



Cytoskeleton disorganization



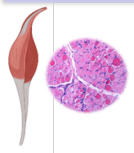
Absence/Reduction of dystrophin

SOCE over-activation

Apoptosis and Necrosis

Muscle damage
Functional impairment

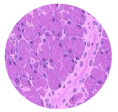
Duchenne muscular dystrophy (DMD)



- Large calf muscles
- Muscle pain and stiffness
- Frequent falls
- Trouble running and jumping
- Learning disabilities
- .....

Acute pancreatitis

SOCE over-activation in pancreatic acinar cells (PACs)



Intracellular Ca^{2+} overload in PACs

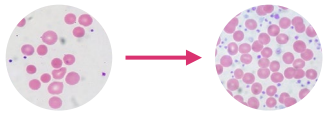
Uncontrolled release of intracellular digestive proenzymes

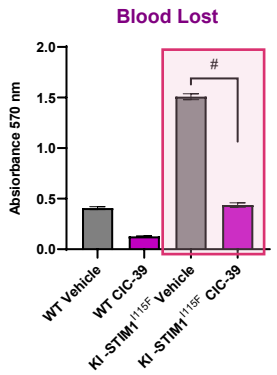
Suden and painfull inflammation of pancreas and peri-pancreatic organs

Target pathologies – Scientific Results

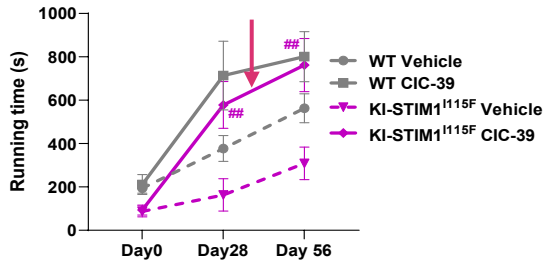
Tubular aggregate myopathies

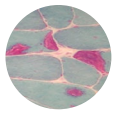
Reducing blood loss
CIC-39: 60 mg/Kg/daily 15 days





Contrasting muscle damage
CIC-39: 60 mg/Kg/daily 56 days





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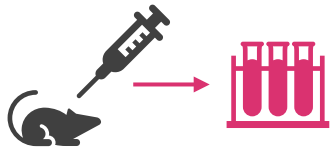
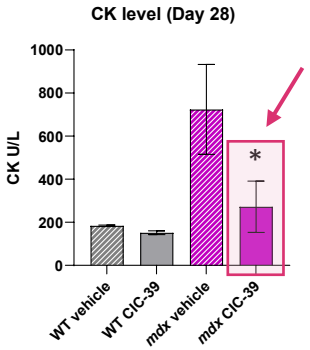
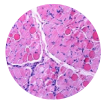
Duchenne muscular dystrophy

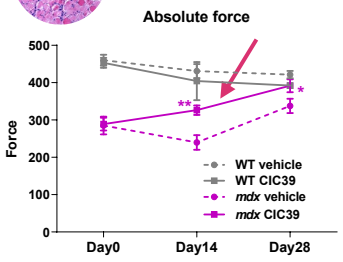
Reduction of creatine kinase plasma levels
CIC-39: 60 mg/Kg/daily 28 days

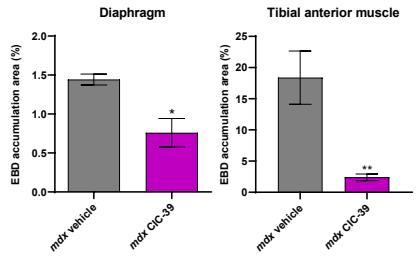




Contrasting muscle damage & reducing apoptosis
CIC-39: 60 mg/Kg/daily 28 days







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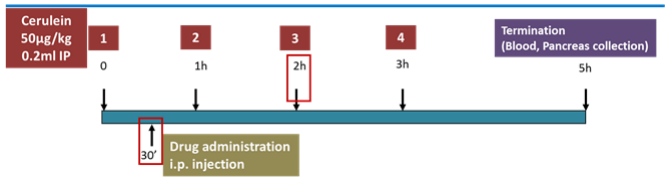
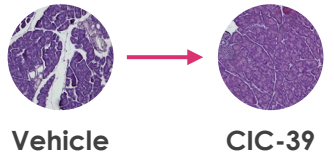
Acute pancreatitis

Experimental protocol



Restoring pancreatic tissue edema
CIC-39: 10 mg/Kg (x2) IP administration



Reducing inflammation
CIC-39: 10 mg/Kg (x2) IP administration