

# Pharmacological activation of AMPK as a therapeutic approach for FBXL4-related Mitochondrial DNA Depletion Syndrome (MTDPS13)

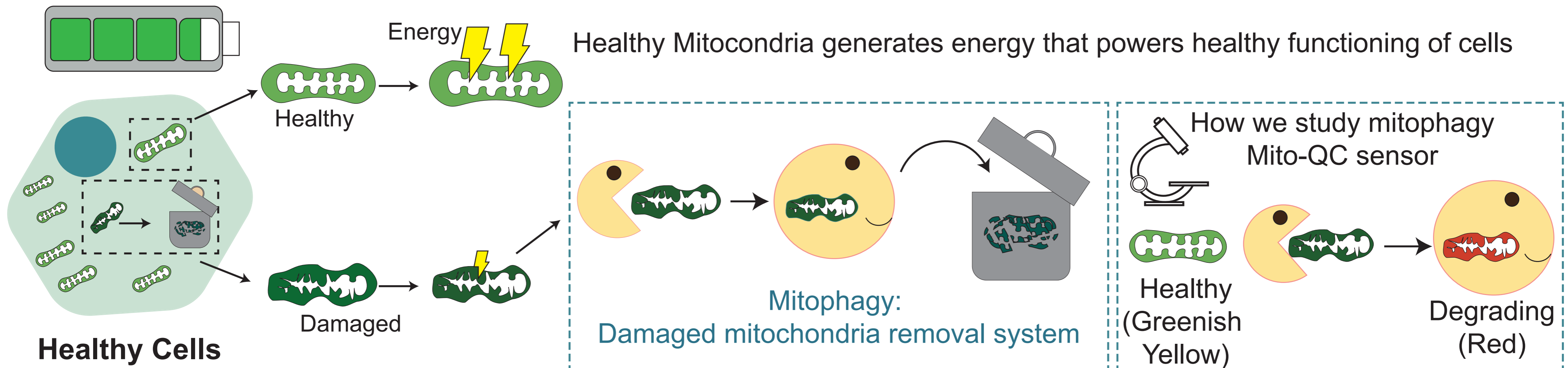
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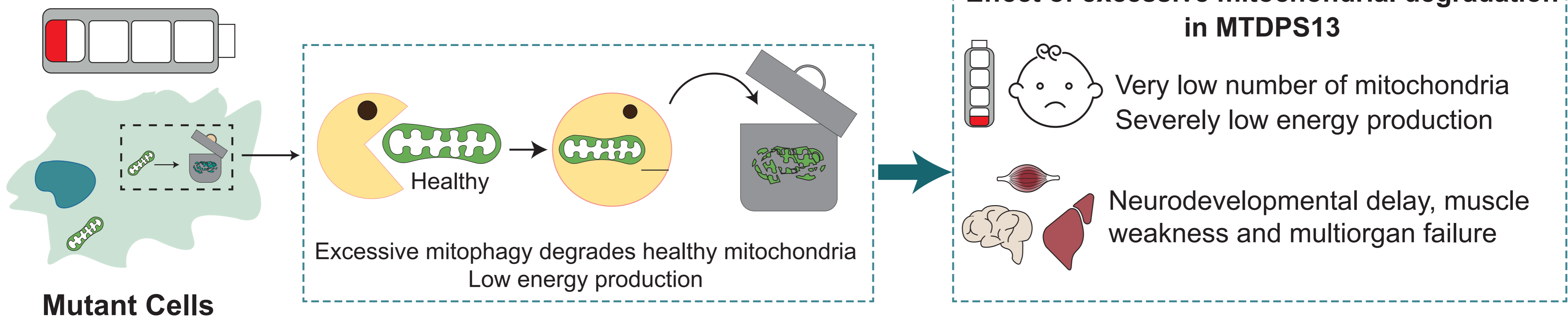


## Quality control of mitochondria, the powerhouse of the cell



## Excessive mitochondrial degradation causes MTDPS13

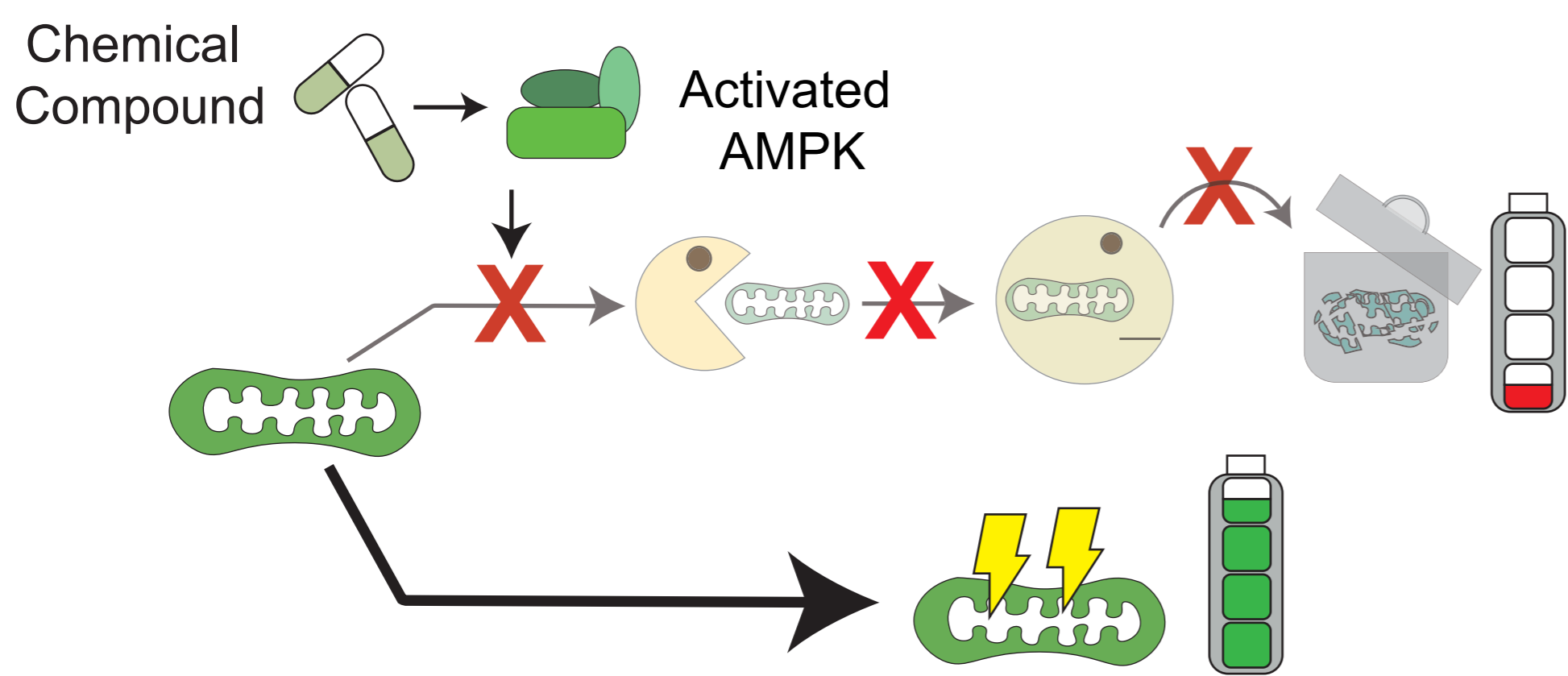
MTDPS13 is a devastating disease caused by the FBXL4 gene mutation



**Half of the newborns with MTDPS13 barely survive till 3 years of age. No treatment currently available**

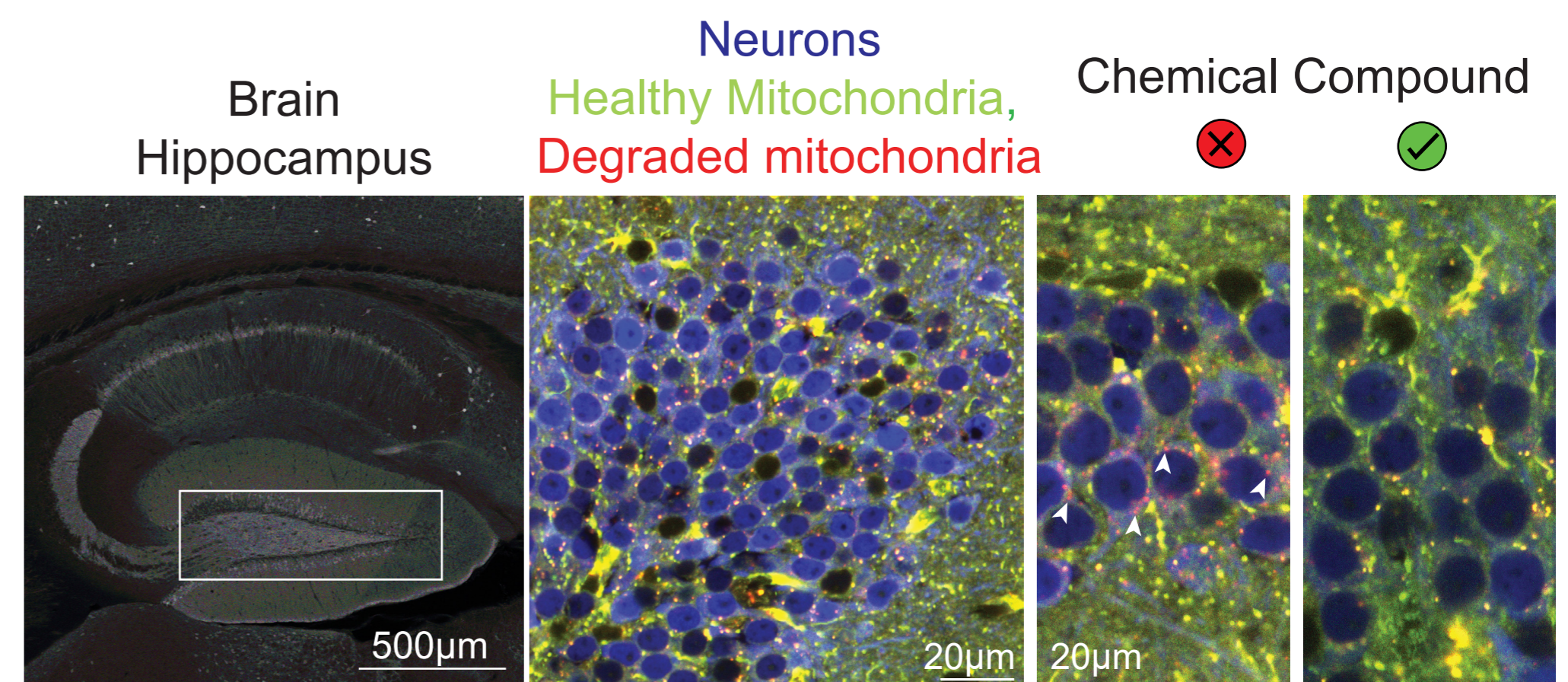
## Our Solution: Activate Energy Sensing protein AMPK

We recently uncovered that AMPK activation blocks degradation of healthy mitochondria

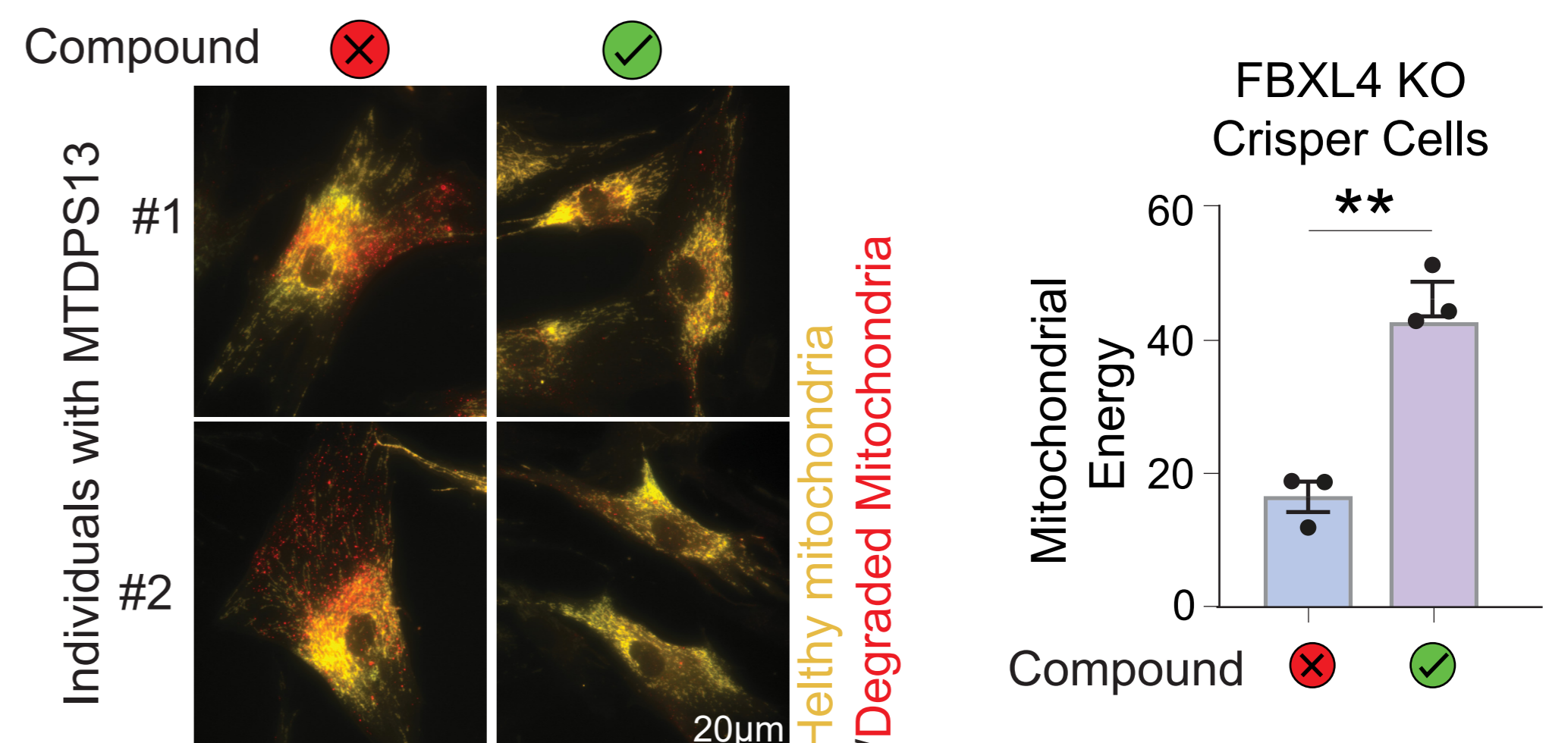


## Key Results

AMPK activation reduces mitophagy in MTDPS13 mice model

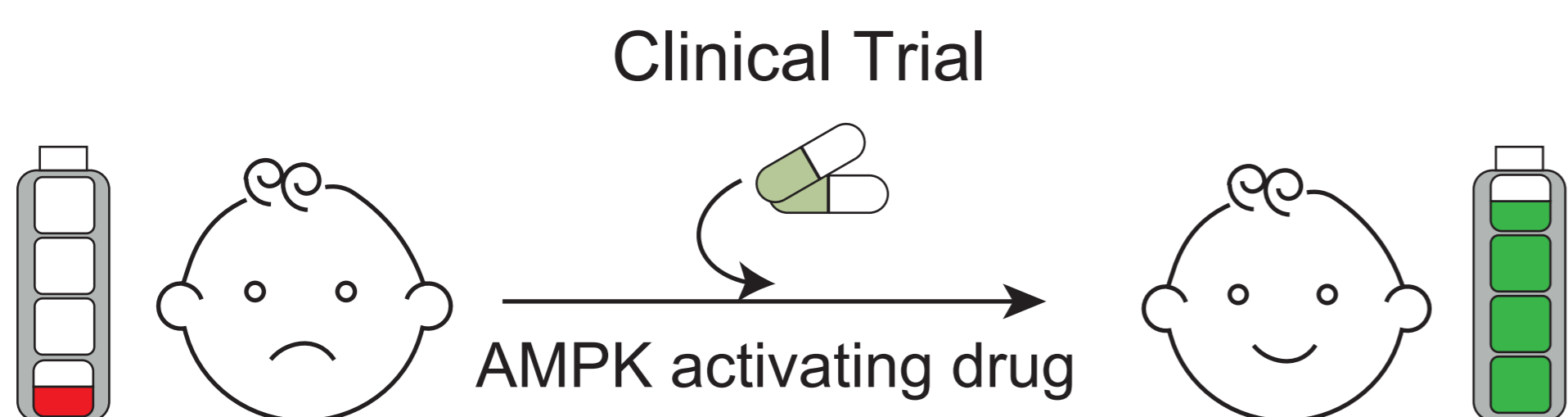


AMPK activation reduces mitophagy in cells of individuals affected with MTPDS1 and energy levels in cell model



## Summary and Future Plan

Our data suggests AMPK activation restores mitochondrial level in cell line, mouse model and patient derived cells



**Our findings provide strong evidence to form the basis of the first effective treatment for MTDPS13, offering new hope for affected children and their families.**