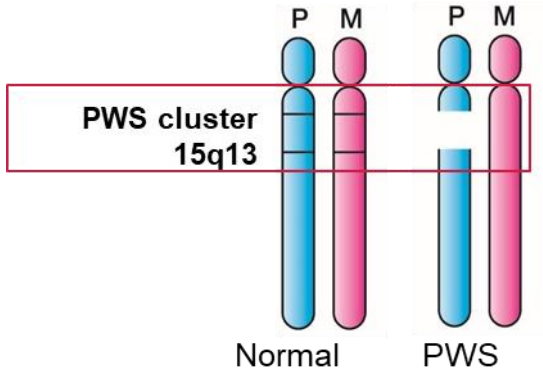
SMCHD1 inhibition to treat Prader-Willi Syndrome (PWS)

The Problem

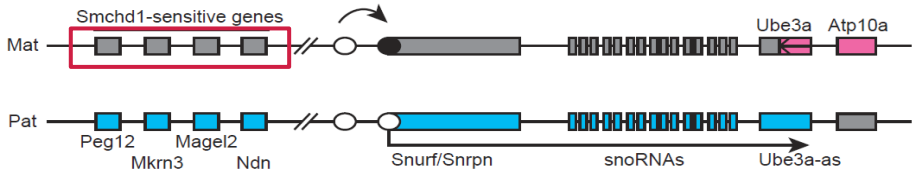
- PWS is a genetic disorder that causes developmental delays, cognitive disabilities and an insatiable appetite leading to obesity.
- PWS symptoms stem from a defect in the hypothalamus of the brain. Symptoms are identified at infancy and are life-long.
- This genetic disorder is caused by failure to express critical PWS genes in a paternal allele.
- Current treatments target some symptoms; however, no treatment targeting the genetic cause is available.



The Solution

- Almost all PWS patients have a functioning set of PWS genes on the maternal side. SMCHD1 is an epigenetic regulator that switches off the maternal PWS genes.
- We have shown that SMCHD1 deletion in human neural stem cells reactivates PWS critical genes in the maternal allele but does not affect other SMCHD1 targets.
- Our goal is to develop a small molecule clinical candidate to address the genetic cause of PWS.

Mouse PWS cluster



Our Program

- Only active program to undertake this approach, which has the potential to effectively treat multiple symptoms of this syndrome by targeting the cause.
- Chemistry: Two hit series identified and validated. Medicinal chemistry program currently in progress.
- Biology: Our compounds recapitulate SMCHD1 deletion and show expression of key PWS genes.

Seeking ***license/partnership/investment***

Our Team

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