# The Current State of Rett Research

雷特研究当前状态

The last few years has brought unprecedented attention to Rett Syndrome by academic scientists, researchers and executives of pharmaceutical and biotech companies and more recently, life sciences investors. The interest stems from the advantages enjoyed by the Rett field: a known and single genetic cause, expectation of reversibility, NIH funded natural history study and Rett clinics with significant patient populations.

在过去的几年里，越来越多的科学家、研究人员、制药业高管，生物科技公司，特别是生命科学投资者关注了雷特综合症。研究人员之所以感兴趣是因为雷特综合症研究的独特优势。这一优势包括了雷特综合症是由以知单基因引起的，可逆转的中枢神经系统疾病。该疾病还得到了美国国家卫生研究院（NIH）提供的资金研究支持。此外还有大量等待临床实验的雷特病患者。

The graphic below encapsulates the various approaches that are currently being pursued.

以下图标表囊括了当前正在进行的几项治疗措施研究。

Since Rett Syndrome is caused by defective MECP2 it stands to reason that approaches that attack the root cause, MECP2 itself, hold the greatest promise of significantly improving symptoms. Therefore approaches such as activating the silent MECP2, gene therapy and protein replacement can be classified as potentially curative approaches.

由于雷特综合征是由 MECP2 单一基因突变导致的，从而靶向 MECP2 的治疗方法为改善症状并最终攻克雷特综合提供了希望。因此激活处于静默状态的 MECP2，基因治疗和蛋白质替换治疗均成为潜在的有效治疗措施。

On the other hand therapeutic interventions that are downstream of MeCP2 will likely improve a symptom or subset of symptoms. These approaches are treatments rather than cures.

另外对 MECP2 下游的治疗干预将很可能改善一个或多个症状，这种方法属于改善型治疗而不能达到根本治愈的目的。

## Current Project

## 当前项目

### CLINICAL TRIALS & STUDY

- 临床试验和研究

### CONSORTIA

- 联盟合作

### DNA/RNA/PROTEIN THERAPY

- 脱氧核糖核酸/核糖核酸/蛋白质治疗

### MODIFIER GENES

- 修饰基因

### DOWNSTREAM TARGET

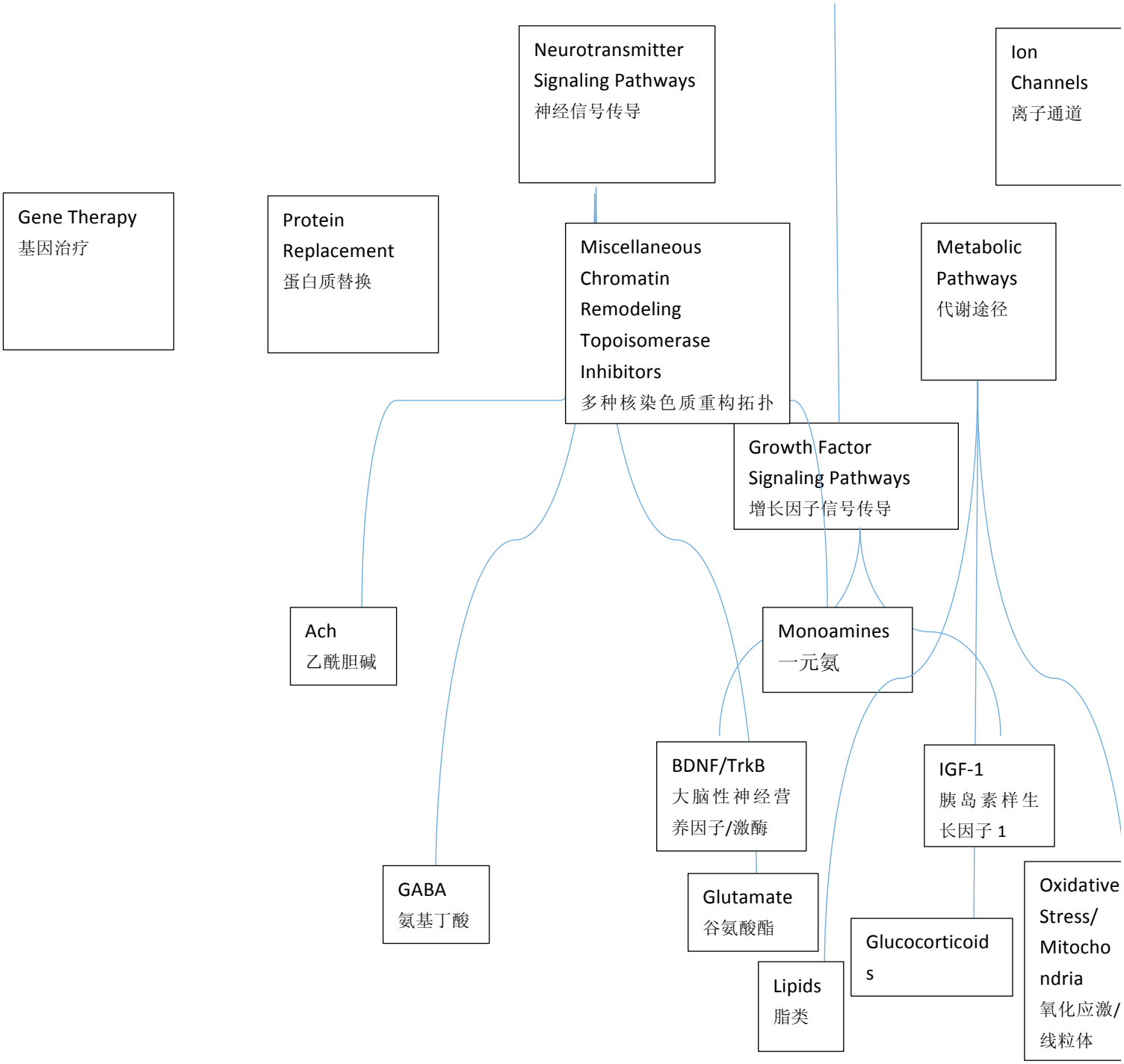
- 下游标靶

### MECP2 DUPLICATION

- MECP2 基因复制

## CHART OF TREATMENTS & STRETIGIES

## 治疗战略图


Neurotransmitter Signaling Pathways
神经信号传导
Ion Channels
离子通道
Gene Therapy
基因治疗
Protein Replacement
蛋白质替换
Miscellaneous
Chromatin Remodeling
Topoisomerase Inhibitors
多种核染色质重构拓扑
Metabolic Pathways
代谢途径
Growth Factor Signaling Pathways
增长因子信号传导
Ach
乙酰胆碱
Monoamines
一元氨
BDNF/TrkB
大脑性神经营养因子/激酶
IGF-1
胰岛素样生长因子 1
GABA
氨基丁酸
Glutamate
谷氨酸酯
Glucocorticoids
Lipids
脂类
Oxidative Stress/ Mitochondria
氧化应激/线粒体