ANGELMAN SYNDROME THERAPEUTICS PIPELINE*

**Discovery**
- Finding Potential Therapies/Drugs

**Pre-Clinical Development**
- Testing in Laboratory

**IND Filed**
- Investigational New Drug; FDA Filing

**Phase I**
- Human Safety Trial

**Phase II**
- Human Safety and Efficacy Trial

**Phase III**
- Definitive Trial

**NDA Filed**
- New Drug Application; FDA Filing

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**FINAL STAGE - AVAILABLE TO PATIENTS**

- **Improve Tonic Inhibition**
  - OV-101
    - Ovid

- **Symptomatic**
  - SAGE-324
    - Sage (seizures, myoclonus)
  - Ketone Esters
    - Disruptive Nutrition (seizures)

- **Improve Synaptic Function**
  - NNZ-2591
    - Neuren (motor and cognitive function)

- **Activate Paternal UBE3A**
  - Topoisomerase Inhibitors
    - UNC
  - Ionis/Biogen, Roche
    - GeneTX/Ultragenyx
  - CRISPR
    - UNC, UC Davis
  - shRNA
    - UConn/UTSW
  - AAV-Based Approaches
    - PTC Therapeutics, UPenn, UNC, Bamboo/Pfizer

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*This is a list of therapeutics under development that ASF is aware of to date. Other therapeutics may be under development.*