ANGELMAN SYNDROME THERAPEUTIC PIPELINE

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

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

**Improve Synaptic Function**
- NNZ-2591 by Neuren
- LB-100 by Lixte

**Activate Paternal UBE3A**
- Topoisomerase Inhibitors: UNC - AskBio
- Antisense oligonucleotides (ASO): IONIS, GeneTx/Ultragenyx, Roche/Genentech
- CRISPR: UNC - AskBio, UC Davis
- shRNA: UConn-OVID/UTSW-Taysha
- Small Molecules: UNC-Pinnacle Hill

**Gene Therapy**
- AAV-Based Approaches: PTC therapeutics, UPenn, UNC - AskBio, Bamboo/Pfizer, StrideBio/Sarepta