### SMA Drug Pipeline

We're funding and directing research with more breadth and depth than ever before. We know what we need to do to develop and deliver new therapies, which could also work in combination, to reach our goal of treatments for all ages and types. And we're on the verge of further breakthroughs that will continue to change the course of SMA, and eventually lead to a cure.

**Organizations/Drug Name or Approach**

- Biogen/Ionis-Spinraza
- Novartis-Zolgensma (IV)
- Roche-Genentech/GYM329 (Muscle Directed)
- Biohaven-Taldefgrobep alfa (BHV-2000)
- NMD Pharma-NMD-670
- Alnylam Pharma-MXRNA
- Biogen - Higher Dose Nusinersen
- Columbia/NU-p38a MAPK Inhibitor
- Monani-Modifier Program
- Meriney-Calcium Channel Modifier
- Patten-Zebrafish Screen
- Jablonka-Calcium Channel Modifier
- Voyager Therapeutics: AAV Gene Therapy
- Praxis Biotech-Protein Synthesis Enhancers
- Scholar Rock - Aplegrumab (SRK-019) [Muscle Directed]
- Novartis-OAV101 (AXS 101) IT
- Biogen - Higher Dose Nusinersen
- Roche-Genentech/GYM329 (Muscle Directed)
- Biohaven-Taldefgrobep alfa (BHV-2000)
- NMD Pharma-NMD-670
- Alnylam Pharma-MXRNA
- Biogen - Higher Dose Nusinersen
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- Jablonka-Calcium Channel Modifier
- Voyager Therapeutics: AAV Gene Therapy

**BLA** = Biologics License Application  **IND** = Investigational New Drug  **NDA** = New Drug Application  **Last updated: April 2023**