

INDUSTRY COLLABORATION

YEAR END REPORT


2023

Established in 2016, the SMA Industry Collaboration (SMA-IC) is a multi-faceted partnership that brings together pharmaceutical companies, Cure SMA, and other nonprofit organizations to share information, ideas, and data. The SMA-IC works together to address scientific, clinical, and regulatory topics critical to advancing drug development in spinal muscular atrophy (SMA). It is currently comprised of our partners at Scholar Rock, Biogen, Biohaven Pharmaceuticals, Genentech/Roche, Novartis, Alcyone Therapeutics, NMD Pharma, and SMA Europe.

Cure SMA would like to thank the members of the SMA community for your continued support. No single group can research and develop a treatment for SMA alone—it requires collaboration between academics, industry, government, and the SMA community.

During Phase 8, the SMA-IC remained steadfast in our approach to ensure developing treatments address the unmet needs of the SMA community; research efforts include:

SURVEY AND FOCUS GROUP PARTICIPATION



874 Individuals Affected by SMA

927 Caregivers

62 Healthcare Providers

PROVIDER AND RESEARCHER ENGAGEMENT



7 Poster Presentations



CURE SMA COMMUNITY OUTREACH

4 Latest News Posts

Article in the Spring Issue of Directions Newsletter

Article in the Winter Issue of Directions Newsletter

ENGAGEMENT WITH THE FDA VIA A PATIENT-LED LISTENING SESSION

COLLABORATION WITH ACADEMIC RESEARCHERS

Pediatric Neuromuscular Clinical Research Network (PNCRN)

- SMA-PRO
- ATEND
- Bulbar Assessments for Voice/Speech, and Swallow
- SMA-Functional Composite Score (SMA-FC)

PEER REVIEWED PUBLICATIONS

5 Published Articles

8 Manuscripts in Peer Review / in Development