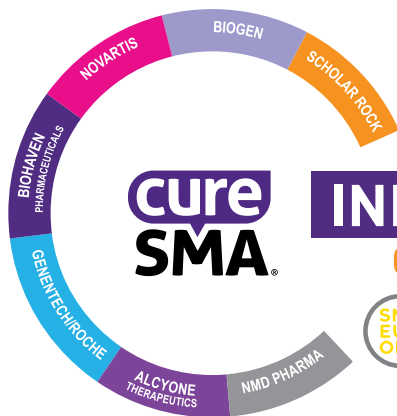




INDUSTRY COLLABORATION



YEAR END REPORT

2024

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 Biogen, Novartis, Scholar Rock, Genentech/Roche, argenx, 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 9, the SMA-IC remained steadfast in our approach to ensure developing treatments address the unmet needs of the SMA community; research efforts include:



SURVEY PARTICIPATION

509 Individuals Affected by SMA

535 Caregivers

PEER REVIEWED PUBLICATIONS

2 Published Manuscripts

10 Manuscripts in Peer Review / in Development

CAMPAIGN TO SUPPORT COMMUNITY ACCESS AND AWARENESS OF SMA CLINICAL TRIALS

5070 Engagements with the Cure SMA Clinical Trials Page and Resource Handouts

6670 Engagements with the SMA Registry and Clinical Trial Finder

PROVIDER AND RESEARCHER ENGAGEMENT



Poster
Presentations

COLLABORATION WITH ACADEMIC RESEARCHERS

Adult & Pediatric Neuromuscular Clinical Research Network (APNCR)

- SMA-PRO
- ATEND
- SMA-Functional Composite Score (SMA-FC)

PUBLICATION OF UPDATED CURE SMA CLINICAL TRIAL READINESS PROGRAM TOOLKITS

Basics of SMA and the Effective Clinical Trial Conduct

Best Practices for Clinical Research Coordinators in SMA