



INDUSTRY COLLABORATION

YEAR END REPORT

2021

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 Novartis Gene Therapies, Biogen, Genentech/Roche Pharmaceuticals, Scholar Rock, 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 6, the SMA-IC remained steadfast in our approach to ensure developing treatments address the unmet needs of the SMA community; research efforts include:

PEER REVIEWED PUBLICATIONS

3 Published Articles **9** Manuscripts in Peer Review / in Development



SURVEY PARTICIPATION

406 Individuals Affected by SMA
669 Caregivers
40 Healthcare Providers

COLLABORATION WITH ACADEMIC RESEARCHERS

Pediatric Neuromuscular Clinical Research Network (PNCRN)

- STEP-IN SMA Training Program
- SMA-PRO
- ATEND

University of Rochester

- SMACR-HI

PROVIDER AND RESEARCHER ENGAGEMENT

12 Poster Presentations
228 attendees of the Clinical Trial Readiness Program Webinar Series
6,742 Provider Engagements at Professional Organization Conferences (APTA, NDTA, AAN, AAP)

PUBLISHED TOOLKITS

SMA Europe - Spinal Muscular Atrophy: Pathology, Diagnosis, Clinical Presentation, Therapeutic Strategies and Treatments

Cure SMA Clinical Trial Readiness Program Toolkit Updates

CURE SMA COMMUNITY OUTREACH

- **5** Latest News Posts
- Articles in the Spring and Summer Issues of *Directions* Newsletter
- Dedicated Issue of *Compass* Newsletter

