



INDUSTRY COLLABORATION

YEAR END REPORT

2022

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, Genentech/Roche Pharmaceuticals, Scholar Rock, Novartis Gene Therapies, Biohaven Pharmaceuticals, Epirium Bio, 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 7, 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



1097 Individuals Affected by SMA
355 Caregivers
60 Healthcare Providers

ENGAGEMENT WITH THE FDA VIA A PATIENT-LED LISTENING SESSION

PEER REVIEWED PUBLICATIONS

5 Published Articles
6 Manuscripts in Peer Review / in Development

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)

University of Rochester

- SMACR-HI



CURE SMA COMMUNITY OUTREACH

7 Latest News Posts
Article in the Spring Issue of Directions Newsletter

PROVIDER AND RESEARCHER ENGAGEMENT

9 Poster Presentations

1178
 total views of video series entitled

'The Identification and Evaluation of Suspected Spinal Muscular Atrophy (SMA) in Children and Adolescents During a Telehealth Appointment'