Academic and private researchers identified promising areas of SMA research that required basic research seed funds to further explore the proof of concept.

Cure SMA provided basic research and drug development seed funding for academic and private SMA research proposals that have led to all three SMA FDA-approved treatments.

Researchers leverage the Cure SMA seed grants and learnings to win competitive basic research grants at the National Institutes of Health (NIH) to further develop their SMA research.

Positive findings from the Cure SMA/NIH funded basic research in SMA are published in peer review journals, which attracts industry interest and funds.

Industry partners acquired Cure SMA/NIH funded basic research projects to continue to the next phase, investing in and managing the research through clinical trial phases (1, 2, and 3).

Industry submitted New Drug Applications to the FDA and won approval following fast-tracked rare disease authority created by Congress.

The Result of Public-Private Action & Investment into SMA Research

U.S. Food and Drug Administration Approves Therapies to Treat Spinal Muscular Atrophy

Approved on December 23, 2016

Approved on May 24, 2019

Approved on August 7, 2020