

FROM RESEARCH IDEA TO APPROVED TREATMENT

Researchers Develop Promising Research Idea

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 Funds Early Basic Research

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

Cure SMA Funded-Research Secures Secondary NIH Research Grants

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

Researchers Publish Findings in Peer Review Articles in Journals

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 License & Invest in Research Begin Clinical Trial Phases

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 Submits Drug Application FDA Reviews and Approves SMA Drug

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







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Approved on May 24, 2019

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