

Families of SMA Research 25 Year Report



Jill Jarecki Ph.D. Research Director
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FSMA has Funded \$50 Million in SMA Research

- Basic Research to understand the disease.
- Drug Discovery / Translation Research to find therapies.
- Clinical Trials to test drugs.

Basic Research

- FSMA has funded 150 research grants at over 70 institutions.
- **Our funding has helped lead to the following discoveries:**
 - Identification of SMN protein and its role in cells.
 - Discovery of the back-up gene SMN2.
 - Development of multiple SMA animal models.
 - Identification of HDAC inhibitors as drug candidates.
 - Demonstration that cellular replacement therapy leads to functional benefit in rats with motor neuron disease.

Drug Discovery Translational Research

- Our Goal is to build a pipeline of SMA drugs:
 - A new drug candidate is available if another fails.
 - Diversity of approaches increases success.
- In 2000 FSMA funded the first ever industrial SMA drug discovery program

Families of SMA Therapeutic Pipeline

- FSMA is currently funding 3 distinct drug discovery programs:
 - Quinazolines to boost SMN2 expression.
 - Tetracyclines at Paratek Pharmaceuticals to correct SMN2 splicing.
 - Motor neuron replacement program at California Stem Cell and UCI.
- **These three programs represent an investment of over \$16 Million so far to build a SMA drug pipeline.**

Clinical Research Project Cure SMA

- In 2001 FSMA single handedly set-up the clinical trial network Project Cure SMA:
 - 7 clinical sites in North America, 2 affiliate sites in Argentina and Germany.
 - FSMA funds the entire infrastructure to run a clinical trial, including over 30 professionals.
- **Accomplishments:**
 - \$6 Million invested to date.
 - Project Cure SMA has conducted 5 clinical trials on repurposed drugs.

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