

Since 1984, our legacy is one of unparalleled research progress and patient support, from grassroots fundraising to global scientific leadership. FSMA has funded over \$50 million in SMA research. We have funded more than 140 Basic Research Grants at 70 institutions around the world which have led to the promising new therapies now in development.

FSMA is dedicated to creating a treatment and cure by funding and advancing a comprehensive research program. Our research leadership consistently encourages others in industry and government to invest in SMA research. Our broad research-funding program has three distinct parts:

## 1. Basic Research (to unravel the biology of SMA)

Over the last 25 years FSMA research funding has enabled many critical SMA breakthroughs:

- Mapping and cloning of the SMA gene, SMN1.
- Identification of the SMN protein and its roles in the cell.
- Discovery of the back-up SMA gene, SMN2, that provides a unique and straightforward approach to develop a treatment.

## 2. Drug Discovery Projects (to make new SMA drugs)

Building a therapeutics pipeline with multiple therapies in development dramatically increases our chances of successfully developing a treatment. Our critical role in research is to invest in the discovery and development of safe and effective therapies for SMA, and inspire others to do the same. We need to increase the size of our SMA drug pipeline, which simply means increasing the number of viable SMA candidate therapies in pre-clinical and clinical development. To date we have funded and advanced five such ventures:

- I. Since 2000 with an investment of \$13 million, the **Quinazoline Compound** which increases production of the back-up SMN2 gene, licensed to Repligen in 2009 for clinical development. This new drug candidate recently received the first ever Orphan Disease Designation from the FDA for SMA.
- II. Since 2003, the **Oligonucleotide Program** at UMASS, recently licensed to ISIS Pharmaceuticals, which uses genetic material to modify the back-up SMN2 gene.
- III. Since 2003 with an investment of \$2 million, the **Cellular Therapy Program** at California Stem Cell Inc, University California, Irvine and Johns Hopkins, which completed the first ever pre-Investigational New Drug Meeting with the FDA for SMA.
- IV. Since 2004 with an investment of \$2 million, the **Tetracycline Program** at Paratek Pharmaceuticals to correct SMN2 splicing, which is now being funded in part from a multi-million dollar award by the NINDS.
- V. Since 2009, the **Gene Therapy Program** at Nationwide Children's Hospital in Ohio, to replace the entire SMN1 gene.

**The SMA Community now has 9 new therapeutic development programs in progress compared to just 1 in 2000.**

## 3. Clinical Trial Initiatives (to test drugs in humans)

FSMA's investment of \$7 million in multi-center clinical trials is helping to test existing drugs and has built the clinical trials network Project Cure SMA. The network has successfully completed 5 clinical trials with repurposed drugs for all SMA Types.

These programs attract leading medical professionals and researchers, as well as companies and government who take on the vision and support the large costs of the later stages of therapeutics development. We have demonstrated success in advancing research programs and have become experts in building a broad pipeline of new drug programs.

## Patient Care and Support for SMA Families

*Communication. Connection. Assistance.*

Along with funding and directing the leading SMA research, FSMA provides core resources and assistance that help families navigate life with SMA, providing a stable, unbiased platform for SMA families to live active, engaged, hopeful lives.

- We connect families with medical professionals and the SMA community. We have a dedicated network of local chapters throughout the United States who provide direct and caring support for families.
- Our equipment pool has loaned over 4,200 pieces of much needed medical equipment to families.
- We collaborate with family support groups to help all newly diagnosed SMA families receive care packages.
- FSMA has developed *The SMA Care Series* – resource guides for families and professionals with the latest information on patient care.
- Newsletters: FSMA publishes 2 newsletters, “*Directions*” and “*Compass*,” and sends monthly E-blasts to all our members. These keep the entire SMA community up-to-date on breakthroughs in SMA research and clinical trials, along with sharing interesting and uplifting community news.

## FSMA Annual Conference

The Annual SMA Conference hosts the nation’s largest gathering of those affected by SMA and leading researchers from around the world to share ideas and hope for the future.

The Annual Conference allows families to learn about SMA research developments, increase their knowledge of the disease, share the latest in disease management techniques and network with other families and leading medical and scientific professionals. Our approach has the crucial component that both researchers and families work closely together as a team. FSMA is dedicated to educating families and healthcare professionals and to bringing the entire community together for growth and support.

## Grassroots Fundraising

We continue to answer the call to action, promote awareness and leverage our funding toward the development of a treatment and cure. These funds are generated through incredible chapter and family fundraising events and private donations. Fundraisers and donations have allowed us to make great progress. This continued support is what will get us to our goal. Our membership is over 70,000 strong with 7,500 passionately involved families and 30 active Chapters in the United States.

## FSMA Advisory Boards

FSMA has three independent key advisory boards, compiled of medical and scientific experts, whose mission is to set the agenda for leadership in SMA research and the quality of medical care.

- **Scientific Advisory Board** (SAB) governs basic research funding and makes sure we invest our dollars in the best researchers and projects.
- **Translational Advisory Council** (TAC) governs drug discovery and development projects, creating dynamic collaborative programs which are an avenue for promising new drugs and therapies.
- **Medical Advisory Council** (MAC) governs patient care, support services, and sets agendas for leadership on issues that improve medical care and quality of life for all those affected by SMA.



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