

## FightSMA Launches Gene Therapy Fundraising Campaign: “Realizing the Dream”

*Fight SMA and Gwendolyn Strong Foundation partner on effort to raise money for spinal muscular atrophy gene therapy.*

**Online PR News – 07-July-2010** RICHMOND, VA – Richmond-based [Fight SMA](#) announced today a new fundraising campaign for SMA gene therapy, 'Realizing the Dream.' FightSMA is collaborating with the Santa Barbara, California-based [Gwendolyn Strong Foundation](#), to form a bi-coastal partnership with a common goal: to bring [spinal muscular atrophy \(SMA\)](#) gene therapy to clinical trial.

“Ten years ago, it would have been unheard of to say scientists were approaching a treatment or cure for spinal muscular atrophy,” said FightSMA President Martha Slay. “Today, the dream is being realized in some of the most prestigious labs across the country. Never before has there been such promise for SMA gene therapy.”

Beginning now and for the next three years, families and groups in the SMA community will raise funds to build a safe foundation, deliver genes to an SMA model, and produce adequate vector (gene delivery) supply. These efforts will support Dr. Brian Kaspar of Nationwide Children’s Hospital and The Ohio State University and other collaborating scientists.

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FightSMA, working with the Gwendolyn Strong Foundation, a funding source for critical SMA science and awareness initiatives, invites the SMA community to make a decade-old dream come true. “Our SMA gene therapy program at Nationwide Children’s Hospital and the Ohio State University continues to show great promise for treating SMA patients,” said Dr. Kaspar.

FightSMA’s objective for the balance of 2010 is to complete funding for Phase One, and for Year-One of Phase-Two of the research program.

“The first objective is to build a solid foundation of safety and to eliminate toxicity,” said Dr. Chris Lorson, FightSMA Science Director.”

Additionally, FightSMA plans to raise another \$250,000 to fund the first year of Phase Two (Delivery & Efficacy). The 'Realizing the Dream' program will be accomplished through a series of campaigns. Completing these two Phases will bring SMA gene therapy significantly closer to clinical trial.

FightSMA has been instrumental in helping to develop a gene therapy strategy to cure spinal muscular atrophy (SMA), including oligonucleotides and gene replacement vectors. The strides that SMA researchers have made in the gene therapy arena have provided insights into a range of genetic disorders, including other neurodegenerative disease (ALS/Lou Gehrig's disease, myotonic dystrophy, Huntington disease) and

other diseases such as Duchenne muscular dystrophy.

For more information on the FightSMA – Gwendolyn Strong Foundation partnership and 'Realizing the Dream' campaign, visit [www.fightsma.org](http://www.fightsma.org) or call 804-515-0080.

FightSMA was created to strategically accelerate the search for a treatment and cure for spinal muscular atrophy (SMA), the number-one inherited cause of infant death. The organization pursues this objective by raising awareness and funding for SMA research.

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## Image Gallery



Spinal Muscular Atrophy - Fight SMA

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