

# COMPASS

A Publication Dedicated To Research Updates



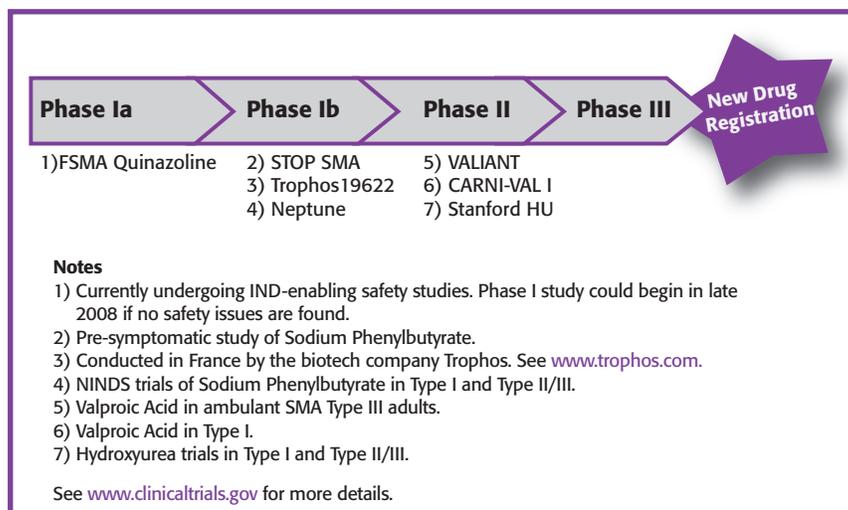
## Dear Families and Friends,

Once a year Families of SMA publishes an issue of "Compass" focusing on clinical trials and Project Cure SMA. Project Cure SMA is a clinical trials network, developed in collaboration between FSMA and the participating clinicians. Currently, the multi-center network consists of seven clinical testing sites and the entire supporting infrastructure needed to perform multiple clinical trials simultaneously. This is the largest network currently performing multiple clinical trials for SMA. ***This leading network has two trials already completed, three more are in progress, and we have plans to launch two additional trials over the next year.*** It is especially noteworthy that we are now able to perform several trials at the same time.

- Just this month, Project Cure SMA completed all study visits for the 90 patients participating in the Phase II CARNI-VAL Trial – this is over 450 total site visits by children and their families! This is a major milestone: to successfully recruit and then maintain these patient numbers in a trial. Results are expected to be released in 2008.

- Project Cure SMA is currently enrolling for the VALIANT trial, which assesses the efficacy of valproic acid in ambulant adults with SMA.

### SMA Clinical Trials Ongoing or Projected to Begin in 2008



- ***In addition, Families of SMA is excited to announce in this issue of "Compass" that Project Cure SMA recently received an Investigational New Drug (IND) from the FDA to initiate the multi-center CARNI-VAL Type I Trial.*** This trial is expected to begin in early 2008 and will assess the safety and tolerability of the combined regimen of valproic acid and carnitine in infants with Type I SMA. This trial will round out the studies of VPA in all types of SMA, one of our major goals.

I was heartened when drawing the above chart that documents ongoing and projected new SMA trials in 2008. It is amazing that seven or more trials could be conducted for SMA during the course of the next year.

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### The Drug Testing Process

FDA approval typically requires three stages of clinical trials, with each stage specifically designed to test different aspects of the drug.

**Phase I** is intended to test the safety of the drug, but generally it is not intended to evaluate whether or not it works. A Phase Ia study is typically conducted in healthy adults, and a Phase Ib study in patients.

**Phase II** is a "Proof of Concept" study. It is used to demonstrate whether or not the drug has benefit, to further assess its safety and side effect profile, and to better define the best dose to be used in the final stage.

**Phase III** or Pivotal Studies are designed to determine definitively whether a drug has clinical benefit in a particular patient population.





**SMA patients being evaluated in Project Cure SMA clinical trials.**

**SMA Registry:** We are excited to announce that other SMA advocacy groups (FightSMA, SMA Foundation, and the MDA) will be joining with us to fund the SMA Registry at Indiana University in 2008.

**Families of SMA has invested \$3.4 Million in clinical trials to date.** Our current plan is to fund an additional \$1.5 Million next year. We plan to add additional locations to our network if new funding becomes available.

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One of the particularly exciting aspects right now is that drug companies are getting involved rather than just non-profits. It has been a long-standing goal of FSMA to encourage companies to both participate in SMA drug programs and to ultimately invest money in SMA drug development, and now this is happening! Tikvah Therapeutics is even collaborating with the Project Cure SMA network to test their proprietary new drug formulation.

***With a clinical candidate in hand for the FSMA quinazoline project, we have started generating the data needed for an IND application for the FDA. If the data indicates a safe drug and the FDA accepts the application, first-in-human testing can be initiated with safety studies in healthy adults in 2008.***

I would like to take this opportunity to highlight an important event designed to facilitate SMA drug development called the "The SMA Summit on Drug Development". The SMA Drug Summit was organized and hosted by the Patient Advisory Group (PAG) of the International Coordinating Committee for SMA Clinical Trials. In the US, the PAG consists of representatives from Families

of SMA, FightSMA, The SMA Foundation, and the MDA. As a group, we meet regularly to organize and fund joint community projects in the area of clinical trials. The specific goal of the summit was to advance SMA drug development and SMA clinical trials. This meeting convened representatives of international SMA advocacy groups, representatives of the biotech and pharmaceutical industries, representatives of the U.S. Federal Government (FDA and NIH), and SMA clinicians.

***Over 13 drug companies attended the event. Nine companies gave updates on SMA drug programs ongoing within their organizations, and FSMA has funded three of the nine projects presented. The experience and results from the Project Cure SMA network have been critical in attracting companies to invest in SMA drug development.***

During the final presentation at the close of the Summit, the FSMA quinazoline project was highlighted as the most promising and advanced novel program in the whole SMA field.

An experienced and operational clinical network will continue to be a critical factor in bringing in companies to the

SMA field. Attracting industry funds for clinical development of new drugs will be essential to our success. Our major clinical goals now are to expand the coverage given by sites in the Project Cure SMA network. We need to have dispersed regional locations to allow for easy access for patient travel and recruitment. **To add an additional site to the network requires approximately \$120,000 per year in new funding.** Ideally we would like to add 2 or 3 new sites over the next year if funding becomes available.

Sincerely,

*Jill Jarecki, Research Director FSMA  
and Sandra Reyna, Clinical Coordinator  
Project Cure SMA*

**Current Project Cure SMA Clinical Site Locations:** Utah, Wisconsin, Michigan, Maryland, Ohio, Montreal, Argentina.



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