



# Clinical Trials-Update

By: Dr Petra Kaufmann

The term "Clinical Trial" typically refers to a research study that evaluates a new treatment or intervention. The new treatment is given to patients under very controlled circumstances to make sure that participants are safe and so that any important changes noted among treated patients can be attributed to the treatment and not to external factors.

As with every serious disease for which there is no cure, physicians and patients alike strive for effective treatment and are understandably ready to embrace any new hope. This aspect of human nature is well captured in the expression "grasping at straws" and makes it difficult to establish whether a new therapy is helpful or harmful. Everyone wants to take the new treatment and everyone involved wants to believe that it works. The history of medicine, however, has many examples of treatments that were associated with considerable discomfort, cost, or even danger, and that were used on many patients until finally they were proven ineffective or even harmful in controlled clinical trials. Also, so-called "open" trials in which everyone receives the new treatment are prone to providing overly optimistic and inaccurate results concerning treatment benefit because of the so-called "placebo effect". For this reason, good clinical trial design requires a comparison group that is not receiving the new treatment. Controlled clinical trials are now widely accepted standards in medical research. Most patient advocacy groups, including networks of cancer and HIV patients, support controlled clinical trials. The FDA requires proof of safety and effectiveness in controlled trials before approving any new medication. The National Institutes of Health guidelines for funding of clinical research encourage medical researchers to design rational, controlled trials.

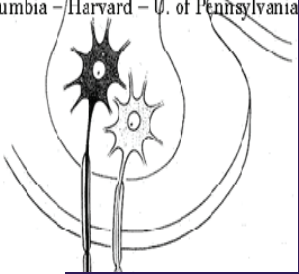
One question that families frequently ask is why some participants will receive a placebo (inactive drug) during at least some periods of a trial. They are wondering whether it would not be better for them to be sure to receive the active medication. We understand this reasoning and we try to keep the number of patients on placebo as small as possible without compromising our ability to determine the actual effects of the treatment. However, we feel that it is our responsibility as physicians and researchers to subject new treatments to controlled clinical trials for several reasons.

Continued on page 5

## Increased Survivability in SMA Type I

SMA type 1 is the most severe subtype of SMA with age of onset prior to 6 months of age. These children never acquire the ability to sit independently and most are thought to die before their second birthday. The standard of care for these children has changed in recent years, with more pro-active management of pulmonary and nutritional status. We hypothesize that advances in medical and respiratory management have an impact on the natural history of SMA. Updated natural history data is imperative in the care of these patients and in planning future clinical trials. To assess medical care and survival in patients with SMA type 1 between 1980 and 2006, de-identified information from participants in the International Spinal Muscular Atrophy Registry was analyzed comparing cohorts by year of birth. Survival data was obtained from 245 patients in the registry, and more detailed information on care parameters were obtained from 145 participants.

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On behalf of the Kiley & Dylan's Sweet Dream & Columbia University's SMA Clinical Research Center, we would like to thank everyone who attended and supported the first annual "Kiley and Dylan's Sweet Dream...A Night at the Tavern on the Green".

The event was a huge success! Please save the date for next year:

**June 26, 2007**

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If you are interested in participating in the planning of next year's event, please contact Mariel at [cureforkiley.com](http://cureforkiley.com)

Volunteers are needed for:

- Organizing the journal
- Collecting silent auction items
- Decorating
- Writing Letters
- Raising Awareness

We would like to send a special thank you to:

**The Kostychenko Family**  
**The Cuevas Family**  
**The McDonnell Family**

# Upcoming Educational Seminars

SEPTEMBER 20, 2007  
Stem Cell Research Update

Dr Wichterle is an assistant professor holding a joint appointment in the Departments of Pathology, Neurology and Neurobiology and Behavior at Columbia University Medical center. He is currently focusing on the use of embryonic stem cells to study motor neuron development and function. His work will help us understand why some nerve cells are susceptible to degeneration in neurodegenerative diseases. These methods may also provide a source of cells for both cell replacement therapies and identification of new neuroprotective drugs.

OCTOBER 18, 2007  
REHABILITATION MEDICINE

Nancy E. Strauss, M.D., Associate Clinical Professor of Rehabilitation Medicine is Board certified in Physical Medicine and Rehabilitation. She is Director of the Residency Training Program and acting chair of Physical Medicine and Rehabilitation at New York Presbyterian Hospital-Columbia and Cornell. Her main clinical and research interest is in the Rehabilitation Management of patients with Neuromuscular Disorders. She will speak to us about the challenges individuals with neuromuscular disease face.

November 15, 2007  
GENETICS

Dr Wendy Chung is the director of the clinical genetics program at Columbia University, a co-director of the molecular genetics diagnostics lab, and heads a research laboratory in the division of molecular genetics investigating the genetic bases for a variety of Mendelian and complex traits. She will explain the genetic make up of individuals with neuromuscular diseases and the future genetic therapies.



Please see our website for educational seminar calendar and details!

[www.columbiasma.org](http://www.columbiasma.org)

## Welcome Dr Andrei Constantinescu & Megan Montgomery

We would like to introduce you to two new Columbia University SMA Center staff members,:

Dr Andrei Constantinescu and Megan Montgomery.

Dr Andrei is a pediatric pulmonologist with much experience and interest in neuromuscular disease. He has worked at

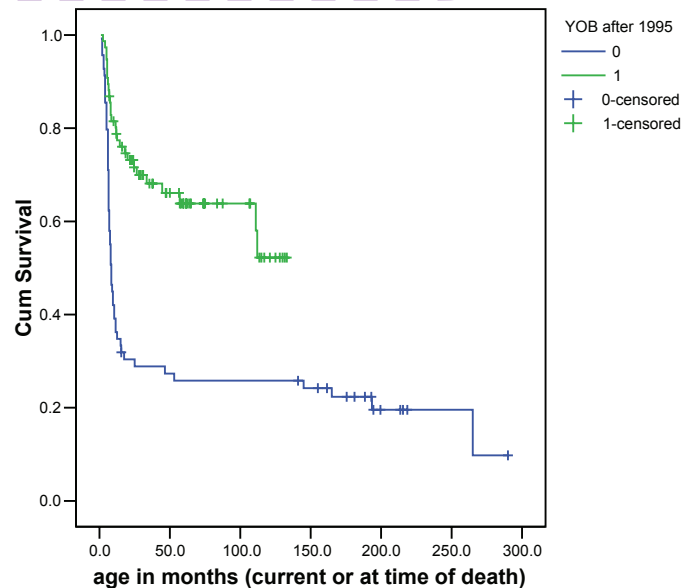
Megan Montgomery is originally from Minneapolis MN. She graduated from Duke University with a Biology and Neuroscience major. Before coming to Columbia, she worked in clinical research at Emory University in Atlanta GA. She is our new clinic coordinator.

**Please welcome our two new team members!**



## Increased Survivability in SMA Type I continued from page 2

The cohort born after 1995 had significantly greater use of ventilation, gastrostomy, prolonged home ventilation greater than 16 hours a day, and ventilation at a younger age. Those born after 1995 had significantly improved survival (Figure on the right). Most of this difference was accounted for by ventilatory support. Survival in SMA type 1 patients has increased in recent years, associated with a change in care with more pro-active management of pulmonary status and nutrition.



# Clinical Trials-Update continued

If we were certain that a new treatment would help patients with SMA, we would not have to do a clinical trial. If at any point during the trial it became clear that patients taking a new medication do significantly better or worse than those without the new medication, the trial would be stopped and we would be able to give the appropriate recommendations to SMA patients. Until and unless we have these results, the best way that individual patients with SMA, their family members, and the community of SMA patients can obtain crucial information on the safety and efficacy of a new medication is through a randomized, double-blind (whereby neither the patients nor the clinicians know who is receiving the new treatment), appropriately controlled trial. We strongly believe that participation in controlled clinical trials is in the best interest of patients with SMA and their families. Especially when dealing with relatively rare diseases, it is of crucial importance that potential therapies are investigated at designated research centers and in controlled trials. In doing so, patients and researchers will have the greatest chance to reach their common goal, that is, to improve the situation of patients with SMA as soon as possible. These studies greatly reduce the inconvenience, risk, and expense that many patients and family members encounter when they feel obliged to take suggested treatments without knowing whether the treatment will actually help or when they should stop taking it. Clinical trials allow us to rationally test treatments, eliminate harmful or ineffective treatments rapidly, and, hopefully, find an effective and safe treatment soon.

## Kids Page - Jokes, Fun Facts, and a Word Find

### Fun Jokes!!!



What is a baby's motto?

If at first you don't succeed, cry cry cry again.

Why did the Dinosaur cross the road?

Because Chickens hadn't evolved yet.

How do we know the world won't come to an end?

Because its round.



Knock Knock.

Who's there?

Boo.

Boo who?

Don't cry its only a joke.

What do u get when you cross a dog with an elephant?  
A very nervous postman.



### Cool Facts !!!

The inventor of the flushing toilet was Thomas Crapper.

On average, a 4 year-old child asks 437 question each day.

Kermit the frog, is left-handed.

### Words to find:

- |        |         |       |      |
|--------|---------|-------|------|
| Cars   | Frogs   | Snow  | Win  |
| Coal   | Insects | Soil  | Wind |
| Energy | Lake    | Trees | Won  |
| Fish   | Rain    | Walk  |      |



C	E	A	C	I	D	I	C	W	I
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