



Having a Good Website is Not Enough: *Communication Tactics to meet the Needs of both the ALS Patient and the ALS Organization*

Robert A. Goldstein

ALS Therapy Development Institute

12/3/2013

Ready, set, go.....

- Using the media to spread awareness of ALS and your organization
- Clinical Trials are about the PALS not the org or the country they live in
- PALS want you to give them information about ALS TODAY (not just the stuff you fund)
- Couple of TDI things you should know....





Most PALS are smarter than you
or I are. Except it.



Problem is, most PALS rely on the internet for information. The internet is not policed.

(many of you will remember Rick Bedlack's talk from a couple years ago)

What do we do then?

- ALS Forum (www.als.net)
- First online bulletin board devoted to a single disease topic.
- More than 10,000 users to date with 100,000+ unique posts since inception.
- Many “old timers” have taken on the role of helping “newbies” catch up quickly.
- You can’t buy this level of credibility....but it comes with risks for an organization...





TOP STORIES IN HEALTH AND WELLNESS



Insurers Seek to Bypass Health Site



Cellphones Leave Gaps for 911 Services

2 of 5



Food for Healthy Living: What About...

HEALTH & WELLNESS

Frustrated ALS Patients Concoct Their Own Drug

Email Print Save 63 Comments f t g+ in A A

By AMY DOCKSER MARCUS

April 15, 2012 7:25 p.m. ET

Some patients with fatal Lou Gehrig's disease, frustrated by the slow pace of clinical drug trials or unable to qualify, are trying to brew their own version of an experimental compound at home and testing it on themselves.

This effort has important implications for both people's health and scientific research. Until clinical trials are conducted, no one knows for sure if a drug is either safe or effective, and patients could potentially harm themselves or even accelerate the course of their disease. Some researchers worry that patients who report online that a do-it-yourself drug isn't working may discourage patients from joining or staying in a formal trial.



The patients who are experimenting with these do-it-yourself drugs say it is a calculated risk worth taking, given the average life expectancy is only two to five years after diagnosis and there are no effective treatments. Many patients say they want to slow down the progression of the disease, in hopes that an effective therapy will be found in

I called the WSJ and told them about this interesting DIY trial.

You can do the same...





Ben passed away in August 2013

PS: NP001's third CEO in two years they will be in another trial by 2015. Also, Bob Miller will present data this week on it again.

Two things we did in response.

1. Added security and reduced the anonymity of who was using the site. Everyone is required to register now to read or post a comment. No negative impact on usage since implementing it in September 2013.
2. Brought back the email blast for the ALS Forum that used to be run by a PALS (Will Hubben). Monthly blast highlighting the latest usage and trends.



Clinical Trials Database

- 50 drug trials
- 51 biomarker/observational trials
- 4 continents
- 20 different countries
- Not just clinicaltrials.gov or WHO...but all...everywhere
- Monthly email blast to list (*no appeal for money*)

Why?

1. What PALS/CALS crave
2. Keeps people on your site
3. Gives customer what they want



Dear {first_name},

Currently, we are monitoring 96 clinical trials worldwide for ALS patients. There have been 15 updates to this list since our last email to you. [Click here to view all trials](#) being monitored by the ALS Therapy Development Institute (ALSTDI).

5 Most Recent Updates to Clinical Trial Database

Tuesday, November 26, 2013

[Efficacy and Safety Study of MYOBLOC® Followed by Open-Label Multiple-Treatment With MYOBLOC® in the Treatment of Troublesome Sialorrhea in Adult Subjects](#)

Type: Observational

Phase: III

Recruiting?: Currently Recruiting

Sponsor: [US WorldMeds LLC](#)

Tuesday, November 26, 2013

[Clinical Trial Nuedexta in Subjects With ALS](#)

Type: Interventional

Phase: II

Recruiting?: Currently Recruiting

Sponsor: [Center for Neurologic Study](#)

Tuesday, November 26, 2013

[Mexiletine in Sporadic Amyotrophic Lateral Sclerosis \(SALS\)](#)

Type: Interventional

Phase: II

Recruiting?: Currently Recruiting

Sponsor: [University of Washington](#)

Tuesday, November 26, 2013

[Immunosuppression in Amyotrophic Lateral Sclerosis \(ALS\)](#)

Type: Interventional

Phase: II

Recruiting?: Currently Recruiting

Sponsor: [Emory University](#)

Monday, November 25, 2013

[Study of miRNA Expression Pattern as Diagnostic and Prognostic Biomarker in Amyotrophic Lateral Sclerosis](#)

Type: Observational

Phase: N/A

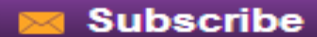
Recruiting?: Not Yet Recruiting

Sponsor: [University Hospital, Bordeaux](#)













Everyone's trials...PALS/CALS will go anywhere!

Clinical Trials

 **Subscribe**

With no effective ALS treatments currently available to slow or stop the disease, many ALS patients opt to participate in clinical trials. ALS TDI provides unbiased and up-to-date information about ALS clinical trials taking place worldwide. Search our database by type and category to find and compare trials. Input your zip code to find clinical trials closest to you. [Click here to learn more about the clinical trial process >](#)

Active Trials

LAST UPDATED	TRIAL	PHASE	RECRUITING	SPONSOR	LOCATION(S)
Within a Week					
11/26/2013	 Clinical Trial Nuedexta In Subjects With ALS	II	Currently Recruiting	Center for Neurologic Study	San Francisco, CA, US Washington, DC, US View all 7 locations
11/26/2013	 Mexiletine In Sporadic Amyotrophic Lateral Sclerosis (SALS)	II	Currently Recruiting	University of Washington	Los Angeles, CA, US Iowa City, IA, US View all 10 locations
11/26/2013	 Immunosuppression In Amyotrophic Lateral Sclerosis (ALS)	II	Currently Recruiting	Emory University	Atlanta, GA, US Boston, MA, US View all 3 locations
Within a Month					
11/19/2013	 Study of Ozanezumab (GSK1223249) Versus Placebo In the Treatment of Amyotrophic Lateral Sclerosis	II	Currently Recruiting	GlaxoSmithKline	Randwick, NSW, Australia Herston, Queensland, Australia View all 35 locations
11/14/2013	 Early Stage Amyotrophic Lateral Sclerosis Phrenic Stimulation (RespiStimALS)	III	Currently Recruiting	Assistance Publique - Hôpitaux de Paris	Paris, France
11/13/2013	 Gilenya In Amyotrophic Lateral Sclerosis (ALS)	II	Currently Recruiting	Massachusetts General Hospital	Orange, CA, US Augusta, GA, US View all 4 locations
11/6/2013	 Phase III/III Randomized, Placebo-controlled Trial of Arimoclomol In SOD1 Positive FALS	III/III	Ongoing, But Not Recruiting	University of Miami	Miami, FL, US Boston, MA, US
11/5/2013	 Evaluation of Masitinib In Amyotrophic Lateral Sclerosis	III	Currently Recruiting	AB Science	Madrid, Spain
11/5/2013	 A Study of the Safety and Tolerability of Tocilizumab In ALS	II	Not Yet Recruiting	ALS Association	Phoenix, AZ, US Kansas City, KS, US View all 3 locations
11/5/2013	 DIPALS Diaphragm Pacing In MND	N/A	Currently Recruiting	Sheffield Teaching Hospitals NHS Trust	London, United Kingdom Sheffield, United Kingdom

Closed Trials

Closed clinical trials include those listed as fully enrolled, concluded, or cancelled [Click to view more closed trials >](#)

Sponsor More Science

Your support accelerates research! Donate money. Run an event. Start a fundraising campaign for someone you know and love. Get involved and keep drug development going. [Here's how >](#)



Immunosuppression in Amyotrophic Lateral Sclerosis (ALS)

#1: Open or Closed?

Status:
Currently Recruiting

Estimated Enrollment:
30

Treatment Type:
Oral and by injection (IV)

Sponsor:
Emory University

Primary Investigator:
Jonathan D. Glass, M.D





Phase:
II

Trial Type:
Open label

Contact Information:
617-724-3314; 617-724-5984
aknox1@partners.org;
AJMCCOURT@PARTNERS.ORG

#3: A real contact with power

#2: Am I eligible?

Enrollment Criteria:	
 Breathing Ability	SVC >80%
 Months Since Onset	≤ 24 months
 Non-Invasive Ventilation (NIV)	No
 Diaphragm Pacer (DPS)	No

Update Notes:

11/26/2013	Contact information updated
10/17/2013	Recruitment status changed
9/6/2013	Contact information updated
8/16/2013	Protocol updated.

Locations:

- Emory University, Atlanta, GA, 30322, United States
- Massachusetts General Hospital, Boston, MA, 02114, United States
- University of Massachusetts Medical School, Worcester, MA, 01655, United States

Other Information:

Purpose:	This is a multicenter, 15-month study evaluating the effect of immunosuppression treatment on the rate of change on the ALS Functional Rating Scale (Revised) (ALSFRS-R) score in 30 subjects with Amyotrophic Lateral Sclerosis (ALS).
Eligibility:	18+, both genders, no healthy volunteers
Details:	It is postulated that the immunosuppression treatment given to the stem cell patients may have exhibited neuroprotective effects by favorably promoting the ratio of regulatory T cells and other protective immune mediators in relation to neurotoxic immune modulators. It is hoped that this trial will optimize the chance of replicating these findings and allow the learning more about the complex changes that occur within the immune system in patients with ALS before and after



ALS, Plan B2?

Posted By Michelle Pflumm, Ph.D. November 26, 2013

A new EphA4 receptor antagonist unveiled at SfN13 may pave the road to a new potential treatment strategy for ALS.

RobGoldstein ▾

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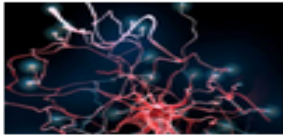
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A new player in the ALS Q

Categories: Podcast

Posted By Michelle Pflumm, Ph.D. October 31, 2013

Johns Hopkins' Nicholas Maragakis MD talks about astrocyte replacement, a new stem cell-based strategy approaching the clinic.



NEALS 2013: ALS, North by Northeast

Categories: Meeting Report

Posted By Michelle Pflumm, Ph.D. October 2, 2013

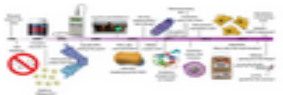
Clinicians weigh in on the latest research and clinical trials today - including stem cell therapy.



Reaching the Bench

Posted By Michelle Pflumm, Ph.D. September 30, 2013

Improving the ability of the motor nerves to reinnervate may be key to designing treatments that help people with ALS keep moving longer.

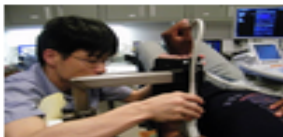


What's in the ALS pipeline - 2013?

Categories: In The Pipeline

Posted By Michelle Pflumm, Ph.D. September 16, 2013

Learn more about potential treatment strategies for ALS being evaluated in the clinic in this interactive timeline.



Breaking the ultrasound barrier in ALS

Categories: In Translation

Posted By Michelle Pflumm, Ph.D. August 26, 2013

Checkups may become easier for people with ALS with neuromuscular ultrasound, an emerging non-invasive alternative to EMG.



Cognition in ALS – Measure for Measure

Categories: Postcards

Posted By Michelle Pflumm, Ph.D. June 19, 2013

A new screening measure may enable clinicians to identify people with ALS experiencing cognitive challenges more quickly.

No one has a monopoly on good ideas...not even you!!

ALSToday [twitter](#)

[ALSToday](#) @ALSToday 27 Nov

A new peptide unveiled at #SfN13 may open the door to a new treatment strategy for #ALS #MND #EphA4 #ephA4B2 bit.ly/1a4hM6U Expand

[ALSToday](#) @ALSToday 21 Nov

A phase IIIA #ALS #MND clinical trial of #Qcells is planned 4 2014 QTherapeutics said today. Learn about #Qcells bit.ly/1tP64k Expand

[ALSToday](#) @ALSToday 21 Nov

Could #astrocyte #stemcell strategies approaching the clinic benefit people w many forms of #ALS #MND? bit.ly/1b8B8Om #SfN13 #Qcell Expand

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Tags

ABC transporters
AMP-associated protein kinase AMPK
antibodies astrocyte axonal guidance
BCRP biomarker BloMOx bulbar onset
C9ORF72 chronic hypoventilation
CK-357 clinical trial collaboration
Cystatin B Cystatin B



Own your bias on your website.

- 2011 we hired a full-time science journalist out of Nature to be the first “beat reporter” assigned to ALS.
- ALS Today was born.
- Coupled with social media, this has been a great success in generating leads!!
- Developed website, email sub-list and social media following.
 - It is now our 2nd most popular...wonder why?



Thing 2: ALS Pharmaceutical Consortium (ALPACA)



Company



Company



Company



Company

- **No cost** to pharma/biotech
- Start with 3-6 of world's largest pharma
- 100+ preclinical study data
- 600+ gene expression human data
- 1000+ gene expression ALS mouse data (SOD, TDP43, LOA, TAF15)
- Will add more data forever
- Convene group 2/year



Thing 3: Neurimmune, ALS TDI and Anelixis Partnership



**Thursday Presentation
here in Milan!!!!**

- Nonprofits can be hugely innovative.
- Some of the best ideas are unfunded
- Venture/angel funders are used to risk
- SOD1 implicated potentially in SALS
- Human monoclonal antibody that targets misfolded SOD1
- \$6-8 million to get it to the clinic





Thank you.

rgoldstein@als.net

Twitter: @ALSTDI

Please visit our labs in Cambridge!!