

Truth in Advertising: Evaluating Therapeutic Claims



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Co-director, Johns Hopkins Myositis Center

TMA 2017

CBS News Features Dr. Ashraf Hanna - Breakthrough Treatment Helps a Wheelchair Bound Patient with Inclusion Body Myositis Walk Again

NEWS PROVIDED BY

ivketamine.com →

Oct 19, 2016, 14:15 ET

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CLEARWATER, Fla., Oct. 19, 2016 /PRNewswire/ -- A United States veteran who was unable to walk for 5 years tries a new breakthrough treatment and is now able to stand and walk and is improving everyday! As an officer and aviator in the United States Coast Guard, Terry Stagg saw his fair share of challenges in his 21 years of serving his country. But it was a viral meningitis infection in 1983 that may have triggered his biggest challenge yet. Terry was diagnosed with [Inclusion Body Myositis \(IBM\)](#) in 2006, and everyday is a struggle to do what most people take for granted. [CBS news recently featured a news story](#) about a new breakthrough in treating his condition.

Ketamine





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Dr Hanna and Guest
Joellen Treherne discuss
Multiple Sclerosis and
her very Successful
IV Ketamine Treatment



FINALLY, a BREAKTHROUGH treatment in RSD / CRPS, Lyme Disease, Fibromyalgia Pain and many other conditions involving chronic pain, depression and post traumatic stress disorder or PTSD.

Our Ketamine Patients travel from all over the world. Our Map is utilizing actual patient zipcode data.

Map Satellite

Peer-Reviewed Journal Articles



Welcome to our research library! We understand that finding reputable scientific literature with public access can be difficult and frustrating. Here, we regularly update our database with peer-reviewed, published scientific articles with “open access” so that anyone may access the full text. We urge everyone to research topics that interest them. Don’t hesitate to reach out to us with any additional requests.

Chronic Pain

Title: **Ketamine for chronic pain: risks and benefits.**

Authors: Niesters, M., Martini, C. and Dahan, A.

Journal: Journal of Clinical Pharmacology

Abstract: The anaesthetic ketamine is used to treat various chronic pain syndromes, especially those that have a neuropathic component. Low dose ketamine produces strong analgesia in neuropathic pain states, presumably by inhibition of the N-methyl-D-aspartate receptor although other mechanisms are possibly involved, including enhancement of descending inhibition and anti-inflammatory effects at central sites. Current data on short term infusions indicate that ketamine produces potent analgesia during administration only, while three studies on the effect of prolonged infusion (4–14 days) show long-term analgesic effects up to 3 months following infusion





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Home

Dr Hanna

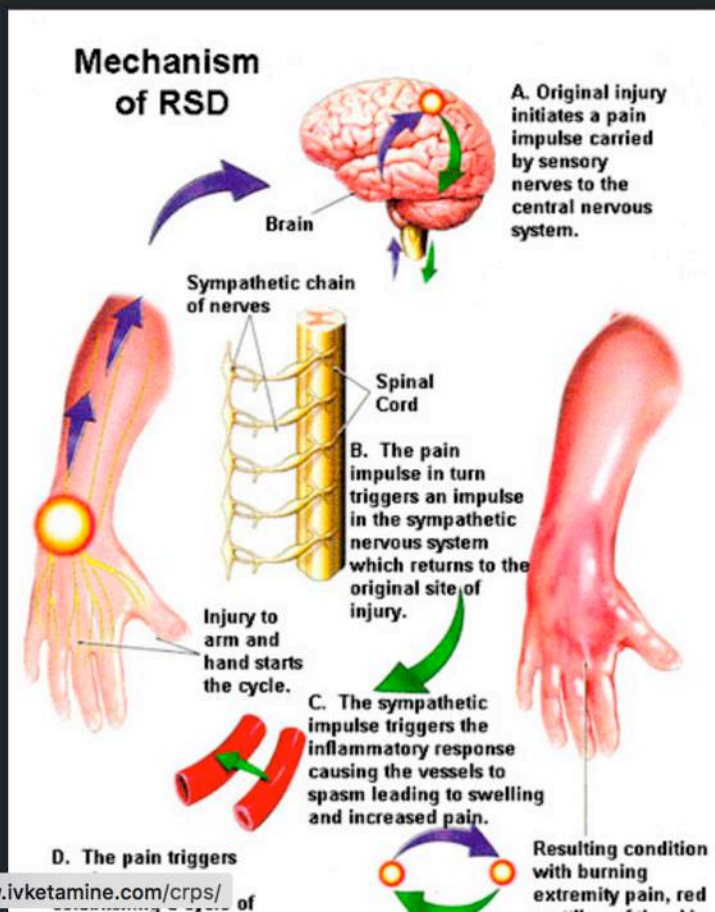
IV Ketamine Treatment

Conditions

Team

Re

What is complex regional pain syndrom (CRPS / RSD)?



www.ivketamine.com/crps/

Anxiety

CRPS / RSD

Crohn's Disease

Depression

Fibromyalgia

Inclusion-Body Myositis (IBM)

Lichen Sclerosus (LS)

Lyme Disease

Multiple Sclerosis (MS)

Phantom Limb Pain

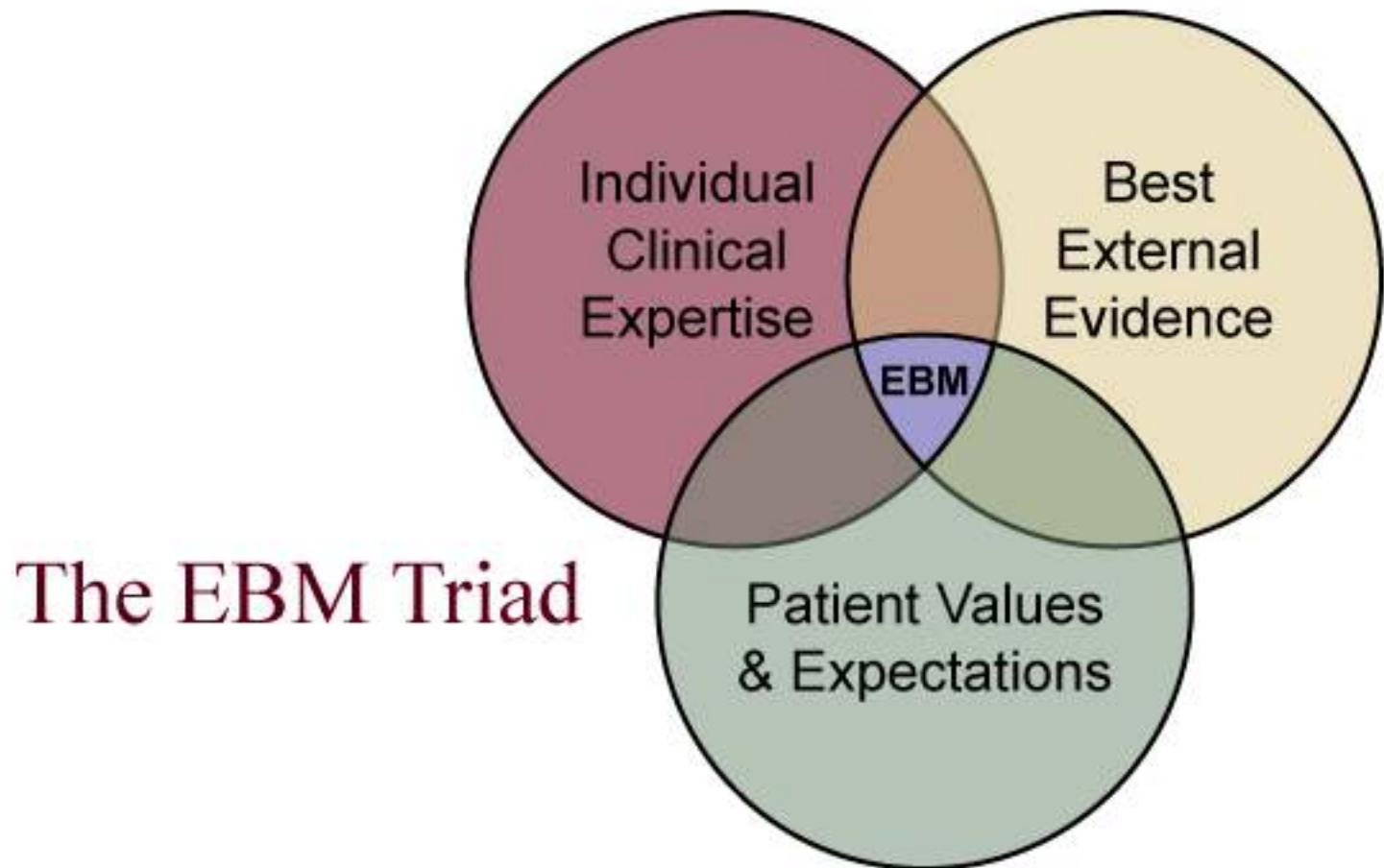
PTSD Depression

Rheumatoid Arthritis (RA)

No drug is approved for CRPS. No single drug works for every person. Drugs

• non-steroidal

Evidenced Based Medicine (EBM)



EBM for IBM

Conventional treatments

- *Diet*
- *Medications*
 - Immunosuppressive meds
 - Muscle growth promoting meds
 - Mainstream supplements
- *Devices*
 - Walking aids
 - Ankle-foot orthoses
- *Therapies*
 - Physical therapy / exercise
 - Occupational therapy
- *Procedures*
 - Esophageal dilation
 - Cricopharyngeal myotomy

“Alternative” treatments

- *Diet*
- *Medications*
 - “Antiinflammatory” supplements
 - “Bodybuilding” supplements
 - Other nutraceuticals
- *Devices*
 - Exoskeleton
 - E-stim (electrical stimulation)
- *Therapies*
 - Massage
 - Accupuncture
- *Procedures*
 - Stem cell injection
 - Hyperbaric oxygen chamber

Medications

Conventional treatments

- Immunosuppressive meds (SE)
 - IVIG
 - Methotrexate
 - *Statins*
- Muscle growth meds (SE)
 - Oxandrolone / testosterone
 - Bimagrumab
 - AAV-Follistatin
 - Growth Hormone
- Mainstream supplements
 - Creatine (cheap)
 - CoenzymeQ10 (can be pricey)

“Alternative” treatments

- “Anti-inflammatory” supplements
- “Bodybuilding” supplements
 - Myo-X

Intravenous immunoglobulin in DM

Vol. 329 No. 27

HIGH-DOSE IMMUNE GLOBULIN FOR DERMATOMYOSITIS — DALAKAS ET AL.

1993

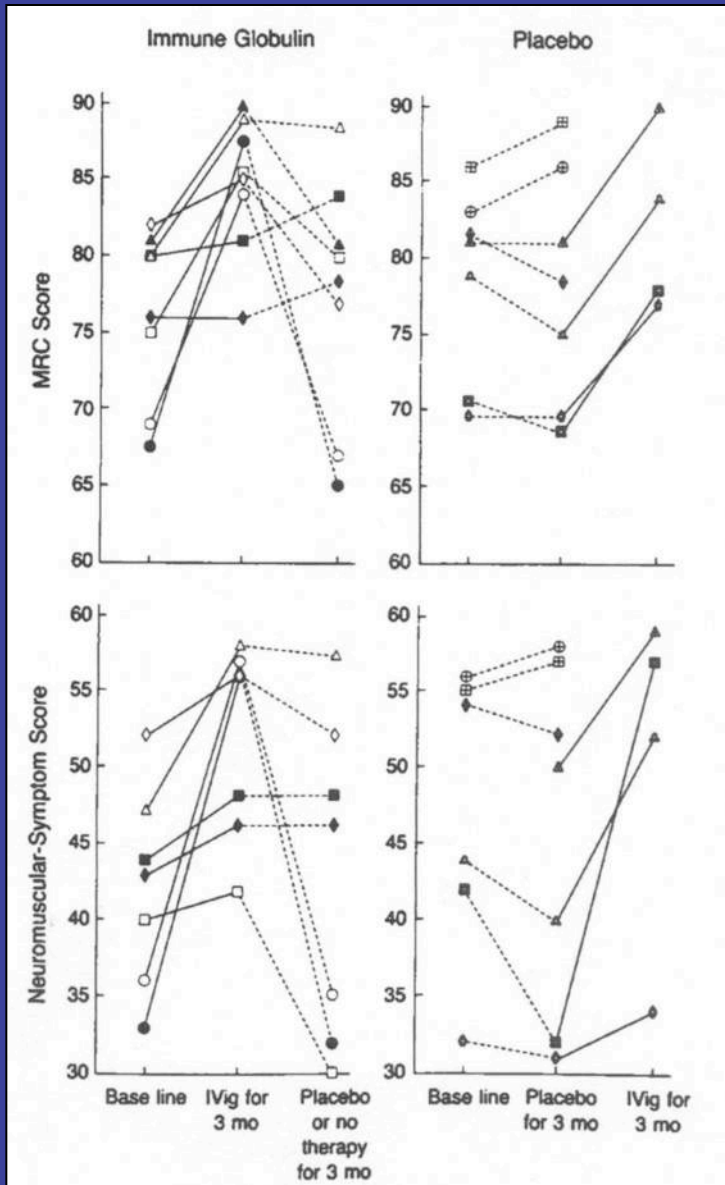
A CONTROLLED TRIAL OF HIGH-DOSE INTRAVENOUS IMMUNE GLOBULIN INFUSIONS AS TREATMENT FOR DERMATOMYOSITIS

MARINOS C. DALAKAS, M.D., ISABEL ILLA, M.D., JAMES M. DAMBROSIA, PH.D., SHAWKE A. SOUEIDAN, M.D.,
DANIEL P. STEIN, M.D., CARLOS OTERO, M.D., STEVEN T. DINSMORE, D.O., AND SUSAN McCROSKY, R.N.

NEJM (1993)

- 15 patients with biopsy-proven, refractory DM
- Continued to receive prednisone
- Random assignment: IVIg vs. placebo monthly for 3 months
- Option to cross-over after 3 months

IVIg leads to improvement



- 12 received IVIg
 - Major improvement = 9
 - Mild improvement = 2
 - No change = 1
- 11 received placebo
 - Major improvement = 0
 - Mild improvement = 3
 - No change = 3
 - Worsening = 5
- Repeat biopsies in those who improved
 - Increased fiber diameter
 - Increased capillary number
 - Resolution of complement on capillaries
 - Reduced MHC I expression

Creatine Supplements + Exercise Beneficial in PM/DM

- Chung et al., *Arth & Rheum*, 2007
- Creatine 20 grams/day for 8 days, then 3 grams/day
- Improved “aggregate functional performance time” with creatine at 6 months
 - 50-foot timed walk
 - Stair ascent and descent tests
 - “get up and go test”
- **Shoulder abduction and hip flexion strength improved with creatine at 3 and 6 months**
- Conclusions: Works in DM and PM. I also use it in IBM.

Creatine for treating muscle disorders

Published:

5 June 2013

Authors:

Kley RA, Tarnopolsky MA, Vorgerd M

Primary Review Group

Hereditary muscle diseases usually lead to a progressive muscle weakness. Treatment is mainly symptomatic because curative therapies are lacking. Creatine, a popular nutritional supplement among athletes, improves muscle performance in healthy individuals. This is an update of our [review](#) evaluating creatine treatment in muscle disorders that was first published in 2007. At this update we identified no new studies but we had previously found 14 randomised controlled trials with 364 participants which met our defined [selection criteria](#). The methodological quality of these studies was high, with only one exception. Analysis of pooled results showed a

Print



PDF



Citation



Authors' conclusions:

High quality evidence from [RCTs](#) shows that short- and medium-term creatine treatment increases muscle strength in [muscular dystrophies](#). There is also evidence that creatine improves functional performance in [muscular dystrophy](#) and idiopathic inflammatory myopathy. Creatine is well tolerated in these people. High quality but limited evidence from [RCTs](#) does not show significant improvement in muscle strength in metabolic myopathies. High-dose creatine treatment impaired activities of daily living and increased muscle pain in McArdle disease.



MYO-X MYOSTATIN INHIBITOR

- **CLINICALLY TESTED NATURAL MYOSTATIN INHIBITOR**
- **REDUCES MYOSTATIN IN TEST SUBJECTS BY AN AVERAGE OF 46%**
- **SHOWN TO WORK ON 100% OF STUDY TEST SUBJECTS**



MYO-X supplement helped me with my workouts thanks Carlon Colker
 — Justin Bieber (@justinbieber) January 7, 2015



12 WEEK TRANSFORMATION WITH MYO-X MYOSTATIN INHIBITOR!

*IFBB PRO Bodybuilder, Victor Martinez, used new MHP MYO-X in conjunction with his regular workout, diet, and supplement regimen to achieve this amazing transformation in just 12 weeks. Results may vary.

Alternative supplements

Pinyin

White peony root (Bai shao)

Paper mulberry fruit (Chu shi zi)

Cyathula root (Chuan niu xi)

Chinese Angelica root (Dang gui)

Codonopsis root (Dang shen)

Rhemannia root (Di huang)

Homalomena rhizome (Qian nian jian)

Acanthopanax (Wu jia pi)

Japanese teasel root (Xu duan)

Processed fleeceflower root (Zhi he shou wu)



Evidence for MYO-X

There's one massive problem with MYO-X: there's no scientific evidence behind it. If you look at the official product website for MYO-X, you'll see a study cited like this:

"Colker C., Effect on Serum Myostatin Levels of High-Grade Handled Fertile Egg Yolk Powder; Journal of the American College of Nutrition, Volume 28, No.3, Abstract 47; Page 309. October 2009."

This study does not exist and never existed. There was no page 309 in the October 2009 issue of the Journal of the American College of Nutrition, so that's a huge red flag.

MHP, however, does claim that it performed a clinical study at the Tampa Human Performance Laboratory. This study showed that subjects were able to add 8 pounds of lean muscle mass and gain "four times greater muscle thickness" compared to a control group which did not take MYO-X.

*MYO-X supplement helped me with my workouts thanks Carlon Colker
— Justin Bieber (@justinbieber) January 7, 2015*

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Devices

Conventional treatments

- **Walking aids**
- **Ankle-foot orthoses**

“Alternative” treatments

- **Exoskeleton**
- **E-stim (electrical stimulation, aka functional neurostimulation)**

Ankle-foot orthoses



\$45

**Ossur
“Foot-up”**



\$26

AFO



\$260

**Carbon
Fiber
AFO**



**Carbon
Fiber
GRAFO
“ground
reaction”**



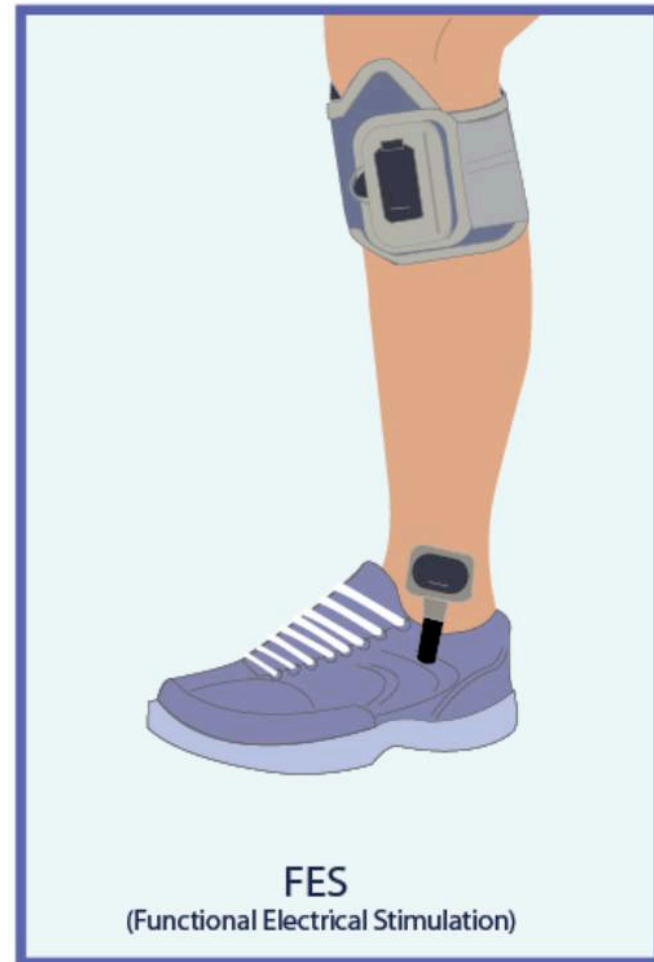
**Stance
Control
Orthosis**

COMBO



**Custom fitted carbon fiber
ground reaction AFO with
removable custom fabricated
knee orthosis addition to
control genu recurvatum**

“Alternative” Devices



Therapy

- Speech therapy
 - SLP referral for dysphagia
- Physical therapy and Exercise: Tae Chung, MD
 - Stretching to maintain flexibility/avoid contractures
 - Low impact (eg water aerobics)
 - High frequency, endurance exercises
- Occupational therapy
 - Mild: “Hand Helper” device to maintain strength and flexibility
 - Moderate: Occupational/Hand therapy
 - Exercises
 - Bracing, interpharyngeal (IP) fusion



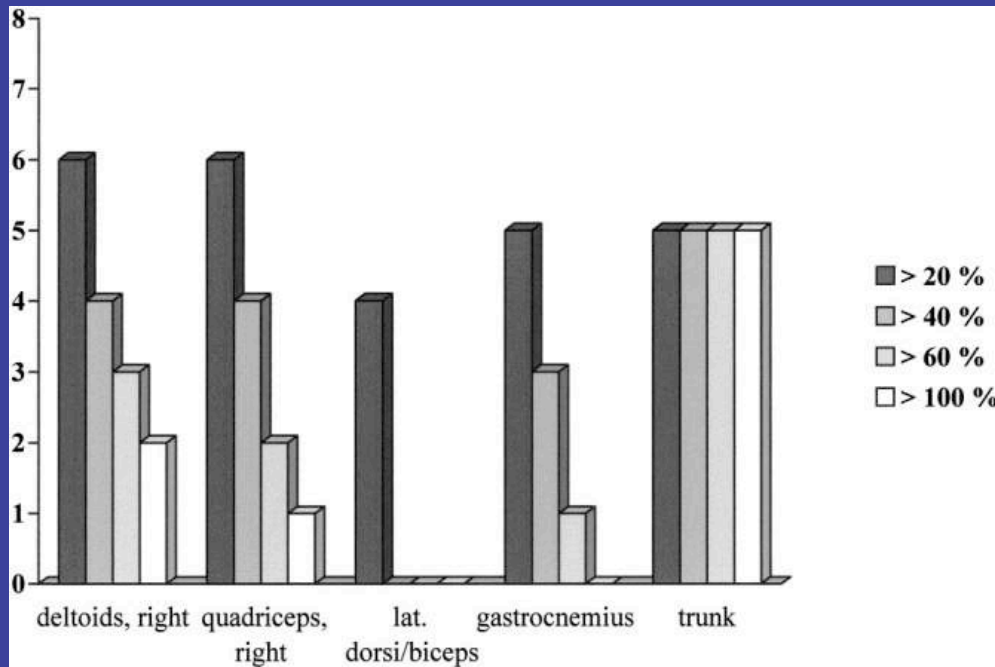
Ultimate Hand Helper®
\$20

MedDev Corporation

<http://shop.hand-helper.com/main.sc>

Benefits of Intensive Resistance Training in Patients With Chronic PM and DM

- Alexanderson et al., *Arth & Rheum*, 2007
- 5 DM and 3 PM patients on prednisone and other meds.
- Exercised 3 days/week



- **Improved strength**
- **No increased muscle inflammation (biopsied before and after)**
- **Conclusions: Patients with chronic, stable PM and DM can safely perform an intensive exercise program with beneficial effects**

Improvement in Aerobic Capacity After an Exercise Program in Sporadic Inclusion Body Myositis

Journal of Clinical Neuromuscular Disease. 10(4):178-184, JUN 2009

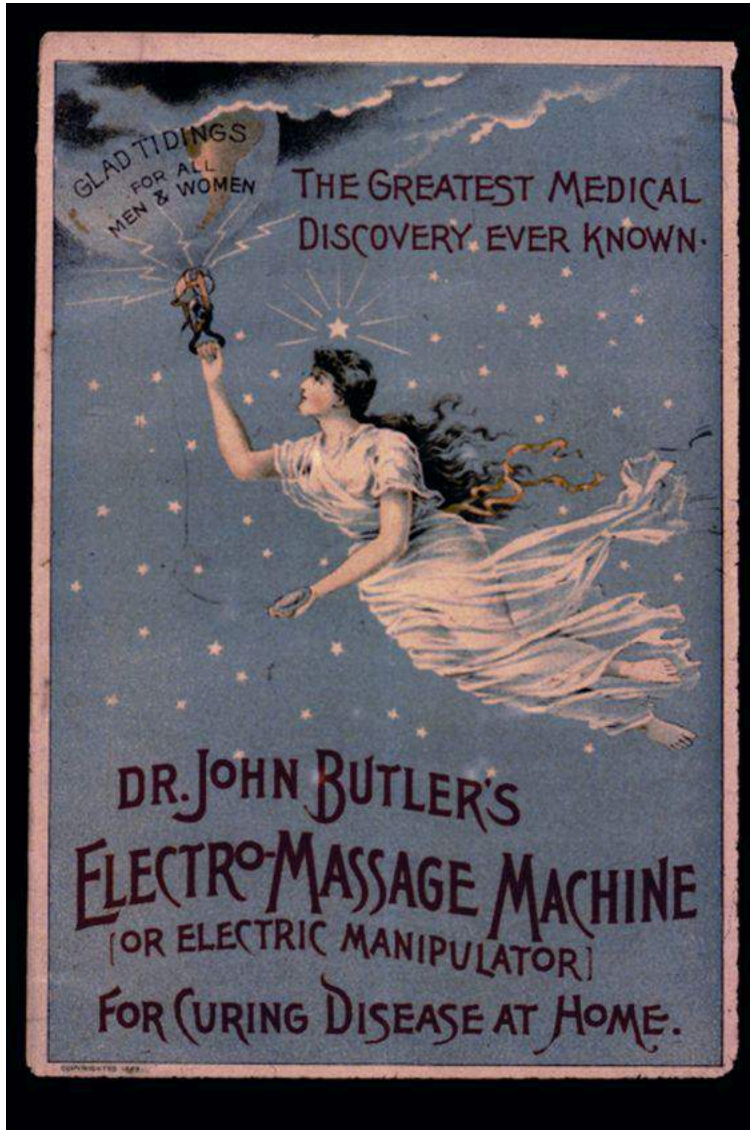
Liam G Johnson; Kelly E Collier; Dylan J Edwards; Danielle L Philippe;
Peter R Eastwood; Susan E Walters; Gary W Thickbroom; Frank L
Mastaglia [show less](#)

TABLE 3. Mean (\pm SE) Pre- and Post-Training Muscle Strength Values of the Participants (n = 7)

Muscle	Mean \pm SE		<i>P</i>
	Pre-Training	Post-Training	
Untrained			
Grip strength (mm Hg)	150.2 \pm 49.9	138.3 \pm 55.3	0.122
Shoulder external rotation (kgf)	7.4 \pm 0.0	7.6 \pm 0.9	0.652
Trained (kgf)			
Knee extension	7.3 \pm 0.2	6.6 \pm 0.1	0.805
Wrist extension	9.8 \pm 0.4	9.1 \pm 0.5	0.271
Elbow extension	7.4 \pm 0.5	6.8 \pm 0.3	0.067
Elbow flexion	10.9 \pm 0.5	11.0 \pm 0.3	0.402
Shoulder abduction	12.3 \pm 0.6	17.2 \pm 0.5	0.000**
Hip flexion	11.5 \pm 1.2	15.6 \pm 0.7	0.008*
Hip abduction	9.0 \pm 0.4	10.5 \pm 0.2	0.041*
Knee flexion	10.4 \pm 0.4	11.5 \pm 0.3	0.027*

P* < 0.05. *P* < 0.001.

Massage, Accupuncture



- Little scientific evidence that it is helpful
- Essentially impossible to conduct placebo-controlled trial
- Minimal risk
- Placebo effect is real and can be beneficial!
- So, if it works for you that's great!

Procedures

Conventional treatments

- **Esophageal dilation**
- **Cricopharyngeal myotomy**

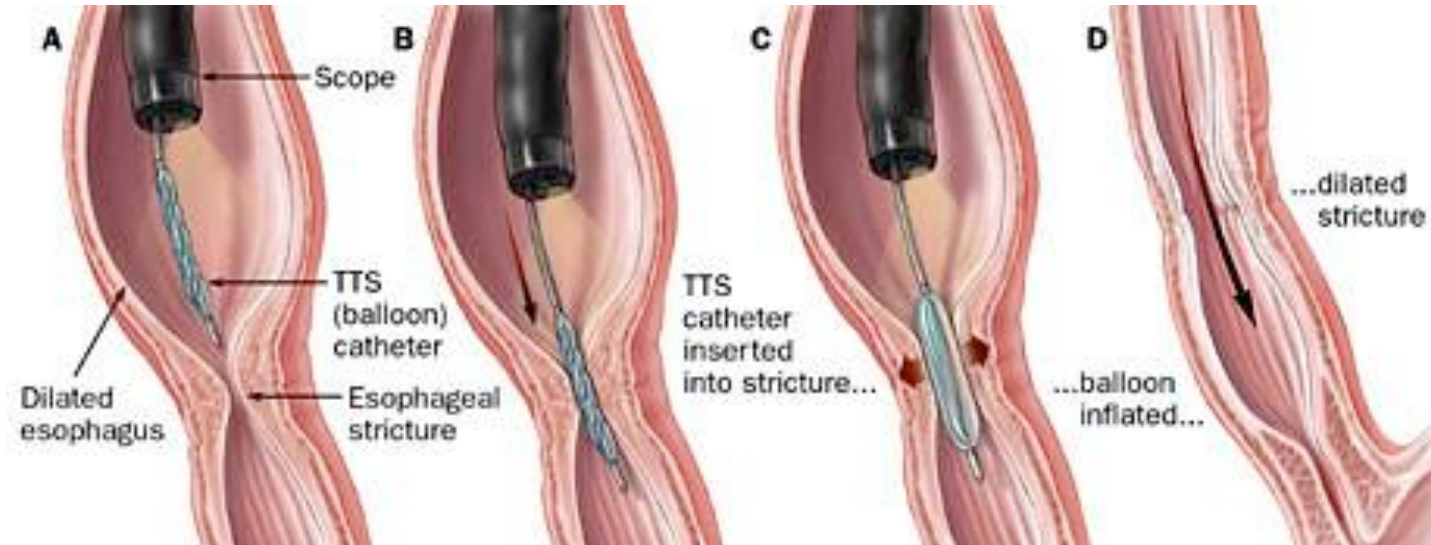
“Alternative” treatments

- **Stem cell injection**
- **Hyperbaric oxygen chamber**
- **Tendon transfer (extensor to flexor)**

Dysphagia in IBM

- Usually caused by constriction of upper esophageal sphincter (UES) due to cricopharyngeus involvement
- Diagnosis:
 - Video Fluoroscopy Swallow Study with SLP Guidance
- Management:
 - Mild:
 - Heimlich maneuver training
 - SLP referral
 - Moderate:
 - GI referral for esophageal dilation
 - Severe/Refractory:
 - ENT referral for cricopharyngeal myotomy
 - PEG tube if weight loss

Balloon Dilation

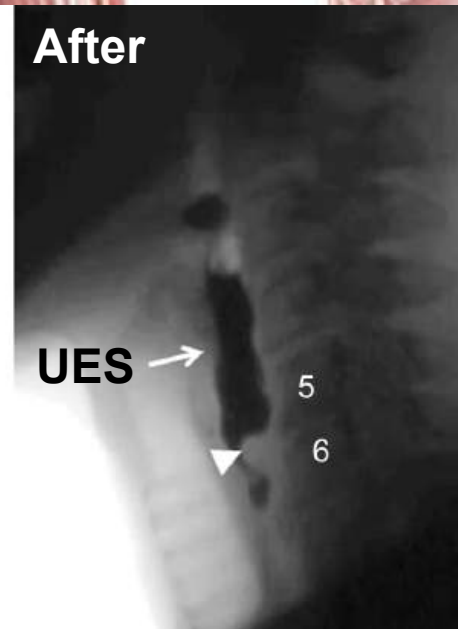
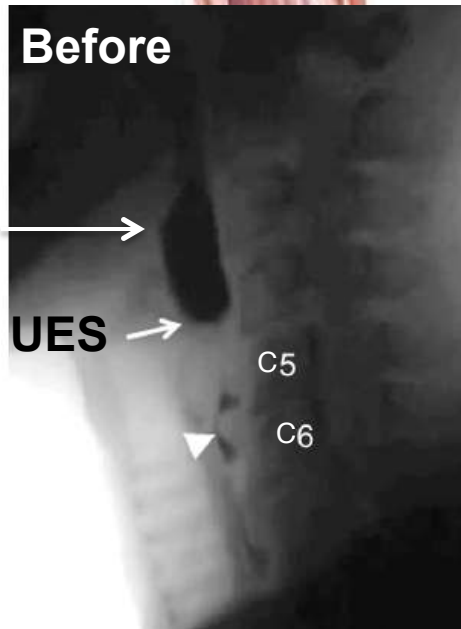


Before

During

After

Barium



Tendon transfer

Case:

- 74 yo man w/ 15 yr h/o slowly progressive proximal leg > hand weakness.
- Meets definite IBM diagnostic criteria
- Exam shows absent distal finger flexion but 4+/5 wrist and 4/5 finger extension.

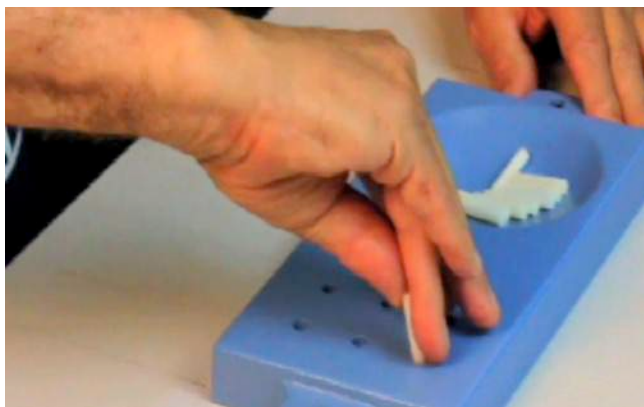
Referral to Orthopedic Hand Surgeon: Tom Brushart.

- Thumb IP fusion
- ECRB to FDP index and middle finger.

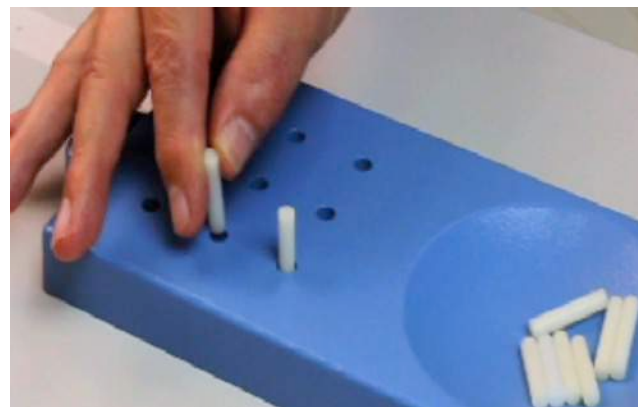
**Severely fibrosed FDP
muscle adjacent to healthy
ECRB muscle**



Pre-op



4 month post-op



Clinical Trials

Ethics

Health Fraud

What's the harm? Stem cell tourism edition

Stem cells have become big business. Offshore clinics claim to use stem cells to treat anything from aging, diabetes, stroke, cancer, and even autism, all without compelling evidence that these treatments have any meaningful effect.

Unfortunately, the potential for harm, both financial and to health, is high, as the case of Jim Gass demonstrates.

David Gorski on June 27, 2016

<https://sciencebasedmedicine.org/whats-the-harm-stem-cell-tourism-edition/>

last week. To recap, I wrote about a man named Jim Gass, a former chief legal counsel for Sylvania, who had suffered a debilitating stroke in 2009 that left him without the use of his left arm, and weak left leg. He could still walk with a cane, but was understandably desperate to try anything to be able to function more normally in life. Mr. Gass was both driven enough, credulous enough, and wealthy enough to spend \$300,000 pursuing stem cell tourism in China, Mexico, and Argentina over the course of four years. The result is that he [now has a tumor growing](#) in his spinal column, as reported in [The New England Journal of Medicine \(NEJM\)](#) and [The New York Times \(NYT\)](#). Genetic analysis has demonstrated that the cells in this tumor mass did not come from Jim Gass, and the mass has left him paralyzed from the neck down, except for his right arm, incontinent, and with severe chronic back pain. Worse, although radiation temporarily stopped the tumor from growing, apparently it's growing again, and no one seems to know how to stop it. Given that the traits that make stem cells so desirable as a regenerative treatment, their plasticity and immortality (ability to divide indefinitely), are shared with cancer, scientists doing legitimate stem cell research have always tried to take precautions to stop just this sort of thing from happening in clinical trials. Clearly, “stem cell tourist” clinics, which intentionally operate in countries where the regulatory environment is—shall we say?—less than rigorous are nowhere near as cautious, as they charge tens of thousands of dollars a pop for stem cell treatments that might or might not actually have real stem cells in them.

“ As noted by numerous scholars, only a few stem cell therapies are currently supported by good scientific data. However, despite this clinical reality, unproven stem cell therapies are being marketed to patients throughout the world. The clinics that offer these services often operate outside of ethical or regulatory oversight and exploit individuals at their most vulnerable by offering unproven treatments for incurable and debilitating diseases.

She raged against the clinics, telling them: “You ought to be ashamed for charging \$40,000 a shot. You prey on people like my brother-in-law who is desperate for help.”

Then came her kicker: “I said, If what you are saying is true, you should get the Nobel Prize. If not, you ought to go to hell. Shame on you.”

But Mr. Gass was undeterred. He was willing to spend his money and go anywhere. What did he have to lose? The worst that could happen, he thought, is that he would have no improvement.

Clinical Trials

Medical Ethics

Politics and Regulation

Forget stem cell tourism: Stem cell clinics in the US are plentiful

It's generally thought that quack stem cell clinics are primarily a problem overseas because the FDA would never allow them on US soil. As a new survey shows, that assumption couldn't be more wrong.

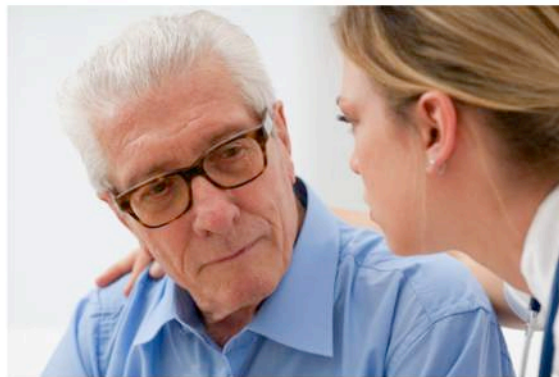
David Gorski on July 4, 2016



Inclusion Body Myositis

by beccyrockett | May 16, 2013 | Auto Immune | 0 comments

Inclusion body myositis (IBM), an inflammatory and degenerative muscle disease, is characterized by slow progressive weakening and wasting of skeletal muscles. Though the cause of IBM is currently unknown, it likely results from both genetic and environmental factors, triggering an autoimmune response in otherwise healthy muscle tissue. The muscle fiber degeneration seen with IBM is characterized by the appearance of vacuoles, or holes in the muscle cells. These holes are created by inflammatory cell invasion of healthy muscle tissue. These cells then discard pockets of abnormal proteins into the muscle fibers, leading to further muscle deterioration and weakness.



Inclusion body myositis is regarded as an age-related disease, most commonly affecting men over 50, but can also target women. IBM typically affects the muscles of the arms and legs, where common early symptoms include frequent tripping, falling, and difficulty manipulating the fingers. Foot drop is seen, but is considered a late manifestation of the disease process. Decreased mobility and ability to care for one's self are the primary concerns that IBM patients must deal with. Although it is not

Research Programs

Orthopedics

- Options for Neck Arthritis and Cervical Disk Disease
- Options for Back Arthritis and Disk Disease
- Alternative to Knee Surgery
- Alternative to Hip Surgery
- Alternative to Elbow & Hand Surgery
- Alternative to Shoulder Surgery

Metabolic Diseases

- Diabetes Mellitus

Ophthalmic Diseases

- Dry Eyes
- Glaucoma
- Retinitis Pigmentosa

Auto-Immune Diseases

- Lyme Disease
- Lichen Sclerosis
- COPD
- Cardiomyopathy
- Hair Restoration
- Crohn's Disease
- Inclusion Body Myositis
- Rheumatoid Arthritis
- Scleroderma
- Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)
- Sarcoidosis
- Polymyositis
- Takayasu's Arteritis
- Temporal Arteritis
- Lichen Planus

Neurologic Conditions

- Amyotrophic Lateral Sclerosis (ALS)
- Multiple Sclerosis
- Peripheral Neuropathy
- Parkinsons
- "Potts Disease" Muscular Dystrophy
- Stroke Recovery and Regeneration
- Cerebral Palsy
- Early Dementia / Alzheimer's Disease

Urology

- Interstitial Cystitis
- Peyronies Disease
- Erectile Dysfunction
- Male Incontinence

Pulmonary / Lung Conditions

- COPD
- Emphysema
- Pulmonary Fibrosis
- Asthma
- Bronchiectasis

Musculoskeletal System and Connective Tissue

- Fibromyalgia

Stem Cell Treatment for Inclusion Body Myositis

Through an intravenous (IV) deployment of the patient's own stem cell rich solution, or stromal vascular fraction (SVF), these cells have been shown to be attracted to areas of tissue degradation and inflammation. These specialized cells then may develop into healthy muscle fiber cells, replacing damaged cells and thus, restoring muscle function. Our group is looking into the effects of a stem cell rich solution (SVF) that is harvested from a patient's own fat and deployed in the same setting, on the progression and status of IBM patients.

At Phoenix Stem Cell Treatment Center we have been studying the effects of SVF (rich in mesenchymal stem cells and growth factors) for a number of conditions. Many of our innovative deployment methods are based on techniques used around the world and also used in the veterinary experience.

All of our deployments are performed by a board certified surgeons with decades of medical experience. We have several types of injections that are IRB Institutional Review Board approved and we have specially designed a deployment that is best suited for conditions which combines systemic with local approaches and emphasizes safety for our patients. The SVF is taken from your own fat and the deployment injections are done intravenously and deployed in the area of concern. Multiple treatments may be required. The entire procedure is done with local anesthesia and takes approximately three hours.

We care about our our patients at Phoenix Stem Cell Treatment Center and take pride in the time we provide to our patients to deploy the best investigational protocols to help our patients achieve their goals.

ALS

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Using Social Networking to Bring Patients, Clinicians and Scientists Together for Investigation of Alternative or Off-Label ALS Treatments.

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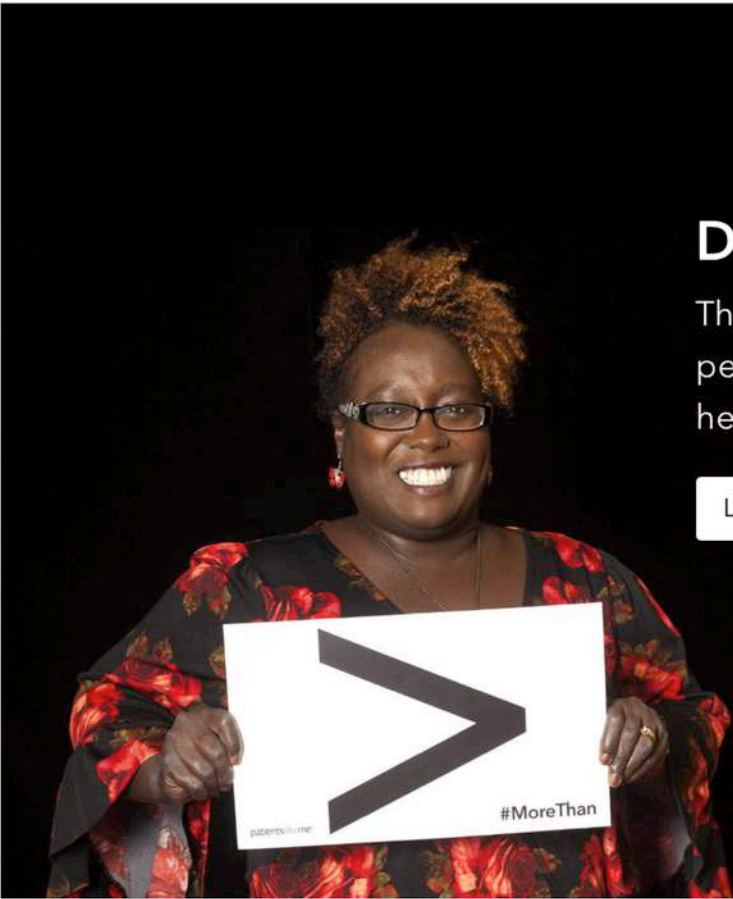


ALSUntangled @ALSUntangled

So glad these talented reporters got interested in ALS and our ALSUntangled and ALS Reversals programs tinyurl.com/ybb73qt8



ALSUntangled helps patients with amyotrophic lateral sclerosis (ALS) to review alternative and off-label ALS treatments. Instructions for using ALSUntangled, as well as our published and active reviews can all be found on this website.



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*"Without our
voices, things
would remain
status quo."*

- Cris, member living with
ALS

[Learn More](#)

ALS-UNTANGLED

ALSUntangled: Introducing The Table of Evidence

ALSUntangled

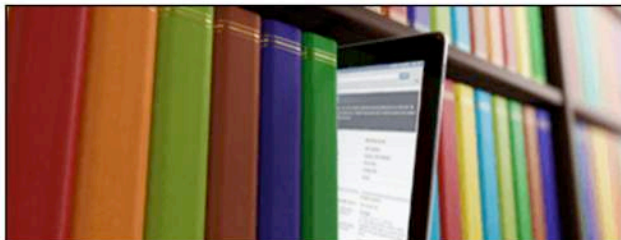
Patients with ALS (PALS) often consider alternative or off-label treatments (AOTs) they read about on the internet (1,2). Internet information about AOTs is not always accurate (3). In 2009, the North American ALS Research Group (ALSRG) started ALSUntangled to scientifically review AOTs and allow PALS to make more informed decisions about them (4). Our review team now consists of 95 clinicians and scientists from 10 different countries. To date we have received requests to investigate more than 160 different AOTs, and have completed 26 reviews which are available free on our website (5).

ALSUntangled has always had a protocol for conducting its reviews. This starts with attempts to contact the proponents of the AOT. Materials used to advertise the AOT are gathered to determine what claims are being made about it. A PubMed search is conducted to determine if there are relevant scientific publications. A Google search is conducted to review any related news items or blogs. The ALSUntangled review team is polled to see if any patients under their care have tried the AOT and what happened to them. The PRO-ACT database and Patient-sLikeMe are queried, and if patients trying the AOT are identified, their available outcome measures before and after the AOT are reported. Attempts are made to visit the clinic offering the AOT to review its infrastructure, oversight and consent processes, interview patients who are trying it and review medical charts on the clinic's 'best successes'. Finally,





Filters activated: Clinical Trial. [Clear all](#)



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[LinkOut](#)

Table I. ALSUntangled TOE.

Evidence category	Grade					
	U	F	D	C	B	A
Mechanistic plausibility	Unknown	Implausible; would violate known principles or laws of biology	Acts on a biological mechanism but it is not clear than this mechanism is relevant in ALS	Theoretically and plausibly acts on an ALS-relevant mechanism in humans	Shown in a peer-reviewed publication to act on a relevant mechanism in pre-clinical model(s)	Shown in a peer-reviewed publication to act on a relevant mechanism in humans
Pre-clinical models (animal or cell models recognized by ALSUntangled reviewers to be relevant to ALS)	None	The only studies available show no benefit	One or more non-peer reviewed studies reporting benefits (published on a website or in an abstract)	One or more peer-reviewed publication(s) reporting benefits in flawed studies (*)	One peer-reviewed publication reporting benefits in a well-designed study (*)	Two or more peer-reviewed publications reporting benefits in well-designed studies (*)
Patient case reports	None	The only reports available show no benefit	Subjective report(s) of benefit without validated diagnoses and/or benefits	One unpublished report of benefit with validated diagnosis and benefits	More than one unpublished report of benefit with validated diagnosis and benefits	One or more peer-reviewed publications reporting benefits with validated diagnosis and benefits
Patient trials	None	The only trials available show no benefit	One or more peer-reviewed publications reporting benefits in a flawed trial (**)	One or more peer-reviewed publications reporting benefits in a well-designed randomized, blinded, placebo-controlled phase I or II trial	One peer-reviewed publication reporting benefits in a well-designed randomized, blinded, placebo-controlled phase III trial	Two or more peer-reviewed publications describing benefits in well-designed randomized, blinded placebo-controlled phase III trials
Risks (harms that occurred on this treatment)	Unknown	At least 5% of exposed patients experienced death or hospitalization	More than 0% but less than 5% of exposed patients experienced death or hospitalizations	At least 10% of exposed patients experienced harms (no hospitalizations or deaths)	More than 0% but less than 10% of exposed patients experienced harms (no hospitalizations or deaths)	No exposed patients appear to have experienced harms

*Animal studies are assumed to be ‘well designed’ when they follow published guidelines (8). When they deviate from these they are considered ‘flawed’.
**Flawed trials means those in which there are identifiable problems with patient selection, randomization, blinding, controls or follow-up. These have ‘high or unclear risk of bias’ according to published criteria (9). Well-designed trials are those that have ‘low risk of bias’.

Table II. Conversion of all prior ALSUntangled reviews into TOE.

	Mechanism	Pre-Clinical	Cases	Trials	Risks
Iplex	D	B	B	D	F
Hickey Wellness	D	U	C	U	B
San Jose Tec	D	U	U	D	F
XCell Center	C	U	D	U	D
Stowe	C	U	U	U	U
Mary Murray	F	U	D	U	U
Aimspro	C	C	D	D	C
Naltrexone	D	U	D	U	B
Spirulina	C	C	U	U	A
Lutimax	C	U	D	U	B
NuTech	C	U	F	U	U
Dean Kraft	F	U	C	U	A
Bee Venom	C	C	D	U	B
MotoTab	C	U	U	U	U
Coconut Oil	C	U	D	U	B
Cannabis	C	C	D	F	U
Lyme Treatment	D	U	D	U	B
Prevagen	C	U	D	U	B
Sodium Chlorite					
A. NP001	A	D	U	C	B
B. WF10	A	D	D	U	C
C. ORAL	F	U	D	U	F
Deanna Protocol	B	C	D	U	B
Fecal Transplants	D	U	D	U	B
Propofol	C	U	D	U	D
Rife Machine	F	U	D	U	B
Vitamin D	C	C	F	D	B
Ursodiol	C	U	D	D	C
Lunasin	C	U	C	U	B

IBM Untangled?

Table I. ALSUntangled TOE.

Evidence category	Grade					
	U	F	D	C	B	A
Mechanistic plausibility	Massage Accupuncture	Ketamine Myo-X		Prednisone Campath Arimoclomol Oxandrolone	Creatine CoenzymeQ10 Bimagrumab	Exercise
Pre-clinical models (animal or cell models recognized by ALSUntangled reviewers to be relevant to ALS)	No valid preclinical models for sporadic IBM					
Patient case reports			Hyperbaric chamber		Creatine*	Exercise
Patient trials		Prednisone	Campath	Arimoclomol Oxandrolone	Creatine* Bimagrumab	Exercise
Risks (harms that occurred on this treatment)			Prednisone Campath Oxandrolone		Bimagrumab Exercise Arimoclomol	Creatine

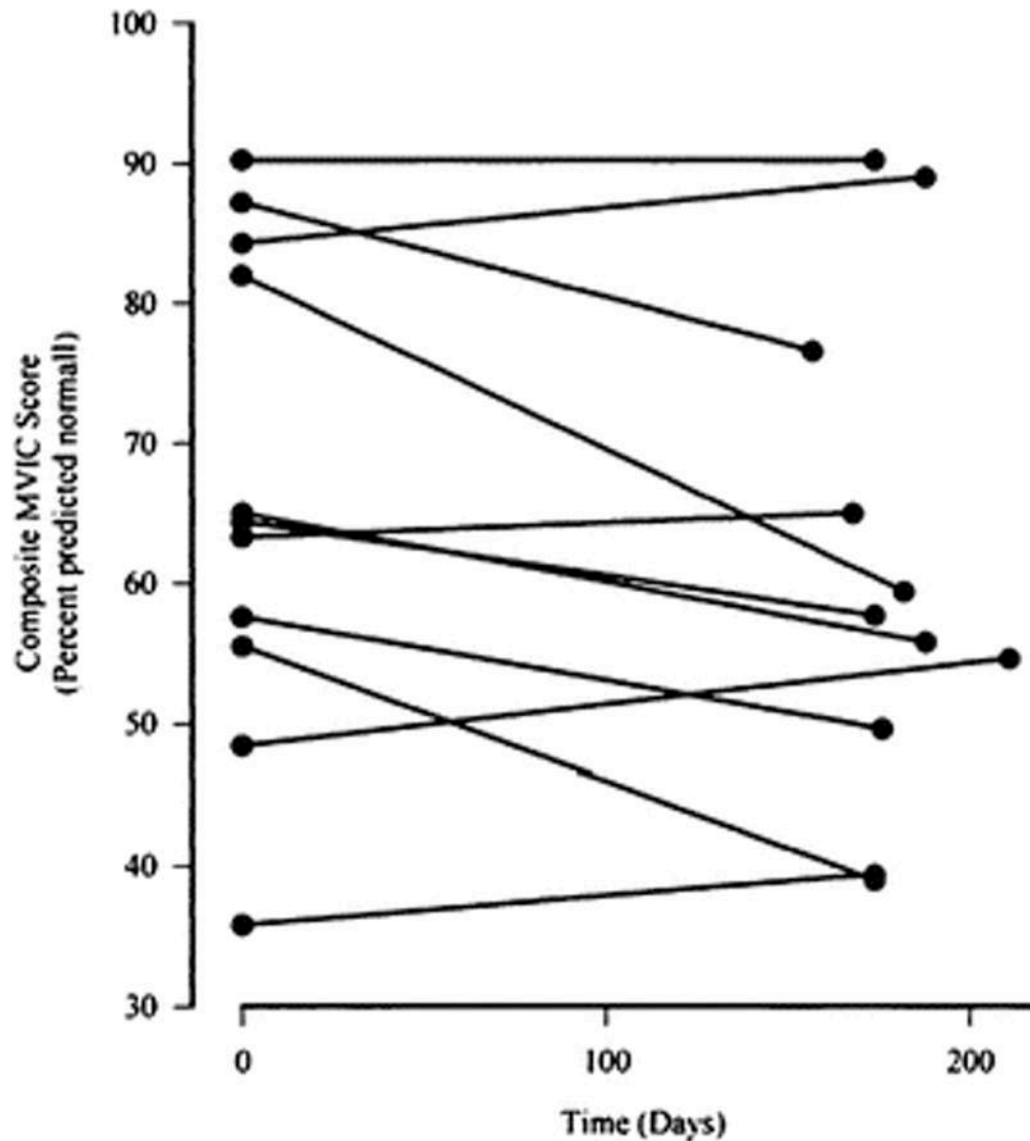
*Animal studies are assumed to be 'well designed' when they follow published guidelines (8). When they deviate from these they are considered 'flawed'.

**Flawed trials means those in which there are identifiable problems with patient selection, randomization, blinding, controls or follow-up. These have 'high or unclear risk of bias' according to published criteria (9). Well-designed trials are those that have 'low risk of bias'.

Statins for prevention of heart attack / stroke / death

Evidence category	Grade					
	U	F	D	C	B	A
Mechanistic plausibility						Statin
Pre-clinical models (animal or cell models recognized by ALSUntangled reviewers to be relevant to ALS)						Statin
Patient case reports						Statin
Patient trials						Statin
Risks (harms that occurred on this treatment)					Statin	

Natural History of IBM



**Quantitative
Myometry**

**Griggs R C Neurology
2006;66:S30-S32**

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Row	Saved	Status	Study Title	Conditions	Interventions
1	<input type="checkbox"/>	Completed Has Results	Arimoclomol in Sporadic Inclusion Body Myositis	Inclusion Body Myositis	Drug: Arimoclomol; Other: Placebo
2	<input type="checkbox"/>	Completed	Efficacy, Safety and Tolerability of BYM338 in Patients With Sporadic Inclusion Body Myositis	Sporadic Inclusion Body Myositis	Biological: BYM338; Biological: Placebo
3	<input type="checkbox"/>	Completed	Study of Long-term Safety, Efficacy Tolerability of BYM338 in Patients With Sporadic Inclusion Body Myositis	Sporadic Inclusion Body Myositis (sIBM)	Drug: BYM338 (Bimagrumab)
4	<input type="checkbox"/>	Completed	Lithium in Inclusion Body Myositis (IBM)	Inclusion Body Myositis	
5	<input type="checkbox"/>	Recruiting	Study of Arimoclomol in Inclusion Body Myositis (IBM)	Inclusion Body Myositis	Drug: Arimoclomol; Other: Placebo
6	<input type="checkbox"/>	Completed	Muscle Strength and Inflammatory Response in Patients With Inclusion Body Myositis	Inclusion Body Myositis	
7	<input type="checkbox"/>	Completed	Double-blind, Randomized, Placebo-controlled Trial of Etanercept for 12 Months in Subjects With Inclusion Body Myositis	Inclusion Body Myositis	Drug: Etanercept
8	<input type="checkbox"/>	Completed	An Extension Study of the Efficacy, Safety and Tolerability of BYM338 (Bimagrumab) in Patients With Sporadic Inclusion Body Myositis Who Previously Participated in the Core Study CBYM338B2203	Sporadic Inclusion Body Myositis	Drug: Bimagrumab; Drug: Placebo
9	<input type="checkbox"/>	Completed	Blood-flow Restricted Exercise in Inclusion Body Myositis	Sporadic Inclusion Body Myositis	Other: Blood-flow restricted training; Drug: Care as usual
10	<input type="checkbox"/>	Active, not recruiting	Natalizumab in Inclusion Body Myositis (IBM)	Inclusion Body Myositis (IBM)	Drug: Natalizumab

Funding Source - FDA OOPD. The purpose of this study is to evaluate the safety and efficacy of the study drug, Arimoclomol in IBM patients.

Condition	Intervention	Phase
Inclusion Body Myositis	Drug: Arimoclomol Other: Placebo	Phase 2

Study Type: Interventional

Study Design: Allocation: Randomized

Intervention Model: Parallel Assignment

Masking: Double (Participant, Investigator)

Primary Purpose: Treatment

Official Title: Phase II Study of Arimoclomol for the Treatment of Sporadic Inclusion Body Myositis (IBM)

Primary Outcome Measures:

- Decline in Inclusion body myositis functional rating scale (IBMFRS) [Time Frame: Change from Baseline to Month 12]
Measured by rate of decline in the IBMFRS between experimental and placebo groups. The IBMFRS is a 10 item questionnaire. Scores for each item range from 0 to 4. There is a total maximum score of 40 and minimum score of 0. The higher the score the better functional status of the person.

Secondary Outcome Measures:

- Manual Muscle Testing (MMT) [Time Frame: Change from Baseline to Month 20]
- Maximum voluntary isometric contraction (MVICT) of quadriceps [Time Frame: Change from Baseline to Month 20]
- Grip and pinch test [Time Frame: Change from Baseline to Month 20]
- modified timed up and go (mTUG) [Time Frame: Change from Baseline to Month 20]
- 6 minute walk test with 2 minute distance captured [Time Frame: Change from Baseline to Month 20]
- Health Assessment Questionnaire (HAQ-DI) [Time Frame: Change from Baseline to Month 20]
- SF-36 [Time Frame: Change from Baseline to Month 20]

- Measured using the Short form health survey with 36 items (SF-36).
- Count of adverse events [Time Frame: Change from Baseline to Month 20]
 - Decline in Inclusion body myositis functional rating scale (IBMFRS) [Time Frame: Change from Baseline to Month 20]
Measured by rate of decline in the IBMFRS between experimental and placebo groups. The IBMFRS is a 10 item questionnaire. Scores for each item range from 0 to 4. There is a total maximum score of 40 and minimum score of 0. The higher the score the better functional status of the person.

Estimated Enrollment:	150
Anticipated Study Start Date:	August 2017
Estimated Study Completion Date:	December 2021
Estimated Primary Completion Date:	December 2021 (Final data collection date for primary outcome measure)

► Eligibility

Ages Eligible for Study: 45 Years and older (Adult, Senior)
Sexes Eligible for Study: All
Accepts Healthy Volunteers: No

Criteria

Inclusion Criteria:

- Meet any of the European Neuromuscular Centre Inclusion Body Myositis research diagnostic criteria 2011 categories for IBM
- Demonstrate being able to arise from a chair without support from another person or device
- Able to ambulate at least 20 ft/6 meters with or without assistive device. Once arisen from the chair, participant may use any walking device, i.e. walker/frame, can, crutches, or braces. They cannot be supported by another person and cannot use furniture or wall for support.
- Body weight of ≥ 40 kg
- Pre-menopausal women must have a negative pregnancy test prior to dosing with study medication.
- If a participant in the bimagrumab study, the participant must be off of the study medication for at least 6 months.
- Able to give informed consent

Exclusion Criteria:

- History of any of the following excludes subject participation in the study: chronic infection particularly HIV or Hepatitis B or C; cancer other than basal cell cancer less than five years prior; or other chronic serious medical illnesses.
- Presence of any of the following on routine blood screening: WBC < 3000 ; platelets $< 100,000$; hematocrit, 30%; BUN > 30 mg%; creatinine > 1.5 mg%; symptomatic liver disease with serum albumin < 3 g/dL.
- History of most recent creatine kinase > 15 times the upper limit of normal without any other explanation besides IBM.
- History of non-compliance with other therapies
- Use of testosterone except for physiologic replacement doses in case of androgen deficiency. Participants must have documented proof of the androgen deficiency.
- Coexistence of other disease that would be likely to affect outcome measures.
- Drug or alcohol abuse within past three months
- Participation in a recent drug study in the last 30 days prior to the screening visit or use of a biologic agent less than 6 months prior to the screening visit.
- Women who are lactating or unwilling to use adequate method of birth control who are not surgically sterile. Adequate birth control includes use of intrauterine device, abstinence, or oral contraceptives or a double barrier method, e.g. condom plus diaphragm will be necessary for both male and female participants.

Where to get information on different therapies?

- Reliable Websites

- Myositis.org
- Cureibm.org
(Kevin Dooley MD)
- MDA website
- For doctors:
 - Uptodate
 - Pubmed



- Be cautious

- Patientslikeme
- Facebook
- Blogs
- Google

Asked what he would like others to learn from his experience, Mr. Gass said, "Don't trust anecdotes."

His sister-in-law had a different reply: "If something sounds too good to be true, it is."

Topic Outline

INTRODUCTION

GOALS OF THERAPY

OUR APPROACH

- Nonpharmacologic therapy
 - Exercise
 - Ambulation and fall prevention
 - Hand strengthening
 - Speech therapy
 - Nutritional support

• Creatine

IMMUNOSUPPRESSIVE
THERAPY IN SELECTED
PATIENTS

- Glucocorticoids
- Methotrexate or azathioprine

AGENTS WITHOUT CLEAR
BENEFIT

PROGNOSIS

INFORMATION FOR PATIENTS

SUMMARY AND
RECOMMENDATIONS

REFERENCES

GRAPHICS

TABLES

- IBM versus polymyositis

Management of inclusion body myositis

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INTRODUCTION

Sporadic inclusion body myositis (IBM) is classified along with polymyositis, dermatomyositis, and autoimmune necrotizing myopathy as one of the idiopathic inflammatory myopathies. However, despite some histologic similarities, the clinicopathologic manifestations, treatment, and prognosis of IBM are clearly distinct from the other disorders ([table 1](#)). (See "[Clinical manifestations of dermatomyositis and polymyositis in adults](#)" and "[Initial treatment of dermatomyositis and polymyositis in adults](#)" and "[Treatment of recurrent and resistant dermatomyositis and polymyositis in adults](#)".)

The treatment and prognosis of IBM will be reviewed here. The clinical manifestations and diagnosis are presented separately. (See "[Clinical manifestations and diagnosis of inclusion body myositis](#)".)

GOALS OF THERAPY

The primary goal of therapy in inclusion body myositis (IBM) is to optimize muscle strength and function. Given the slowly progressive and variable course of the disease, it can be quite challenging to determine if treatment leads to an objective improvement in or stabilization of muscle strength [[1](#)]. It is well known that immunosuppressive medications will lower muscle enzyme levels in IBM patients despite continued progression of weakness, and also that creatine kinase (CK) levels decrease with muscle atrophy [[2,3](#)]. Therefore, CK levels cannot be used to monitor response to therapy in this disease. Based on the existing data, we only consider a trial of immunosuppressive medications in IBM patients with an atypical presentation or in patients with another autoimmune disease.

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SUMMARY - I

- Evidenced-based medicine in myositis
 - Good evidence that immunosuppressive therapy works for DM, PM, and IMNM though very few comparison studies.
 - Very few well performed clinical trials for sIBM
- Why so few clinical trials in sIBM?
 1. Pathogenesis poorly understood!
 - Good drug targets are unknown.
 - Lack of preclinical animals models
 2. Trials are expensive.
 - Pharma will fund clinical trials for rare diseases if can patent.
 - Better surrogate biomarkers need to be developed.
 - “Humanized” mouse models may help.

SUMMARY - II

- For alternative/off-label treatments (AOTs) consider risks/costs vs benefits
 - If plausible mechanism, evidence of efficacy in other muscle diseases, and low risk/cost – **I recommend.**
 - Exercise
 - Creatine
 - Consider: CoenzymeQ10
 - If doctor recommends statin, I recommend rosuvastatin lowest dose.
 - If lack of clear mechanism or efficacy but negligible side effects – **I am neutral/supportive.**
 - Massage
 - Accupuncture
 - Placebo! -- the power of positive thinking.

QUESTIONS?

JHU Management of IBM

- Physical therapy and Exercise
 - Stretching to maintain flexibility/avoid contractures
 - Low impact (eg water aerobics)
 - High frequency endurance exercises
- Orthopedic/Orthotic
 - Carbon fiber Ground Reaction Ankle foot orthosis for foot drop and gait? (GRAFO)
 - Stance Control Orthosis (SCO) – rarely helps
 - Finger splints, tendon transfer
 - Enhance Muscle Strength / Energy
 - Nutrition (minimize waist, maximize muscle)
 - Creatine monohydrate (3 grams/day)
 - Consider Anabolic steroids (oxandrolone)
- Swallowing
 - Learn Heimlich maneuver
 - Speech (SLP) therapy and video swallow study
 - Esophageal dilation if narrowing
 - Surgery if severe / refractory to dilation



Carbon
Fiber
GRAFO