



## HIBM Research Group

California not for profit organization

HRG is an ARM-sponsored non-profit laboratory specialized in research on HIBM. Besides research, HRG offers genetic testing for HIBM and other genetic diseases.



### What does HRG do?

- Accelerate research toward HIBM clinical trials by developing needed biomaterials (e.g. mouse model, gene clone, GNE antibody);
- Develop key collaborations required for trials (e.g. with NIH, MCMRC/Texas);
- Provide patient referrals (e.g.. for the IVIg Trial at NIH);
- Develop/provide patient services (e.g. genetic testing, patient/family counseling, and physician consultations).

### What Has HRG Accomplished?

Recently, HRG has developed a Middle-Eastern genetic testing panel and expanded clinical services to include genetic testing for other serious genetic diseases common in people of Middle Eastern decent.

HRG has validated and optimized several important tests relevant to both animal research and clinical trials. These tests include rapid genotyping of mouse models, muscle sialic acid measurement, developed animal cell culture for testing gene therapy vector bio-activity, and muscle histology/immunohistochemistry tests.

HRG's immediate goals include research plans that will lead to clinical trials on patients with gene therapy and stem cell therapy using a patient's own muscle stem cells.

#### HIBM Test (Simple and Affordable)

Testing for HIBM is simple, painless, and may be covered by your insurance. The test does not require blood draw and is performed by just gently rubbing a Q-Tip inside the mouth (buccal swab). Additionally, the test is 100% confidential and may be performed anonymously. For more information call (818) 789-1033 or send an email to [ddarvish@hibm.org](mailto:ddarvish@hibm.org)

HRG is accredited by CAP and licensed by CLIA and LFS for high complexity molecular diagnostic testing.

-College of American Pathologists (CAP)  
-US. Department of Health and Human Services  
-California Department of Health Services

## Recent Developments

### First HIBM Gene Therapy Trial Result

Following more than two years of development work, FDA approved a single patient gene therapy trial in the last quarter of 2008. This was the result of multi-center collaboration, including Mary Crowley Medical Research Center (MCMRC), Gradalis Inc (Pharmaceuticals), and HIBM Research Group (HRG). The GNE gene clone was provided by HRG during the preclinical development, and the trial is ongoing at MCMRC. Such single patient trials are uncommon, and are sometimes higher risk than more common multi-patient clinical trials. However, important safety and efficacy data can be collected, which may support accelerated development and approval for a larger multi-patient trial. Ms. Julie Ogden, HIBM patient. received several injections in her forearm muscles. Following each injection Julie had increased strength of the injected and surrounding muscles. Unfortunately, the strength gain returned back to baseline levels after a few weeks. Furthermore, this gene therapy method did not show any unforeseen side effects.

This was the first human trial towards developing effective gene therapy for HIBM, and it was a result of many years of hard work by various groups worldwide. The knowledge gained through contributions of all the scientists working on HIBM made this initial trial possible. Read more about Julie's story and watch a video of her trial online at [www.hibm.org](http://www.hibm.org).

### Dr. Tremblay's work towards HIBM treatment

Dr. Jacques P. Tremblay of CHUQ Research Center in Quebec has been working for years on the development of a stem cell therapy for recessive muscular dystrophies. His research group has recently demonstrated in a Phase 1 clinical trial in 9 patients affected by Duchenne Muscular Dystrophy, that the transplantation of myoblasts obtained from a healthy donor restored the expression of dystrophin, the protein which is absent in this type of dystrophy. However, because the transplanted cells were obtained from another person, an immunosuppression had to be used to prevent the rejection of the donor cells. To avoid the requirement for immunosuppression, Dr. Tremblay is currently working on the transplantation of genetically corrected stem cells as a possible treatment for HIBM. With this therapeutic approach, the muscle stem cells would be obtained from a muscle biopsy of the patient himself. The cells would be genetically corrected and then be retransplanted in the patient's muscle without immunosuppression. Dr. Tremblay hopes that this therapeutic approach can lead to a clinical trial in 1 to 2 years.

### HRG Leadership & Consultants

Marvin Pietruszka, MD, JD  
Clinical Laboratory Director, HRG

Yadira Valles-Ayoub, MD, PhD  
Director of R&D, Clinical Molecular Laboratory, HRG

Dr. Daniel Darvish, MD  
Founder and Clinical Consultant, HRG

Marlene E. Haffner, MD, MPH  
Executive Director, Regulatory Affairs, Amgen, Inc.  
Thousand Oaks, California and Washington, DC  
(Former Director of FDA Office of Orphan Products Development)

Wayne Grody, MD, PhD  
Professor, Division of Molecular Pathology  
Pediatrics, and Human Genetics  
UCLA School of Medicine, Los Angeles, CA

Perry Shieh, MD, PhD  
Director, Neuromuscular Program  
UCLA School of Medicine, Los Angeles, CA

Assad J. Kazeminy, PhD  
Founder/CEO Irvine Pharmaceuticals

### Recognition for Outstanding Achievement

Arman Haghighatgoo, BS: Since joining HRG, Arman Haghighatgoo has been involved in different research projects, including manuscript writing and research development. Arman completed his Bachelor's degree in Neuroscience at University of California, Los Angeles (UCLA) in 2006 and subsequently joined HRG, where he contributed to developing molecular assays critical to HIBM. Arman is scheduled to begin pharmacy school in the fall of 2009. He plans to continue his work on HIBM during and after his post-graduate training. We would like to thank Arman for his relentless efforts and continued enthusiasm toward developing a therapy for HIBM.



# ARM

Advancement of Research for Myopathies  
Non-profit 501(c)(3) Org.

# ARM Newsletter

July 2009

### Our Vision

The vision of ARM is to unite communities worldwide in our fight against HIBM. Together, we can significantly shorten the path to the development of an effective treatment. Please join in this fight.

### this issue.



## ARM Launches Dollar Campaign

one minute, one dollar, pass it on.

You can make a REAL difference with very LITTLE money and very LITTLE time. We're asking for two things: give \$1 and pass this email onto people you know. We're trying to raise \$1,000,000 in 3 months to enable human therapeutic trials to fight HIBM, a debilitating disease. We're asking for two things:

**First, go to WEBSITE ([www.hibm.org](http://www.hibm.org)) to donate \$1. Second, send this campaign on to all your friends and family.**

In the Internet age, reaching one million people in that amount of time is surely do-able. If you are interested in becoming a bigger influence in this campaign, host your own dollar collecting campaign in your community, church, workplace, among your friends and family and donate what you've collected as one donation.

HIBM (Hereditary Inclusion Body Myopathy) is a progressive and debilitating muscle-wasting disorder caused by a gene defect. It touches those between the ages of 20 and 40 and, although progression is slow, it typically leads to total disability within 10-15 years. Studies suggest that HIBM may be easier to cure than

## ARM is a partner of Rare Disease Day



Rare Disease Day is a campaign dedicated to increasing awareness with policy makers and the public of rare diseases and of their impact on patients' lives. Rare Diseases are chronic, progressive, debilitating, disabling, severe and often life-threatening.

The hope is that every year on February 28 Rare Disease day will increase awareness of rare diseases, the special challenges encountered by those affected, and the need for research to develop safe, effective treatments or cures. The rare disease patient is the orphan of most health systems often without diagnosis, without treatment and without research.

## Newsletter Program

Our supporters are important to us and ARM wants to keep you informed and up to date on the happenings of ARM. Therefore, ARM has begun two periodic newsletter programs:

1) Update, the newsletter for donors, and 2) Review, the newsletter for healthcare professionals and scientists. These newsletters will include important information regarding scientific updates, ARM funded research, activities, and ARM's financial position. If you or someone you know, would like to receive one or both of the newsletters, please email a request to [arm@hibm.org](mailto:arm@hibm.org) and we would be happy to add you to the distribution list.



# ARM

Advancement of Research for Myopathies

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



Corporations Will Donate on Your Behalf!  
Support ARM while you shop at your favorite stores



Log on to [www.iGive.com/forARM](http://www.iGive.com/forARM) and register to shop at more than 700 stores. Corporations will donate a portion of your purchases to ARM. Among them, you will find brand names such as: Staples, eBay, GAP, Expedia, Barnes & Noble, Toys “R” Us, Overstock.com, Office Depot, Home Depot, Lancôme and Victoria's Secret.

Thank you for supporting ARM and HIBM research by shopping at: [www.iGive.com/forARM](http://www.iGive.com/forARM). ARM will be rewarded if you chose <http://www.iSearchiGive.com/forARM> as your search engine. [www.Igive.com/forARM](http://www.Igive.com/forARM)



Dr. Babak Darvish, an awardee of the Jefferson Award

Dr. Babak Darvish, Co-Founder of ARM, was nominated by co-worker and friend, Crystal and selected as an awardee of the Channel ABC7 (Los Angeles) Jefferson Award. His story, “Doctor determined to cure rare disease”, was profiled Friday, July 24 Jefferson on Channel ABC7.

LOS ANGELES (KABC) – Throughout the year ABC7 recognizes individuals making a difference in their communities. The Jefferson Awards is an award organization co-founded by Jacqueline Kennedy-Onassis in 1972 to encourage and honor people for their contributions through community service. It is an organization based on a simple idea – the belief that one person can make a difference. Through media partnerships across the country, The Jefferson Awards are presented to individuals on both national and local levels. Local winners are ordinary people who do extraordinary things. ABC7 honors one individual in Southern California each month. The honoree receives a Jefferson Awards medal and a \$1,000 donation to the charity or community organization of their choice. Visit, [www.hibm.org](http://www.hibm.org) for more details or watch his profile on, [www.abclocal.go.com](http://www.abclocal.go.com)

When Will We Have A Cure?

A cure for HIBM may mean either stopping the muscle wasting (prevention), or returning the lost muscles (muscle regeneration). We believe therapies for “prevention” will be developed years before therapies for “muscle regeneration.” Each proposed therapy must be tested in people through clinical trials, before we can be certain the treatment is effective for patients. Scientifically, it might be easier to stop the muscle damage caused by HIBM than by other more common muscle diseases. There are several promising proposed therapies for HIBM. Testing these therapies on people are very expensive and a team of specialized scientists. Unfortunately, the amount of funds currently raised by ARM each year is not adequate for clinical trials. Therefore, we have tried and succeeded in raising the interest of well-funded scientists to work towards developing a treatment for HIBM. These scientific investigators, including those at the National Institutes of Health (NIH), have realized the potential for developing an effective treatment for HIBM. To raise the funds needed for finding a cure as soon as possible, we must unite our community. Every dollar brings us closer to a cure. The pace at which we can get there is in YOUR hands.

Corporate Sponsorship Needed

Corporate matching gifts are an easy way to increase the impact of your contributions to ARM. Many companies offer matching gift programs to encourage employees to contribute to charitable organizations. Most of these programs match contributions dollar for dollar, and some will even double or triple the amount of your gift!

Here's how to make a matching gift: Check with your company's human resources department to find out about its matching gift policy. Many employers provide a form which you may fill out and send to us with your contribution. We will verify your gift and return the form to your company, which will then issue a matching gift contribution to ARM in your name.

You may also mail your completed matching gift form to: ARM (Advancement of Research for Myopathies). P.O. Box 261926 Encino, CA 91426-1926 Telephone: (800) ARM-2000 Fax: (818) 337-7250



No Barriers. My Trip to Japan.  
By: Kam Redlawsk

No matter how many times I meet a new HIBM Patient each time is unique in its own right. It was March of 2008 and ARM had just launched their new vision and brand campaign. Not even a year of being with ARM and yet I felt like I've known the patients, the organization and the work for an eternity.

My name is Kam and I am an HIBM patient. At 20 years old I started noticing physical changes in my body. I was active and played soccer my whole life, but suddenly small things like climbing stairs was getting difficult. This was the starting point of a journey. The journey of finding a diagnosis, finding hope and crossing paths with unexpected people who would add so much to my life.

After 7 years of searching it wasn't until I had moved to California that I would feel confident about finding a real diagnosis and more than that meet my first HIBM patient-Dr. Daniel Darvish.

After the ARM Gala I received an email from Mayu Kojima, an Artist, Designer and an HIBM patient In Japan. Mayu expressed that Japanese patients were in the midst of forming their own organization and that they had heard about me and the work I'm doing with ARM. She wanted to know more and see what types of opportunities could be created for patients to spread awareness for this unknown disease.

This email immediately sparked excitement in me and from then on Mayu and I shared our stories, our struggles and our journey. Over the next few months we built a relationship and I felt a strong connection with her. Because of the rarity, it is always special and joyful gift when patients cross paths especially when it is in different corners of the world.

I decided I was going to travel to Japan and meet Mayu and the Japanese Patient Association. Through email our interaction was limited, especially due to language, but in person our week together would be unforgettable.

My boyfriend, Jason and I visited in November of 2008 and the trip was truly amazing. It was more than meeting patients, but rather meeting new life-long friends. Mayu organized our entire itinerary and every day we were meeting new people, giving speeches and gaining new experiences.

A great advantage for patients in Japan is that there are so many patients in one area and together they can do a lot. In other countries patients are few

and not always close to each other. I always think no one can tell the story and express the struggles of a patient more accurately than a patient. I was impressed to see patients come together and spread awareness by telling their story. It is difficult to be open about such a difficult and personal aspect of one's life and on top of that share it openly with the world.

Over the eight days we were in Japan we traveled to various cities like, Tokyo, Kyoto and Osaka. Each city welcomed us with a group of curious HIBM patients. I met the founder of PADM, Aki, a young Japanese woman who was already wheelchair bound. Each patient was at different levels of progression and some were walking and some could barely move.

The purpose of my visit was to build relationships and share what each of our organizations was doing to promote awareness of a cure within reach. As we all sat in a room I was emotionally overwhelmed. As the eyes of each patient, each at their own level of progression, I felt humble and an enormous weight. Every time I meet another patient, it is a reminder of the seriousness of this disease and that there are so many people struggling. It reminds me of the sense of urgency we all need to have, because every minute that passes a patient's body worsens.

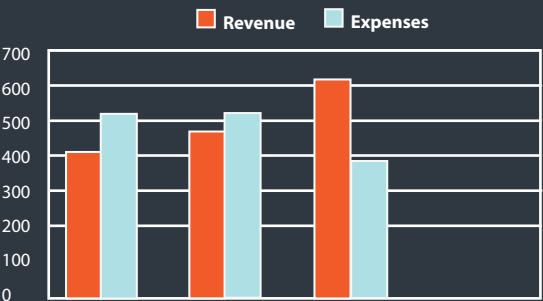


Our experience with HIBM is difficult and though there was a language barrier during my visit, there is no barrier in the language of the heart. I felt a connection with each patient and I think together we felt each other's story. I think the world is more similar than different and it is in those similarities where we find hope, love, understanding, confirmation and connection. I was honored to meet PADM and we stay in touch on a regular basis updating each other in this journey toward a cure. To a patient, it doesn't matter who cures it, but moreso that it will be done. All we can feel is the weakness of the body and the strength of our spirit and there is no room for ego in that equation. When I step back and see the big picture, all that matters is the person involved and the larger vision. It is the journey of sharing and working together for the good of one cause that matters the most and that will remain in the story of our lives. My trip to Japan was amazing and so were the patients.

We are all connected and together we can do it.

How Your Donation Dollars Are Used

At ARM, we strive to stretch every dollar to the maximum value and keep administrative and fundraising costs to an absolute minimum. All your generous donations are received and organized with great care. ARM's qualified committee disperses the donations efficiently as grants to the leading research labs around the world that are devoted in developing therapy and a cure.

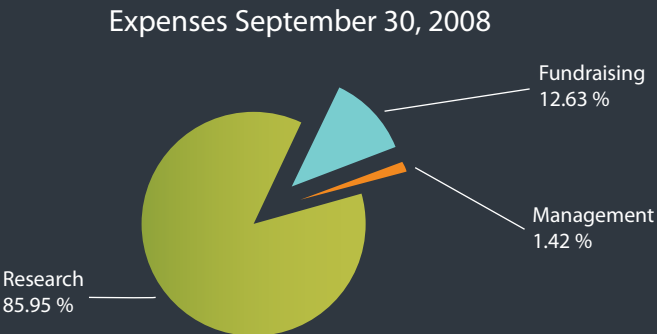


Financial Report

ARM accounting is based on the fiscal year beginning on October 1, and ending on September 30. The following chart shows the revenue and expenses of the years that ended on Sep. 30, 2008, 2007, 2006.

Financial Report

For the year that ended Sep. 30, 2008, the total revenue was \$618,746, which included 47.16% funds raised from special events, 52.64% direct donations, and 0.2% from bank interest. The total expenses were \$390,875, which included 85.95% research grants awarded, 12.63% fundraising costs, and 1.42% management costs.



Calendar of Events

The following events are planned for 2009:

October 29th: Costume Party - Los Angeles (TBA)  
December: Telethon - Los Angeles (TBA)

To receive detailed information, call 1-800-ARM-2000.