



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.



Genetic Consultation and Testing HIBM Test (Simple and Affordable)

Over the past few years, HRG has become a leader in HIBM genetic sequencing and Iranian-Jewish genetic testing services. HRG is accredited by CAP, licensed by CLIA and LFS for high complexity molecular diagnostic testing.

Testing for HIBM is simple, painless, and may be covered by your insurance. The test does not require blood draw and is performed by 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

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

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,

HRG has published collaborative scientific papers with investigators at NIH, Harvard University, Ohio State University, and others. HRG provides genetic testing services internationally as well as domestically for doctors and patients at Cedars-Sinai, Genzyme Genetics, Johns Hopkins University, UCLA, Mayo Clinic, and others.

HRG Leadership & Consultants

Marvin Pietruszka, MD, JD
Clinical Laboratory Director, HRG

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

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

HRG Expansion

For the past two years, HRG has collaborated with local universities to send outstanding students to HRG. This collaborative group brings together academia and a research community through mutually beneficial basic and applied research opportunities. HRG believes that university collaboration is integral in fostering motivation into the biomedical research field to develop hands-on skills and knowledge. HRG has set up a satellite laboratory at California State University, Northridge so students will be able to work, learn, and contribute to helping HRG find a cure for HIBM.

Current Students at HRG are: Rosangela Carbajo, Saghi Esfandarifard, Zeshan Khokher, Daniel No, Chai Saechao, Ishita Shah, Pedram Sinai.

HRG Current Projects

1. Mouse model development for HIBM. Over the past few years, HRG was involved with development of an HIBM mouse model that was useful for proof of efficacy studies on N-Acetylmannosamine (ManNAc) as potential initial therapy for HIBM (Galeano, Klootwijk et al. 2007). The effectiveness of this potential therapy was also verified using another mouse model developed in Japan (Malicdan, Noguchi et al. 2009). Due to non-muscle pathology, late onset muscle disease, and/or unpredictable survival, neither mouse model is optimal for rapid testing of other potential therapies. Thus, HRG, with collaboration of VA/UCLA Medical Center, is developing another mouse model with hope that it will develop muscle disease at an earlier age. This model will hopefully allow for more rapid translational research towards clinical trials. The development of this mouse model was partially funded by Muscular Dystrophy Association (MDA, USA).

2. ManNAc Toxicology Study. Following proof of efficacy in animal studies (Galeano, Klootwijk et al. 2007), an investigational new drug (IND) application was filed with FDA for ManNAc clinical trials for 20 patients affected with HIBM. Due to lack of adequate safety/toxicology data, the clinical trial was placed on hold pending performance of animal toxicology studies on both rodent and non-rodent models for the duration of 3 months. This toxicology study is estimated to cost \$0.5M – \$1M. ARM and HRG are currently working closely to ensure enough funds become available to perform this urgent study in the most cost effective and efficient manner possible.

3. Gene therapy vector development. HRG is involved in both developing and validating promising gene therapy vectors for potential HIBM treatment. This work is the continuation of Julie Ogden (Texas) trial, for which the GNE expression vector was developed in collaboration with Gradalis and MCMRC (Jay, Nemunaitis et al. 2008). This single patient gene therapy trial is currently ongoing, and we hope to see promising results soon.

4. Biomarker Development for HIBM Clinical Trials. One of the most important needs for HIBM clinical trials is development of robust, reproducible, and reliable clinical assays that can be used to determine if a therapy being tested in human trials is biochemically effective. HRG is currently active in improving assays for clinical use. One current priority includes a reliable testing of Sialic Acid levels from a very small amount of human muscle samples obtained by needle-core biopsies. An initial progress of this work was presented at the American Society of Human Genetics (ASHG) meeting in October 2009 and the Society for Glycobiology meeting in November 2009.



ARM Newsletter

April 2010

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 us in this fight.

Who is ARM and What is HIBM?

ARM (Advancement of research for Myopathies) is a 501(c)(3) non-profit organization with the primary goal of speeding up bio-medical research on IBM2, the Autosomal Recessive form of Hereditary Inclusion Body Myopathy (HIBM).

The purpose of ARM is to accelerate biomedical research aimed at developing treatments for HIBM, and skeletal muscle regeneration. ARM's mission is to accomplish this goal in the most efficient manner possible. The vision of ARM is to unite communities worldwide in the fight against HIBM. Confidential genetic testing for HIBM is available at the HRG lab in Reseda for a nominal fee. Together, we can significantly shorten the path to development of an effective treatment.



ARM 10 Year Anniversary

Come Celebrate With Us!

It is no longer about FINDING the cure; it is about FUNDING the cure.

On Sunday June 27, 2010, ARM will host it's 10 Year Anniversary Gala to celebrate accomplishments and share the future of HIBM research. We are at an unprecedented moment in the search for a cure for HIBM. It truly is no longer about finding the cure, it is only about funding the cure. All necessary knowledge is available and all necessary research has been done to move towards clinical trials with multiple patients. To be able to do this, all that is needed is funding. ARM has been working closely with HRG, HIBM Research Group, the only non-profit laboratory working full-time on HIBM and the laboratory with the most detailed knowledge and understanding of HIBM research.

ARM's 10 Year Anniversary will be celebrated with excellent music, food and entertainment, and ARM hopes to celebrate with you! For information and tickets please contact the ARM office or visit the ARM website.

ARM Cordially Invites You To Their
10th Year Anniversary Gala Dinner
On Sunday, June 27th, 2010

Larry Klug Jazz Band
Comedian Maz Jobrani
The Gypsy Kings

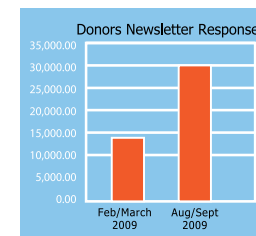
And the premiere of the new ARM promotional video by David Victory

Time: 6:00pm
Location: Marriott Los Angeles Airport, Marquis Ballroom
5855 West Century Boulevard, Los Angeles, CA 90045

\$150.00 per ticket (open seating)
\$175.00 reserved seating

Purchase tickets by phone or online:
1.818.789.1044 / www.hibm.org

Kosher Catering by Sharon



Newsletter Program

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. If you would like us to include other specific information regarding HIBM or ARM in the next newsletter, please email a request to arm@hibm.org.

ARM needs more funds to be able to support new research projects vital for funding clinical trials for HIBM. If every household makes a donation, regardless of the amount, we will be able to fund more research towards developing an effective treatment. Please take a moment and donate as much as you can afford. Your donations are crucial for developing a treatment for HIBM.

How Can I Help?



Join ARM as a Volunteer!

ARM needs volunteers of all ages with different skills, to organize parties, gala dinners, or informative seminars, to write articles, to design and write newsletters, or produce documentaries. Various groups representing different demographics are currently active in forwarding ARM's objective. If you would like to be involved as a volunteer, and join our great journey towards a cure for HIBM, please contact ARM at 1.800.ARM.2000.

Visit www.hibm.org for more ways to help.

Calendar of Events

The following events are planned for 2010:

- May 13, 2010** - ARM's 10th Year Anniversary Gala (NY)
- June 27, 2010** - ARM's 10th Year Anniversary Gala (LA)
- October 28, 2010** - Young ARM's Costume Party (LA)
- December 5, 2010** - Annual Telethon (LA)

To receive detailed information, call 1.800.ARM.2000.

Young ARM Party (Vanguard)

The annual Young ARM Costume Party was on Thursday October 29th, 2009 at Club Vanguard in Hollywood. ARM would like to thank the Young ARM group for organizing the party and their dedication to ARM throughout the years. ARM strives to improve its events and make it bigger and better. For 2010, Young ARM will give a new look to ARM's annual Costume Party by using a different venue and bringing new entertainment and surprises to the event. We thank everyone who attended the event and hope to see you again next year. For more information and to submit your suggestions on ways to improve the costume party please email ARM@hibm.org

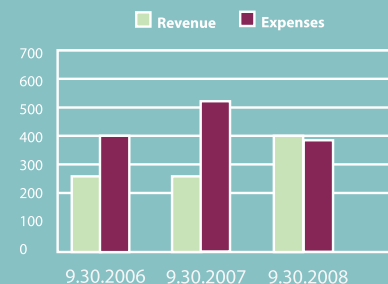
Board of Directors

The Board of Directors of ARM includes the following dedicated and hardworking individuals:

- Nadia Adhami
- Babak Darvish, MD, President and Co-Founder
- Kam Redlawsk, Creative Director
- Mahrokh Eshaghian (NY)
- Minoo Koutal
- Hamid Soleimani, Attorney at Law

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.



Expenses September 30, 2008



ARM Dollar Campaign



ARM 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) 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.

I-Give

Support ARM while you shop at your favorite stores



Log on to 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. ARM will be rewarded if you chose <http://www.iSearchiGive.com/forARM> as your search engine. www.Igive.com/forARM

Financial Report

For the year that ended Sep. 30, 2008, the total revenue was \$424,028 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 67.47% research grants awarded, 26.61% fundraising and 5.92% management cost.

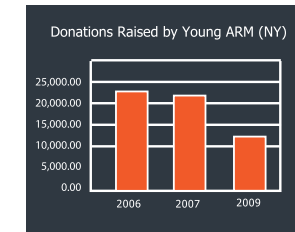


Patient Corner

Roya Nourioff – An Incredible Journey

My name is Roya Nourioff and I have had HIBM for 13 years. I was born in Iran. After graduating from high school in Iran, I was banned from attending college there due to my religion. That did not dissuade me from pursuing my dreams in regard to the study of art. I started privately meeting with famous artists in Iran, studying their techniques and style. It was during this time I noticed difficulty walking. I became progressively worse and doctors were unable to correctly diagnose my condition for years. After numerous consultations, the shocking news came that I have Hereditary Inclusion Body Myopathy (HIBM). HIBM is a group of genetic disorders, which are very rare in the general population, but cause progressive muscle wasting and weakness leading to severe disability. At once, I felt my dreams were shattered. It seemed my whole life was changing and I would surely have to abandon my dream of being an artist. This idea was unacceptable to me and I pushed even harder to hone my skills as an artist. Finally, my family and I decided to come to America to have an easier life. When I came to America three years ago, I met doctors Daniel and Babak Darvish at ARM. Meeting with other patients helped me to not give up. However, it has not been easy, as my illness has progressed and I am now forced to use a wheelchair. Despite this setback, I have been attending college ever since arriving in America. Living in Los Angeles has allowed me the incredible opportunity to have a better life. I am in the final stages of completion on a work of poetry and art that will be my second book of poems. Thus far, my life has been an incredible journey of life experience and lessons, hardships and struggles, setbacks and major leaps forward. I am grateful for every moment and hope to continue my dream of becoming an artist. I wrote a novel, in Farsi, entitled "Hasti". It is based on my own life story, and has been published 3 times in Iran. I wrote the 520 page work, and designed the cover. My second book is ready to be published in America, which is a collection of 260 poems. Four years ago, I realized that my hands have started to become weaker. I cannot paint on a huge canvas as I used to, but it does not make me stop. I draw and paint at any opportunity and hope someday a cure will be found. I have learned to communicate with others through various languages, I found a medium in art that transcends language and spoken word, and is a way for me to express my deepest thoughts and emotions. I see the world differently than others and take normal human interactions and gain inspiration for a poem, painting, or even a sculpture. It connects me to a world that sometimes feels distant due to my disability.

It is no longer about FINDING the cure; it is about FUNDING the cure.



Young ARM New York

Young ARM of New York has successfully raised over \$50,000 for ARM through their efforts since 2006. We truly appreciate their hard work and dedication. The Young ARM of New York Committee has done an amazing job in both spreading awareness, and raising funds for HIBM research. We applaud the members for their organization skills and hard work. For more information you can contact ARM by email.

The Young ARM New York members include the following dedicated individuals:

- David Amirian
- Michael Azizi
- Ben Haghani
- Misha Haghani
- Melody Kohandarvish

Telethon 2009

Currently, ARM is gathering all the donations pledged on Sunday December 6th, 2009 Telethon. If you have not yet sent in your pledged donation please do so. Remember, every dollar brings us closer to a cure. ARM would like to thank all of you for your continued support. The Telethon total revenues will be available in the next newsletter.

Please Mail your Donations to:
ARM
P.O. Box 261926
Encino, CA 91426

Telethon 2009. Major Contributors

Mrs. Mahrokh Eshaghian

Mrs. Mahrokh Eshaghian is the President of ARM in New York and has donated over \$150,000 to ARM over the past two years. She has been responsible for many more contributions to ARM and has actively been involved in organizing ARM events in New York.

The Yashouafar Family (Milbank Real Estate)

The Yashouafar family has been a solid and generous contributor to ARM for many years, and continues to support ARM in every way they can. The location of the 2009 ARM Telethon was donated by the Yashouafar family, which resulted in a very successful fundraising event.

ARM board members extend their warmest gratitude to Mrs. Eshaghian and her loving family, and the Yashouafar family, for their commitment and continued support of ARM. It is with the generosity of active members of our community that ARM is able to continue its fight against HIBM.

ARM Supporters / Sponsors Telethon 2009

- The Yashouafar Family - Millbank Real Estate www.milbankre.com
- Mr. Bijan Pakzad - Bijan Menswear Perfume Jewelry www.bijan.com
- Mr. Bruce Hakimi - Cybernet Communication Inc. www.Cybernetcom.com
- GV Medical Supplies (310) 542-6575
- Sharon Glatt Kosher Catering (818) 342-7204
- Mr. Simon P. Etehad - Etehad Law Firm www.etehadlawfirm.com
- Elat Market (310) 659-7070
- Beverly Blossoms (323) 655-7171
- United Fabrics International, Inc. www.unitedfabrics.com
- Video Audio Center - www.videoandaudiocenter.com
- Mansour Modern - www.mansourmodern.com
- Hollywood Dj - www.hollywooddj.com
- Sadaf - www.sadaf.com
- Golchin - www.golchinproducts.com
- Omid Travel & Tours (310) 477-2031
- Imperial Party Rentals (323) 931-5566