



# ARM UPDATE

March 2009  
Donor Newsletter

Advancement of Research  
in Neurofibrosarcoma (A.R.A.M.)  
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## Recent Developments

**First HIBM Gene Therapy Trial:** 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 HIBM Research Group (HRG) and Mary Crowley Medical Research Center (MCMRC). The GNE gene 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. With great hope we all wait for the final results of this important initial trial. In the next newsletter, we will cover the personal story of the brave selfless individual who is the subject of this trial.

## Current Research

We are at a very promising stage in research. Our immediate goals include two clinical development research plans that will lead to *Phase I Therapeutic Trials* on human patients; one is for gene therapy and the other for stem cell therapy using a patient's own muscle stem cells. Unfortunately, the costs of moving forward with these plans are higher than ARM has been able to raise over the past few years. We estimate that roughly \$2-5 million will be needed for these trials. If adequate funding is available, we are hopeful that both trials could begin within the next two years. With your help we know that we can succeed and we are closer than ever in reaching our goals.

## HIBM Test (Simple and Affordable)

The HIBM test 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 and to make an appointment call (818) 789-1033.

## Calendar of Events

*The following events are planned for 2009:*

- April:** Los Angeles (TBA)
- July:** 4<sup>th</sup> of July Party (TBA)

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

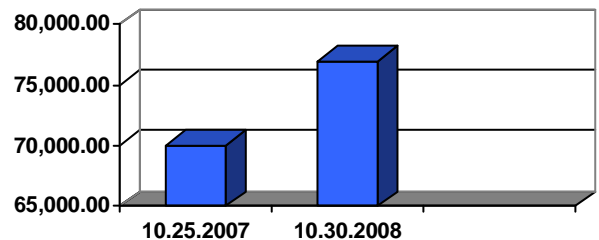
## Young ARM's 8th Annual Costume Party Great Success!

Young ARM's *8th Annual Costume Party*, which took place in October, raised \$77,000 for HIBM research.

Thanks to all Young ARM volunteers that organized this party, and thanks to all guests, HIBM research will move another step closer to a cure. Almost 1200 people attended the event, and of course all were dressed up in the most amazing costumes. Prizes were given to the most original and best costumes, a traditional contest at this party. To see some of the pictures taken at the event, please visit [www.hibm.org](http://www.hibm.org).



Costume Party 2007-2008





Minoo Koutal  
Hamid Soleimani, Esq.  
Rodney Yashouafar, JD

The board members serve a term of one year, and elections are held November of every year. The Board of Directors of ARM is the driving force behind the fundraising efforts and the promise to find a cure for HIBM. They are responsible for awarding funds to research institutes and laboratories in such a way that a cure for HIBM will be achieved as soon as possible.

## What is ARM

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 Myopathies (HIBM).

Founded by HIBM patients in 2000, ARM's mission is to support and inform patients and their families, to raise funds for research, to encourage researchers to study this rare disorder and to ultimately find a cure for Hereditary Inclusion Body Myopathy (HIBM). ARM, a non-profit organization, is the leading source of information, hope and knowledge for HIBM patients worldwide, and is the only non-profit organization specialized in this rare disorder. ARM offers scientific grants to research centers who are interested in working towards developing a treatment for HIBM.

Every year unsuspecting young adults are diagnosed with Hereditary Inclusion Body Myopathy (HIBM), a rare muscle wasting disorder for which there is no treatment or cure yet available. For them and all HIBM patients, ARM offers a ray of hope with research funding, support and an expanding campaign of public awareness. Only with your help can ARM continue its efforts to cure this debilitating disease.

## ARM Mission and Vision

The purpose of ARM is to accelerate biomedical research aimed at developing treatments for IBM2 (recessive HIBM - Hereditary Inclusion Body Myopathy), 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 our fight against HIBM. Confidential genetic testing for HIBM is available at the HRG lab in Encino (using saliva) for a nominal fee. Together, we can significantly shorten the path to development of an effective treatment.

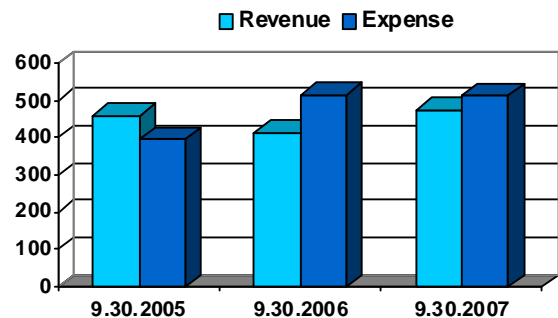
## Board of Directors

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

Nadia Adhami  
Babak Darvish, MD  
Kam Redlawsk  
Mahrokh Eshaghian (NY)

## Financial Report

For the year that ended Sep. 30, 2007, the total revenue was \$474,751, which included 47.16% funds raised from special events, 52.64% direct donations and 0.2% from bank interest. The total expenses were \$513,039, which included 87.26% research grants awarded, 12.62% fundraising and administrative costs.



The total revenue of the year 2008 will be available in the next newsletter.

## 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 who are devoted in developing therapy and a cure.

## 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](mailto:arm@hibm.org).



## Telethon 2008

On December 7, 2008, ARM held their yearly telethon, broadcasted on Persian media, including KSCI 18, satellite TV and radio. A total of \$267,200 was pledged, out of that amount only \$144,933 pledges had the correct information. Currently ARM is gathering all the donations pledged at the 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.

### Private Costume Party for ARM Fundraising

Last October, Mrs. Roya Musighi organized a Costume Party at her residence to raise funds for HIBM research. More than 200 people, all creatively dressed in an amazing array of costumes, attended the party. ARM would like to thank Mrs. Musighi and all involved for this great initiative and for her effort in helping HIBM research move forward!

### A Great Fundraising Initiative!

Harmony, an HIBM patient, has taken the initiative to raise funds for ARM in a fun way! At the office where she works, her co-workers agreed to "pay" to wear jeans on Fridays. They donate \$5 each week, \$20 a month, to ARM. With this great idea, she has raised almost \$3,000 so far, and she hopes to encourage more people to take the initiative with a simple and easy fundraising idea.

### A Special Thanks to the Moinian Family

ARM would like to express special gratitude for the continued generous support of Mr. and Mrs. Moinian and their family. Their selfless dedication to the cause will help us getting closer to a cure for HIBM. They certainly are an example and inspiration for all of us!

### 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, Overstock.com, Toys R Us, 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). Also, ARM will be rewarded if you chose <http://www.iSearchiGive.com/forARM> as your search engine.



For more information about ARM and the telethon, please visit ARM's website or the website of the American Iranian Jewish Federation to read the following documents in Farsi:

- Telethon Report by Mr. Mansour Pour Etehad
- An interview with ARM's Board Members and HIBM Researchers

[http://www.iajf.org/organization/the\\_arm/the\\_arm.php](http://www.iajf.org/organization/the_arm/the_arm.php)



## 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.*

آرم با تلاشی خستگی ناپذیر همچنان هدف بزرگ خود را دنبال می کند و به دنبال نتایج موفقیت آمیز آزمایشات بر روی نمونه حیوانی موش، اکنون با شروع تست های داروئی بر روی انسان به نقطه عطف تحقیقات خود رسیده است. پرداخت هزینه سنگین FDA برای تائید داروهای مورد نظر و رسیدن به مراحل ثمربخش پایانی، در گرو دستان بخشنده شماست. در کنار شما راهی دراز را پیموده ایم و با سخاوت شما نیز نجات بخش نسل خود و جوانان آینده خواهیم بود.

چشم به راه مهر همیشگی شما هستیم و تقاضا داریم جزئیات فعالیت ها و بیلان مالی را در صفحات انگلیسی و یا در سایت آرم [WWW.HIBM.ORG](http://WWW.HIBM.ORG)

مشاهده فرمائید.