



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. 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.



www.hibm.org

Watch ARM Telethon Sunday, December 4, 2011 12-7pm



ARM's yearly Telethon will be broadcasted **LIVE on Sunday, December 4th, 2011 from 12-7pm.**

As usual, the program will include many Persian artists and entertainers, and will be shown on TimeTV, Highvision, ParsTV, TiTV and other stations.

Over the years, the Telethon has become one of the best shows on Persian tv, and has been very important for ARM's fundraising for HIBM research.

Thanks to all donations of the Telethon, research for HIBM has moved forward fast, and different therapies are now being tested on patients in clinical trials. To help make a cure available for all patients, your donations are more than needed, especially now, as we reach the end phase of curing and treating this debilitating disease.

Help ARM to help all patients, and make your donation during the Telethon!

For updated information OR if you want to be involved in the Telethon and volunteer, please contact ARM or visit www.hibm.org

Let's make 2011 the best telethon yet!



HIBM Clinical Trials!
Read story inside!!



Community Involvement

The term "grassroots" is a term used to denote any kind of effort that derives most of its power and reason for being from a community, and from common ordinary people. Grassroots fundraising means an organization invites as many people as possible to give donations of widely varying amounts, small or large. You too can create your own grassroots fundraising project. Gather your community and get involved today!

Bike for Kam (www.bikeforkam.com)



Over the years ARM has reached out to the community and the community has responded by organizing their own fundraising efforts. This method allows 100% of the donations raised from the community to go directly to research while empowering them to make a difference.

One such example is **Bike for Kam**, a grassroots fundraising project that took place in spring of 2011.

Kam has been an HIBM patient for more than ten years, and although it limits her physically she carries on her passion for life as if she didn't have HIBM. An industrial designer and an illustrator, Kam has had the great opportunity of meeting interesting people through ARM and HIBM, who have morphed into great friends.

Once a toy designer, her coworkers didn't quite understand what HIBM was when she told them, but during years of lasting friendships they came to understand the severity and depth of the condition through their personal experience with her. They met her while she was walking and are still with her as she has moved into a wheelchair.

Painful to see their dear friend debilitate, and knowing that something could be done, six guys, Kam, and a whole lot of passion, dreamed up Bike for Kam, a 500 mile coastal bike tour from San Francisco to Santa Monica in six days.

The seven of them put together the entire project, without ARM funding, and during the month of April raised awareness for HIBM while raising over \$22,000. The donations raised went directly to HIBM research.



It was all for a worthy cause, but in the midst they had fun doing it and created lasting memories. Read their story, their six days of journeying, and watch their videos by visiting: www.bikeforkam.com.

Look for BikeforKam 2012!

YOU can make a difference

Like us on Facebook

www.facebook.com/curehibm



Follow ARM on Twitter

www.twitter.com/hibmarm



Be a part of the cure



Donate online:
www.hibm.org

Donate by mail:
P.O. Box 261926
Encino, CA 91426

Patient Corner



My name is Rushabh Madhu Pranlal Desai and I am 23 years old. I am from India. Until a few years ago, I was leading a normal life and

helping people around me do the same. However, I was not destined for a normal life, as I was diagnosed with a rare type of condition called HIBM (Hereditary Inclusion Body Myopathy).

I was recently diagnosed with HIBM and it is very difficult to express what I feel and think about this condition and what it is like living with it. It has had a tremendous impact on everything and everyone in my day-to-day life. It is a frustrating struggle and a never ending painful challenge.

My diagnosis came as a surprising shock to me. My parents, in my best interest, hid the news from me so that I could complete my last year of graduation. When I finally came to know about it I couldn't even imagine in my wildest nightmares that I would have a disease so devastating which has no cure; my dreams, ambitions and wishes all felt shattered in the fraction of that moment.

I have cried endlessly. I have had sleepless nights and got angry because the most simple things in life have suddenly become so difficult, especially when you know that you once could do all those things. I always wanted to drive a car, go on an adventure, travel the world, but now I can only sit and ponder and see others living my life. I want to run, I want to play, I want to be independent. "What is the use of living life when you are fighting with yourself to move your body to do all these things?"

Stepping out of the shock and sorrow, I have diverted my attention towards understanding the disease, and searching for a solution. With the support of my parents, family, faith in God, belief in the doctors, and an optimistic approach I have done everything in my power to overcome this ailment and return to the life that I once took for granted. I yearn to get back to life as before, not just for myself, but for my parents, family and well-wishers who have been very supportive.

I have now successfully completed my graduation and am assisting my father in his business. I take each day as it comes and I am trying to help everyone in the best way I know how.

Though HIBM is very rare it touches my life and others like me in a deep way. We live each day with the hope that there will be light at the end of this dark tunnel. We are very hopeful that one day we would be able to do all the things which we used to do and missed out in during the years of having HIBM. Life is very unpredictable, so please do not waste it!!!

I am told that treatment is possible and with the years of dedication from ARM, HRG and others I am hopeful that HIBM does not need to continue in my life much longer. Thank you to all those who have supported and donated. Please know that it makes a difference even for those across the world like me.

HIBM Clinical Trials

You might have heard the exciting news that finally, HIBM clinical trials have started!

- ARM has been collaborating with Ultragenyx, a start-up pharmaceutical company that specializes in rare diseases. Ultragenyx is working on clinical trials with HIBM patients. HIBM patients don't make enough sialic acid, which is needed for healthy muscles. This first trial is to test the safety and pharmacokinetics of extended release form of sialic acid. For more information visit www.clinicaltrials.gov
- At the same time, NIH (National Institutes of Health) is collecting data in their Natural History study, to better understand HIBM. NIH will start clinical trials with ManNAc. You might be familiar with ManNAc, which is turned into sialic acid by the body. NIH and HRG have been collaborating for years, resulting in the final steps towards a treatment. For more information visit www.nct.nih.gov/27544213
- ARM/HRG is working on pre-trial research and with enough funding, will move forward to clinical trials with gene therapy for HIBM patients.

We are ever so thankful to Ultragenyx to choose HIBM for their first main project, and for NIH Department of Rare Diseases for their work in HIBM research!

This wouldn't have been possible without the relentless efforts of Dr. Daniel Darvish (ARM/HRG), and all other researchers working on HIBM, all donors, volunteers, and all the work that we have done together in the past 12 years!

It is no longer
about **FINDING**
the cure;

it is about
FUNDING
the cure.



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.

Ultragenyx Collaboration (Sialic Acid by mouth)

Since summer last year, we have been working with Ultragenyx, a bio pharmaceutical company focused on rare diseases. The founder of the company has over 18 years experience in successful development of treatments for rare diseases caused by enzyme dysfunction. We are actively involved, and we have made significant contributions by identifying a potential HIBM blood marker to follow disease progression, currently accepted for publication. This test has been used in mouse models treated with sialic acid. We are also providing patient genetic testing and referrals, HRG has been testing patients for phase I and phase II for Ultragenyx. There are two trial sites, one in NY and one in LA.

Bio Marker

We are identifying a potential HIBM blood marker for a new test to follow disease progression. This will be very helpful with following patients after clinical trials, and progression of HIBM in general. The research paper describing this new marker has been accepted for publication. This test is already being used with the animal model treated with sialic acid and we are working on making it available as a clinical test.

Middle-Eastern Genetic Screening

We have obtained IRB approval to collect DNA samples from 1000 individuals of Middle-Eastern Jewish and 1000 individuals of non-Jewish ancestry. We will be screening for HIBM (muscle disease), Usher Syndrome (deaf-blind), Wolman (death by age of 1yr), Dubin-Johnson (Liver disease), G6PD deficiency (favism, or allergic blood reaction, rarely lethal).

Employees of HRG

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 laboratory collaborations at California State University Northridge, UCLA, and Los Angeles Mission College, so students will be able to work, learn, and contribute helping HRG find a cure for HIBM.



Daniel No came to HRG from Los Angeles Mission College and has been working at HRG for nearly three years. Given his professionalism and tremendous attention to detail, he was appointed as a DNA sequencing analysis associate to assist in the discovery and diagnosis of DNA variations responsible for HIBM. Additionally, Daniel has been listed as a co-author for several publications and manuscripts submitted by HRG. Daniel shares the vision and values of

HRG and approaches every day with moral integrity, hard work, selflessness, and teamwork and has clearly proven himself to be an invaluable asset to HRG.

Rosangela Carbajo is currently a CSUN student, and joined HRG almost two years ago. Ever since her arrival, she has devoted her time to learn as much as she could for the benefit of the organization. Her ultimate goal of becoming a physician is her driving force to serving the community. Driven by her talents, Rosangela is a visionary thinker. She reaches for the sky in every task she partakes. She was a vital part in the process of developing the blood marker for HIBM. Currently, she utilizes tissue culture to determine drug effects on human cells.

Zeshan Khokher was the first student who came to HRG from Los Angeles Mission College. With his previous research experience, he was a good addition to the research projects. The tenacity of Zeshan's determination to help society, in many ways as possible, gives him the drive to do the level of work that he contributes to the lab with unalterable precision. He was selected to maintain and manage the entire mice colony to develop an animal that can be used to study HIBM and test potential treatments. Currently, the person who assists Zeshan is Oliver Morton; he is the newest member of HRG.

Arman Haghighatgoo and Chai Saechao are both previous employees of HRG that are now attending the University of California, San Francisco School of Pharmacy. Saghi Esfandiari and Pedram Sinai will both be attending St. George's University School of Medicine in 2012. All four have been an integral part in the research and development in the treatment for HIBM. Congratulations to all of them and they will be greatly missed.



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