



Non-profit 501(c)(3) Org.

ARM Newsletter

May 2013

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

ARM Telethon

Sunday, June 9th, 2013 from 12-7pm



On Sunday June 9th, ARM will be hosting the semi-annual Telethon. Please join us, as we will be having many Persian entertainers and artists. It will be shown on the following stations: Time TV, KSCI Channel 18, Pars TV, and many more.

For the past ___ years, ARM has been able to broadcast successful Telethons yearly. Now, we have the opportunity to host two telethons. The funds that have been raised during the telethon have advanced research tremendously and we are now moving on to clinical trials.

We would like to thank everyone who has continued donating and supporting ARM over the years, because if it were not for you, we would not have made it this far.

If you would like to be involved in the telethon and volunteer please contact ARM or visit www.hibm.org.

Remember, TUNE in June 9th from 12pm - 7pm and help ARM help all patients and make a donation during our LIVE telethon.

Community Involvement

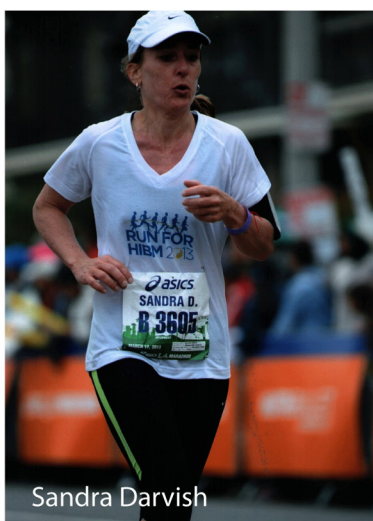


The term "grassroots" is a term used to denote any kind of effort that derives most of its power and reason for being from a community, and from common ordinary people. Grassroots fundraising means an organization invites as many people as possible to give donations of widely varying amounts, small or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

YOU can make a difference

Run for HIBM

(www.crowdrise.com/RunforHIBM)



Sandra Darvish

"Running a marathon always seemed impossible, almost inhuman, to me. Or at least something so far from what I thought I would be able to do, that it would be a great challenge to use as a fundraiser for ARM. And that's how my journey to the finish line of the Los Angeles Marathon started, with a thought.

As HIBM patients like my husband face challenges each and every day, challenging myself to run a marathon would be "peanuts" compared to their struggles. I started training, first just to be able to finish a marathon, but soon my competitive spirit kicked in and I set a time goal for myself. Not only did I have to finish, I had to do it in a reasonable time...

I had no idea how much commitment it takes to train for a marathon! It's like having a part-time job. You don't just go for a run when you feel like it; you HAVE to run. And getting closer to the marathon, you will have to drop all other activities and focus on this one task at hand.

Three more runners were willing to run the Los Angeles Marathon for ARM and help me raise funds for HIBM research: Andrew Voogel, the son of an HIBM patient; Bryan McElroy, a close friend; and Hyung Kim, a colleague of my husband. Together we raised almost \$10,000, which will all be used for HIBM research."

-Sandra Darvish

ARM thanks all the runners for running for HIBM patients!



Andrew Voogel with HIBM patients; Debbie Scott and his mother, Tara Voogel

Bike for Kam 2013

Los Angeles to San Diego

Bike for Kam will be biking from LA to San Diego June 1-2, 2013. If you would like to ride, register @ bikeforkam.com OR show your financial support the month of May/June! Share with all your friends!



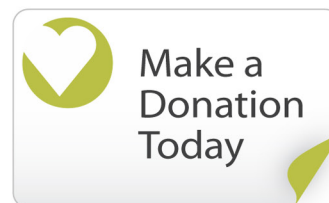
Follow ARM on Facebook and Twitter

www.facebook.com/curehibm
www.twitter.com/hibmarm



Be a part of the cure

Consider making a tax deductible donation today OR become a monthly donor towards the cure!



Donate online:
www.hibm.org

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



Tara Voogel

I was born in a rural village in South America. I am of East Indian origin with six siblings. **FIVE out of the SEVEN** of us have Hereditary Inclusion Body Myopathy (HIBM) and two siblings are unaffected.

As a teenager growing up, I noticed my eldest sister started walking in an unusual way - a waddling kind of gait. We thought it was because she had just given birth to her first child. As time progressed, however, we noticed that she avoided coming to our home, which was only one mile from her house. She used to walk almost daily to visit us and bring me detective magazines to read. I always looked forward to her visits. Sadly to say, we thought that she had become "lazy" and did not want to walk the mile to visit us anymore. Decades later we now understand why she avoided visiting us...

During the late sixties, significant political unrest developed in my country. No proper medical facilities existed to diagnose my sister, and thus she went undiagnosed for many years. It was not until fifteen years later when my second sister acquired the same "waddling" gait pattern that she was able to get a (mis) diagnosis of Limb Girdle Muscular Dystrophy (LGMD). I decided to get myself examined by a neurologist but the tests for LGMD came back inconclusive. As the years went by this pattern of "waddling" gait continued for yet another sister, then

my only brother, and then me. Some of my siblings started showing symptoms in their mid-twenties and some in their mid-thirties.

My symptoms started in my late thirties, which caused me to stop working in the year 2000 due to progressive weakness. Since then I have devoted my time to adaptive exercises and pursuing doctors and researchers to get a definitive diagnosis.

One day in June 2010 I got a phone call from my doctor. She said, "I have news for you". There was a long sigh on my side with my thoughts traveling at lightning speed. "What will she tell me?", I thought. She said, "Your family does not have LGMD but something called Nonaka Myopathy". She then explained to me what Nonaka Myopathy was, and that it is called by different names e.g. HIBM, Distal Myopathy with Rimmed Vacuoles (DMRV), and GNE Myopathy.

Finally, after forty years we have a disease with a name HIBM. I felt such an enormous relief, a kind of freedom it was as if I could fly as freely as a butterfly. In many aspects I felt fortunate. The most immediate benefit was that my family and I have finally gotten a definitive diagnosis. What ails us has been given a name and we can now attempt to learn all about this disease. The team member then referred me to some people, and related resources.

One of those people I contacted was Dr. Daniel Darvish who is an HIBM patient, founder and research scientist from ARM/HRG. I called his office and I told him that I just found out that I have HIBM. He was very patient and empathetic to me. I am unable to express in words how I felt talking to Dr. Darvish that day. I felt very hopeful that a cure will most likely be available in my lifetime. I have connected with someone who not only is a doctor who has HIBM but, most importantly, understands what the challenges are to those who have HIBM. Since, I have met many individuals with HIBM, researchers who are studying HIBM, and have myself participated in a few clinical trials.

I know that getting and funding a cure costs multiple millions, and that this is an almost insurmountable task. For this reason, recently my son and others participated in the Los Angeles Marathon to help raise funds for this cause. Patient involvement is essential, and ARM/HRG has truly been at the forefront of research in attempting to find a cure for people with HIBM.

My parents are in their mid-eighties, and what a precious gift it would be if a cure became available for their children. They have lived through our declining function, our sorrows, tears, thwarted aspirations, and our disappointment in not being able to help them in their old age. They have always kept a positive outlook, and have continued to help us and do so with grace, love, and enduring empathy. My current focus is to stop the progression of my ever-failing muscles, and to help in ways that I can facilitate a cure not only for myself but others here and now and for all future generations.





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.

Be a part of the cure

Research Update

Over the past decade, your support and ARM funding has led to development of critical laboratory bio-materials necessary for HIBM research, including cell culture, gene clone, antibodies, and an animal model for HIBM. Additionally, ARM funding enabled us to develop rapid clinical genetic testing using mouth swab, identifying numerous non-Iranian HIBM patients, and obtaining Orphan Drug Designation by FDA for several treatment options for HIBM. We have developed a biomarker for HIBM that we will use in clinical trials. These significant advances have led to collaborations with both government and biotech industry towards accelerating the development of an effective treatment.

This is just the beginning of clinical trials. HRG/ARM is working hard to make gene therapy trials a reality too. Gene therapy may show a more direct effect on the muscles. Gene therapy may also be more effective long term. To start gene therapy trials, we need to finish preliminary research for FDA approval. This phase of the research still needs to be funded. If finished by the end of the year, we could start gene therapy trials in 2013.

For the past 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, Lucia Sandoval, Jorge Garcia-Figueroa and Sarah Stein.

Our progress to date would not have been possible without the relentless efforts and support of our donors and volunteers! To continue our research efforts, we need your support for these critical stages of our journey. It will be one of the best and most rewarding investments you can make for all HIBM patients, and for keeping our current and future children healthy and productive!



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

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

It is no longer about
FINDING the cure;

it is about **FUNDING**
the cure.