



Celebrating ARM's 10th Anniversary!!

Dear Supporters and Friends,

It is truly a blessing and an immense honor to celebrate ARM's 10 Year Anniversary with you. Your support through all these years is a humbling endorsement and the sustaining source of our success.

We are at an unprecedented moment in the search of a cure for HIBM. It is definitely no longer about finding the cure; now it is about funding the cure. It is no longer about discovery, but rather about development of an effective treatment.

When my brother and I started our fundraising efforts in 1997 in my parents' living room, helping our community to find a cure for HIBM became the mission of our lives.

Since then, ARM has raised over 3 million dollars and has supported vital HIBM research projects all over the world, both financially and with critical research materials. ARM has significantly impacted research across the Globe; from Israel to Japan, from Canada to Australia, from Washington to California.

In 2002, ARM established its own laboratory specialized in HIBM, in order to centralize all HIBM knowledge and to make resources available to all. This laboratory became known as HRG, HIBM Research Group, the only non-profit laboratory in the world working full-time on HIBM. HRG is led by my dear brother, Dr. Daniel Darvish, who volunteers his expertise full-time towards a definitive cure for HIBM.

Through the work of my brother and HRG, we have learned that HIBM affects people from all walks of life, from every corner of the world, but still predominates in Middle Eastern and especially the Iranian communities. ARM has moved research forward from no diagnosis for patients to testing available for everyone, from no hope for patients, to a cure at hand. We have moved research forward with the limited funds we had, and made the best use of every dollar we raised. We have managed to stretch ourselves and accomplish all this at an estimated 1/10th the usual cost.

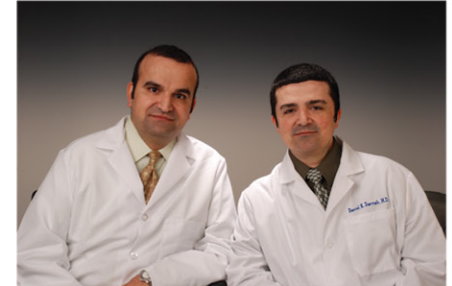
This celebratory newsletter tells the story, where we came from and where we are today.

For the final stages of our remarkable journey, we are taking strides with other institutions to design a unified pathway toward our final destination, which is now nearly in view, causing tremendous excitement.

Because HIBM is such a rare disease, there is little prospect for government and institutional financial support; therefore, we need your continued generous support to reach the finish line and help all patients. Soon, HIBM will no longer lead to muscle wasting and disability, and our future generations will no longer suffer from this devastatingly debilitating disease.

Without your support, we would not be celebrating our successes; without your support, there would be no hope as there is today. Thank you all very much, and we hope to see you all at our future fundraising events and projects!

Dr. Babak Darvish
Co-Founder and President
www.hibm.org



Dr. Babak Darvish, MD, ARM Co-founder and President | Dr. Daniel Darvish, MD, ARM Co-founder and HRG Founder

FDA ORPHAN DRUG STATUS APPROVAL
Read story inside!! ▶▶▶

It is no longer about **finding** the cure; it is about **funding** the cure.

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.

1997-2000

Dr. Daniel and Dr. Babak Darvish, both HIBM patients, spearhead an organized effort toward **public awareness and fundraising** to advance research on HIBM

ARM funds early research and starts collaborations with Hadassah in Israel, and ARM collects blood samples from patients all over the world to accelerate **identification of the gene** responsible for HIBM

ARM starts research funding and collaboration with UCLA and USC

NIH TRND Program Tackles HIBM

The National Institutes of Health (NIH) program for Therapeutics for Rare and Neglected Diseases (TRND) has selected HIBM as one of the five rare/neglected disease pilot projects.

The TRND program was established last year with \$24 million of funding. TRND will work together with scientists, advocates and others to do the required research and testing on drugs before a compound can be tried in humans in a clinical trial.

TRND is funding the needed toxicology studies for N-Acetylmannosamine (ManNAc) as a promising HIBM therapeutic. This toxicology work is needed before clinical trial on human patients can begin under FDA regulations.

2001

ARM-funded research at Hadassah leads to **finding the responsible gene mutation**, using blood samples collected by ARM

ARM recognizes urgent need for greater scientific community involvement and identification of **worldwide expertise** to accelerate development of a treatment for HIBM

Research projects are funded at Hadassah in Israel, UCLA, and USC in Los Angeles

2000

ARM is registered and incorporated as an **independent non-profit** organization, IRS approved as 501(c)(3)

ARM expands fundraising efforts, community outreach, and public awareness for HIBM

2002

Dr. Daniel Darvish decides to give up private practice to be able to **volunteer** full-time for ARM, and devote all his time to research and the quest for a cure for HIBM (2002-present)

To make HIBM biomaterials available for all interested researchers worldwide, **ARM sponsors and establishes its own laboratory**

ARM's laboratory formulates the **first therapeutic hypotheses** including substance therapy (ManNAc), gene therapy, and stem cell therapy, and identifies worldwide leading experts for all three

ARM offers streamlined testing for HIBM and ongoing patient counseling and support

FDA ORPHAN DRUG STATUS APPROVAL

HIBM Research Group (HRG) is two steps closer to finding a cure for the muscle-wasting rare disorder that is gradually robbing HIBM patients of their ability to live healthy and productive lives. In 2010 Dr. Darvish was notified that both of the orphan drug designation applications he filed in February were approved by the U.S. Food and Drug Administration.

The Orphan Drug Act (ODA), is meant to encourage pharmaceutical companies to develop drugs for diseases that have a small market. Under the law, companies that develop such a drug (a drug for a disorder affecting fewer than 200,000 people in the United States) receive specific benefits outlined under the orphan drug act.

"It's a significant boost to our efforts, both in terms of fund raising and partnering with Pharmaceutical companies specializing in clinical development of orphan drug products." Dr. Darvish said.

2003

ARM proposes the potential cause of **muscle wasting**: the lack of sialic acid

ARM sponsors HIBM workshop at UC Irvine

NIH hosts HIBM workshop, with Dr. Daniel Darvish as keynote speaker, inspiring global research, and ARM's own laboratory, now known as **HRG (HIBM Research Group)**, begins collaboration with NIH

ARM and HRG begin development of mouse model, critical biomaterials, and other reagents for HIBM research

Research projects are funded at Hadassah in Israel, USC in Los Angeles, and HRG

2004

HRG laboratory establishes **cell culture and distribution center** for HIBM research samples, much needed biomaterials and reagents to support researchers worldwide

ARM and HRG introduce simple and efficient **mouth (buccal) swab test** for HIBM

HRG receives accreditation by **CAP** (College of American Pathologists), an internationally recognized program for laboratories achieving the **highest standards of excellence in clinical testing quality assurance**

Research projects are funded at Laval University in Canada, Johns Hopkins University, and HRG

2005

ARM and HRG create the **first mouse model** of HIBM, causing tremendous excitement in the scientific communities

HRG innovates and develops a remarkably elegant and **rapid HIBM test** with results in 1-2 days for the Middle Eastern mutation of GNE

HRG develops clinical **sequence analysis of GNE gene**, leading to discovery of new mutations and identification of HIBM patients in different communities across the globe

Research projects are funded at Laval University in Canada, Johns Hopkins University, and HRG

2006

ARM-funded HRG refers patients to NIH and makes **first therapeutic clinical trial** possible for HIBM patients by administering IVIG (IntraVenous Immune Globulins)

Research projects are funded at Laval University in Canada, Hadassah in Israel, University of Wisconsin, UC Irvine, and HRG

2007

ARM launches a new campaign to increase awareness worldwide and to unite HIBM patients from all communities, creating a support group for all who are affected by this disorder

ARM-supported research by HRG and NIH proves **ManNAc** to be beneficial in HIBM mouse model

ARM and HRG support pre-clinical work on **gene therapy** and vector production with biomedical materials

Research projects are funded at Wisconsin, UC San Diego, and HRG

HRG collaborations are ongoing at Mary Crowley MRC in Texas

2008

ARM and HRG support the world's **first** gene therapy trial with one patient (approved on a compassionate basis by FDA) by providing highly purified essential biomedical genetic material

Dr. Babak Darvish receives **MDA grant** to develop additional mouse models for HIBM research

Research fellowship is funded at UCLA, and research project funded at Gulbenkian Science Institute

HRG collaboration with Mary Crowley MRC in Texas

ARM/HRG introduce other organizations to HIBM researchers and share more than 10 years of research experience and results in the hope that research will move forward even faster

2010

ARM and HRG continue their fervent efforts focused on facilitating FDA approval for **multi-patient clinical trials with gene therapy**

HRG receives FDA Orphan Drug designation approval for gene therapy vector and ManNAc, opening the way for a rapid and streamlined drug development pathway

ARM and HRG continue to **raise awareness, offer HIBM testing, support and counsel** patients and their families, seek financial resources and raise money to **FUND** the cure for HIBM

2009

ARM and HRG continue to work on **improving gene therapy delivery and vector design**, and make progress toward improving **tests to measure sialic acid** in HIBM patients in order to measure the effectiveness of gene therapy

Dr. Babak Darvish receives **Jefferson Award** for his dedication to ARM and volunteering for this noble cause to find a cure for HIBM

ARM focus is on funding HRG gene therapy

hope

2011

Only with your financial support will ARM and HRG be able to start multi-patient clinical trials with gene therapy, with promise of stopping muscle wasting in HIBM patients and cure this debilitating disease

It is no longer about **finding** the cure; it is about **funding** the cure

Take part and pride in funding this remarkable journey as we embark on our proverbial **sprint to the finish line!**

www.hibm.org



Patient Corner

Cara Elizabeth Yar Khan, HIBM Patient

"My darling friend Bradley once told me that, in allowing ourselves to be vulnerable and ask for help, is in fact a strength rather than a weakness, because it allows us to love more honestly and be loved in return. These words of wisdom poetically describe my life since realizing that something was happening to me, that something was wrong.

My story is similar to that of other patients, a tale of confusion, uncertainty and disbelief that does not ever sit comfortably, even when the repeated misdiagnoses end with HIBM. My diagnosis came three and a half years ago, over the phone, with even more confusion, uncertainty and disbelief, simply because the doctors could not tell me what was going to happen. And I never imagined it would be like this.

How do I, living with HIBM, put into just a few words here, what I feel and think about this condition when it affects everything, everyone in and every day of my life? The struggle is exhausting; the challenge is neverending and the pain is frustrating.

Since I was young I always loved babies and dreamed of growing up to be a mother with five children; five hungry mouths to feed (I love cooking); five adorable faces to cherish. Without a cure for HIBM, I am not sure that I will ever have even one child.

When I was in grade school I started dancing. By the time I was in university latin dance was my most profound passion, a way to express myself, share with others, teach others, exercise, and feel totally alive! Because of HIBM, I cannot dance anymore, at least not the way I used to. My heart hurts when I hear salsa music and see dancers twirl around but cannot join them. The movements and steps and turns are all inside me. I feel them still and I want to move. But I cannot. I remain to one side, at the edge of the dance floor, balancing on my cane, wishing I did not need the hidden leg braces, which without, I cannot walk straight anymore.

I would be lying if I said I have never burst into tears, fed up and angry, because the most simple movement or task has become so difficult. When you grow up with a full and active life, running, playing sports, hiking, skiing, skating, climbing and dancing, what are you left with when these joys are no longer possible? I will tell you.

You are left with a new found appreciation for the smallest pleasures in life; the courage to ask a total stranger to lend a hand; and an unimaginable tender compassion and loving kindness that comes with sharing this journey with others.

We figure it out, day by day. And by "we" I mean my beloved parents, extended family, friends and supporters. And most important we remain hopeful. Hopeful because we know that thanks to the enduring dedication and hard work of Dr. Daniel Darvish, Dr. Babak Darvish, other patient involvement and ARM's generous volunteers and donors, that a cure IS within reach. They discovered it. Now we, together, are trying to realize its development."



Donate online:
www.hibm.org

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

ARM needs more funds to be able to support new research projects vital for funding clinical trials for HIBM. Please take a moment and donate as much as you can afford. Your donations are crucial for developing a treatment for HIBM.

Become a "Fan" of ours on Facebook!

In ARM's expanding campaign of public awareness we are increasing our visibility within the online social media landscape.



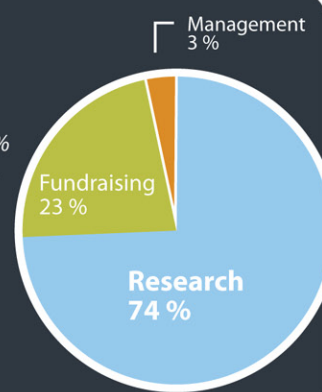
Contribute by spreading awareness by becoming a fan on our Facebook **ARM Fanpage**:
www.facebook.com/curehibm

It's simple and easy. Click "like" and then personally encourage your Facebook friend's list to become a fan, too. Internet era something as simple as "liking" quickly spreads the word virally. You may also find ARM on Facebook's **Cause Page**:
www.causes.com

1999-2009 Breakdown of Expenses

Total Revenue = \$4,283,660

Total Expenses = \$4,216,815, includes 74% research grant awarded, 23% fundraising and 3% management costs.



For the year that ended September 30, 2009:
Total Revenue = \$511,552 which included \$502,098 from contributions and grants, and \$9,454 from interest income. **Total Expenses** = \$723,653, includes 64.41% research grants awarded, 31.26% fundraising and 4.32% management costs.

Future EVENTS: (Los Angeles)

October - **Young ARM's Annual Costume Party**

December - **ARM's Annual Telethon**

ARM Quarterly Newsletter put together by volunteers:

Kam Redlawsk (HIBM patient & ARM Creative Director), Sandra Darvish and Sanaz Imani