



**Advancement of
Research for Myopathies**

Table of Contents

Preface	4
ARM Mission Statement	5
History and Inception of ARM	5
Accomplishments of ARM	8
- Community Awareness	8
- Speeding Biomedical Research	9
- Genetic Testing for HIBM	10
ARM Today	11
- Board of Directors	11
- Young ARM Group	14
- Research Project Grant Program	16
- Scientific Advisory Group	17
- Collaboration with HRG	17
- Financials	18
ARM Future Plans	19
Summary	20
ARM Volunteers	21

**Life's problems wouldn't be called
"hurdles" if there wasn't a way to get
over them. ~Author Unknown**

Preface

Hereditary Inclusion Body Myopathies (HIBM) are a group of muscle wasting disorders, which are uncommon in the general world population. An autosomal recessive form of HIBM is also known as IBM2, and is a very common genetic disorder amongst certain groups of the Middle East. However, IBM2 has also been identified in other minorities throughout the world, as patients of Asian, European, and South American origin have been identified.

Throughout this booklet, the terms HIBM, IBM2 and DMRV will be used somewhat interchangeably to refer to the same clinical syndromes.

HIBM causes progressive muscle weakness and wasting. People affected with HIBM notice muscle weakness between the ages of 20 to 40. As such, it strikes within the most productive years of its victim's lives. In most cases it leads to severe disability within 10 years.

Advancement of Research for Myopathies (ARM) was founded in 1997 to speed up the biomedical research towards development of a treatment for HIBM. ARM has accomplished its goal by raising community awareness, recruiting interested scientists for research on HIBM, and offering biomedical research grant awards. The awareness raised by ARM made more patient samples available to researchers and significantly increased the enthusiasm of biomedical investigators towards research on HIBM. Funds raised by ARM were used to find the gene mutation responsible for HIBM. Since 1998, ARM has awarded a total of over \$900,000 to various institutions such as Hadassah Hospital, UCLA, USC, UCSD, and the Johns Hopkins University.

Preliminary scientific data suggest that HIBM may be much simpler to treat than many other muscle wasting disorders such as Duchenne's Muscular Dystrophy (DMD). Better understanding and ultimately finding a cure for HIBM will undoubtedly shed light and result in at least advances, if not remedy, for other muscle disorders.

We invite you to review this booklet, and join us in our fight against HIBM. We cannot speed up development of a treatment without your support.

For more information on HIBM and the activities of ARM, please refer to the following online sites:

<http://www.hibm.org/arm>

<http://www.ncbi.nlm.nih.gov/htbin-post/Omim/dispim?600737>

ARM Mission Statement

ARM, Advancement of Research for Myopathies, was created to accelerate biomedical research aimed at developing treatments for HIBM (also known as recessive HIBM, DMRV or IBM2 - Hereditary Inclusion Body Myopathy, Mendelian Inheritance in Man Catalogue #600737), and skeletal muscle regeneration.

The mission of ARM is to accomplish this goal in the most efficient manner possible. ARM envisions to fund FDA approved clinical trials with an effective treatment for HIBM by the year 2008. Due to the progressive and debilitating nature of this disease, it is imperative that a treatment is found as soon as possible.

History and Inception of ARM

As young adults, brothers Babak and Daniel Darvish, born less than two years apart, were avid athletes, music lovers and medical students who planned to become surgeons. In their late twenties, both were diagnosed with Hereditary Inclusion Body Myopathy (HIBM), a rare genetic muscular disorder experienced by only several hundred individuals worldwide.



Babak & Daniel Darvish, 1996

Babak first detected something during his medical training years.

"I was an avid guitar player and realized that I was having progressively more difficulty playing the guitar." Around the same time, Daniel, who by then had completed his medical training, noticed he wasn't running and bounding up the hospital steps as quickly as usual.

None of numerous neuromuscular specialists could offer a satisfactory diagnosis. Under the microscope, the muscles of Daniel and Babak resemble a disorder known as Inclusion Body Myositis (IBM), which is

the most common muscle disorder among people over age 55. There are other muscle diseases that resemble IBM, such as hereditary forms of IBM (HIBM), Limb Girdle Muscular Dystrophy (LGMD), and Occulopharyngodistal Myopathy (OPDM).

However, to Babak and Daniel it was clear that they were suffering from something yet unknown. They searched the medical literature and finally discovered a few research papers that seemed to accurately describe their condition. Autosomal Recessive HIBM (IBM2), they learned, typically strikes in the 20s or 30s. It gradually weakens the muscles of the limbs and eventually leads to severe disability. The disease predominantly affects people of Iranian-Jewish descent, who have a 5 -10 percent chance of carrying the gene mutation responsible for HIBM. When both parents carry the gene, their children have a 25 percent chance of developing the disease.

Once they realized what they were facing, the brothers traveled to Israel with blood samples in hand to meet with researchers Drs. Zohar Argov and Stella Mitrani-Rosenbaum, the only scientists actively investigating the genetic cause of this condition.



Babak and Daniel returned home determined to generate support for research on HIBM. The two spoke extensively at public gatherings and numerous events to raise awareness within their community.

"The daunting limitations that threatened scientific progress became apparent," Daniel noted. "There were not enough patients known to provide blood samples for research. There was inadequate awareness of the disease due to its small target population, and there existed

very little interest in the wider general and medical communities. This was an orphan disease, in an orphan community."

There were no realistic avenues to pursue for securing public funding and financial support for urgently needed biomedical research. The necessary resources and required elements to allow for the mere thought of success were simply nonexistent. Daniel, Babak and their family realized that unless they became proactively involved, an effective treatment would not be developed anytime soon.

The non-profit organization Advancement of Research for Myopathies (ARM) had its beginnings in the guest room of the Darvish family home in Los Angeles, California. The daily routine of the Darvish family

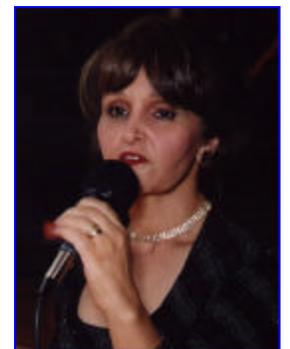
eventually evolved into organized tasks and activities related to the objectives of ARM. Initial funds came from close friends and family. Eventually the need to build awareness and solicit support within the Iranian-Jewish community, which was disproportionately affected with the disorder, was further realized. ARM was rapidly becoming well known in the greater Los Angeles area. Efforts were ultimately aimed at identifying new and possibly misdiagnosed patients, as well as raising funds, to expedite biomedical research. As the name of the organization implies, the focus is to speed up the process that would undoubtedly spare future generations of this potentially debilitating disease, and hopefully lead to preventive medicine for those at risk.

ARM began to contact and catalogue those who had HIBM, or seemed to exhibit symptoms. The two dozen patients initially described in the medical literature ballooned to hundreds across the world in just months after the Darvish brothers began their public outreach and campaign.



In 1998, Mr. Mansour Pourettehad joined ARM following a meeting with Dr. Babak Darvish during the offering of the first grant of ARM awarded to UCLA. Following efforts of Mr. Pourettehad, ARM made a significant financial jump. Along with his contagious enthusiasm, Mr. Pourettehad infused a tremendous sense of dedication to all of the volunteers of ARM. Currently, Mr. Pourettehad is the President and Board Chairman of ARM.

Next to Mr. Mansour Pourettehad, Mrs. Shokouh Darvish has been the strongest driving force behind the fundraising efforts of ARM. As the mother of Daniel and Babak Darvish, Shokouh Darvish has endured many challenges. Prior to her activities with ARM, Shokouh was known for her elegant poetry and writing. Following her involvement with ARM, she earned the "Mother of the Year Award" from the Iranian Jewish Women's Organization of Southern California in Los Angeles in 1999.



Recognizing that the objective of ARM is not of religious or ethnic affiliation, it was incorporated as a nonprofit biomedical research funding organization during 2001. In 2002, ARM was recognized as an established 501-(C)-(3) organization.

Accomplishments of ARM

Community Awareness

The first objective of ARM was to increase the awareness of the community. This was accomplished by many private and public gatherings. In 1999, Mr. and Mrs. Asherian hosted the first fundraising gala, which started the momentum in fundraising efforts of ARM.



Asherian Residence, 1999

Dr. Babak Darvish spoke about HIBM at local hospitals as a member of panels composed of healthcare professionals and patient advocate groups. This led to wider recognition of HIBM as a prevalent genetic disorder of the Jews of Iran. However, HIBM is not exclusive to this group and it is expected that HIBM is still underdiagnosed in the Middle East, Japan and other geographic regions.



Meeting with Moshe Katsav
8th President of State of Israel 2000

Other activities to increase awareness included various radio and TV interviews, which were broadcasted both locally and internationally, and meetings with political leaders. Because Israel has the largest HIBM population known to the medical community, ARM tries to encourage the Health Ministry of Israel to increase funding for this disorder.

Dr. Daniel Darvish spoke about HIBM to close family and friends of patients in Israel. As exciting as it is to finally know what is going on, it is also heartbreaking to realize the relentless progression of muscle wasting and the inevitable disability that lays ahead for many young patients.

During April 2000, ARM had its first internationally televised telethon. This telethon raised over \$250,000 for biomedical research on HIBM, with overwhelming help from volunteers, family and friends.



ARM Telethon 2000

Speeding Biomedical Research

Since its inception, ARM has awarded over \$1,000,000 to various research groups worldwide. For many centers, ARM's support has been the only support available for HIBM research. Such seed money will help fund meritorious HIBM research programs in the US.

In addition to funds, patient's biological samples are critical for biomedical research advances. ARM has played a pivotal role in delivering patient samples to Hadassah Hospital in Jerusalem. Many volunteers, including friends and family members of patients, have helped towards speeding up research to find the gene mutations responsible for HIBM. Such worldwide efforts resulted in the identification of the gene mutations responsible for HIBM during 2001.

Using funds granted and DNA samples provided by ARM, the Israeli research group identified the gene defect that is responsible for HIBM and published their findings in August 2001 in the Nature Genetics journal. The defects are on a gene commonly called "GNE", which codes for the enzyme UDP-N-Acetylglucosamine 2-Epimerase / N-Acetylmannosamine Kinase (GNE/MNK).

Following this discovery, several groups worldwide, including HIBM Research Group in Encino, California, confirmed this finding in all patients. Subsequently, it became more clear that disorders commonly known as Nonaka's Myopathy, or Distal Myopathy with Rimmed Vacuoles (DMRV), and other unknown Quadriceps Sparing Myopathies (QSM) are clinically and genetically the same disorder. GNE/MNK is the rate-limiting enzyme of sialic acid biosynthesis. Although sialic acid and sialylation of cell surface macromolecules have been studied with regards to the immune system, cancer cells, and viral or bacterial infective ability, it is still unclear why these human mutations lead to muscle damage (for further information regarding scientific advances, please contact us or visit our website at <http://www.hibm.org/arm>).

In June 2003, ARM and University of California at Irvine (UCI) held the first Inclusion Body Myositis/ Myopathies (IBM) scientific conference. The purpose of this, and other such conferences planned, is to foster close collaboration amongst the leaders in IBM biomedical research towards speeding up scientific



HIBM Conference at UCI, 2003

discovery in HIBM. Researchers from National Institute of Health (NIH), and University of Southern California (USC), University of California at Los Angeles (UCLA), Irvine (UCI), San Diego (UCSD), The Burnham Institute at La Jolla, California, and Laval University at Quebec, Canada, attended the conference. This was a milestone event, which will undoubtedly lead to significant scientific progress on HIBM.

In November 2003, the National Institutes of Health organized a workshop on HIBM. Researchers from all over the world came together to discuss problems, progress and future research plans for HIBM. Dr. Daniel Darvish was invited as a special guest speaker, as he is a patient and researcher.

Genetic Testing for HIBM

Currently, several centers worldwide offer HIBM Genetic testing. In the United States, HIBM Research Group, a Non-Profit Organization in Encino, California, offers genetic testing at minimal or no cost to patients and donors of ARM. In Europe and the Middle East, Hadassah Hospital in Israel offers HIBM genetic testing. And in Asia, the National Center for Neurology and Psychiatry in Japan (<http://www.ncnp.go.jp>) offers genetic testing for Nonaka's Myopathy or Distal Myopathy with Rimmed Vacuoles (DMRV), which are genetically and clinically the same disorder as HIBM.

Because of the high prevalence of HIBM in minorities of Iranian-Jewish descent, it has been advised that people of this origin who are between the ages of 10 - 40 should be tested. Additionally, even though weakness begins at adult age, it is unclear at what age actual muscle wasting starts, and at what age prevention should start. Answers to



HRG Laboratory in Encino, California

some of these questions can be found with the help of patients and their families. During 2002, HIBM Research Group optimized the technique to perform screening for the IBM2 mutation (GNE-2186t>c) by a simple mouth swab. This eliminates the need for a blood draw for those interested in HIBM genetic testing.

Although not studied yet, based on recent theories, some currently available diet supplements may ultimately prove to be effective in slowing down the progression of muscle wasting. Risks and benefits of each recommendation should be evaluated on per patient basis (contact Dr. Daniel Darvish at (818) 789-1044 for more information).

ARM Today

ARM is a 501(C)(3) nonprofit organization that supports research on IBM2 and GNE/MNK basic science, and development of an effective treatment for the current and future patients. Today, effective muscle regeneration and gene therapy are two essential goals of ARM research funding. ARM places special emphasis on the time/cost value of each research project.

The president and the board of directors of ARM mandate that more than 90% of funds raised by ARM is to be spent directly for HIBM biomedical research and public education. Thus, the administrative costs of both research and organizational management are critically evaluated on a monthly basis. Through such process, ARM has been successful at keeping its administrative cost below 5%.

Board of Directors

ARM currently has 8 board members. They are selected at an annual election based on past and current accomplishments, and their dedication to ARM objectives.



President: Mr. Mansour Pourettehad

Mr. Pourettehad has been with ARM since 1998. He is the current chairman of the board. Mr. Pourettehad is the guardian angel to all patients affected with HIBM. Since his involvement, ARM has excelled to becoming one of the most prominent nonprofit research funding organizations in the Iranian-Jewish community of the United States. Mr. Pourettehad has been involved with fundraising and nonprofit activities in Iran, Israel, and the United States.

"For the health of the society, one has to sacrifice."



Vice President: Babak Darvish, MD

Babak Darvish is one of the original founders of ARM. Following graduation from Medical School, Dr. Darvish completed his specialty training at the UCLA-Multicampus Physical Medicine and Rehabilitation Program in Los Angeles. After realizing that he and his brother, Dr. Daniel Darvish, are both affected with HIBM, they founded ARM with the help of their family and selfless community members. Dr. Darvish now serves as the medical research and HIBM healthcare liaison to the board.

"As physicians and sufferers of HIBM, I have long felt that my brother and I have been charged with the responsibility to bring this devastating disease to a definitive end. As such, the mission of ARM and our own personal life-pursuits are one and the same."



Co-founder: Mrs. Shokouh Darvish

Shokouh Darvish, along with her sons, Drs. Daniel and Babak Darvish, is the co-founder of ARM organization. She is well known for her elegant writing style and her poetic talent in the Iranian community in Los Angeles. In 1999, she received the "Mother of the Year" award from the Iranian Women Organization in USA. Her life story

encourages many women in the community who endure similar difficulties. She is a role model for all family members of people affected with HIBM.

"I feel that I am the mother of all HIBM patients. I cannot rest, until we beat this disease."



Secretary: Mrs. Minoo Koutal

Mrs. Minoo Koutal has been with ARM since 1997. Throughout her life, Mrs. Koutal has served on the board of different nonprofit organizations, and she has had numerous accomplishments in nonprofit activities. Driven by her dedication and compassion, Mrs. Koutal is one of the most active members of ARM community awareness programs and committee.

"If everyone in this world spends part of their time and money for others, we will have a better world to live in".



Treasurer: Nadia Adhami

Nadia Adhami has been an active member of ARM since 1998. She assists in organizing ARM programs, managing funds for grant recipients of HIBM research, and other sponsored activities. Prior to her involvement with ARM, Ms. Adhami has served on the board of several other nonprofit organizations. Ms. Adhami's education includes

a Master of Science in Computer Sciences. Following 10 years of experience at NASA Jet Propulsion laboratory, she became a recipient of a NASA patent.

"After witnessing the devastation that HIBM unleashes on its victims at the prime of their lives, I was propelled to devote myself to this most deserving cause. It has been a personal privilege for me to work

alongside Daniel, Babak, and other dedicated volunteers who are also passionate about ridding the world of this genetic illness. The groundbreaking research we are funding at institutions around the globe will one day alleviate the debilitating effects of this illness and ultimately lead to a cure for our generation and the ones to come."



Board Member: Sunny S. Nassim, Attorney at Law

Sunny Nassim has been an active member of ARM since 2000. She is also the president of the Young ARM Group. For the past two years, she has been an integral part of the ARM community awareness and fundraising campaigns. Ms. Nassim is also volunteering her expertise as legal counsel and advisor for other non-profit

organizations in the community.

"I am deeply committed and attached to this cause, the people and it's goal. I envision a future without this disease based on the dedication of ARM to finding a cure and educating the public."



Board Member: Mrs. Farah Nassim

Farah Nassim has been a member of ARM board of directors since 2001. Mrs. Nassim is also an active member of ARM community awareness programs, fundraising committee and other committees. An inspired mother of two daughters and a proud grandmother of two, Mrs. Nassim has dedicated her time and energy to help

further this cause.

"My desire to help ARM stems from my good fortune of becoming a mother and grandmother at a young age. I feel the pain of all the patients' mothers with HIBM. Therefore, I will do all that is in my power to help support ARM and look forward to beating this disease."



Board Member: Yolanda Moradzadeh Sadighpour

Yolanda Moradzadeh is the newest member of ARM Board of Directors. She is a dedicated mother of two daughters and an active member of society, involved with several other organizations. She has a bachelor degree in Interior and Graphic Design.

"To feel complete is to give back to human kind. I am inspired and dedicated to be a part of the journey to find the cure for this disease."

Young ARM Group

The Young ARM Group was established in 1999 to promote awareness, education and involvement of the younger generation. As the age group most likely to be directly impacted by HIBM, this group will be instrumental as a unified force in the fight against this disease. To date, the volunteers of Young ARM have held various informational seminars, formal lectures and social events to meet their objectives.

Information about activities of Young ARM can be found on their website: www.youngarm.org



President: Sunny S. Nassim, Attorney at Law

Sunny Nassim is the president of Young ARM since 2000. She is also a Board Member of ARM. Under her leadership, Young ARM has become a very active group with clear objectives and successful fundraising efforts.

Vice President: Shawn Gabbaie, JD

Shawn Gabbaie has volunteered his time to organize special events, raise capital from different organizations, and increase the awareness of HIBM and the organization to the general public.

"With hard work, persistence, and dedication we can rid the world of this debilitating disease and provide a healthy life for our future generations. ARM and Young ARM are devoted to make a difference in our community. It is our duty in life to help those in need. The support from our community and the numerous volunteers are critical to the progress of this cause."



Treasurer: Nazy Yadkarim

Nazy Yadkarim has been involved with the Young ARM Group by doing Fundraising and Special Events Planning for the past 4 years.

"I am committed to make a difference in our community, by bringing awareness and education about HIBM. My passion is driven from reaching the goal and vision of removing this disease forever".



Secretary: Mitra Habibzadeh

Mitra Habibzadeh has contributed her time and energy for the Young ARM group since 1998.

"The first time I heard of HIBM, I was devastated. I didn't know that there is such a disease that can easily ruin the life of a young person. I think of the patients as part of my family and I always ask this question from my self as well as others; how would it feel if one of our loved ones had this debilitating disease. My conviction is to help, give time and energy and hopefully with everybody's support, we can find the cure for HIBM."

Young ARM has a network of dedicated volunteers who help organize ARM fundraising events and activities.



Research Project Grant Program

For speeding up HIBM research towards development of an effective treatment, ARM has established a grant program, which adheres to the highest standards of biomedical research. Special emphasis is given to the results/duration/cost value of each project. ARM offers research and research development grants based on strict policies and guidelines, which can be reached online in both HTML and PDF formats at: <http://www.hibm.org/grantguideline/>

Since its inception in 1997, ARM has awarded over \$900,000 in grant awards to various universities and institutions.

Grant Description	Amount
- 1998, UCLA Richard Gatti, MD; Patient identification and sample collection	\$10,000
- 1999, Hadassah, Mt. Scopus, Jerusalem Stella Mitrani-Rosenbaum, PhD; Genotyping and sequencing efforts	\$59,000
- 1999, UCLA Richard Gatti, MD; Patient identification and linkage analysis	\$2,000
- 2000, Hadassah, Mt. Scopus, Jerusalem Stella Mitrani-Rosenbaum, PhD; Genotyping and sequencing efforts	\$52,000
- 2000, Molecular Genetic Lab, LA, CA Chaim Jacob, MD, PhD. Arastoo Vojdan, PhD Sample collection and genotyping efforts	\$50,000
- 2000, UCLA Richard Gatti, MD; Linkage Analysis	\$44,510
- 2000, USC Chaim Jacob, MD, PhD; Linkage Analysis	\$19,826
- 2001, UCLA Richard Gatti, MD; Linkage analysis	\$17,760
- ARM Laboratory -Daniel Darvish, MD., Yong Huo, PhD Laboratory Startup Cost & sequencing	\$30,000
- 2002, Hadassah, Mt. Scopus, Jerusalem - Stella Mitrani-Rosenbaum, PhD Grant #006 - "Mechanism of pathogenesis of GNE/MNK in HIBM"	\$120,000
- 2002, UCSD; Hudson Freeze, PhD Grant #002 - "Initial Analysis and Potential Therapy for HIBM"	\$55,806
- 2002, USC; Valerie Askanas, MD, PhD; W. King Engel, MD Grant #007 - "h-IBM Research"	\$55,000
- 2002, USC; Jianping Zheng, MD, PhD Grant #003 - "Initial Study Of Antisense GNE/MNK And Over Expression Of Mutant GNE in Human Skeletal Muscle Primary Culture System"	\$40,535
- 2003, Laval University; Jacque P. Tremblay, PhD Grant #008 - "Transplantation of genetically modified myoblasts for IBM2"	\$73,000
- 2003, HIBM Research Group - Yong Huo, PhD, Daniel Darvish, MD Grant #010 - "Establishment of Cell Culture & Distribution Center for HIBM research samples and reagents."	\$21,603
- 2003, Johns Hopkins University; Kevin J. Yarema, PhD Grant #005 - "Probing the role of GNE/MNK mutations in HIBM"	\$77,159
- 2001 - present, Every year from 2001 an additional \$65,000 per year has been granted to HIBM Research Group for Research Development, Sample and Reagent Depository Center Development & Maintenance.	\$65,000 p/year
Total Grants Awarded	\$923,199

Currently, both gene therapy and muscle regeneration efforts are given serious consideration for funding. For further information regarding ARM financial data, please read the corresponding financial section of this booklet.

Scientific Advisory Group

Through HIBM Research Group (HRG), ARM benefits from scientific decision making with the help of over 35 renowned scientists throughout the world who act as advisors and perform grant application evaluation on volunteer basis. All grant application evaluations are confidential. To avoid any conflict of interest, HRG requires a statement from all evaluators that they do not have an active relationship with the applicants. All independent evaluators are rotated each season. Those who are applying for ARM grants are not eligible to participate as an evaluator in the same season.

Collaboration with HIBM Research Group (HRG)

Besides funding, ARM also works with and supports HRG in providing research reagents and resources that are difficult to obtain otherwise.

Interested biomedical investigators who are new to the field may feel they are at a disadvantage compared to established investigators in the field. ARM recognizes that biomedical research is a multidisciplinary endeavor and may require the expertise and collaboration of many established as well as new investigators. To level the playing ground, ARM has started an effort to make research resources available to all investigators. This is accomplished through HIBM Research Group, a Non-Profit Organization located at Encino, California.

HRG has established a depository center for maintaining and distributing reagents and resources necessary for HIBM biomedical research. Below is a brief list of what is currently available through HRG:

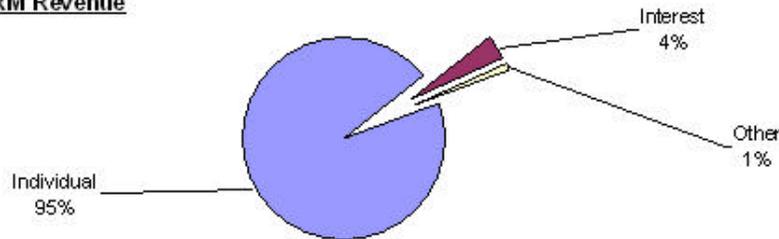
1. Polyclonal Antibody against GNE
2. GNE-M712T homozygous fibroblasts
3. GNE-M712T and wildtype clones
4. HIBM mutation screening for GNE-M712T (Iranian HIBM) by buccal mouth swab.
5. HIBM animal genotype model with GNE-M712T mutation

As research goes forward, there will be increasingly more resources available through HRG.

Financials

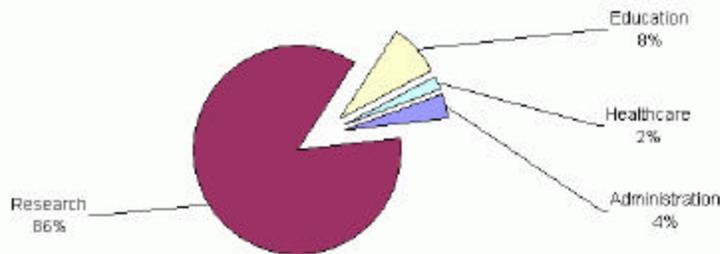
Revenue pie chart, private donations, program revenue, interest earned:

ARM Revenue

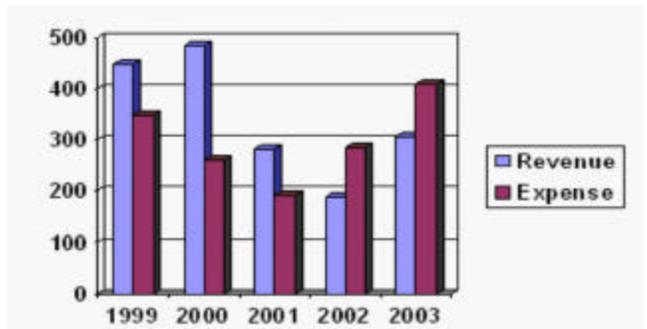


Expense pie chart, grants, fundraising, administration, program expenses:

ARM Expenses & Grants Awarded



Yearly revenue and expense bar chart:



ARM Future Plans

The future plans of ARM continue to be focused on speeding up development of an effective treatment.

ARM has divided the research goals into three parts:

- 1) Clarifying how the gene defects lead to muscle damage;
- 2) Developing an intervention or treatment;
- 3) Muscle regeneration.



Goals 1 and 2 are specific to HIBM.

Goal 3 may apply to many muscle wasting disorders and injuries. Fortunately, many renowned scientists are working on muscle regeneration. ARM has recently started communicating with groups of scientists who are working on muscle regeneration, to raise interest in working on HIBM.

Based on preliminary scientific data, it seems that HIBM would be much easier to treat than the Duchennes (DMD) and many other Muscular Dystrophies. This is primarily because the defective gene is a relatively small gene and may be replaced by currently available gene therapy methods. Additionally, the basic problem seems to be a modest reduction of enzyme activity, which is in contrast to defects in structural protein affecting the dystroglycan complex in other dystrophies. The dystroglycan complex is composed of many large proteins that help to anchor and stabilize the cellular membrane of muscle fibers.

Additionally, a major problem in treating other muscle disorders is the daunting task of treating all muscle groups in the human body. Because HIBM clinically affects specific major muscle groups, HIBM patients may regain much of their physical abilities following treatment of only those specific muscle groups. Furthermore, only a small amount of the GNE/MNK enzyme may be needed to rescue the muscle fiber, which is in contrast to the large amount of other proteins needed in many other muscular diseases. In conclusion, the technology to treat HIBM is available, but the bottleneck at this moment is financial support.

Summary

Although patients have been identified all over the world, HIBM is most common in young members of Middle-Eastern and Japanese communities. Amongst people of Iranian-Jewish decent, this disorder has a prevalence rate as high as 1/500, and a carrier (or heterozygote) rate of roughly 1/15. HIBM is a recessive genetic disorder, which means it can happen to anyone without any warning. More than 98% of the patients have healthy parents. More than 85% of the patients never even heard of HIBM when the doctor told them their muscles are wasting.

Because of the nature of HIBM and its prevalence in a relatively small community, it is an excellent opportunity for researchers. Understanding HIBM will eventually lead to understanding other muscle wasting disorders. Finding a cure for HIBM will undoubtedly be the start of curing a number of other disorders. Fortunately, institutions such as the National Institutes of Health have acknowledged this opportunity and support the work of ARM as much as they can.

ARM Volunteers

The volunteers of ARM include those who selflessly contributed to the objective of ARM over the years, and volunteered in organizing events and raising funds for HIBM research.

ARM strongly depends on its volunteers for all work inside and outside the office. ARM has only two part-time payed employees, ensuring that 95% of the income of ARM is used for research on HIBM.

Although we cannot thank every one of you personally, ARM is extremely thankfull for all your efforts. Without you ARM would not be as succesful as it is now. Thanks again for taking part in our journey to find a cure for HIBM!

**Advancement of Research for Myopathies
(A.R.M.)**

**P.O. Box 261926
Encino, CA 91426-1926
Phone: (800) ARM-2000
Fax: (818) 337-7250
www.hibm.org**