



Patient Advocacy Organizations as Partners in Clinical Trails

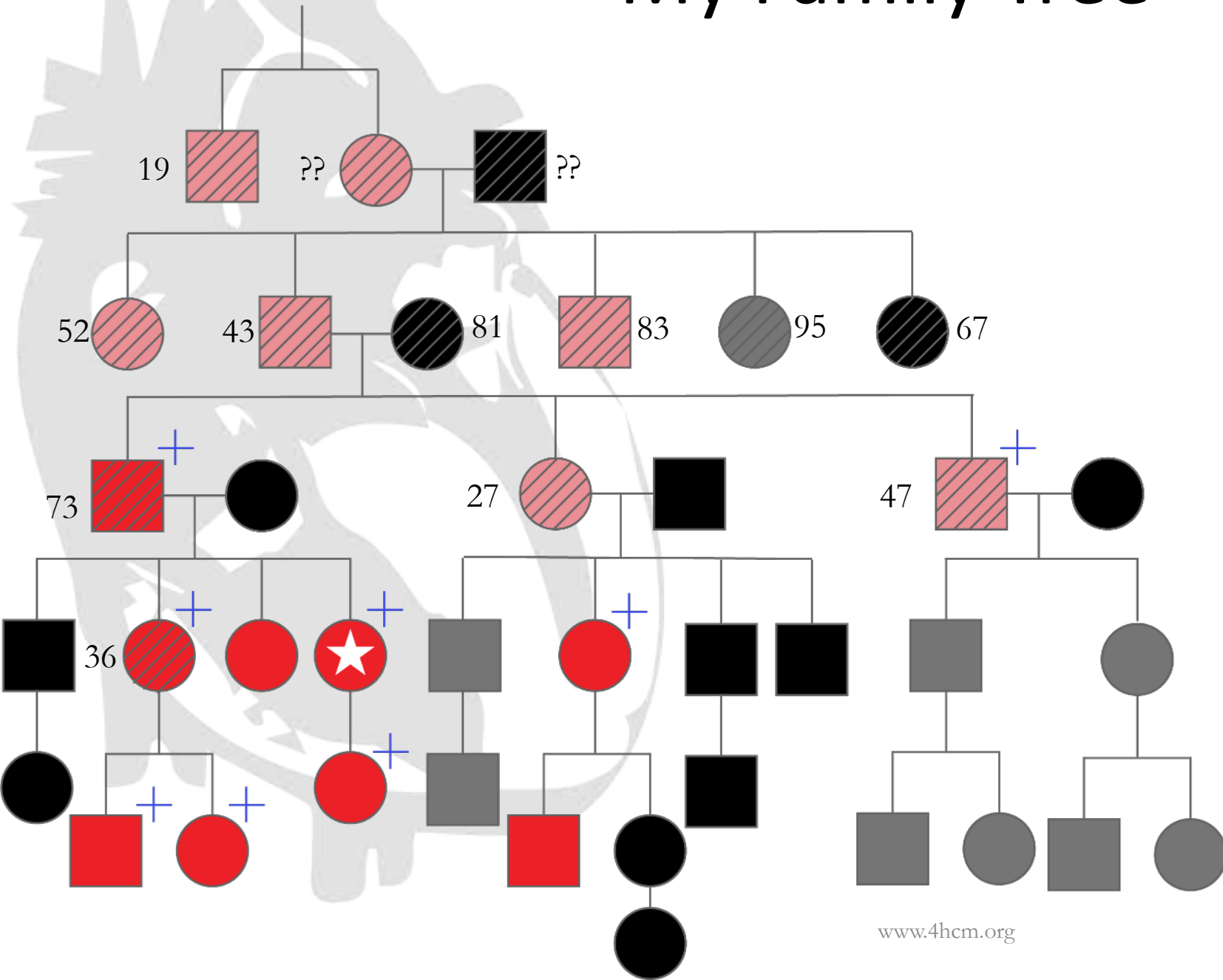
By: Lisa Salberg

CEO and Founder

Hypertrophic Cardiomyopathy Association



My Family Tree



Key:

- — Male
- — Female
- ◻ — No HCM (genetic testing complete or not)
- ◑ — HCM status unknown
- ◒ — Positive genetic test for HCM
- ◓ — Suspected HCM based on cause of death or descendants genetic testing
- +
 — Positive echocardiogram indicating clinical expression of HCM
- ▨ — Deceased



Undiagnosed

SCA Awareness

Community
Awareness

CPR/AED Legislation

Professional
Development

Dispelling the
Myths

Media Outreach



**Risk
Assessment**



Undiagnosed

SCA Awareness

Community Awareness

CPR/AED Legislation

Professional Development

Dispelling the Myths

Media Outreach



Risk Assessment



Diagnosed

HCM Education

HCM Center of Excellence

Living with HCM

Insurance Appeals

Emotional Support

Grief Support

Setting a plan and adjusting the sail

Clinical Trial education

Family Screening





Is HCM really rare?

Japan
N=3,354
20-77yrs
0.17%

Cardia
N=4,111
23-35 yrs
0.17%

Rural Minnesota
N=15,137
16-87 yrs
0.19%

Native Americans
N=3,501
51-77 yrs
0.2%

China
N=8,080
18-74 yrs
0.16%

**General
Population**
1 in 500



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N=3,354
20-77yrs
0.17%

Cardia
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N=3,501
51-77 yrs
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N=8,080
18-74 yrs
0.16%

Echo positive...
How many are
MRI positive?

General
Population
1 in 500

Imaging positive...
What about gene
positive?

70,000
people in
U.S.

At risk for
SCA: 75,000
– 125,000



Japan
N=3,354
20-77yrs
0.17%

Cardia
N=4,111
23-35 yrs
0.17%

Rural Minnesota
N=15,137
16-87 yrs
0.19%

Native Americans
N=3,501
51-77 yrs
0.2%

China
N=8,080
18-74 yrs
0.16%

Echo positive...
How many are
MRI positive?

General
Population
1 in 500

Imaging positive...
What about gene
positive?

700,000
people in
U.S.

At risk for
SCA: 75,000
– 125,000

1 million+
people in
U.S.

General
Population
1 in 250

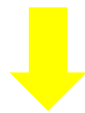
High risk
200,000?



Asymptomatic/
Gene Positive



Profiles in Pathways for HCM



Symptom
Progression



End-stage



Transplant



Atrial
Fibrillation



Sudden
Death
Risk

Is it really?

- Asthma
- Athletically-induced asthma
- Panic attack
- Anxiety attack
- Depression
- Mitral valve prolapse
- An innocent murmur

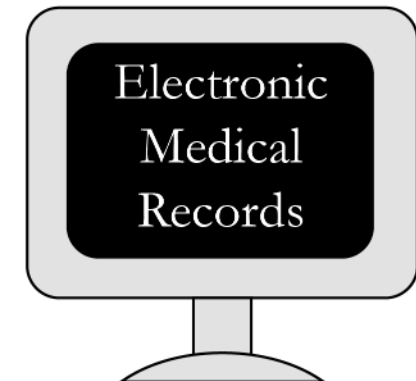
Or is it HCM?

Mitral Valve Prolapse
 Innocent Murmur
 Depression

Asthma

Anxiety

Misdiagnosis	Prevalence	HCM
Asthma	1 in 15 = 76	19.1% (290)
Mitral Valve Prolapse	1 in 50 = 22	13.9% (210)
Anxiety or Panic Attacks	1 in 113 = 10	16.8% (254)
Depression	1 in 18 = 63	18.0% (273)
Innocent Murmur	No data available	51.3% (777)
Other	No data available	15.6% (237)



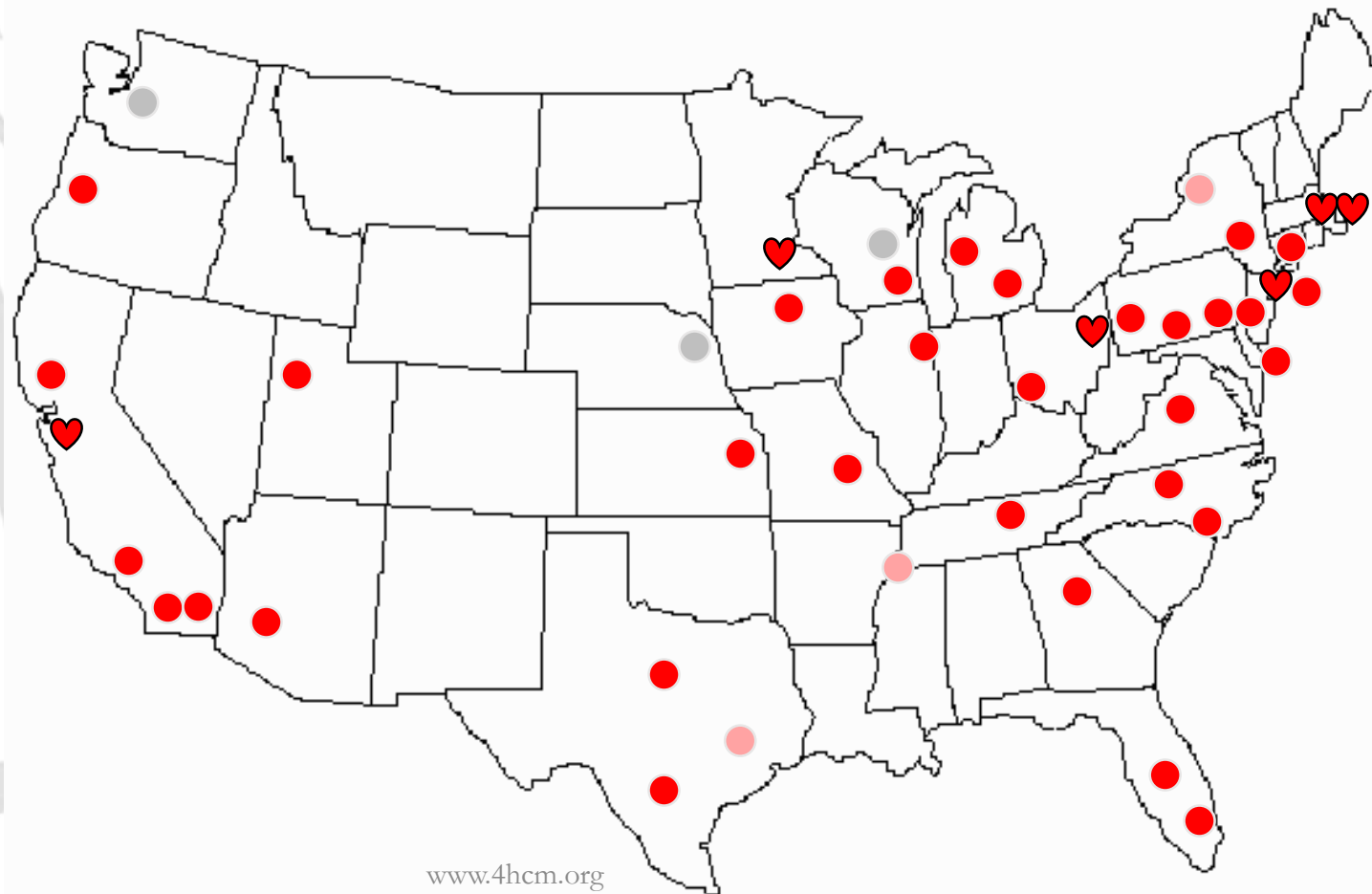
HCM



HCM Center of Excellence Program Destinations

- ♥ COE over 1000+ pts
- COE over 400 pts
- Program under evaluation
- Program pending evaluation

Today an estimated
45,000+ are
managed each year
at HCMA
recognized COE's!



www.4hcm.org





**Clinical
Trial #1**

Education only
Recruitment
Sponsor
controlled
method

**Clinical
Trial #2**

- Education
- Webinars
- Survey based
recruitment

**Clinical
Trial #3**

- Education
- Mailing
 - Social
media
- Survey based
recruitment
- Client hand
off to ensure
connection



**Clinical
Trial #1**

Education only
Recruitment
Sponsor
controlled
method

168 participants:
Took over a year to recruit
Trial terminated

**Clinical
Trial #2**

- Education
- Webinars
- Survey based
recruitment

2200 participants:
1850 retained and in a 5 year
follow up.
Recruitment target reached

**Clinical
Trial #3**

- Education
- Mailing
- Social
media
- Survey based
recruitment
- Client hand
off to ensure
connection

2 arms:
70 participants in arm A
220 in arm B
Recruitment completed in
under 6 months

Thank you:

- To all our HCMA Recognized COE partners
- To the staff of the HCMA
- To the Board of the HCMA
- All the HCMA volunteers

**And... of course to
Brandy and her family**

