Patient Advocacy Organizations as Partners in Clinical Trails

By: Lisa Salberg
CEO and Founder
Hypertrophic Cardiomyopathy Association

www.4hcm.org
Undiagnosed

SCA Awareness

CPR/AED Legislation

Professional Development

Community Awareness

Dispelling the Myths

Media Outreach

Risk Assessment

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Diagnosed

HCM Education
Living with HCM
Insurance Appeals
Grief Support
Clinical Trial education
HCM Center of Excellence
Emotional Support
Setting a plan and adjusting the sail
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

Maron: 2001
70,000 people in U.S.

At risk for SCA: 75,000 – 125,000

General Population
1 in 500

Echo positive…
How many are MRI positive?

Imaging positive…
What about gene positive?

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N=3,354
20-77 yrs
0.17%

Cardia
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Maron: 2001
1 million+ people in U.S.

General Population 1 in 250

Japan
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18-74 yrs
0.16%

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

High risk 200,000?

Echo positive…
How many are MRI positive?

Maron: 2001

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Asymptomatic/Gene Positive Profiles in Pathways for HCM

Sudden Death Risk

Symptom Progression

End-stage

Atrial Fibrilation

Transplant
Is it really?

- Asthma
- Athletically-induced asthma
- Panic attack
- Anxiety attack

Or is it HCM?

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

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