CDG AWARENESS AND DISSEMINATION KIT:

• Kit materials include:
  - A powerpoint with an introduction about Rare Diseases and Living with a Rare Disease (CDG in our case)
  - Fundraising and Conference presentation course

For CDG AWARENESS AND DISSEMINATION KIT materials, please contact: cdgawareness@gmail.com

©Original Idea and Coordination of the CDG awareness and Dissemination project: Vanessa Ferreira
(Portuguese Association for CDG and other Rare Metabolic Diseases)
CONGENITAL DISORDERS OF GLYCOSYLATION (CDG) AWARENESS AND DISSEMINATION PROJECT

QUESTIONS ABOUT PRESENTATION SUPPORT CONCERNING:
- INTRODUCTION ABOUT RARE DISEASES (RD)
- THE PATIENT’S VOICE
- CONFERENCE PRESENTATIONS COURSE

CONTACT: Vanessa Ferreira (sindromecdg@gmail.com)

- HAVING A RARE DISEASE: LIVING WITH CDG
CONTACT: Bas Holten (basesdownunder2003@hotmail.com)

TUTORIAL FOR FUNDRAISING:
- SIMPLE STEPS FOR FUNDRAISING SUCCESS
CONTACT: Andrea Berarducci (maui911@yahoo.com)
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Please, all information should be used with respect and integrity. Thanks.

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PLEASE IF YOU USE THIS AWARENESS MATERIAL WE KINDLY REQUEST YOU TO MENTION AS SOURCE:

CDG AWARENESS DONE BY CDG PATIENT’S VOICE
CONGENITAL DISORDERS OF GLYCOSYLATION (CDG)

Name, CDG patient representative
Country
OUTLINE

• INTRODUCTION ABOUT RARE DISEASES (RD)
• THE CDG PATIENT’S VOICE
• LIVING WITH CDG
• SIMPLE STEPS FOR FUNDRAISING SUCCESS
WHAT IS A RARE DISEASE?

A rare disease in Europe is a disease affecting less than 1 in 2,000 citizens

In the United States, a rare disease is any disease or condition that affects 1 in 1,500 people

- 29 million people affected in the EU
  - 3 million people Spain
  - 3 millions people in France (1 in 20)
  - 600 000-800 000 people in Portugal
  - 3.5 million people in the UK
  - 1 million people in the Netherlands

- 25 million people USA
A number of scientific publications about rare diseases continues to increase.
## CHARACTERISTICS OF RARE DISEASES

<table>
<thead>
<tr>
<th>Chronic</th>
<th>Patients are few and geographically spread</th>
</tr>
</thead>
<tbody>
<tr>
<td>Progressive</td>
<td>Research is fragmented</td>
</tr>
<tr>
<td>Degenerative</td>
<td>Resources are limited</td>
</tr>
<tr>
<td>Life-threatening</td>
<td>Experts are few</td>
</tr>
<tr>
<td>Disabling</td>
<td>Specialised care centres for each disease cannot exist in every country</td>
</tr>
<tr>
<td>Lack or loss of autonomy</td>
<td>Relevant information is little</td>
</tr>
<tr>
<td>Difficult to manage</td>
<td></td>
</tr>
</tbody>
</table>

- Chronic
- Progressive
- Degenerative
- Life-threatening
- Disabling
- Lack or loss of autonomy
- Difficult to manage
RARE DISEASES ORIGINS

- Clinical manifestations
- Causes
- Populations they affect
- Severity and age of onset

DNA (Deoxyribonucleic acid)

80% Genetic origins

- Bacterial or viral infections
- Allergies
- Environmental causes
- Degenerative or proliferative basis

Non-genetic origins
- Suggestions that susceptibility may be genetically determined

The cell

http://www.freedigitalphotos.net/images/view_photog.php?photogid=2888
DIAGNOSIS OF RARE DISEASES

Several Specialists
Several medical exams
Delays in diagnosis

Skin
- Inverted nipples, abnormal subcutaneous fat pads at birth; fat pads disappear with increasing age

Neurologic
- Global developmental delay: axial hypotonia, reduced deep-tendon reflexes, cerebellar atrophy, epilepsy; joint contractures, most children do not walk without support; stroke-like episodes

Nutrition/gastrointestinal
- Feeding problems (need for tube feeding common), gastroesophageal reflux, failure to thrive, hepatitis, liver failure

Ophthalmologic
- Strabismus, impaired night vision, retinitis pigmentosa

Cardiac
- Hypertrophic cardiomyopathy, pericardial effusion

Renal
- Nephrotic syndrome early in life, renal cysts

Hematologic
- Coagulation disorder (thrombosis or hemorrhage)

Box 2: Differential diagnosis of global developmental delay
- Infection (congenital)
- Trauma
- Exposure to toxins
- Uncontrolled seizures
- Asphyxia or hypoxic ischemic encephalopathy
- Structural brain abnormality
- Genetic, syndromic or neurocutaneous disorder
- Inborn error of metabolism
- Endocrinopathy
- Severe environmental deprivation or neglect
- Malnutrition or failure to thrive

Underrecognized

BARRIERS IN ACCESS TO MEDICAL AND SOCIAL SERVICES
OUTLINE

• INTRODUCTION ABOUT RARE DISEASES (RD)
• THE CDG PATIENT’S VOICE
• LIVING WITH CDG
The CDG Patient’s Voice

Links from different associations:
Portugal: http://sindromecdg.orgfree.com/
Spain: http://webs.ono.com/aescdg/SINDROME_CDG/Bienvenidos.html
France: http://www.lesptitscdg.org/
USA: http://www.cdgfamilynetwork.org/
Canada: http://www.thefog.ca/
   http://thelittlefightersfoundation.com/
Germany: https://www.cdg-syndrom.de/
Denmark: http://www.cdgforeningen.dk/
Sweden: http://www.cdgs.se/

CDG active patient representatives nucleus:
Netherlands, UK, Ecuador, Brazil, Finland, Australia
The CDG Patient’s Voice aims to:

- **Improve and enhance understanding and awareness** of CDG amongst the general public and medical professionals.
- Facilitate tools for Patient Advocacy at International, national & EU levels.
- Support, promote and disseminate information to affected CDG families.

**FINAL GOAL:**

**TO INCENTIVE AND SUPPORT RESEARCH**

**TO ESTABLISH A CDG NETWORK: PATIENTS, RESEARCHERS AND MEDICAL DOCTORS = RESULT!**
Examples of CDG patient organizations initiatives:

http://www.rarediseasecommunities.org/en/community/cdg
Examples of CDG patient organizations initiatives:

- **Scientific, Medical and Family Meetings**

Glycokids CDG – Family-Meeting 2011
Examples of CDG patient organizations initiatives:

- Information/Dissemination for families, Medical Doctors and Researchers:
  - Newsletter
  - Access to scientific articles
  - Leaflets
  - Webpage
  - Practical guide for CDG families

- Awareness
  - High School and Universities projects
  - CDG Community Store
    - http://www.cafepress.com/CDG_Community
  - Fairy-tale about CDG: Glycoland and the coloured antennas

- Understanding
  - Families give seminars: Having a rare disease
OUTLINE

• INTRODUCTION ABOUT RARE DISEASES (RD)
• THE CDG PATIENT’S VOICE
• LIVING WITH CDG
Having a rare disease: LIVING WITH CDG
29 month 200?
15 November 200?
• You are entering a ‘new’ strange world...

• ... with ‘1001’ questions,

• But no answers what so ever!

• And the world outside keeps on going, and going and...
Finally at home..!! Although..?!?!

“...Also the feeling of uncertainty is coming back. Especially now that – name of the child- is sleeping in his bed for the first time. No monitor just to check once a while, no sister who can call a doctor. No doctor that checks your child. Learning to trust on what you see, learning to trust on what your hear, learning to trust your child... Just learning to trust on your own intuintion makes it even more complicated”

24-7

Non stop...
• From living to ‘survival’
Structuring & Organizing
<table>
<thead>
<tr>
<th>Activity</th>
<th>Hours</th>
</tr>
</thead>
<tbody>
<tr>
<td>Visiting doctors</td>
<td>1</td>
</tr>
<tr>
<td>'Happiness'</td>
<td>1</td>
</tr>
<tr>
<td>Administration</td>
<td>1</td>
</tr>
<tr>
<td>Household</td>
<td>2</td>
</tr>
<tr>
<td>Patients tools</td>
<td>2</td>
</tr>
<tr>
<td>Medication</td>
<td>3</td>
</tr>
<tr>
<td>Therapies</td>
<td>7</td>
</tr>
<tr>
<td>Taking rest &amp; sleep</td>
<td>12</td>
</tr>
</tbody>
</table>

*24-7 Non stop...*

*‘Our 24 hour care language’*
“What did you miss??”

‘Questions & answers’ don’t match!!

supply and demand do not exclude each other
Protocols
Rules
Management
Laws
Agreements
Regulations etc...

knowledge and experience is up for grabs...
Knowledge and experience of Parents
Thanks to:

- Families and members
- We are grateful to .....
Sources of photos:

• Slide 5: from the left to the right
• Slide 20:  
  http://www.freedigitalphotos.net/images/view_photog.php?photogid=2125

• Slide 21  
  http://www.freedigitalphotos.net/images/view_photog.php?photogid=3062

• Slide 22  

• Slide 23  

• Slide 28  
  http://www.freedigitalphotos.net/images/view_photog.php?photogid=3062
  http://www.freedigitalphotos.net/images/view_photog.php?photogid=3062

• Slide 29  
  http://www.freedigitalphotos.net/images/view_photog.php?photogid=3062

• Slide 30  
  http://www.freedigitalphotos.net/images/view_photog.php?photogid=2664
Before starting the awareness and dissemination talks

• Think on the targeted places where you plan to do the awareness and dissemination activities

• Send them a letter explaining who you are and the goals that you want to achieve;

• Inform us about the GREAT results that you got with your activity by sending an e-mail to cdgawareness@gmail.com

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CDG PATIENT’S VOICE

AIMS:

TO INCENTIVE AND SUPPORT RESEARCH

TO ESTABLISH A CDG NETWORK: PATIENTS, RESEARCHERS AND MEDICAL DOCTORS=RESULT!

Simple Steps TO Fundraising success
Sources of Funding - Overview

- Individuals

- Foundations – Large, Community, Corporate

- Corporations – Large, Community-based

- Government
Fundraising Basics

• Set a goal
  – Identify the need
  – Be clear about what the money will be used for

• Target an audience
  – Know or learn who your audience is
  – What will attract your audience to the cause?

• Formulate a plan
  – How much money do you want to raise?
  – Who will be donating toward the cause?
  – Who can help you put this plan/event together?
  – What is a realistic time frame?
Fundraising Options

• Auctions
• Selling merchandise / services
• Themed breakfasts / dinners
• Run/Walk/Bike – athons
• Events with a twist... comedy night, trivia night, bowling events, dance-a-thons
• The “Non-event” event
  – A mailing or solicitation in place of an actual event
  – Cash cans, percentages of breakfast / dinner sales, etc
Selecting the “Right” Fundraiser

• Group dynamics
  – Research your target audience
  – “Think globally, act locally”

• Establish a time frame
  – Consider time, money and resources available

• Consider costs
  – Network! Look for those who may be willing to donate goods and/or services toward your fundraiser

• Identify resources
  – What materials are needed? What manpower is needed?
  – Spread tasks among volunteers according to experience, strengths and capabilities
The Details of the Fundraiser

- **Identify a location** – look for donated space, work with the venue coordinator
- **Setting the “right” date** – prepare for “seasons” and weather considerations, if applicable
- **Organizing and recordkeeping** – be accountable, track donors, and collect contact information for follow-up!
- **Finding volunteers** – who and how can people help?
  - Churches, civic groups, schools, etc
- **Set a schedule**
  - Be comprehensive, include details, track progress
- **Invitees and special guests**
  - Think of anyone who will draw the audience to the cause
- **Contingency plans** – always have a back-up plan
Community Fundraising

• **Involving the community** – focus on everyone, not just the “big” players in the community
• **Make things happen** – start small, get people on board, and build group enthusiasm
• **Work with your community Board** – build awareness
• **Find local sponsors** – find sponsors, co-sponsors and make opportunities for recognition
• **Promote locally** – pre-event coverage as well as post-event coverage; give yourself at least 3 months to promote your fundraiser
• **Social networking** – post event information, pictures, videos and blogs
The Budget

• Assess costs – “Raising money costs money”
  – Typical expenses – food/refreshments, speakers/equipment, travel/lodging, items to be sold/raffled, event supplies

• Budget for expenses – Determine how you will spend money and how you will make money
  – Sell tickets, hold raffles, auction items, etc

• Find sources of funding
  – Develop a list of prospects
  – Individuals, businesses, civic organizations, government agencies, foundations and trade associations
Fundraising Tools

• Communication infrastructure
  – Determine how technically savvy your group is
  – Identify technology available to the group, as well as budget or equipment that will be necessary for your communication strategy

• Know who to contact
  – Make a list of contact persons for each aspect of planning

• Using a website for fundraising
  – Post pictures, videos, interviews and blogs
  – Social networking, email and facebook

• E-fundraising
  – Low cost way to generate revenue in support of a specific cause
  – Causes.com, Justgive.org, etc
Spreading the Word

- **Advertising** – Define your advertising needs:
  - Radio – PSAs, on-air interviews/appearances
  - Internet & Email – post stories, pictures & videos
  - Online newsletters – always link back to your website
  - Signs, Flyers and Posters – simple, clear and strategic placement
  - Promotional Activities – free giveaways, kickoff event, photo opportunities and print stories prior to the event
  - Print – use community resources, newspapers, grocery stores, community centers, etc
  - Visual Marketing – “A picture is worth a thousand words”
    - Design the graphics, text and photos for your event in a visually appealing and clear way
    - Hire or recruit a professional to donate services to help
Alternative Ways of Fundraising

• Foundations

• Corporations

• Grants

• Other major donors
Foundations

• Can be public, private, corporate or government foundations

• Typically fund worthwhile causes and activities including educational, scientific, environmental, political and charitable needs

• Foundations are typical funders of special projects and do not fund ongoing operating expenses

• Seeking foundation grants require well established plans and submission of a proposal, including measuring evaluation and follow-up

• Research the Foundation Center – www.fndcenter.org
Corporations

• Approaching Corporations
  – Who do you know?
  – Build relationships
  – Give something back – public image, corporate recognition

• Employee Donations
  – Many companies have employee contribution programs or “matches”
  – Contact the company’s Human Resources and to discuss matching gift programs (either adding to or establishing a new one for your cause)

• The United Way – currently contributes to 1,300 community-based organizations
Grants

• Finding grants
  – Do your research, be aware of geographical restrictions, guidelines of the funder, application criteria and whether your needs are a match for the funding programs available

• The application process
  – Collect data, be specific, state your cause, following grant guidelines, develop collaborations and support, prepare the presentation

• Timing and follow-up
  – Grant funding is competitive, prepare reasonable time-lines

• Corporate grants – make sure that your cause if a good match for the corporation

• Federal grants – search for grants applicable to your cause; www.grants.gov
Measuring Success

• **Evaluate fundraising efforts** – post-event evaluations... “what worked” and “what needs improvement”

• **Evaluate the process** – did you make the right choices, what are areas that you can measure for future success

• **Make improvements** – seek feedback, judge your weak points, focus on the positives

• **Fundraising wrap-up** – maintain a log of resources and event highlights, prepare for the future, report results

• **Say “thank you”, “thank you”, “thank you”** to everyone who helped make your event successful, ensure that proper acknowledgements are made for “repeat performances”!
Conference presentation Course

• IDEAS TO REACH A GREAT TALK

Tutorial by Vanessa Ferreira
(sindromecdg@gmail.com)
Points to consider:

- Effective public speaking
- Anatomy of a seminar
- Common mistakes in slide preparation
Effective public Speaking

- Voice Projection
  (Speak loud and clear, but don't shout!)

- Make eye contact with your audience

- Be animated and enthusiastic about your work
How Do I Motivate Others?

• Verbalize an Inspiring Vision and Mission
• Project a Courageous Spirit
• Behave with Enthusiasm
Anatomy of a seminar

• It’s important to structure your content!

• Structure helps memory and orientation.

http://www.freedigitalphotos.net/images/view_photog.php?photogid=659
Anatomy of a talk

It’s like a sandwich!

http://www.freedigitalphotos.net/images/view_photog.php?photogid=2365
INTRODUCTION

- Name / Role
- Purpose of Conference
- Agenda
- Elucidate the public if they can interrupt you during the talk or if the questions should be at end
INTRODUCTION

• **Your purpose and/message**
  – What will they learn once they leave your talk that they do not have now?

• **Importance of the message**
  – Why should they bother?

• **Orientation**
  – Starting point, what will be covered, where end?
BODY OF THE TALK

To organise your:

- EVIDENCE
- INFORMATION
- ARGUMENT

Into a logical flow.
CONCLUSION

• State message (again)
• Summarise
• Why it is important
• Thank them (motivate the audience to questions!)
SECRET IS....
Common mistakes in slide preparation

• Time: 1 minute per slide! And do not prepare long talk (25-30 minutes)
• Choice of font and point size
• Choice of text color
• Improper use of animation
Choice of font and point size

• 24 point font use for text
• 32 point font use for list of points
• 48 point font use for titles
• In fact the option to use color is very subjective. Although, it is important to be aware about the combination of colours that you use:
  – Blue and black it is is a terrible combination, like yellow and white

Then, we cannot forget that 7% of male population is red/green blind
Improper use of animation

• Use the animation only when required, otherwise it can distract.

• And make sure that you trained the use of the animation.
GOOD LUCK!!!!!! AND....

Bibliography:


http://www.weizmann.ac.il/mcb/UriAlon/nurturing/HowToGiveAGoodTalk.pdf